NCT02704429

STATISTICAL ANALYSIS PLAN

Protocol Title:	An Open-Label, Phase 2, Pilot Study Investigating the Safety, Clinical Activity, Pharmacokinetics, and Pharmacodynamics of Oral Treatment with the BTK Inhibitor PRN1008 in Patients with Newly Diagnosed or Relapsing Pemphigus Vulgaris (Part A)
Protocol Number:	PRN1008-005
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Table of Contents

List of Abbreviations

ABSIS	Autoimmune Bullous Skin Disorder Intensity Score
ABQOL	Autoimmune Bullous Diseases Quality of Life (assessment)
AE	Adverse event
ALT	Alanine Aminotransferase or SGPT
ALP	Alkaline Phosphatase
AST	Aspartate Transaminase or SGOT
AUC	Area under the plasma concentration-time curve
BCR	B-cell receptor
BID	Twice daily (morning and evening)
BMI	Body mass index
BPM	Beats per minute for Heart Rate; Breaths per minute for Respiratory Rate
BTK	Bruton's Tyrosine Kinase
CDA	Control of Disease Activity
CI	Confidence Interval
Cmax	Maximum observed plasma concentration
CPK	Creatine phosphokinase
CR	Clinical Response
CRF	Case report form
CS	Corticosteroid(s)
CTCAE	Common Terminology Criteria for Adverse Events
DILI	Drug Induced Liver Injury
DSG	Desmoglein
EADV	European Academy of Dermatology and Venereology
ECG	Electrocardiogram
ELISA	enzyme-linked immunosorbent assay
FcγR	Fc-gamma receptor
FcεR	Fc-epsilon receptor
FSH	Follicle Stimulating Hormone
HIV	Human Immunodeficiency Virus
LPLV	Last participant last visit
MedDRA	Medical Dictionary for Regulatory Activities
N	Sample Size
PBMC	Peripheral Blood Mononuclear Cell
PD	Pharmacodynamic
PDAI	Pemphigus Disease Area Index
PK	Pharmacokinetic
PV	Pemphigus vulgaris
Q12H	Every 12 hours
QD	Once a day
QTc	QT interval corrected for heart rate
SAE	Serious adverse event
SI	Système international d'unités (International system of units)
SMC	Safety Monitoring Committee
SNAQ	Simplified Nutritional Appetite Questionnaire

TABQOL	Treatment of Autoimmune Bullous Diseases Quality of Life (assessment)
TBL	Total Bilirubin
TEAE	Treatment-emergent AEs
Tmax	Time of observed maximum plasma concentration
TSH	Thyroid stimulating hormone
ULRR	Upper limit of the Reference Range
WHODD	World Health Organization Drug Dictionary

1. Introduction

Pemphigus vulgaris (PV), which is characterized by intraepidermal blisters in skin and mucosae, is known to be driven by autoantibodies to epidermal proteins and is responsive acutely to the anti-inflammatory effects of CSs and within five to 35 weeks to B cell depletion by anti-CD20 therapy (Horváth et al. 2012).

Bruton's agammaglobulinemia tyrosine kinase (BTK) is an essential signaling element downstream of the B-cell receptor (BCR), Fc-gamma receptor (FcγR) and Fc-epsilon receptor (FcεR). A selective BTK inhibitor has the potential to target multiple pathways involved in inflammation and autoimmunity. These include modulation of BCR-mediated B-cell pathways, inhibition of FcγR-induced cytokine release from monocytes and macrophages, FcεR-induced mast cell degranulation, granulocyte migration and mediator release. These effects lead to the prediction that a BTK inhibitor could block the initiation and propagation of various inflammatory diseases and mitigate the resulting tissue damage.

PRN1008 is a novel, highly selective, small molecule inhibitor of non-T cell white blood cell signaling (via B cell receptor, FcγR, FcεR signaling of the Bruton's Tyrosine Kinase, i.e. BTK, pathway). In rodent models of autoimmune arthritis, PRN1008 rapidly reverses disease with maximal efficacy similar to dexamethasone. In naturally occurring canine pemphigus foliaceus, an oral BTK inhibitor sharing an identical kinase inhibition profile to PRN1008 (PRN473) can rapidly reverse disease without the need for CS treatment.

To date, PRN1008 has been administered orally to 98 healthy volunteers in four Phase 1 studies (PRN1008-001, PRN1008-002, PRN1008-004, and PRN1008-006). No clinically significant changes in laboratory parameters or in vital signs or ECG parameters were observed in 97/98 patients tested. In Study PRN1008-001, healthy volunteers received 10 or 11 days of dosing, at dose regimens of placebo, 300 mg *qd*, 300 mg *q12h*, 600 mg *qd*, and 450 mg *q12h* (n=8 per dose level), with a liquid formulation, under fasted conditions on all but one day. PRN1008 was safe and well tolerated at the dose level tested. No severe or serious adverse events (SAEs) were reported, and no patients required discontinuation or interruption of dosing. Reported adverse events were mild or moderate in severity, with gastrointestinal adverse events being most common (nausea/vomiting, loose stools). Study PRN1008-006 compared the relative bioavailability of the liquid formulation to a tablet formulation, whereby patients (n=12) received 300 mg single doses of both PRN1008 formulations, as well as the tablet after food and after several days of esomeprozole treatment. The tolerability was improved with the tablet compared with the liquid formulation, primarily by the elimination of acute throat irritation reported with the liquid formulation, and elimination the most common of gastrointestinal adverse events (AEs) reported in PRN1008-001 study when the tablet was administered following food.

Clinical pharmacokinetics showed that with therapeutic multiple dosing, the terminal half-life of PRN1008 is approximately 3 to 4 hours and the time of maximum concentration (T_{max}) is 1.5 to 2.0 hours. There is no effect of food on the AUC or C_{max} of the tablet formulation although the T_{max} is slightly delayed from 1.5 hours to approximately 2.5 hours. The volume of distribution is high (on the order of 2500 L) and plasma protein binding is 98%.

Following single and multiple oral doses of PRN1008 in Study PRN1008-001, mean BTK occupancy in peripheral blood mononuclear cells (PBMCs) of more than 70% was observed 4 hours or later following doses of 300 mg or more on Day 1. The target trough BTK occupancy levels were safely and consistently exceeded, suggesting PRN1008 may be a safe and highly effective treatment in human PV and other autoimmune diseases.

2. Objectives

2.1. Primary Objectives

- To evaluate the safety of PRN1008 in patients with PV
- To evaluate the clinical activity of PRN1008 in patients with PV, per criteria in the European Academy of Dermatology and Venereology (EADV) 2014 Pemphigus S2 Guideline (Hertl et al. 2015)

2.2. Secondary Objective

- To evaluate the pharmacokinetics (PK) and the pharmacodynamics (PD) of multiple doses of PRN1008 in patients with PV

2.3. Exploratory Objective

- To evaluate the relationship of PK and PD to each other and to efficacy and safety in this patient population

3. Investigational Plan

3.1. Overall Study Design and Plan

This is a Phase 2, open-label, pilot cohort study to evaluate the safety, clinical activity, pharmacodynamics, and pharmacodynamics of PRN1008 in patients with newly diagnosed or relapsing PV. An open-label design was chosen for several reasons: PV does not spontaneously remit so there is no placebo effect to account for; clinicians need to know when to use rescue CSs, if necessary, in a timely manner; and lastly, the pilot nature of the investigation itself is best explored in an open-label fashion.

Up to 28 days before enrollment in the study, patients will be required to sign an informed consent form, after which screening assessments will be carried out. Patients must fulfill all entry criteria to be enrolled into the study. Patients who fail to meet the entry criteria may be rescreened at the discretion of the Investigator. Up to 25 male or female patients with newly diagnosed (i.e., naïve to an effective induction treatment regimen) or relapsing, biopsy-proven, mild or moderate PV patients (Pemphigus Disease Activity Index [PDAI] 8 to 45) will be enrolled in the study. Enrolled patients will receive twice daily (*bid*) PRN1008 treatment for 12 weeks, starting on Day 1 and ending on study Day 84, followed by 12 weeks of follow up (total duration of individual patient participation is 28 weeks). The expected study duration is approximately 18 months from the first patient treated to the last patient completing the study. The end of the study is defined as the date of the final safety follow-up after the last participant last visit (LPLV).

Based on available data from the Phase 1 clinical studies, a 400 mg *bid* oral dose of PRN1008 is expected to be safe and well tolerated. Initial PRN1008 dosing in this study will be 400 mg *bid*, with intra-patient dose-adjustment based on clinical response and tolerability, supplemented by BTK occupancy. CS rescue treatment will be initiated, if indicated. The maximum dose in this study, after dose adjustment, will be 600 mg *bid*. Clinical response and tolerability will be assessed at each visit.

Patients will complete the visit procedures per the Schedule of Assessment (Appendix 15.1). After providing written informed consent, patients will complete the screening assessments within 28 days before the first dose of PRN1008. Patients will return at specified times on an outpatient basis for assessment of vital signs, physical examination, assessment of adverse events, assessment of concomitant medication use, assessment of clinical benefit, and provision of blood samples for PK and PD, and other clinical laboratory tests. Height and weight, full physical examination, 12-lead electrocardiogram (ECG) (an extra ECG may be taken at additional visits, if indicated), FSH for postmenopausal women who are not surgically sterile, and some clinical laboratory testing (HIV, hepatitis B, hepatitis C, TB) will be taken at screening only. Skin biopsy will be also collected at screening if not already performed. During study drug treatment and follow up visit, Disease activity scores (PDAI and Autoimmune Bullous Skin Disorder Intensity Score [ABSIS]), Autoimmune Bullous Diseases Quality of Life (ABQOL) assessment, Treatment of Autoimmune Bullous Diseases Quality of Life (TABQOL) assessment, and Simple Nutritional Appetite Questionnaire (SNAQ) will be performed. Patient PDAI and ABSIS scores at screening will be collected as part of the medical history review. Brain MRI with macrocyclic contrast will be conducted at baseline and the Day 15 visit for patients from French sites. If

headache, nausea, vomiting or visual disturbances have occurred, a full neurological examination, including cranial nerves, will be performed for patients from French sites.

The first PRN1008 dose on Day 1 of Week 1 will be supervised in the clinic and the patients will under observation for approximately two hours after administration of the first dose and until PK/PD sample is drawn. BTK occupancy in PBMCs will be measured at baseline (pre-dose), and 2 and 24 hours after the first dose, then at various times of the day during follow up visits. For the Day 2 (24 hour) BTK occupancy, PRN1008 should be taken as usual in the evening and the PRN1008 morning dose withheld in order to take the blood sample approximately 12 hours after the prior evening dose. Where follow-up is not feasible on Day 2, another day in the first week of therapy may be used to get trough occupancy instead. For all other visits, occupancy is measured at random times after the usual morning dose. Anti-desmoglein-1 and -3 autoantibody titers will be measured by enzyme-linked immunosorbent assay (ELISA).

3.2. Study Endpoints

3.2.1. Safety Endpoints

The incidence of treatment-emergent AEs (TEAEs), including clinically significant changes in physical examination, laboratory tests, and vital signs.

3.2.2. Primary Efficacy Endpoint

The primary efficacy endpoint is the proportion of patients who are able to achieve control of disease activity (CDA) within 4 weeks of starting PRN1008 treatment without the need for doses of prednis(ol)one >0.5 mg/kg at any time on or before the Week 5 visit.

CDA is defined as the time at which new lesions cease to form and established lesions begin to heal per European Academy of Dermatology and Venereology (EADV) 2015 Pemphigus S2 Guideline.

3.2.3. Secondary Efficacy Endpoints

- Proportion of patients able to achieve CDA without CSs within 4 weeks
- Proportion of patients able to achieve a complete response (CR) without CSs within 12 weeks
- Proportion of patients able to achieve CR without the need for doses of prednis(ol)one of greater than 0.5 mg/kg at any point within 12 weeks
- Proportion of patients able to achieve CDA at each visit
- Proportion of patients able to achieve CDA at each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg
- Cumulative incidence of patients ever achieved CDA by each visit
- Cumulative incidence of patients ever achieved CDA by each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg

- Proportion of patients able to achieve CR at each visit
- Proportion of patients able to achieve CR at each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg
- Cumulative incidence of patients ever achieved CR by each visit
- Cumulative incidence of patients ever achieved CR by each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg
- Time to first CDA
- Time to first CR
- Time to first end of consolidation phase
- Time to first relapse after PRN1008 treatment completion or discontinuation
- Cumulative CS usage over 12 weeks
- Change from baseline in Pemphigus Disease Area Index (PDAI) and Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) scores at each follow-up visit
- Change from baseline in Autoimmune Bullous Diseases Quality of Life (ABQOL) and Treatment of Autoimmune Bullous Diseases Quality of Life (TABQOL) scores at each follow-up visit
- Change from baseline in appetite (SNAQ score) at each follow-up visit

CR, End of consolidation phase, and Relapse as defined in European Academy of Dermatology and Venereology (EADV) 2015 Pemphigus S2 Guideline are listed below:

Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN1008 immunotherapy. In this study, "minimal therapy" is defined as daily doses of ≤ 10 mg of prednisolone or equivalent doses of other CS for any period of time.

End of consolidation phase: The time at which no new lesions have developed for minimum of 2 weeks, approximately 80% of lesions have healed, and when most clinicians start to taper steroids.

Relapse/flare: Appearance of ≥3 new lesions/month that do not heal spontaneously within 1 week, or by extension of established lesions, in a patient who has achieved disease control.

3.2.4. PK/PD Endpoints

3.2.4.1. PK Endpoints

- Plasma concentrations of PRN1008 at approximately the time of maximum concentration on Day 1 and at various subsequent times during outpatient dosing

3.2.4.2. PD Endpoints

- Percentage BTK occupancy for individuals in PBMCs at 2 & 24 hours after the first PRN1008 dose and at varied subsequent times during outpatient dosing
- Change from baseline in anti-dsg1-3 autoantibody levels by ELISA at various time points

3.2.4.3. Exploratory PK/PD Analysis

- Exploratory PK/PD analysis will examine the effects, if any, of covariates on PK and/or PD, and the relationship between PK, PD and efficacy in this population.

3.3. Treatments

PRN1008 Dosage Initial PRN1008 dosing will be 400 mg *bid*. The maximum dose in this study, after dose adjustment, will be 600 mg *bid*. Patients will be treated with PRN1008 for a maximum of 12 weeks. PRN1008 tablets should be taken with a glass of water and may be taken with or without food, i.e., a period of fasting is not required.

Based on available data from clinical studies PRN1008-001 and PRN1008-002, a 400 mg *bid* oral dose of PRN1008 to be used in this study is expected to be safe and well tolerated. The 400 mg *bid* starting dose is also based upon the dose known to produce ~70% BTK occupancy at trough (~85% average occupancy over the day), as adjusted by results of the relative bioavailability study, where the tablet had ~70% of the exposure of the equal dose of the liquid formulation.

Each PRN1008 film-coated tablet contains either 100 mg or 300 mg of PRN1008 drug substance. In addition, the tablet contains Microcrystalline Cellulose (filler), Crospovidone (disintegrant), Sodium Stearyl Fumarate (lubricant) and a non-functional film coating. The 100 mg tablet is a round shape and orange in color. The 300 mg tablet is an oval shape and white in color.

3.4. Dose Adjustment/Modifications

Initial PRN1008 dosing will be 400 mg *bid*. Patients will return to the study center and complete the visit procedures per the Schedule of Assessment (Appendix 15.1). The investigator will review the patient's clinical response, tolerability, and BTK occupancy to determine if a dose-adjustment is needed (Appendix 15.2). The maximum dose in this study, after dose-adjustment, will be 600 mg *bid*. Doses may range from 100 mg *bid* to 600 mg *bid* in 100 mg *bid* increments (100 mg is the smallest tablet size). CS rescue treatment will be initiated, if indicated (Appendix 15.3).

"Well tolerated" is defined as the absence of Grade 3 or greater gastrointestinal AEs, or Grade 2 non-gastrointestinal AEs, including liver function changes, related to PRN1008 therapy. Investigators should use their judgment of the risk-benefit of continuing therapy by weighing the extent of clinical response, and AEs potentially related to PRN1008 therapy. In general, PRN1008 should be stopped and conventional therapy started when tolerability is poor and clinical response is also suboptimal.

4. General Statistical Considerations

Continuous data will be described using descriptive statistics (i.e. n, mean, standard deviation, median, minimum, and maximum). Categorical data will be described using the frequency and percentage in each category. For the summary statistics of all numerical variables unless otherwise specified, minimum and maximum will be displayed to the same level of precision as reported. Mean and median will be displayed to one level of precision greater than the data collected. Standard deviation / standard error will be displayed to two levels of precision greater than the data collected. P-values will be rounded to three decimal places. If a p-value is less than 0.001 it will be reported as "<0.001". If a p-value is greater than 0.999 it will be reported as ">0.999".

Due to the small sample sizes, all p-values derived from inferential analyses will be considered informative. In general, all significant testing will be two-sided at significance level 0.05. All tests will be made without adjustment for multiplicity or multiple comparisons. Two-sided 80% and 95% Confidence Intervals of the response rate for efficacy endpoints will be provided.

When frequency are presented, the percentage will be suppressed when the count is zero in order to draw attention to the non-zero counts. A row denoted "Missing" will be included in count tabulations where specified on the shells to account for dropouts and missing values. The denominator for all percentages will be the number of patients within the analysis population of interest, unless otherwise specified.

Unless otherwise specified, baseline will be defined as the last non-missing assessment prior to the first dosing of study drug.

Patients will be identified in the listings by patient number. Patients will be grouped into dose levels according to the maximum dose they received during the 12 week treatment period and all data will be summarized based on dose levels as well as overall unless otherwise specified. If data are to be summarized by visit, the visit name collected on the CRF page will be used. All analyses will be conducted using SAS Version 9.4 or above.

4.1. Sample Size

Sample size for this pilot study was determined pragmatically by the number of patients required to determine a preliminary safety profile and a point estimate for the primary endpoint of efficacy. If the clinical response rate is 50%, 25 patients will result in an 80% CI of ±13%. Results from this pilot study will be used to design confirmatory clinical trials. Sample size will be reevaluated based on an interim analysis when approximately 6 patients have completed 4 weeks of PRN1008 therapy.

4.2. Randomization, Stratification, and Blinding

Not applicable; the study is open-label.

4.3. Analysis Populations

Four study populations will be defined: Screening Population, Safety Population, Efficacy Population, and Pharmacokinetic Population.

4.3.1. Screening Population

All patients who provide informed consent and have screening assessments evaluated for study participation are included in the Screening Population.

4.3.2. Safety Analysis Population

All patients who have received at least one dose of PRN1008 will be included in the safety analysis. The Safety Analysis Population will be defined for all safety analyses.

4.3.3. Efficacy Analysis Population (ITT Population)

All patients who have received at least one dose of PRN1008 will be included in the efficacy analysis. Patient response and disease progression will be determined using PDAI, ABSIS, ABQOL, and TABQOL scores. Efficacy data will be presented in listings, by patient and tabulated for each efficacy endpoint.

4.3.4. mITT Population

Patients with at least Week 5 visit follow up will be included in the mITT population. Patients who terminated therapy prior to the Week 5 visit on the first day of treatment without any further follow up are not included in this population.

4.3.5. Per Protocol (PP) Population

Patients who have
1) No major protocol deviations relevant to data integrity per Medical Monitor review
2) At least 80% compliance based on drug accountability
3) Completed Week 13/Day 85 visit.

4.3.6. Pharmacokinetic Analysis Population

The definition of Pharmacokinetic Analysis Population will be described outside of this Statistical Analysis Plan.

5. Patient Disposition

5.1. Disposition

The number and percentage of patients in each analysis population defined in Section 4.3, the number and percentage of patients enrolled and patients screen failure will be reported overall and by maximum dose level.

The number and percentage of patients completing, withdrawing, along with reasons for withdrawal, will be tabulated overall and by maximum dose level for the Safety Analysis Population. The percentage of reasons for withdrawal will be based on the number of patients who withdraw from the study.

A listing of patient disposition data will be presented for the Safety Population, include informed consent date, study completion status, study completion/termination date, and reasons for withdrawal.

Reasons for withdrawal are specified in the CRF as: Adverse Event, Death, Lack of Efficacy, Lost of Follow-up, Non-Compliance with Study Drug, Physician Decision, Pregnancy, Progressive Disease, Protocol Violation, Recovery, Study Terminated by Sponsor, Technical Problem, Withdrawal by Subject, and Other.

5.2. Protocol Deviations

Protocol deviations are any unanticipated or unintentional divergence or departure from the expected conduct of an approved clinical study that is not consistent with the protocol, ancillary study documents, study plans/guides, or Informed Consent Form. Protocol deviations will be categorized to major and non-major deviations. Major deviations are departures from the protocol that impact patient safety or data integrity.

Major protocol deviation information will be documented and reviewed by the PPD project team and approved by the sponsor.

The number and percentage of patients with major deviations will be summarized overall in a table for the Safety Analysis Population.

All subject level protocol deviations will be listed for the Screening Population. Site level protocol deviations will be presented in the CSR separately.

6. Demographics and Baseline Characteristics

6.1. Demographics

A summary of demographics and baseline characteristics will be presented overall and by maximum dose level for the Safety Analysis Population/ITT Population, mITT Population and PP Analysis Population. The demographic characteristics consist of age (years), sex, race, and ethnicity. The baseline characteristics consist of baseline pemphigus (clinical diagnosis) type, time from pemphigus vulgaris confirmed date to screen date (Months), baseline Anti-DSG 1 and Anti-DSG 3 antibody status/profile, baseline height (cm), baseline weight (kg), baseline body mass index (BMI) (kg/m^2), PDAI total activity score, PDAI mucous membrane activity (% >0), PDAI scalp activity (% >0), PDAI skin activity (% >0), ABSIS oral activity (% >0), and CS dose on screening date and Week 1 Day 1 date, which is summarized in the CS summary table separate from the demographics summary.

A patient's age in years is calculated using the date of the informed consent and date of birth. Age, height, weight, BMI, PDAI total activity score, and CS Dose on the day of screening and Week 1 Day 1 will be summarized using descriptive statistics. The number and percentage of patients by sex (Male, Female), race (White, Black or African American, Asian, American Indian or Alaska Native, Native Hawaiian or Other Pacific Islander, and Other), ethnicity (Hispanic or Latino, Not Hispanic or Latino), baseline pemphigus type (Pemphigus Vulgaris, Bullous Pemphigoid, Pemphigus Foliaceus, Epidermolysis Bullosa Aquisita, Linear IgA Bullous Dermatoses, Mucous Membrane Pemphigoid, and Other). Time from pemphigus vulgaris confirmed date to screen date in months is defined as (Date of Screening – Date of pemphigus vulgaris confirmed +1) / 30.4375.

Baseline Anti-DSG status will be presented in the following categories, using the limits of detection as the basis for positive/negative determination:

- Anti-DSG 1 positive, Anti-DSG 3 negative;
- Anti-DSG 1 negative, Anti-DSG 3 positive;
- Anti-DSG 1 positive, Anti-DSG 3 positive;
- Anti-DSG 1 negative, Anti-DSG 3 negative;

No formal statistical analyses will be performed, and no inferential statistics reported.

Subgroup analyses variables detailed in Section 8.2.11 will also be summarized by categories.

Patient demographic and baseline characteristics will be presented in a listing for the Safety Analysis Population.

6.2. Medical History

Patient medical history data including specific details will be presented in a listing for the Safety Analysis Population.

6.3. Inclusion and Exclusion Criteria

Patients who meet all of the protocol defined inclusion criteria and none of the exclusion criteria will be enrolled into the study. Inclusion/exclusion criteria data will be summarized and listed for the Screening Population.

7. Treatments and Medications

7.1. Prior and Concomitant Medications

All medications taken within 28 days prior to the date of study screening will be recorded in the CRF. Concomitant medication will be coded according to the World Health Organization Drug Dictionary (WHODD, version 01SEP2015). A prior medication is defined as any medication that has a stop date before the date of first dose of study drug. A concomitant medication is defined as any medication that has a stop date on or after the date of first dose of study drug.

The number and percentage of patients who used prior or concomitant medications will be summarized in two separate tables by Anatomical Therapeutic Chemical (ATC) level 4 and preferred term, maximum dose levels and overall using the Safety Analysis Population.

All prior and concomitant medication will be presented in a listing for the Screening Population.

Imputation for missing medication start dates and stop dates can be found in Appendix 15.4.

Medications with missing stop dates after imputation will be classified as both prior and concomitant.

7.2. Study Treatments

A 400 mg *bid* initial oral dose of PRN1008 will be used in this study, and dosage will be adjusted during the treatment of study drug. Patients will be treated with PRN1008 for a maximum of 12 weeks. Tablets may be taken with or without food.

7.2.1. Extent of Exposure

Overall duration of exposure is defined as the total number of days a patient is exposed to the study drug and will be presented as the total number of days from the first dose date (Day 1) to the last dose date:

Overall Duration of Exposure = Date of last dose - Date of first dose + 1.

If date of last dose date is missing, date of last visit during the treatment will be used.

The average daily dose will also be introduced to characterize features of the dose adjustment design adopted in this study, which is calculated as the following:

Average Daily Dose = Cumulative dose / Overall duration of exposure

The cumulative dose will be defined as the sum of doses taken by patients during the treatment.

The overall duration of exposure and average daily dose will be summarized using descriptive statistics overall and by maximum dose level in a table for all patients in the Safety Analysis Population.

The number of adjustments in dose level and the minimum dose level will also be summarized.

Patient's overall duration of exposure, average daily dose, minimum dose level, maximum dose level, and number of adjustments in dose will be presented in a listing for the Safety Population.

7.2.2. Treatment Compliance and Modifications

Study drug accountability will be performed during the treatment. Patients will be asked to bring back all remaining study drug and all study drug packaging at each study visit for drug accountability.

The overall study drug compliance (%) will be calculated as following:

Overall Compliance (%) = Total number of tablets taken during the treatment / Total number of tablets planned during the treatment * 100

The total number of tablets taken will be calculated by subtracting the number of tablets returned from the number of tablets dispensed. The total number of tablets planned will be calculated by multiplying the total number of days of planned exposure to study drug by the number of tablets planned per day.

The overall study drug compliance will be summarized by descriptive statistics overall and by maximum dose level. The number and percentage of patients in compliance category of <80%, >=80 and <=120%, and >120% will also be presented.

Study drug dispensed and returned and study drug compliance will be presented in two listings by patient for the Safety Analysis Population.

8. Efficacy Analysis

All efficacy analyses will be conducted using the ITT, mITT and PP Analysis Population.

All efficacy analyses consider CS used in endpoints to be systemic CS only, i.e. oral (not mouthwash), intramuscular or intravenously administered. Topical CS are not considered systemic CS.

For cumulative by visit summaries, unscheduled visits are not included in the summary. For other by visit summaries, unscheduled visits, if exists in the data, will be presented separately after other visits. All visits will be presented in listings.

8.1. Primary Efficacy Endpoint

The primary efficacy endpoint is "The proportion of patients who are able to achieve CDA within 4 weeks of starting PRN1008 treatment without the need for doses of prednis(ol)one >0.5 mg/kg at any time".

Other types of CS are converted to dose-equivalent prednis(ol)one (see Section 8.2.6).

This is operationalized as meaning the first CDA is achieved on or before the Week 5 visit but after first dose of PRN1008, and will be summarized, by maximum dose level to the Week 5 visit and overall in a table, using frequencies and percentages. In addition, two-sided 80% and 95% CIs (Confidence Intervals) of the response rate will be provided for each maximum dose level and overall using the Exact (Clopper-Pearson) method.

Patients who are able to achieve CDA between first dose of study drug and the Week 5 visit and have no occurrence of daily prednis(ol)one >0.5 mg/kg before the date of the first CDA will be considered as responders, even if the CDA status was not maintained or higher doses of prednis(ol)one were used after the first CDA. Patients who are not able to achieve CDA prior to or at the Week 5 visit or have any occurrence of daily prednis(ol)one >0.5 mg/kg before a first CDA during the study treatment will be considered as non-responders. Patients whose CDA status is unknown at the Week 5 visit for whatever reason (include early termination before Week 5 visit) will be also considered as non-responders. Only systemic CSs (as defined by medication taken orally or intravenously)are to be used to evaluate the efficacy endpoints, topical CSs will not be counted.

8.2. Secondary Efficacy Endpoint

8.2.1. Achieved CDA without CS within 4 Weeks

- The proportion of patients who are able to achieve CDA without CSs within 4 weeks will be summarized in the same way as that described in Section 8.1. Patients who are able to achieve CDA by the Week 5 visit and have no occurrence of any CS on or before the first CDA visit during the study treatment will be considered as responders. Patients who are not able to achieve CDA by the Week 5 visit or have any occurrence of any CS on or

before a CDA visit during the study treatment will be considered as non-responders. Patients whose CDA status is unknown at the Week 5 visit for whatever reason (include early termination before Week 5 visit) will be also considered as non-responders. Only systemic CSs (as defined by medication taken orally or intravenously) are to be used to evaluate the efficacy endpoints, topical CSs will not be counted.

8.2.2. Achieved CR without CS within 12 Weeks

The proportion of patients who are able to achieve CR without CSs within 12 weeks will be summarized in the same way as that described in Section 8.1. Patients who are able to achieve CR between treatment start date and the Week 13 visit and have no occurrence of any CS after the treatment start date and before the first CR visit during the study treatment will be considered as responders. Patients who are not able to achieve CR between start of treatment and the Week 13 visit or have any occurrence of any CS between start of treatment and a first CR visit during the study treatment will be considered as non-responders. Patients whose CR status is unknown at the Week 13 visit for whatever reason (include early termination before Week 13 visit) will be also considered as non-responders. Only systemic CSs (as defined by medication taken orally or intravenously) are to be used to evaluate the efficacy endpoints, topical CSs will not be counted.

8.2.3. Achieved CR without Prednis(ol)one >0.5 mg/kg within 12 Weeks

The proportion of patients who are able to achieve CR without the need for doses of prednis(ol)one of greater than 0.5 mg/kg within 12 weeks will be summarized in the same way as that described in Section 8.1. Patients who are able to achieve CR at any visit on or before the Week 13 visit during the study treatment and have no occurrence of daily prednis(ol)one >0.5 mg/kg between the first dose date of PRN1008 treatment and the day before the date of the first CR will be considered as responders. Patients who are not able to achieve CR at all visits on or before the Week 13 visit during the study treatment or have any occurrence of daily prednis(ol)one >0.5 mg/ between the first dose date of PRN1008 treatment and the day before the date of the first CR will be considered as non-responders. Patients whose CR status is unknown at all visits on or before Week 13 visit during the study treatment for whatever reason (include early termination before Week 13 visit) will be also considered as non-responders. Only systemic CSs (as defined by medication taken orally or intravenously) are to be used to evaluate the efficacy endpoints, topical CSs will not be counted.

8.2.4. Proportion of CDA and CR at each Visit

The proportion of patients who are able to achieve CDA and the proportion of patients who are able to achieve CR will be summarized by maximum dose level and overall by CDA and CR status for each visit by using frequencies and percentages, respectively. This is a cross sectional analysis and differs from the cumulative assessment of efficacy described above. In addition, two-sided 80% and 95% CIs of the response rate will be provided for each maximum dose level and overall using the Exact (Clopper-Pearson) method.

The proportion of patients who are able to achieve CDA and/or CR without the need for doses of prednis(ol)one of greater than 0.5 mg/kg (CS doses from Day 1 to the day before the date of the visit) will also be summarized by maximum dose level and overall for each visit. Two-sided 80% and 95% CIs will be provided by using the Exact (Clopper-Pearson) method.

The disease status at each visit, and the CS dose on the day before the achievement date will be listed.

8.2.5. Cumulative Incidents of CDA and CR at each Visit

The cumulative incidents of patients who are able to achieve CDA and CR by each visit will be summarized by maximum dose level and overall. This is a cumulative analysis compared to section 8.2.4. In addition, two-sided 80% and 95% CIs of the response rate will be provided for each maximum dose level and overall using the Exact (Clopper-Pearson) method.

The proportion of patients who are able to achieve CDA and/or CR without the need for doses of prednis(ol)one of greater than 0.5 mg/kg (CS doses from Day 1 to the day before the date of the visit) will also be summarized by maximum dose level and overall for each visit. Two-sided 80% and 95% CIs will be provided by using the Exact (Clopper-Pearson) method.

If a subject drops out, they will still accommodate to future unattended visits cumulatively, i.e. the denominator for this analysis would not diminish over time.

8.2.6. Time to first CDA, CR, End of Consolidation Phase, and Relapse

Time to first CDA is defined as the following:

Time to first CDA = Date of the first CDA confirmed - Date of first dose + 1.

Time to first CR and End of Consolidation Phase are defined in the same way as Time to first CDA.

Time to first relapse after PRN1008 treatment completion or discontinuation is defined as the following:

Time to first Relapse = Date of the first relapse after Week 13 visit confirmed – Date of last PRN1008 dose + 1.

If the date of last PRN1008 dose is missing, the date of the Week 13 visit during treatment will be used. If patients did not complete the Week 13 visit they will be excluded from the relapse analysis.

The time to relapse analysis will be conducted in two subpopulations for each analysis population: 1) Patients with CDA at the Week 13 visit, 2) Patients with CR at the Week 13 visit.

Patients who have not achieved the above events by the date of study completion or discontinuation will be censored at the date of completion or discontinuation respectively.

Kaplan-Meier survival distribution plots for time to each event will be provided. Kaplan-Meier estimates of quartiles in days and its associated 80% and 95% CI will be calculated by using the LOGLOG transformation. Min and Max survival times in days as well as number of the patients and the probability of experiencing the event on or before specific date will be provided in a table.

The CDA status, CR status, End of Consolidation Phase status, and Relapse status by visit, by patient will be presented in a listing.

Duration of CR will be summarized and listed, and is defined by the last known CR date – first known CR date + 1. Each subject will only have one CR date as a result.

8.2.7. Cumulative CS Usage

Cumulative corticosteroid (CS) usage per patient (mg) over 12 weeks will be presented overall and by maximum dose level in a table. Only subjects who complete at least 12 weeks of treatment (terminated at or after the Week 13 (Day 85) Visit) will be included. To calculate this endpoint, the following steps will be followed:

- Step 1: All other CSs will be converted into prednisolone/prednisone dosage equivalent. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.

- Step 2: The CSs usage will be converted to standard frequency using the following conversion chart, the standard frequency is in the first column (i.e., mg/day).

Standard frequency	Unit	Frequency	Conversion factor
mg/day	mg	QD	1
mg/day	mg	BID	2
mg/day	mg	QOD	0.5
mg/day	mg/day	Any value	1
mg/day	mg	ONCE	1
mg/day	mg	Q24H	1
mg/day	mg	TID	3
mg/day	mg	QS	0.1428571
mg/day	mg	QID	4

  Once the CSs type and frequency are converted as above, each record of CSs taken will be converted to prednisolone with standard frequency (mg/day).

- Step 3: The duration for each record of CSs (CS) taken will be calculated using the formula: Duration = CS end date – CS start date + 1, in days. Any missing start or end date of CSs taken will be imputed using the algorithm specified in Section 15.4.

    - If the CS end date is after the date of Week 13 (Day 85) - 1, then the CS end date will be replaced by the date of Week 13 (Day 85) - 1; if the CS end date is before the study treatment start date, this record will not be used to summarize cumulative dose for CSs.

    - If the CS start date is before the study treatment start date, then the CS start date will be replaced by the study treatment start date (i.e. Day 1); if the CS start date is after the date of Week 13 (Day 85) - 1, this record will not be used to summarize cumulative dose for CSs.

- Note that above date adjustments only apply to cumulative CS dose calculation. Other analyses, where CS dose is needed, will use CS start and end dates as reported.

- Step 4: The "record" level cumulative CSs usage will be calculated as: record level cumulative dose = standard frequency * duration (days).

- Step 5: The patient level cumulative CSs usage will be calculated as the summation of all the non-missing record level cumulative doses.

All CS usage will be presented in a listing showing CS type, dose, frequency, route, start and stop dates.

8.2.8. Mean CS Dose on the Day Before Each Visit

For each subject on the day before each scheduled visit, dosage level of Predniso(lo)ne in mg will be summarized using summary statistics by maximum dose level and overall.

For Week 1/Day 1 visit, however, the CS dose is set as the day of, rather than the day before.

If a subject is still enrolled in the study but does not report any CS, then the dosage level is set as 0. If a subject is terminated prior to a visit, then this subject is not included in the calculation for visit.

8.2.9. PDAI, ABSIS, ABQOL and TABQOL scores

Quantitative assessment of patient response and disease progression will be determined using PDAI, ABSIS, ABQOL, and TABQOL scores. The PDAI, ABSIS, ABQOL and TABQOL questionnaires are located in Appendix 15.5.

For each questionnaire, we assume they are taken on the day of visit dates, and use the visit date indicated on the Visit Date CRF as questionnaire date, as the latter are not rigorously QC'ed.

The PDAI questionnaire has two components including activity and damage. The activity component consists of skin, scalp and mucosa parts and the damage component consists of skin and scalp parts. Actual values and change from baseline in PDAI total activity, total damage scores, and total PDAI score will be summarized by maximum dose level and overall, respectively.

The PDAI activity or damage total scores per body part will be calculated as the sum of the scores on that body part as follows. PDAI total activity and total damage scores are calculated and reported by site investigator into EDC.

- PDAI total activity score = Total skin activity + Total scalp activity + Total mucosa activity

- PDAI total damage score = Total skin damage + Total scalp damage

The ABSIS questionnaire has three components, including skin involvement, oral involvement and (oral) severity. The ABSIS scores will be calculated separately for each component. The ABSIS total activity score and total ABSIS score will also be calculated as follows.

- Skin involvement total score = sum of (%BSA * Weighting factor)
- Oral involvement total score = sum score from each oral part
- (Oral) Severity total score = sum of (Level * Factor of discomfort)
- ABSIS total activity score = Skin involvement total score + Oral involvement total score
- Total ABSIS score = Skin involvement total score + Oral involvement total score + Severity total score

Actual values and change from baseline in ABSIS skin involvement total score, ABSIS oral involvement total score and ABSIS total activity score will be summarized for all patients, and for patient subgroups categorized into two groups by a baseline oral involvement total score equal to 0 and >0, and for patient subgroups categorized into two groups by a baseline skin involvement score equal to 0 or >0.

When calculating skin involvement total score, if sum of %BSA = 0, then skin involvement score component is 0. If %BSA > 0, then weighting factor needs to be considered, and if weighting factor is missing in this circumstance, then the skin involvement total score and ABSIS total score are considered missing.

The ABQOL and TABQOL questionnaires both contains 17 questions with numerical scores. The ABQOL and TABQOL scores will be calculated as the sum of scores from each question. Actual values and change from baseline in ABQOL and TABQOL scores will also be summarized overall and by maximum dose level in a table. If individual scores are missing, then total scores as set as missing.

PDAI, ABSIS, ABQOL and TABQOL scores and subscores (for PDAI and ABSIS) by patient and visit will be presented in separate listings.

8.2.10. Simple Nutritional Appetite Questionnaire (SNAQ) score

Simple Nutritional Appetite Questionnaire (SNAQ) contains 4 questions with numerical scores. The SNAQ score will be calculated as the sum of scores from each question. The appetite questionnaire is located in Appendix 15.6. If individual scores are missing, then total scores as set as missing.

Actual values and change from baseline in SNAQ score will be summarized by visit overall and by maximum dose level in a table.

The SNAQ score will be presented in a listing by patient and visit.

8.2.11. Subgroup Analysis

The rates of the primary endpoint will be calculated in selected subgroups:

1. Age (≥ 50, < 50)
2. Gender
3. Total anti-desmoglein (dsg) antibody (≥ 100, < 100 units) at baseline.
4. Pemphigus History Type: Newly diagnosed (≤ 6 months from screening) vs. relapsed (> 6 months)
5. Pemphigus Anti-DSG Profile at baseline: PV vs. PF vs. negative anti-dsg profile, where PV is defined as anti-dsg3 positive with anti-dsg1 negative or anti-dsg 3 positive with anti-dsg1 positive. PF=anti-dsg1 positive only vs. neither detectable).
6. Pemphigus Severity: Mild vs. moderate disease severity at baseline (PDAI total activity < 15 vs. ≥ 15). If screening PDAI is not available use Day 1.

9. Safety Analysis

Specific assessments to evaluate treatment safety include the following: the frequency and type of adverse events, clinical laboratory testing, brain MRI (baseline and Day 15 in a subset of patients), vital signs, and symptoms and/or signs of headache, nausea, vomiting or visual disturbances. All analyses of safety will be conducted using the Safety Analysis Population.

For by visit summaries, unscheduled visits, if they exist in the data, will be presented separately after other visits.

9.1. Adverse Events

An AE can be any unfavorable and unintended sign (including an abnormal laboratory finding, for example), symptom, or disease temporally associated with the use of an investigational product, whether or not considered related to the product. All AEs encountered during the study will be reported in detail in the CRF, begins at the time of the first screening/eligibility assessment and ends at the end of the study for each patient. Pre-existing conditions that worsen during a study are to be reported as AEs. Clinically significant changes in physical examination, laboratory safety tests, and vital signs will also be defined as AEs and recorded appropriately. All AEs will be coded using the MedDRA Dictionary (MedDRA, Version 18.0).

Imputation for missing AE onset dates and end dates can be found in Appendix 15.4.

A treatment-emergent AE (TEAE) is defined as an AE that begins on or after the first dose of study drug.

If the AE onset date is missing after imputation and the imputed end date is on or after the first dose of study drug, or if onset date and end date are both missing, then the AE will be classified as a TEAE. If the imputed AE onset date is the same as the first dose of study drug, and AE onset time on or after the first dose of study drug or AE onset time is missing, then the AE will also be classified as a TEAE.

An overall summary of the number and percentage of patients with any TEAE, serious TEAE, study drug-related TEAE, study drug-related serious TEAE, TEAE leading to treatment discontinuation, and AEs leading to death will be provided overall and by maximum dose level.

All AEs will be presented in a listing for the Safety Population.

9.1.1. Incidence of Treatment Emergent Adverse Events

The number and percentage of patients with at least one TEAE and the number of TEAEs will be summarized by SOC and PT, maximum dose level and overall. At each level of SOC or PT, a patient will be counted only once if the patient reported multiple events.

9.1.2. Relationship of Adverse Events to Study Drug

The investigator will provide an assessment of the relationship of the event to the study drug. The possible relationships defined in the protocol are “Not Related” and “Related”.

The TEAEs categorized by SOC and PT will be summarized by maximum dose level and relationship to study drug in a manner similar to that described in Section 9.1.1.

TEAEs that are missing a relationship will be presented in the summary table as "Related" but will be presented in the data listing with a missing relationship.

Related TEAEs will also be presented by SOC and PT.

9.1.3. Intensity Grade of Adverse Event

The NCI CTCAE (version 4.0) will be used for grading AEs.

The TEAEs categorized by SOC and PT will be summarized by maximum dose level and intensity grade in a manner similar to that described in Section 9.1.1. Per each SOC or PT, a patient will be counted only once at the highest grade of intensity.

TEAEs with missing intensity grade will be presented in tables as "Grade 3" but will be presented in the data listing with a missing severity.

9.1.4. Serious Adverse Events

The seriousness of an AE should be assessed by the Investigator independently from the severity of the AE.

The treatment-emergent SAEs categorized by SOC and PT will be summarized by maximum dose level in a manner similar to that described in Section 9.1.1. Treatment-emergent SAEs by relationship to study drug will also be presented in a table. SAE information by patient will be presented in a listing.

9.1.5. Adverse Events Leading to Treatment Discontinuation

The TEAE leading to treatment discontinuation will be summarized by SOC and PT, and maximum dose level in the same way as that described in Section 9.1.1.

All patients who have an AE leading to treatment discontinuation will be presented in a listing.

9.1.6. Death

The TEAE leading to death will be summarized by SOC and PT by SOC and PT, maximum dose level in the same way as that described in Section 9.1.1.

All patients who have an AE with an outcome of death will be presented in a listing.

9.2. Clinical Laboratory Evaluations

Laboratory assessments will be performed at a central laboratory, with the provision for occasional local laboratory testing, if required. The International System of Units (SI units; Système international d'unités) will be used and those not reported in SI units will be converted to SI units before analysis.

Summary tables presenting actual values and changes from baseline will be presented for chemistry tests, hematology tests, and coagulation tests overall and by maximum dose level. All relevant clinical laboratory tests will be flagged as Low, Normal, and High according to the normal ranges if applicable. Shift tables will be provided to summarize changes in normality status from baseline to each scheduled post-baseline visit for those tests with normality flags.

The number and percentage of patients for urinalysis tests - will be presented in separate tables. This categorical data will be summarized in shift tables comparing the results at each scheduled post-baseline visit with those at baseline visit.

All lab tests results with normality status will be presented by patient and visit in a listing for the Screening Population.

A special group of clinical laboratory tests which are believed to be the indicators for drug induced liver injury (DILI) will be assessed separately with graphical displays and tabulations. They include:

- ALT (alanine aminotransferase or SGPT)
- AST (aspartate transaminase or SGOT)
- TBL (total bilirubin)
- ALP (alkaline phosphatase)

The test results of the above clinical laboratory tests will be reported in a separate listing and the values outside a number of designated reference ranges will be marked.

Furthermore, scatter plots of the peak TBL divided by the upper limit of the reference range (ULRR) (Y-axis) against peak ALT divided by the ULRR (X-axis), on a log scale, will be created. Lines for Upper limit of the Reference Range (ULRR) for both TBL and ALT, 2xULRR for TBL and 3xULRR for ALT will be presented in the same scatter plot graph. Any patients in the upper right quadrant, which is defined by 2xULRR for TBL and 3xULRR for ALT, would represent cases that were further investigated for potential DILI.

9.3. Vital Sign Measurements

Summary tables presenting observed values and changes from baseline will be provided overall for vital sign measurements, which including systolic blood pressure (mmHg), diastolic blood pressure (mmHg), heart rate (bpm), respiratory rate (bpm), and temperature (°C). All vital sign measurements will be presented in a listing by patient and visit for Safety Population.

Systolic blood pressure values <90 or >140 mmHg or diastolic blood pressure values <60 or >80 mmHg will be flagged as outside the normal range. Resting heart rate <40 or >100 beats per minute will be flagged as abnormal. The number and percentage of patients for each normality category will be summarized overall at each visit.

9.4. Physical Examination

The number and percentage of patients for each body system will be summarized by normality status and overall at each visit.

For the screening visit, the physical examination includes the following body systems: general appearance; skin; eyes, ears, nose, throat; heart; chest/breast; abdomen; neurological; lymph nodes; skeletal; and other. Physical examinations at all other visits will only include: general appearance; abdomen; cardiorespiratory; and other.

Physical examination results for the safety analysis population will be presented in a listing.

For patients from French sites only: if clinically significant headache, nausea, vomiting or visual disturbances have occurred, a full neurological examination, including cranial nerves, will be performed. The neurological symptoms will be documented on AE page. The status of neurological symptoms with relevant AE number, and results of neurological examination will be presented in separate listings.

9.5. Electrocardiogram

Summary tables will be presented for ECG data including ventricular rate (bpm), PR Interval (msec), QRS Duration (msec), QT Interval (msec), QTcF Interval (msec) and QTcB Interval (msec), overall and by maximum dose level for the screening visit.

ECG interpretation at the screening visit will also be summarized overall and by maximum dose level.

If ECG results are collected or interpreted multiple times, the latest one prior to dosing will be used for this summary.

ECG results for the safety analysis population will be presented in a listing.

9.6. Female Reproductive Status and Pregnancy Test Results

The fertility status and method of birth control used for women will be recorded in the CRF at the screening visit. The pregnancy test will be performed for women of childbearing potential only. Pregnancy serum test will be done at the screening visit, urine dip tests will be done at other time points. The reproductive status as well as serum and urine pregnancy test results will be presented in a listing for the safety analysis population.

9.7. Food Intake Status

The food intake status will be recorded in the CRF at each visit. A listing of patient food intake status will be presented for the safety analysis population.

9.8. Brain MRI

Brain MRI with macrocyclic contrast will be conducted on baseline and Day 15 visit for patients from French sites. Date of scan and results will be presented in a listing for the safety analysis population.

10. Pharmacokinetics/ Pharmacodynamics

The analyses of pharmacokinetics and pharmacodynamics (BTK occupancy) will be described and reported outside of the PRN1008-005 study report and attached as appendices.

ITT Analysis Population will be used for TFLs below.

The limit of quantifications for Anti-DSG 1, Anti-DSG 3 and Total Anti-DSG are 14, 9 and one or both of 14 or 9, respectively. Below the threshold is defined as negative, at or above these thresholds is defined as positive.

For Anti-DSG 1 and Anti-DSG 3 data, and a calculated variable of "total DSG" (Anti-DSG 3 and Anti-DSG 1 summed) summary tables presenting actual values and changes from baseline will be presented by visit overall and by maximum dose level. Missing values will not be imputed. This analysis will be performed only using Anti-DSG 1 and Anti-DSG 3 data above the limit of quantification, and for Total Anti-DSG, only use samples where at least one of Anti-DSG 1 or 3 is above the limit of quantification. Two exploratory DSG analyses will be performed:

1) CR status (without more than 0.5 mg/kg of CS prior to Week 13) yes/no at the Week 13 visit will be used to compare the change from baseline to Week 5 & 13 in Anti-DSG 1, Anti-DSG 3 data, and total DSG levels.

2) CDA status (without more than 0.5 mg/kg of CS and as defined for the primary endpoint) yes/no before Week 5 visit will be used to compare the change from baseline to Week 5 & 13 in Anti-DSG 1, Anti-DSG 3 data, and total DSG levels

All pharmacokinetics and pharmacodynamics data, including plasma concentrations of PRN1008, percentage BTK occupancy in PBMCs, and Anti-DSG 1-3 autoantibody levels (including quantified values below limit of quantification), will be presented in listings.

BTK occupancy data will also be described in a separate report.

11. Interim Analysis

An interim analysis to evaluate safety, and to reevaluate sample size and other aspects of study design, was conducted for this study when 6 patients completed 4 weeks or more of PRN1008 therapy (PRN1008-005 Interim Analysis Report March 20 2017), and again when 26 patients had completed 4 weeks or more of PRN1008 therapy (PRN1008-005 Interim Analysis Report August 15 2018). A further interim analysis will be performed when Part A enrollment and follow up is complete.

12. Safety Monitoring Committee

A Safety Monitoring Committee (SMC) will review the emerging safety data (TEAEs, safety labs), and efficacy, PD, and dose modification data approximately every 3 months within the first 6 months following the first patient enrolled in the study, and then with a frequency determined as appropriate by the SMC. The SMC members will include the Sponsor's Medical Monitor, a Principal Investigator, and a pemphigus clinical expert who is not an investigator in the study. The trial statistician will also participate in safety reviews. Documentation of the patient data reviewed at each meeting, including the individual SMC member’s confirmation of data review and the findings and actions of the SMC, will be included in the Trial Master File. SMC findings that impact the safety of patients in this study will be immediately reported to the local Competent Authority (CA) and Ethics Committee (EC).

An SMC Charter outlining the SMC composition and responsibilities will be in place prior to the first scheduled meeting.

13. Changes in the Planned Analysis

In addition to the secondary efficacy endpoints listed in protocol section 11.2, the proportion of patients who are able to achieve CDA/CR at each visit, with or without the need for doses of CS of greater than 0.5 mg/kg will be summarized. Cumulative incidence of patients who ever achieved CDA/CR by each visit will be summarized as secondary efficacy endpoints also.

14. References

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Wilson MM, Thomas DR, Rubenstein LZ, Chibnall JT, Anderson S, Baxi A, Diebold MR, Morley JE. Appetite assessment: simple appetite questionnaire predicts weight loss in community-dwelling adults and nursing home residents. Am J Clin Nutr. 2005 Nov;82(5):1074-81.

15. Appendices

15.1. Schedule of Assessments

	Screen	Day 1, Week 1 Pre-dose	Day 1 Week 1 Post-dose	Day 2, Week 1[a]	Day 15, Week 3 +/- 3 days	Day 29, Week 5 +/- 3 days	Day 57, Week 9 +/- 7 days	Day 85, Week 13 +/- 7 days	Day 113, Week 17 +/- 7 days	Day 141, Week 21 +/- 7 days	Day 169, Week 25 +/- 7 days	Unscheduled Visit
Informed Consent	X											
Inclusion/Exclusion Criteria	X	X										
Height	X											
Weight	X	X			X	X	X	X	X	X	X	X
Physical exam./med. History PDAI/ABSIS	X											
Abbreviated physical exam., PDAI, ABSIS		X			X	X	X	X	X	X	X	X
ECG (12-lead)	X											(X)[b]
Vital Signs	X	X		X	X	X	X	X	X	X	X	X
MRI[c]		X			X							
Urinalysis	X											
Hep B &C, HIV, QuantiFERON® TB	X											
Pregnancy test[d]	X	X				X	X	X			X	
Skin biopsy[e]	X											
Hem, Coag, Chem[f]	X	X			X	X	X	X				X
FSH[g]	X											
BTK occupancy sample[h]		X	X[h]	X	X	X	X	X				(X)[b]
PK sample		X	X[h]	X	X	X	X	X				(X)[b]
Anti-DSG antibodies		X				X	X	X	X	X	X	
Photography (Optional)[i]		X			X	X	X	X	X	X	X	X
ABQOL & TABQOL		X			X	X	X	X	X	X	X	X
SNAQ questionnaire		X				X	X	X	X	X	X	X
AEs[j]		X		X	X	X	X	X	X	X	X	X
Concomitant Medications	X	X		X	X	X	X	X	X	X	X	X
Drug dispensed		X				X	X					
Drug reconciliation					X	X	X	X				

Schedule of Assessments Footnotes:

a. Withhold PRN1008 on morning of Day 2 until PK/PD measurement has been taken as close to 12 hours after second dose as possible. Where follow-up on Day 2 is not possible, PK/PD samples may be taken on another day in the first week of treatment. On all other days, instruct patient to take PRN1008 in the morning as usual prior to clinic and take note of the time taken. Extra PK/PD sample intended for 1 to 5 days after dose adjustment or to replace missing samples–not required for other extra visits.
b. Only if clinically indicated.
c. Patients from French sites only: Brain MRI with macrocyclic contrast. Baseline examination to be conducted during the screening period. Follow up examination at Day 15 ± 3 days.
d. For women of childbearing potential only. Serum pregnancy test done at screening, urine dip test done at other time points
e. Performed only if no suitable prior biopsy.
f. TSH and CPK are only taken at Screening as part of chemistry panel.
g. To confirm postmenopausal status for women who are not surgically sterile only.
h. Two-8mL pre-dose blood tubes to be collected at baseline to ensure sufficient samples for later time point assay calculations. On Day 1 blood draw also taken at 2 hours post-dose (+/- 15 mins)
i. Photography is used to document skin disease changes; ideally in most patients. Strict masking of patient identity is required.
j. Including particular questioning on symptoms of headache, nausea, vomiting and visual disturbances. If any of these symptoms are reported, a full neurological examination, including the cranial nerves, should be performed.

15.2. General Dose Adjustment Guidelines for Dose Selection in the First 4 Weeks

Clinical Response	Trough BTK occupancy	Tolerability*	Action
Responder	≥ 50%	Well Tolerated	Maintain 400 mg *bid* Taper CSs if used in combination
		Poorly Tolerated	Reduce to 300 mg *bid* Taper CSs if used in combination
	< 50%	Well Tolerated	Maintain 400 mg *bid* Taper CSs if used in combination
		Poorly Tolerated	Reduce to 300 mg *bid* Taper CSs if used in combination
Suboptimal Response	≥ 50%	Well Tolerated	*Follow rescue criteria if triggered*, if not maintain dose at 400 mg *bid*
		Poorly Tolerated	*Follow rescue criteria if triggered*, if not maintain dose at 400 mg *bid* if feasible
	< 50%	Well Tolerated	*Follow rescue criteria if triggered, if not,* increase dose to 600 mg *bid*,
		Poorly Tolerated	*Follow rescue criteria if triggered,* if not, increase dose to 600 mg *bid* if tolerability allows.

* "Well tolerated" is defined as the absence of Grade 3 or greater gastrointestinal AEs, or Grade 2 non-gastrointestinal AEs, including liver function changes, related to PRN1008 therapy.

15.3. CS rescue criteria and treatment protocol

Systemic CSs will be avoided during PRN1008 monotherapy unless these "rescue criteria" are triggered, as described below, or patients enter the study on low doses (≤ 0.5 mg/kg per day). In each rescue scenario, the recommended method of CS taper is shown in Appendix 15.7.

Rescue criteria #1: Flare which, in the mind of investigator, is potentially life threatening

- Commence conventional immunosuppressive therapy and discontinue PRN1008

Rescue criteria #2: Development of severe but not life threatening PV (defined as PDAI score ≥ 45) at any time during PRN1008 therapy:

- If most recent BTK trough occupancy was 70% or more, commence conventional immunosuppressive therapy and discontinue PRN1008
- If most recent BTK trough occupancy was <50% and PRN1008 dose has not been increased since last BTK assay, commence, or increase dose to, full dose CS (1.0 mg/kg) orally and increase dose of PRN1008 by an amount agreed to with the Sponsor's Medical Monitor. At next review:
    - If not responding, commence conventional immunosuppressive therapy and discontinue PRN1008
    - If responding, maintain or taper CSs as clinically appropriate while maintaining PRN1008 treatment
- If no BTK trough occupancy is available to guide therapy, maintain PRN1008, and commence, or increase to, full dose CS (1.0 mg/kg) orally. Seek to expedite latest BTK trough occupancy test result or arrange for a new blood draw to measure BTK trough occupancy. At next review:
    - If not responding, commence conventional immunosuppressive therapy and discontinue PRN1008
    - If responding, maintain or taper CSs as clinically appropriate while maintaining PRN1008 treatment (including adjust dose upwards if BTK trough occupancy <50% in consultation with the Sponsor Medical Monitor)

Rescue criteria #3: Patients with non-severe PV but who fail to achieve "Control of Disease Activity (CDA*)" as monotherapy by 4 weeks

- If most recent BTK trough occupancy was 50% or more, commence, or increase dose to, low dose CS (0.5 mg/kg) orally. Review after another ~2 weeks:
    - If not responding, commence conventional immunosuppressive therapy and discontinue PRN1008
    - If responding, maintain or taper CSs as clinically appropriate while maintaining PRN1008 treatment
- If most recent BTK trough occupancy was <70% and PRN1008 dose has not been increased since last BTK assay, increase dose of PRN1008 by an amount agreed with the Sponsor's Medical Monitor. At review after another ~2 weeks:
    - If not responding, commence, or increase dose to, low dose CS (0.5 mg/kg) orally and maintain PRN1008 treatment. If a dose of ≥ 0.5 mg/kg is already in use, or if after further review the patient is still not responding, commence conventional immunosuppressive therapy and discontinue PRN1008
    - If responding, maintain PRN1008 treatment and consider tapering of CS

In each rescue scenario the recommended method of CS taper is shown in Appendix 15.7.

When patients enter the study on CSs, the dose should be maintained for the initial 2 weeks of PRN1008 therapy if possible in order to evaluate the additive effects of PRN1008. During this time and subsequently, the rescue criteria should be followed if indicated. In other words, in some circumstances the CS dose should be increased, with or without cessation of PRN1008. If patients respond well to PRN1008 without increasing the CS dose, the Werth taper (Appendix 15.7) should be commenced after four weeks of PRN1008 therapy.

* As defined by the EADV 2014 Pemphigus S2 Guideline (Hertl et al. 2015)

15.4. Missing date imputation

15.4.1. Missing medication and AE stop dates imputation

For the purpose of inclusion in prior and/or concomitant medication tables, incomplete medication stop dates will be imputed as follows:

- If only day is missing, then set to the last day of the month.
- If only month is missing, then set to December.
- If both month and day are missing, then set to December 31.
- Completely or Year missing date will not be imputed.

15.4.2. Missing medication start dates and AE onset dates imputation

For the purpose of inclusion in TEAE tables, incomplete AE onset dates will be imputed as follows:

Missing day only:

- If the year is the same as the year of the first dose of study drug
    - If the month is before the month of the first dose of study drug, then the last day of the month will be assigned to the missing day.
    - If the month is the same as the month of the first dose of study drug, then the day of the first dose of study drug will be assigned to the missing day.
    - If the month is after the month of the first dose of study drug, then “01” will be assigned to the missing day.
- If the year is before the year of the first dose of study drug, then the last day of the month will be assigned to the missing day.
- If the year is after the year of the first dose of study drug, then “01” will be assigned to the missing day.

Missing month only:

- If the year is the same as the year of the first dose of study drug, then the month of the first dose of study drug will be assigned to the missing month.
- If the year is before the year of the first dose of study drug, then December will be assigned to the missing month.

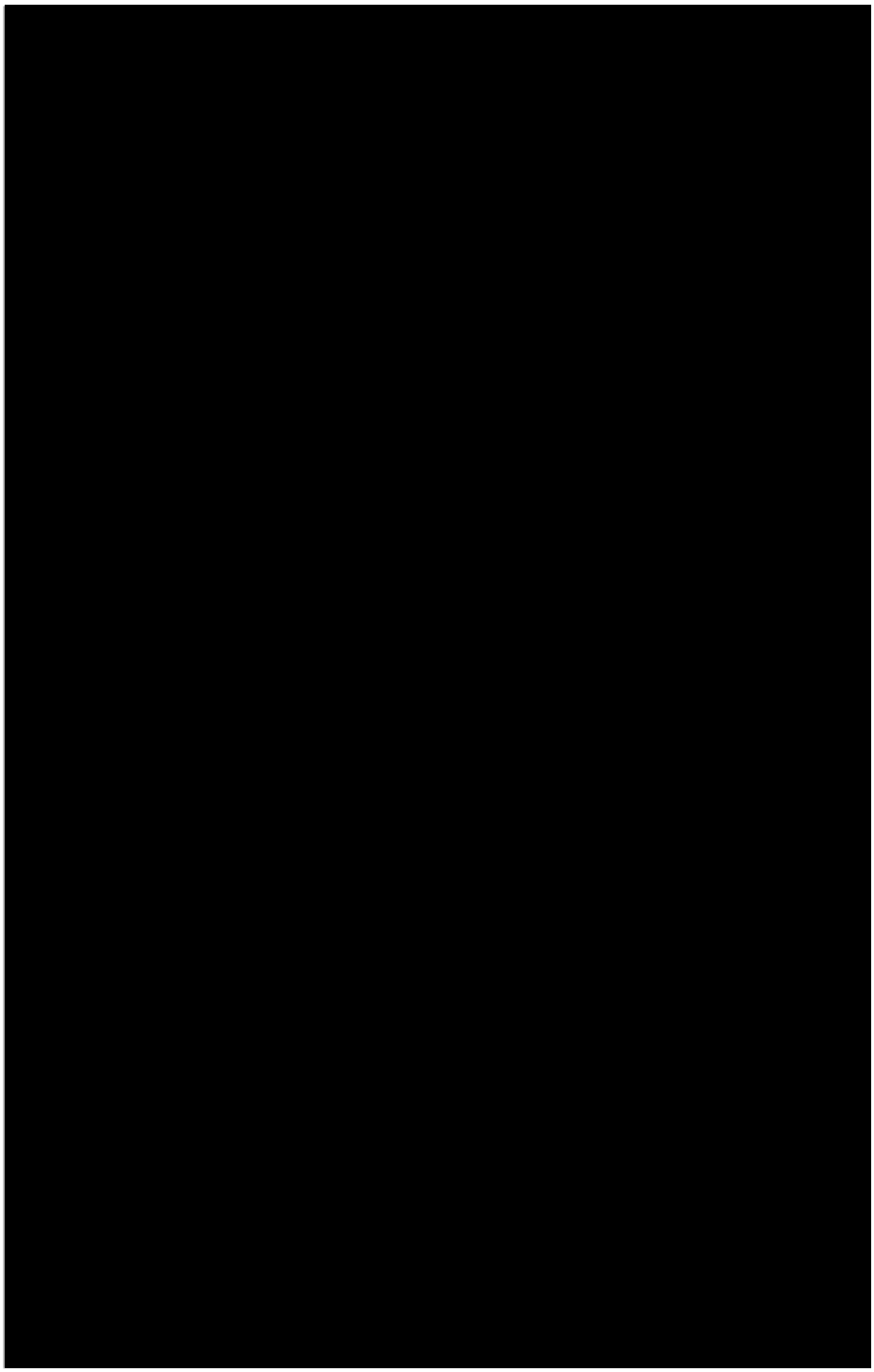
- If the year is after the year of the first dose of study drug, then January will be assigned to the missing month.

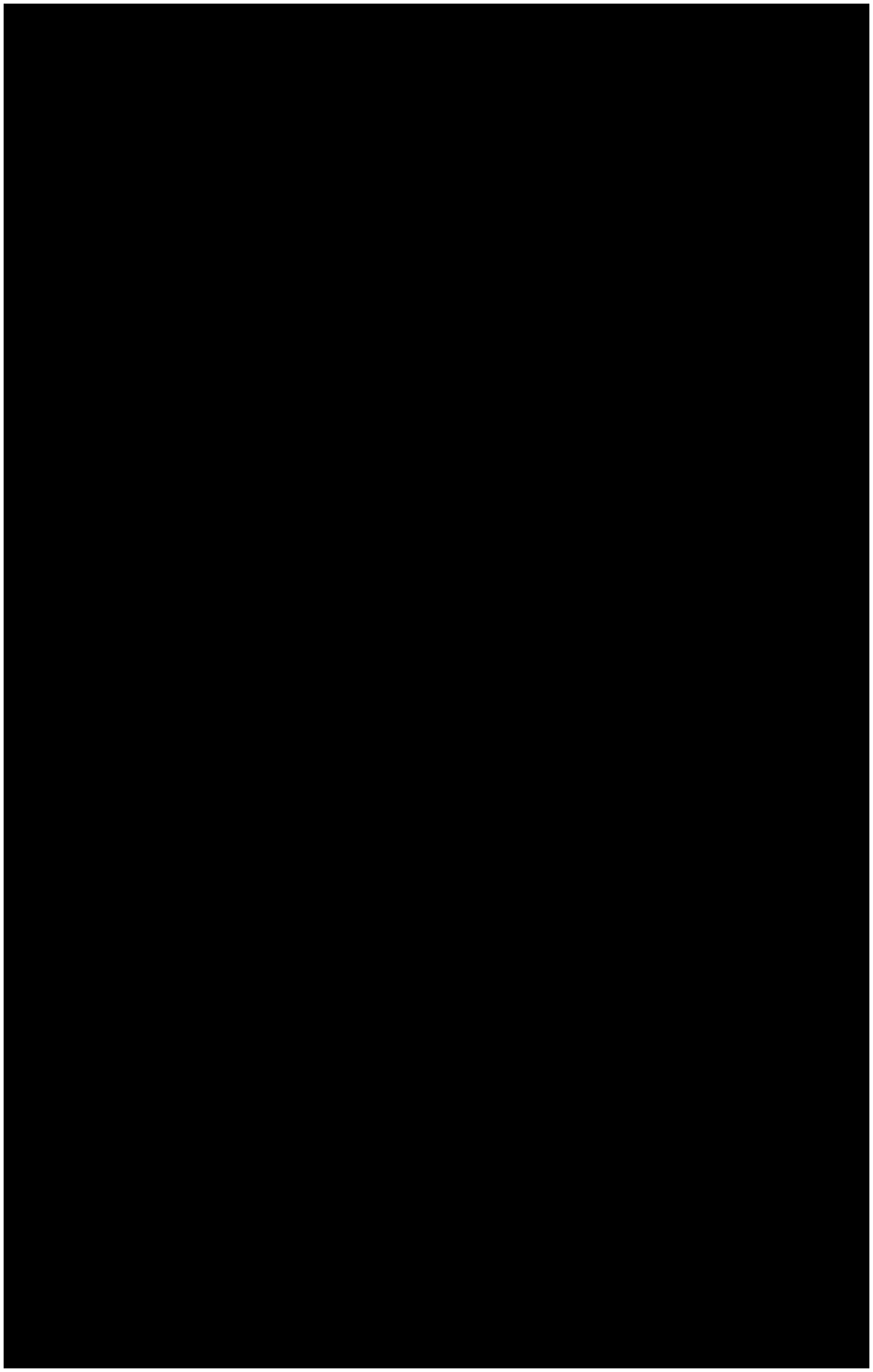
Missing day and month:

- If the year is the same as the first dose of study drug, then the date of the first dose of study drug will be assigned to the missing fields.
- If the year is before the year of first dose of study drug, then December 31 will be assigned to the missing fields.
- If the year is after the year of first dose of study drug, then January 1 will be assigned to the missing fields.

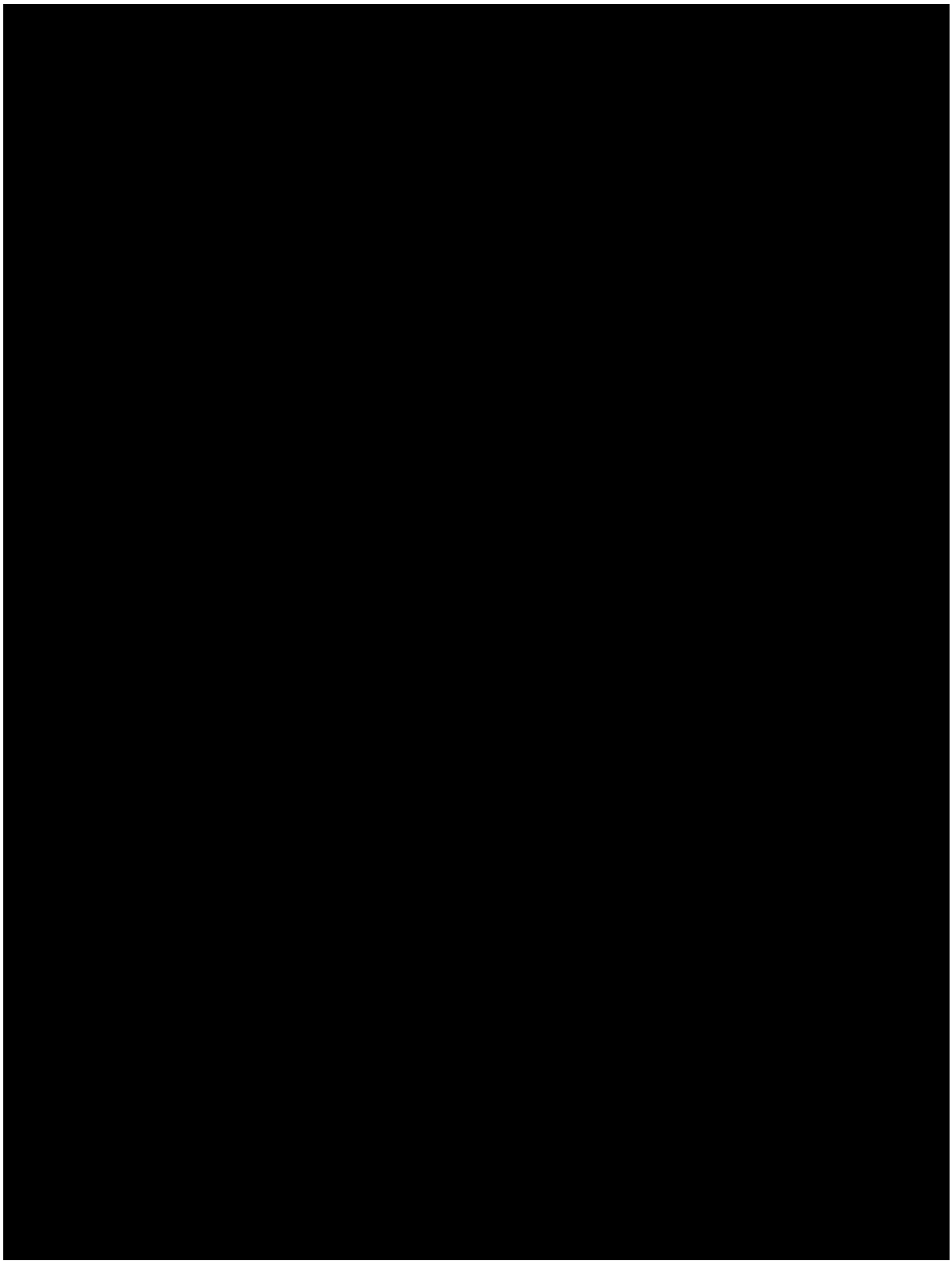
If year is missing, onset date will not be imputed. If the imputed end date is non-missing and the imputed onset date is after the imputed end date, the onset date will be imputed by the imputed end date.

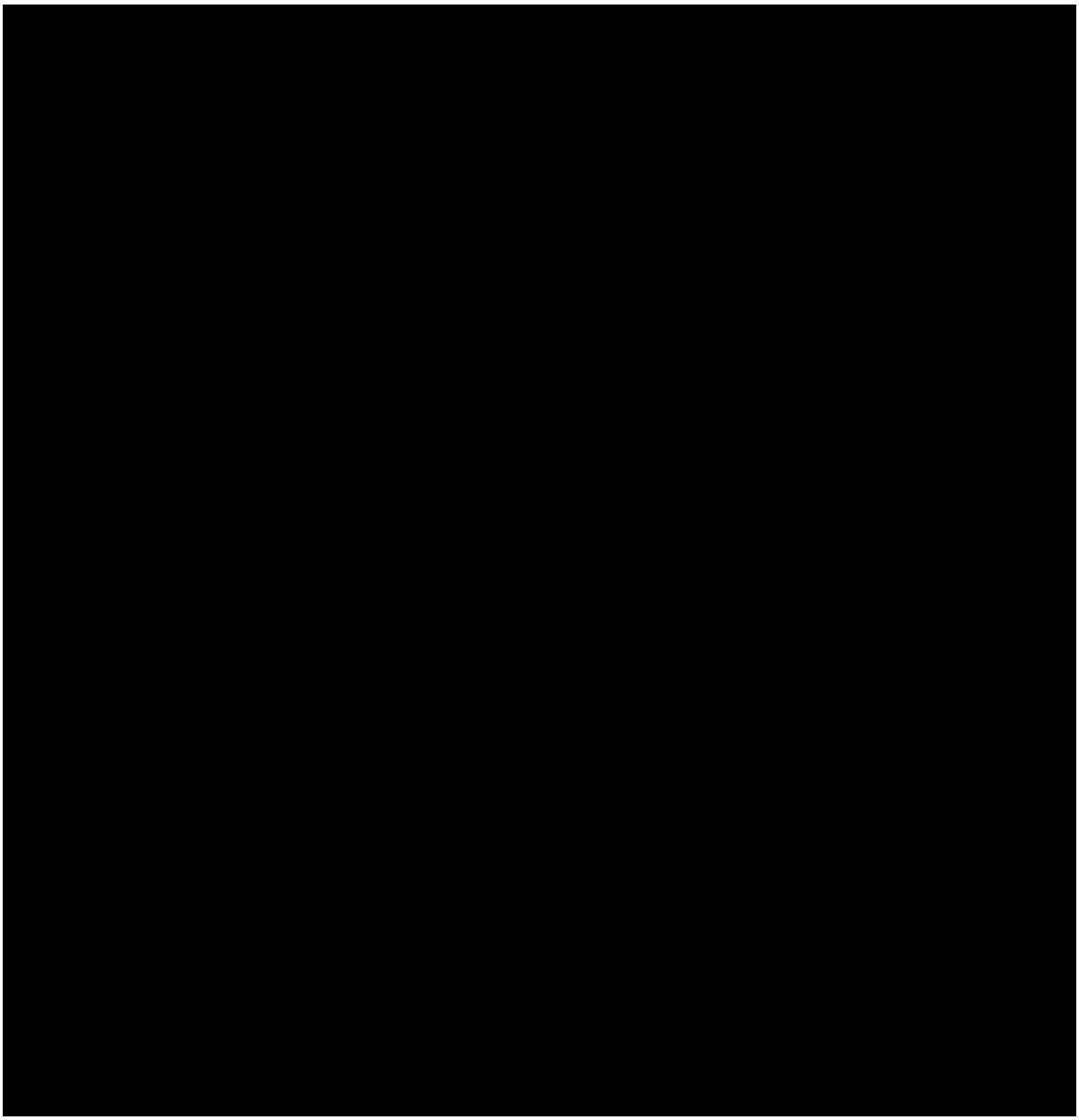
15.5. Pemphigus Disease Activity and Quality of Life Evaluation Instruments: ABQOL, TABQOL, PDAI, ABSIS





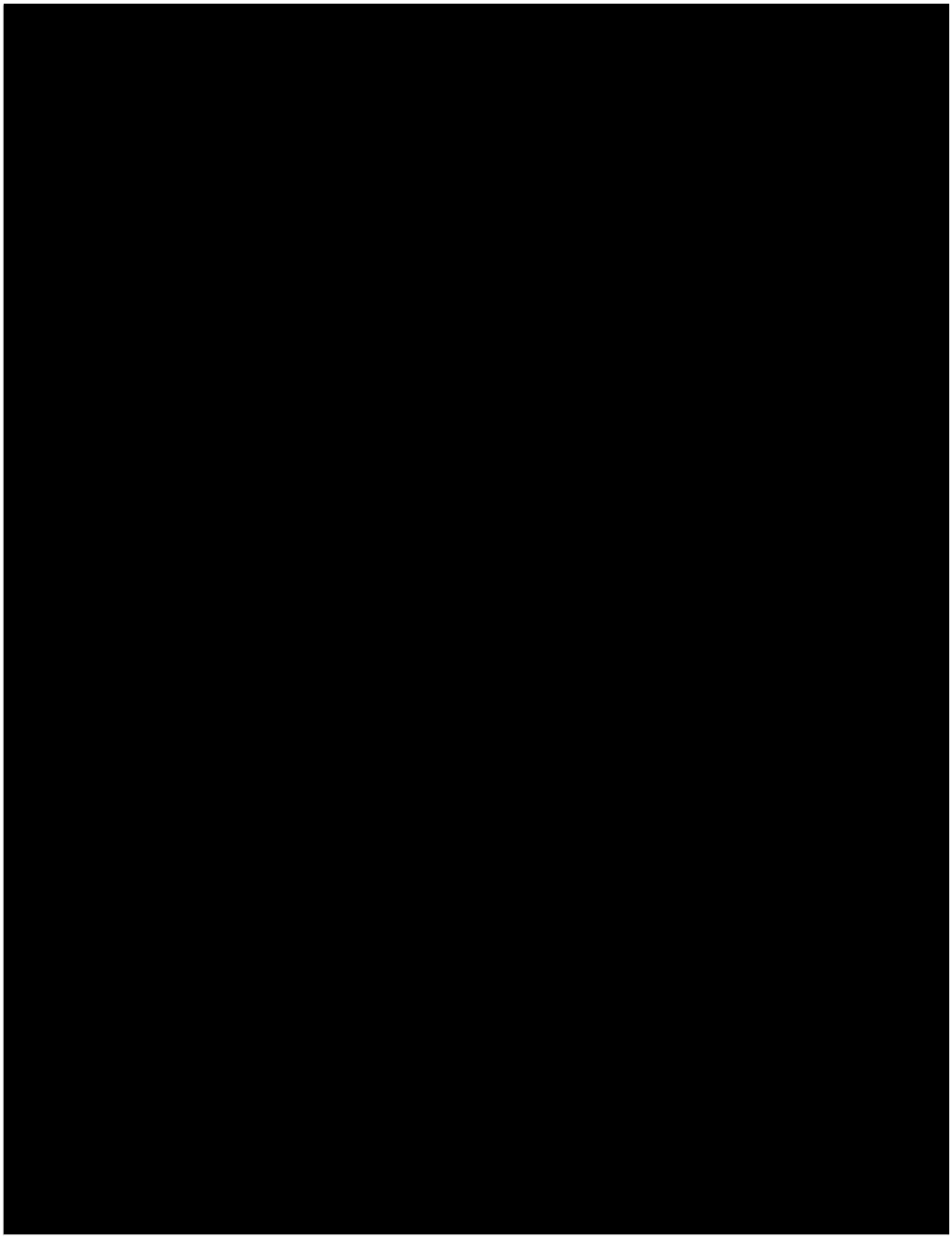
Treatment of Autoimmune Bullous Diseases Quality of Life Assessment (TABQOL)



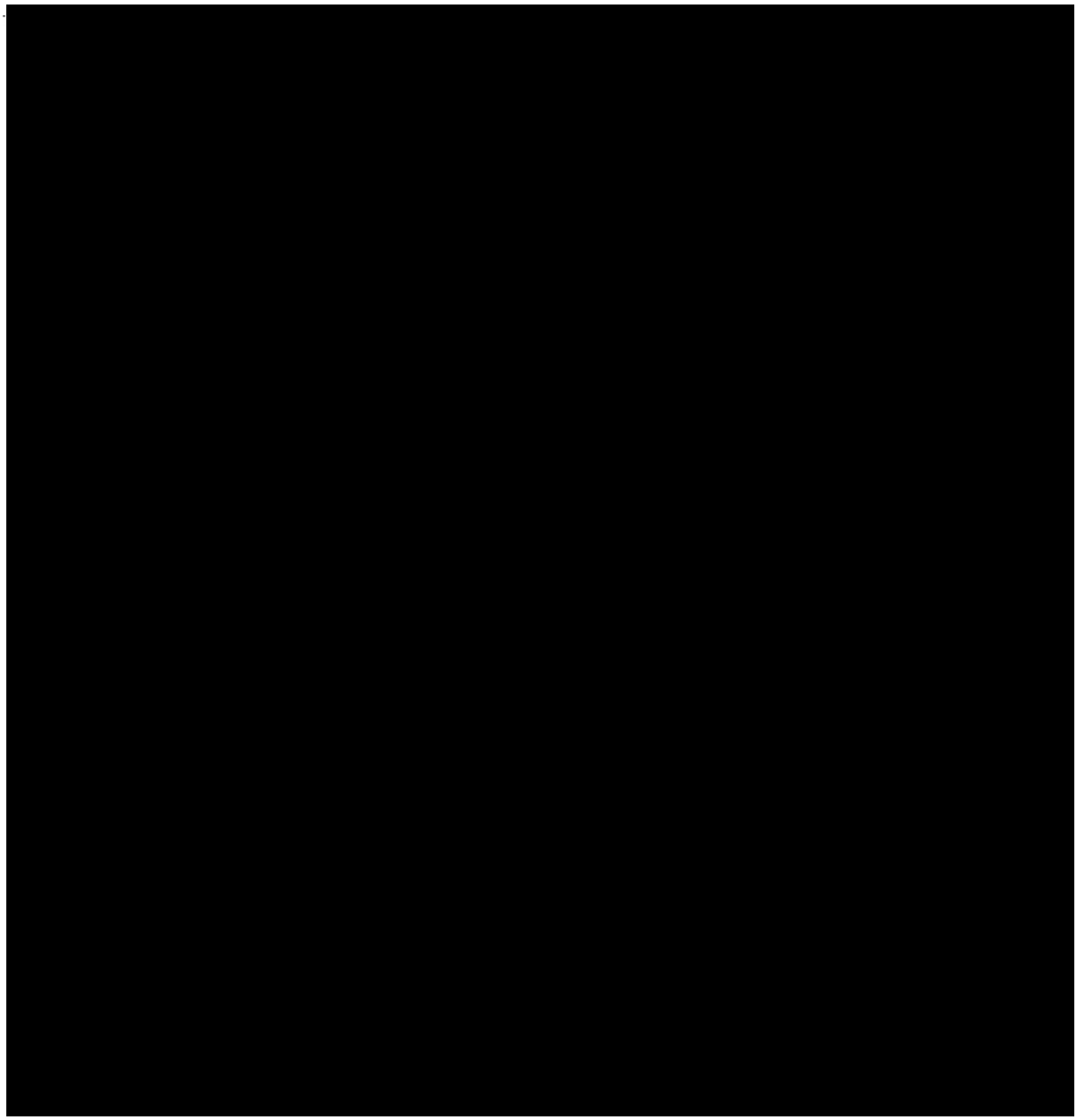


From: Tjokrowidjaja et al. 2013

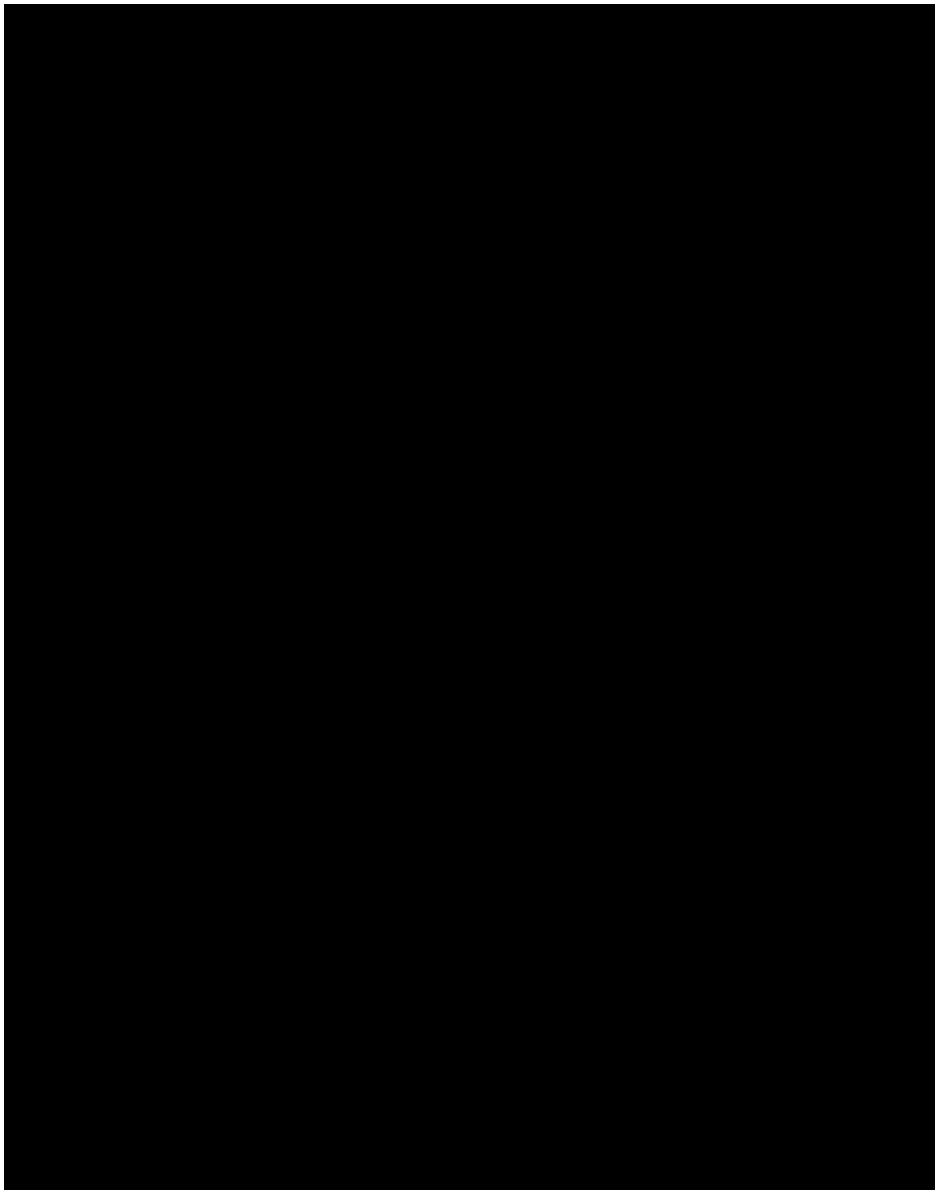
Pemphigus Disease Activity Index (PDAI) (Murell et al. 2008)



Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) (Rosenbach et al. 2009)



15.6. Simple Nutritional Appetite Questionnaire (SNAQ)



From Wilson et al., 2005

15.7. CS taper protocol

Maintain CS dose for 2 weeks after disease control has been achieved. Subsequently, reduce the CS dose by 15% every three weeks.

-OR-

Table 1. Glucocorticoid Taper Schedules

Prednisone Dosage, mg/d × 7 d		
40	17.5	5
35	15	4
30	12.5	3
25	10	2
20	7.5	1
Prednisone, mg Every Other Day × 8 d[a]		
40-35	40-5	12.5-1
40-30	40-4	12.5-0
40-25	40-3	10-0
40-20	35-3	7.5-0
40-18	30-3	6-0
40-15	30-2	5-0
40-15	25-2	4-0
40-13	20-2	3-0
40-10	17.5-2	2-0
40-7.5	17.5-1	1-0
40-6	15-1	0

From: Werth et al. 2008

Sponsor:	Principia Biopharma Inc..
Protocol:	PRN1008-005
Protocol Title:	An Open-Label, Phase 2, Pilot Study Investigating the Safety, Clinical Activity, Pharmacokinetics, and Pharmacodynamics of Oral Treatment with the BTK Inhibitor PRN1008 in Patients with Newly Diagnosed or Relapsing Pemphigus Vulgaris
SAP Version:	Final v2.0
SAP Date:	16JAN2019

The statistical analysis plan has been reviewed and approved.

Sponsor:

[redacted]

[redacted]

Signature Date

Author: [redacted]

[redacted]

Signature Date

STATISTICAL ANALYSIS PLAN TABLES AND LISTINGS MOCK SHELLS AND PROGRAMMING NOTES

Protocol Title:	An Open-Label, Phase 2, Pilot Study Investigating the Safety, Clinical Activity, Pharmacokinetics, and Pharmacodynamics of Oral Treatment with the BTK Inhibitor PRN1008 in Patients with Newly Diagnosed or Relapsing Pemphigus Vulgaris (Part A)
Protocol Number:	PRN1008-005
Sponsor:	Principia Biopharma Australia Pty Ltd. 400 East Jamie Court, Suite 302 South San Francisco, CA 94080 Phone: (650) 416-7700
Author:	Summit Analytical, LLC 8354 Northfield Blvd. Bldg. G Suite 3700 Denver, CO 80205
SAP Date:	2019-01-16
Status:	Final 2.0

Document History – Changes compared to previous version of SAP Shells:

Version	Date	Changes
V1.1	05JAN2018	Protocol amendment for French site: add listings for Brain MRI, Neurological symptoms, Neurological examination
V1.1	05JAN2018	Protocol addendum: update exist listings for PDAI and ABSIS to add screening visit results
V1.1	05JAN2018	ABSIS score: include Total activity score for all patients. Add subset analyses for patients with baseline oral involvement total score >0 and =0
V1.1	05JAN2018	Add new tables for cumulative incidence of CDA and CR by visit
V1.1	22JAN2018	Add baseline anti-DSG 1 and anti-DSG 3 antibody status in T14.1.2.1
V1.2	28JUL2018	Incorporated sponsor comments.
V1.3	02NOV2018	Modify Demographics table for DSG, add Treatment related TEAE table, add CDA/CR by DSG tables. Remove Urinanalysis shift table. Other minor updates.
V1.4	12DEC2018	Updated with new Analysis population
V2.0	20DEC2018	Updated demographics table and corticosteroid by visit table, and upversion to final

Table of Contents

Table 14.1.1.1
Summary of Analysis Populations
Screening Population

	400 mg	500 mg	600 mg	Total [1]
SCREENING POPULATION				xx
ENROLLED				xx (xx.x)
SCREEN FAILURE				xx (xx.x)
SAFETY ANALYSIS POPULATION [2]	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ITT POPULATION [2]	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
mITT POPULATION [2]	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PP POPULATION [2]	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
[1] Percentages are based on the number of patients in Screening Population.
[2] Percentages in respective dose levels are based on the number of patients in Total column.
Source Data: Listing 16.2.1.1 and Listing 16.2.2.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.1.2
Summary of Patient Disposition
Safety (ITT) [1] Analysis Population

	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
COMPLETION STATUS				
COMPLETED	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
EARLY WITHDRAWAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PRIMARY REASONS FOR EARLY WITHDRAWAL [2]				
ADVERSE EVENT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
DEATH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
LACK OF EFFICACY	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
LOST TO FOLLOW-UP	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NON-COMPLIANCE WITH STUDY DRUG	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PHYSICIAN DECISION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREGNANCY	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PROGRESSIVE DISEASE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PROTOCOL VIOLATION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
RECOVERY	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
STUDY TERMINATED BY SPONSOR	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TECHNICAL PROBLEM	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
WITHDRAWAL BY SUBJECT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
[1] Safety and ITT Populations include the same patients.
[2] Percentages are based on the number of patients who withdrew early from the study in the respective dose levels.
Source Data: Listing 16.2.1.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.1.3
Summary of Protocol Deviations
Safety Analysis Population

	Total (N=xx) n (%)
PATIENTS WITH AT LEAST ONE MAJOR PROTOCOL DEVIATION	xx (xx.x)
CATEGORY 1	xx (xx.x)
CATEGORY 2	xx (xx.x)
CATEGORY 3	xx (xx.x)
CATEGORY 4	xx (xx.x)
…	

Note: Patients could have more than one major protocol deviation. Site-level Protocol Deviations are not included in this table.
Source Data: Listing 16.2.1.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.1.4
Summary of Screen Failures
Screening Population

	Total (N=xx) n (%)
SCREEN FAILURES	xx (xx.x)
SCREAN FAILURE REASONS	
<Inclusion Cetieria 1 verbatim>	xx (xx.x)
…	xx (xx.x)
…	xx (xx.x)
	xx (xx.x)
…	

Note: Only Inclusion/Exclusion Critiera with at least one subject are presented.
Each subject could be excluded due to multiple reasons.
Source Data: Listing 16.2.1.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

PROGRAMMING NOTE: All subjects in Part A is included in this table, regardless of whether they have signed ICF.
Percentage for total screen failures uses the Total number of subjects as denominator.
Percentages for each screen fail reasons use total screen failures as denominator.
Use IETEST, IE criteria verbatim as the screen fail reasons, instead of IN01, IN02 etc.
Only include criteria where at least one subject is present.
Total number of screen fail reasons should be at least as many as total screen failed, but may be more if some subjects have more than one screen fail reason.

Table 14.1.2.1
Summary of Demographics and Baseline Characteristics
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
AGE (years)				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
SEX n (%)				
MALE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
FEMALE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
RACE n (%)				
WHITE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
BLACK OR AFRICAN AMERICAN	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ASIAN	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
AMERICAN INDIAN OR ALASKA NATIVE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NATIVE HAWAIIAN OR OTHER PACIFIC ISLANDER	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
FRENCH SITES ONLY - NOT COLLECTED	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ETHNICITY n (%)				
HISPANIC OR LATINO	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT HISPANIC OR LATINO	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
FRENCH SITES ONLY - NOT COLLECTED	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
BMI=(Baseline body weight in kilograms)/(Baseline height in meters)^2
[1] Time from pemphigus vulgaris confirmed date to screen date in months is defined as (Screening Date - Date of pemphigus vulgaris confirmed +1) / 30.4375
[2] Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.
Source Data: Listing 16.2.2.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.2.1
Summary of Demographics and Baseline Characteristics
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
PEMPHIGUS TYPE n (%)				
PEMPHIGUS VULGARIS	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
BULLOUS PEMPHIGOID	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PEMPHIGUS FOLLACEUS	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
EPIDERMOLYSIS BULLOSA AQUISITA	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
LINEAR IGA BULLOUS DERMATOSES	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
MUCOUS MEMBRANE PEMPHIGOID	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TIME FROM PEMPHIGUS VULGARIS CONFIRMED DATE TO SCREEN DATE (Months) [1]				
n	xx	xx	xx	xx
MEAN (SD)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)
MEDIAN	xx.xxx	xx.xxx	xx.xxx	xx.xxx
MIN, MAX	xx.xx, xx.xx	xx.xx, xx.xx	xx.xx, xx.xx	xx.xx, xx.xx
PEMPHIGUS HISTORY TYPE n (%) [2]				
NEWLY DIAGNOSED	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
RELAPSED	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PEMPHIGUS ANTI-DSG PROFILE				
ANTI-DSG 3 POSITIVE (PV)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG 1 POSITIVE AND ANTI-DSG 3 NEGATIVE (PF)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG 1 NEGATIVE AND ANTI-DSG 3 NEGATIVE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
BMI=(Baseline body weight in kilograms)/(Baseline height in meters)^2
[1] Time from pemphigus vulgaris confirmed date to screen date in months is defined as (Screening Date – Date of pemphigus vulgaris confirmed +1) / 30.4375
[2] Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.

Source Data: Listing 16.2.2.1
<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.2.1
Summary of Demographics and Baseline Characteristics
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
PEMPHIGUS SEVERITY AT BASELINE				
MILD (PDAI ACTIVITY < 15)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
MODERATE (PDAI ACTIVITY ≥ 15)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG STATUS AT BASELINE n (%)				
ANTI-DSG 1 + and ANTI-DSG 3 -	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG 1 - and ANTI-DSG 3 +	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG 1 + and ANTI-DSG 3 +	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ANTI-DSG 1 - and ANTI-DSG 3 -	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL ANTI-DSG n (%)				
≥ 100	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
< 100	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PDAI SKIN ACTIVITY > 0 n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PDAI SCALP ACTIVITY > 0 n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PDAI MUCOSAL ACTIVITY > 0 n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABSIS ORAL ACTIVITY > 0 n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
BMI=(Baseline body weight in kilograms)/(Baseline height in meters)^2
[1] Time from pemphigus vulgaris confirmed date to screen date in months is defined as (Screening Date - Date of pemphigus vulgaris confirmed +1) / 30.4375
[2] Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.
Source Data: Listing 16.2.2.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.2.1
Summary of Demographics and Baseline Characteristics
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
HEIGHT (cm)				
n	xx	xx	xx	xx
MEAN (SD)	xxx.xx (xxx.xxx)	xxx.xx (xxx.xxx)	xxx.xx (xxx.xxx)	xxx.xx (xxx.xxx)
MEDIAN	xxx.xx	xxx.xx	xxx.xx	xxx.xx
MIN, MAX	xxx.x, xxx.x	xxx.x, xxx.x	xxx.x, xxx.x	xxx.x, xxx.x
WEIGHT (kg)				
n	xx	xx	xx	xx
MEAN (SD)	xx.xx (xx.xxx)	xx.xx (xx.xxx)	xx.xx (xx.xxx)	xx.xx (xx.xxx)
MEDIAN	xx.xx	xx.xx	xx.xx	xx.xx
MIN, MAX	xx.x, xxx.x	xx.x, xxx.x	xx.x, xxx.x	xx.x, xxx.x
BODY MASS INDEX (BMI) (kg/m^2)				
n	xx	xx	xx	xx
MEAN (SD)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)	xx.xxx (xx.xxxx)
MEDIAN	xx.xxx	xx.xxx	xx.xxx	xx.xxx
MIN, MAX	xx.xx, xx.xx	xx.xx, xx.xx	xx.xx, xx.xx	xx.xx, xx.xx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
BMI=(Baseline body weight in kilograms)/(Baseline height in meters)^2
[1] Time from pemphigus vulgaris confirmed date to screen date in months is defined as (Screening Date - Date of pemphigus vulgaris confirmed +1) / 30.4375
[2] Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.
Source Data: Listing 16.2.2.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Notes: for pemphigus type, do not need to display a category if count of this category equal to 0. The pemphigus type should be all PV since this is a PV trial.
Repeat the above table for the other analysis populations titled below.

Table 14.1.2.2
Summary of Demographics and Characteristics at Screening
ITT Population
Table 14.1.2.3
Summary of Demographics and Characteristics at Screening
mITT Population
Table 14.1.2.4
Summary of Demographics and Characteristics at Screening
Per Protocol Population

Table 14.1.3.1
Summary of Prior Corticosteroid Medications
Safety Analysis Population

ATC Level 4 Preferred Term	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
TOTAL NUMBER OF PRIOR CORTICOSTEROID MEDICATIONS	xx	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE PRIOR CORTICOSTEROID MEDICATION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ATC LEVEL 4 #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #4	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
A prior medication is defined as any medication that has a stop date before the date of first dose of study drug. A patient is counted only once if the patient reported multiple medications per each ATC level 4 or preferred term.
Medications with start date missing or before first dose and missing stop dates are classified as both prior and concomitant. Medications with start date on or after first dose and missing stop dates are classified as concomitant.
Prior medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).
Source Data: Listing 16.2.3.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Medications will be sorted in descending order by ATC level 4 category based on the total column. Within each ATC level 4 category, preferred terms will be sorted in descending order based on the total column.

Table 14.1.3.2
Summary of Concomitant Corticosteroid Medications
Safety Analysis Population

ATC Level 4 Preferred Term	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
TOTAL NUMBER OF CONCOMITANT CORTICOSTEROID MEDICATIONS	xx	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE CONCOMITANT CORTICOSTEROID MEDICATION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ATC LEVEL 4 #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #4	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
A Concomitant medication is defined as any medication that has a stop date on or after the date of first dose of study drug. A patient is counted only once if the patient reported multiple medications per each ATC level 4 or perferred term.
Medications with start date missing or before first dose and missing stop dates are classified as both prior and concomitant. Medications with start date on or after first dose and missing stop dates are classified as concomitant.
Concomitant medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).
Source Data: Listing 16.2.3.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Medications will be sorted in descending order by ATC level 2 category based on the total column. Within each ATC level 2 category, preferred terms will be sorted in descending order based on the total column.

Table 14.1.3.3
Summary of Prior Non-Corticosteroid Medications
Safety Analysis Population

ATC Level 4 Preferred Term	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
TOTAL NUMBER OF PRIOR NON-CORTICOSTEROID MEDICATIONS	xx	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE PRIOR NON-CORTICOSTEROID MEDICATION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ATC LEVEL 4 #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #4	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
A prior medication is defined as any medication that has a stop date before the date of first dose of study drug. A patient is counted only once if the patient reported multiple medications per each ATC level 4 or preferred term.
Medications with start date missing or before first dose and missing stop dates are classified as both prior and concomitant. Medications with start date on or after first dose and missing stop dates are classified as concomitant.
Prior medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).
Source Data: Listing 16.2.3.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Medications will be sorted in descending order by ATC level 2 category based on the total column. Within each ATC level 2 category, preferred terms will be sorted in descending order based on the total column.

Table 14.1.3.4
Summary of Concomitant Non-Corticosteroid Medications
Safety Analysis Population

ATC Level 4 Preferred Term	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
TOTAL NUMBER OF CONCOMITANT NON-CORTICOSTEROID MEDICATIONS	xx	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE CONCOMITANT NON-CORTICOSTEROID MEDICATION	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ATC LEVEL 4 #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #4	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
A Concomitant medication is defined as any medication that has a stop date on or after the date of first dose of study drug. A patient is counted only once if the patient reported multiple medications per each ATC level 4 or perferred term.
Medications with start date missing or before first dose and missing stop dates are classified as both prior and concomitant. Medications with start date on or after first dose and missing stop dates are classified as concomitant.
Concomitant medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).
Source Data: Listing 16.2.3.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Medications will be sorted in descending order by ATC level 2 category based on the total column. Within each ATC level 2 category, preferred terms will be sorted in descending order based on the total column.

Table 14.1.3.5
Summary of Treatment Exposure
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
OVERALL DURATION OF EXPOSURE (Days) [1]				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
AVERAGE DAILY DOSE (mg/day) [2]				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
NUMBER OF DOSE ADJUSTMENTS n (%)				
1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
MINIMUM DOSE LEVEL (mg) n (%)				
100	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
200	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
300	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
400	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
[1] Overall duration of exposure = Date of last dose – Date of first dose + 1
[2] Average daily dose = Sum of doses taken during the treatment / Overall duration of exposure
Source Data: Listing 16.2.3.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.1.3.6
Summary of Treatment Compliance
Safety Analysis Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
OVERALL COMPLIANCE (%) [1]				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
OVERALL COMPLIANCE CATEGORY n (%)				
< 80%	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
>= 80% and <=120%	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
> 120%	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
[1] Overall compliance (%) = (Total number of tablets taken during the treatment / Total number of tablets planned during the treatment) * 100
Source Data: Listing 16.2.3.6

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.2.1.1
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Prednis(ol)one >0.5 mg/kg
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Patients who achieved CDA at or prior to the Week 5 visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg before the first CDA date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Early treatment terminators or subjects whose CDA status are completely missing are considered non-responders.

Repeat this table for the other populations:

Table 14.2.1.2
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Prednis(ol)one >0.5 mg/kg
mITT Population

Table 14.2.1.3
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Prednis(ol)one >0.5 mg/kg
Per Protocol Population

Table 14.2.2.1.1
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Corticosteroids
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Patients who achieved CDA at or before the Week 5 visit and had no occurrence of any corticosteroidbefore the first CDA visit during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Early treatment terminators or subjects whose CDA status are completely missing are considered non-responders.
Repeate this table for the other populations

Table 14.2.2.1.2
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Corticosteroids
mITT Population

Table 14.2.2.1.3
Proportion of Patients who Achieved Control of Disease Activity (CDA) at or Prior to Week 5 Visit without Corticosteroids
Per Protocol Population

Table 14.2.2.2.1
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Corticosteroids
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients who achieved CR at or prior to the Week 13 visit and had no occurrence of any corticosteroid before the first CR date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Early treatment terminators or subjects whose CR status are completely missing are considered non-responders.
Repeat for the other populations.

Table 14.2.2.2.2
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Corticosteroids
mITT Population

Table 14.2.2.2.3
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Corticosteroids
Per Protocol Population

Table 14.2.2.3.1
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Prednis(ol)one >0.5 mg/kg
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients achieved CR at any visit on or before the Week 13 visit during the study treatment and had no occurrence of daily prednis(ol)one >0.5 mg/kg from the first dose taken date up to the day before first CR date will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Early treatment terminators or subjects whose CR status are completely missing are considered non-responders.
Repeat above table for the other populations.

Table 14.2.2.3.2
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Prednis(ol)one >0.5 mg/kg
mITT Population

Table 14.2.2.3.3
Proportion of Patients who Achieved Complete Response (CR) at or Prior to Week 13 Visit without Prednis(ol)one >0.5 mg/kg
Per Protocol Population

Table 14.2.2.4.1.1
Proportion of Patients who Achieved Control of Disease Activity (CDA) at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat the above table for the other populations

Table 14.2.2.4.1.2
Proportion of Patients who Achieved Control of Disease Activity (CDA) at Visit
mITT Population

Table 14.2.2.4.1.3
Proportion of Patients who Achieved Control of Disease Activity (CDA) at Visit
Per Protocol Population

Table 14.2.2.4.2.1
Proportion of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Patients who achieved CDA at a specific visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg before each visit date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat above table for other populations.

Table 14.2.2.4.2.2
Proportion of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg at Visit
mITT Population

Table 14.2.2.4.2.3
Proportion of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg at Visit6uiol
Per Protocol Population

Table 14.2.2.4.3.1
Proportion of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Patients who achieved CDA at a specific visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg before each visit date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat above table with the other populations.

Table 14.2.2.4.3.2
Proportion of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg at Visit
mITT Population

Table 14.2.2.4.3.3
Proportion of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg at Visit
Per Protocol Population

Table 14.2.2.4.4.1
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visits are not included in this summary.
Patients who ever achieved CDA on or before a specific visit during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Repeat above table with other populations:

Table 14.2.2.4.4.2
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) by Visit
mITT Population

Table 14.2.2.4.4.3
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) by Visit
Per Protocol Population

Table 14.2.2.4.5.1
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visits are not included in this summary.
Patients who ever achieved CDA and had no occurrence of daily prednis(ol)one >0.5 mg/kg before the first CDA date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Repeat above table for the following:

Table 14.2.2.4.5.2
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg by Visit
mITT Population

Table 14.2.2.4.5.3
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) without Prednis(ol)one >0.5 mg/kg by Visit
Per Protocol Population

Table 14.2.2.4.6.1
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visits are not included in this summary.
Patients who ever achieved CDA and had daily prednis(ol)one >0.5 mg/kg before a speicfic visit during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Repeat table for following populations

Table 14.2.2.4.6.2
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg by Visit
mITT Population

Table 14.2.2.4.6.3
Cumulative Incidence of Patients who Achieved Control of Disease Activity (CDA) with Prednis(ol)one >0.5 mg/kg by Visit
Per Protocol Population

Table 14.2.2.5.1.1
Proportion of Patients who Achieved Complete Response (CR) at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat for the following populations.

Table 14.2.2.5.1.2
Proportion of Patients who Achieved Complete Response (CR) at Visit
mITT Population

Table 14.2.2.5.1.3
Proportion of Patients who Achieved Complete Response (CR) at Visit
Per Protocol Population

Table 14.2.2.5.2.1
Proportion of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients who achieved CR at a specific visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg on or before the first CR date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Only Systemetic Corticosteroids are considered for this table.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat for the following populations

Table 14.2.2.5.2.2
Proportion of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg at Visit
mITT Population
Table 14.2.2.5.2.3
Proportion of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg at Visit
Per Protocol Population

Table 14.2.2.5.3.1
Proportion of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg at Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients who achieved CR at a specific visit and had daily prednis(ol)one >0.5 mg/kg on or before the first CR date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Only Systemetic Corticosteroids are considered for this table.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat for the following populations

Table 14.2.2.5.3.2
Proportion of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg at Visit
mITT Population

Table 14.2.2.5.3.3
Proportion of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg at Visit
PP Population

Table 14.2.2.5.4.1
Cumulative Incidence of Patients who Achieved Complete Response (CR) by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visit are not included in this summary.
Patients who ever achieved CR on or before a specific visit during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Subject who terminated on the first day of treatment without any further follow up is not included in this analysis. Further, if a subject drop out, they will still accommodate to future unattended visits cumulatively.
Repeat for following populations:

Table 14.2.2.5.4.2
Cumulative Incidence of Patients who Achieved Complete Response (CR) by Visit
mITT Population

Table 14.2.2.5.4.3
Cumulative Incidence of Patients who Achieved Complete Response (CR) by Visit
Per Protocol Population

Table 14.2.2.5.5.1
Cumulative Incidence of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg by Visit
ITT Analysis Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visits are not included in this summary.
Patients who ever achieved CR and had no occurrence of daily prednis(ol)one >0.5 mg/kg before the first CR date during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Only Systemetic Corticosteroids are considered for this table.

Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Repeat above table for the following

Table 14.2.2.5.5.2
Cumulative Incidence of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg by Visit
mITT Population

Table 14.2.2.5.5.3
Cumulative Incidence of Patients who Achieved Complete Response (CR) without Prednis(ol)one >0.5 mg/kg by Visit
Per Protocol Population

Table 14.2.2.5.6.1
Cumulative Incidence of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Cumulative Responder Rate by WEEK3 D15				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
Cumulative Responder Rate by WEEK5 D29				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
…				
Cumulative Responder Rate by WEEK25 D169				
n (%)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy. Cumulative responder rate is used, i.e., any patient achieves the response at any study visit is counted into all the later study visits.
Unscheduled visits are not included in this summary.
Patients who ever achieved CR and had daily prednis(ol)one >0.5 mg/kg before a specific visit during the study treatment will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Only Systemetic Corticosteroids are considered for this table.
Source Data: Listing 16.2.4.1.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Repeat table above for following populations

Table 14.2.2.5.6.2
Cumulative Incidence of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg by Visit
mITT Population

Table 14.2.2.5.6.3
Cumulative Incidence of Patients who Achieved Complete Response (CR) with Prednis(ol)one >0.5 mg/kg by Visit
Per Protocol Population

Table 14.2.2.5.7.1
Duration of Complete Response (CR) Status Among CR Subjects
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients achieved CR at any visit on or before the Week 13 visit during the study treatment and had no occurrence of daily prednis(ol)one >0.5 mg/kg from the first dose taken date up to the day before first CR date will be counted as responders.
Two-sided 80% and 95% confidence intervals are calculated using the Exact (Clopper-Pearson) method.
Percentages are based on the number of patients in the respective dose levels in the Analysis Population.
Duration of CR is defined as the longest time subject is known to have had complete response, i.e. the earliest of either date of last known CR date or last follow up date - start date of CR + 1.
Source Data: Listing 16.2.4.1.10

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog note: Early treatment terminators or subjects whose CR status are completely missing are considered non-responders.
Repeat above table for the other populations.

Table 14.2.2.5.7.2
Duration of Complete Response (CR) Status Among CR Subjects
mITT Population

Table 14.2.2.5.7.3
Duration of Complete Response (CR) Status Among CR Subjects
Per Protocol Population

Table 14.2.3.1.1
Time to First Control of Disease Activity (CDA): Kaplan Meier Estimates
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
TIME TO FIRST CDA (days) [1]				
n	xx	xx	xx	xx
25% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MEDIAN	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
75% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MIN, MAX [2]	xx, xx	xx, xx*	xx, xx	xx*, xx
KAPLAN-MEIER ESTIMATE % (n') [3]				
15 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
29 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
57 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
85 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
113 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
141 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
169 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
[1] Time to first CDA = Date of first CDA confirmed – Date of first dose + 1. Patients who did not achieve CDA by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively. 80% and 95% confidence intervals are calculated using the LOGLOG transformation.
[2] * indicate that this value is from a censored data.
[3] Kaplan-Meier estimate of the probability of experiencing the event on or before the specified timepoint (n' = number of patients remaining at risk beyond the specified timepoint).
Source Data: Listing 16.2.4.1.6

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Repeat the table above with the following population.

Table 14.2.3.1.2
Time to First Control of Disease Activity (CDA): Kaplan Meier Estimates
mITT Population

Table 14.2.3.1.3
Time to First Control of Disease Activity (CDA): Kaplan Meier Estimates
Per Protocol Population

Table 14.2.3.2.1
Time to First Complete Response (CR): Kaplan Meier Estimates
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
TIME TO FIRST CR (days) [1]				
n	xx	xx	xx	xx
25% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MEDIAN	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
75% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MIN, MAX [2]	xx, xx	xx, xx*	xx, xx	xx*, xx
KAPLAN-MEIER ESTIMATE % (n') [3]				
15 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
29 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
57 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
85 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
113 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
141 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
169 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
[1] Time to first CR = Date of first CR confirmed – Date of first dose + 1. Patients who did not achieve CR by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.80% and 95% confidence intervals are calculated using the LOGLOG transformation.
[2] * indicate that this value is from a censored data.
[3] Kaplan-Meier estimate of the probability of experiencing the event on or before the specified timepoint (n' = number of patients remaining at risk beyond the specified timepoint).
Source Data: Listing 16.2.4.1.7

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Repeat for the following populations

Table 14.2.3.2.2
Time to First Complete Response (CR): Kaplan Meier Estimates
mITT Population

Table 14.2.3.2.3
Time to First Complete Response (CR): Kaplan Meier Estimates
Per Protocol Population

Table 14.2.3.3.1
Time to First End of Consolidation Phase: Kaplan Meier Estimates
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
TIME TO END OF CONSOLIDATION PHASE (days) [1]				
n	xx	xx	xx	xx
25% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MEDIAN	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
75% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MIN, MAX [2]	xx, xx	xx, xx*	xx, xx	xx*, xx
KAPLAN-MEIER ESTIMATE % (n') [3]				
15 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
29 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
57 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
85 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
113 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
141 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
169 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. End of consolidation phase: The time at which no new lesions have developed for minimum of 2 weeks, approximately 80% of lesions have healed, and when most clinicians start to taper steroids.
[1] Time to end of consolidation phase = Date of end of consolidation phase confirmed – Date of first dose + 1. Patients who did not achieve end of consolidation phase by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively. 80% and 95% confidence intervals are calculated using the LOGLOG transformation.
[2] * indicate that this value is from a censored data.
[3] Kaplan-Meier estimate of the probability of experiencing the event on or before the specified timepoint

(n' = number of patients remaining at risk beyond the specified timepoint).
Source Data: Listing 16.2.4.1.8

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Repeat for the following population

Table 14.2.3.2.2
Time to First Complete Response (CR): Kaplan Meier Estimates
mITT Population

Table 14.2.3.2.3
Time to First Complete Response (CR): Kaplan Meier Estimates
Per Protocol Population

Table 14.2.3.4.1
Time to First Relapse after PRN1008 Treatment Completion or Discontinuation: Kaplan Meier Estimates
ITT Population

	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
PATIENTS WITH CDA AT WEEK 13	XX	XX	XX	XX
TIME TO FIRST RELAPSE (days) [1]				
n	xx	xx	xx	xx
25% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MEDIAN	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
75% QUARTILE	xx.x	xx.x	xx.x	xx.x
80% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
95% CONFIDENCE INTERVAL	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)	(xx.x, xx.x)
MIN, MAX [2]	xx, xx	xx, xx*	xx, xx	xx*, xx
KAPLAN-MEIER ESTIMATE % (n') [3]				
29 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
57 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)
85 DAYS	xx.x (xx)	xx.x (xx)	xx.x (xx)	xx.x (xx)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Relapse/flare: Appearance of >=3 new lesions/month that do not heal spontaneously within 1 week, or by extension of established lesions, in a patient who has achieved disease control.
Only patients with confirmed CDA before treatment completion or discontinuation will be included into this analysis.
[1] Time to first relapse = Date of first relapse confirmed after Week 13 – Date of last dose + 1.
Patients who did not achieve relapse between Week 13 visit and the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.80% and 95% confidence intervals are calculated using the LOGLOG transformation.
[2] * indicate that this value is from a censored data.
[3] Kaplan-Meier estimate of the probability of experiencing the event on or before the specified timepoint (n' = number of patients remaining at risk beyond the specified timepoint).

Source Data: Listing 16.2.4.1.9

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog note: Repeat for 2) Patients with CR at the Week 13 visit in the same table, and change the first row accordingly.

Repeat the above table with all the subpopulations using the following analysis populations.

Table 14.2.3.4.2

Time to First Relapse after PRN1008 Treatment Completion or Discontinuation Among Subjects who Achieved CDA at Week 13 Visit: Kaplan Meier Estimates
mITT Population

Table 14.2.3.4.3

Time to First Relapse after PRN1008 Treatment Completion or Discontinuation Among Subjects who Achieved CDA at Week 13 Visit: Kaplan Meier Estimates
Per Protocol Population

Table 14.2.4.1.1
Cumulative Corticosteroid Usage over 12 Weeks
ITT Population

Cumulative Corticosteroid Usage (mg)	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Patients who have completed at least Week 13 Visit are included.
All corticosteriods were converted into prednisolone. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.
Source Data: Listing 16.2.3.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Repeat the above table using the other populations

Table 14.2.4.1.2
Cumulative Corticosteroid Usage over 12 Weeks
mITT Population

Table 14.2.4.1.3
Cumulative Corticosteroid Usage over 12 Weeks
Per Protocol Population

Table 14.2.4.2.1
Corticosteroid Usage by Visit
ITT Population

CUMULATIVE CORTICOSTEROID USAGE (mg)	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
SCREENING				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK1 D1				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
All corticosteriods were converted into prednisolone. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.
The dosage level at screening and Week 1 day 1 visit is defined as the dose level on the visit day, and that on all post baseline visits is defined as the dose on the day before the visit date.
Source Data: Listing 16.2.3.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
PROG NOTE: If a subject is still enrolled in the study but does not report any CS, then the dosage level is set as 0. If a subject is terminated prior to a visit, then this subject is not included.

Repeat the above table with the following populations.

Table 14.2.4.2.2
Corticosteroid Usage by Visit
mITT Population

Table 14.2.4.2.3
Corticosteroid Usage by Visit
Per Protocol Population

Table 14.2.5.1.1
Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Activity Score by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Total activity score = Total skin activity + Total scalp activity + Total mucosa activity.
Source Data: Listing 16.2.4.2.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following populations

Table 14.2.5.1.2

Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Activity Score by Visit

mITT Population

Table 14.2.5.1.3

Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Activity Score by Visit

Per Protocol Population

Table 14.2.5.2.1
Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Damage Score by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Total damage score = Total skin damage + Total scalp damage
Source Data: Listing 16.2.4.2.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following population.

Table 14.2.5.2.2

Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Damage Score by Visit

mITT Population

Table 14.2.5.2.3

Summary of Actual Value and Change from Baseline in Pemphigus Disease Area Index (PDAI) Total Damage Score by Visit

Per Protocol Population

Table 14.2.6.1.1
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) by Visit
ITT Population

Score: Skin Involvement Total Score

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.4.3.1 – Listing 16.2.4.3.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programmer note: please repeat "Oral Involvement Total Score" and "Total Activity Score" on the new pages.
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following Populations

Table 14.2.6.1.2
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
by Visit
mITT Population

Table 14.2.6.1.3
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
by Visit
Per Protocol Population

Table 14.2.6.2.1
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) by Visit for Patients with Baseline Oral Involvement Total Score Greater than Zero
ITT Population

Score: Skin Involvement Total Score

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.

Source Data: Listing 16.2.4.3.1 – Listing 16.2.4.3.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programmer note: please repeat "Oral Involvement Total Score", "Severity Total Score", "Total Activity Score", and "Total ABSIS Score" on the new pages.
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat for the following populations:

Table 14.2.6.2.2
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) by Visit for Patients with Baseline Oral Involvement Total Score Greater than Zero
mITT Population

Table 14.2.6.2.3
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) by Visit for Patients with Baseline Oral Involvement Total Score Greater than Zero
Per Protocol Population

Table 14.2.6.3.1
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
by Visit for Patients with Baseline Oral Involvement Total Score Equal to Zero
ITT Population

Score: Skin Involvement Total Score

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.

Source Data: Listing 16.2.4.3.1 – Listing 16.2.4.3.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programmer note: please repeat "Oral Involvement Total Score" and "Total Activity Score" on the new pages.
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.
Repeat for the following populations:

Table 14.2.6.3.2
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
by Visit for Patients with Baseline Oral Involvement Total Score Equal to Zero
mITT Population

Table 14.2.6.3.3
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
by Visit for Patients with Baseline Oral Involvement Total Score Equal to Zero
Per Protocol Population

Table 14.2.7.1
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Diseases Quality of Life (ABQOL) Score by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.4.4.1 and Listing 16.2.4.4.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following populations

Table 14.2.7.2
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Diseases Quality of Life (ABQOL) Score by Visit
mITT Population

Table 14.2.7.3
Summary of Actual Value and Change from Baseline in Autoimmune Bullous Diseases Quality of Life (ABQOL) Score by Visit
Per Protocol Population

Table 14.2.8.1
Summary of Actual Value and Change from Baseline in Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) Total Score by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.4.5.1 and Listing 16.2.4.5.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following populations

Table 14.2.8.2

Summary of Actual Value and Change from Baseline in Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) Total Score by Visit
mITT Population

Table 14.2.8.3

Summary of Actual Value and Change from Baseline in Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) Total Score by Visit
Per Protocol Population

Table 14.2.9.1
Summary of Actual Value and Change from Baseline in Simplified Nutritional Appetite Questionnaire (SNAQ) Score by Visit
ITT Population

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.4.6.1 and Listing 16.2.4.6.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Repeat for the following populations:

Table 14.2.9.2

Summary of Actual Value and Change from Baseline in Simplified Nutritional Appetite Questionnaire (SNAQ) Score by Visit

mITT Population

Table 14.2.9.3

Summary of Actual Value and Change from Baseline in Simplified Nutritional Appetite Questionnaire (SNAQ) Score by Visit

Per Protocol Population

Table 14.2.10.1.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Age Group
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable Age (≥ 50, < 50).

Repeat for the following populations.

Table 14.2.10.1.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Age Group
mITT Population

Table 14.2.10.1.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Age Group
Per Protocol Population

Table 14.2.10.2.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Gender
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable female male.

Repeat for the following populations.

Table 14.2.10.2.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Gender
mITT Population

Table 14.2.10.2.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Gender
Per Protocol Population

Table 14.2.10.3.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Baseline Total Anti-DSG Category
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable baseline total anti-DSG (≥ 100, < 100).

Repeat for the following populations.

Table 14.2.10.3.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Baseline Total Anti-DSG Category
mITT Population

Table 14.2.10.3.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Baseline Total Anti-DSG Category
Per Protocol Population

Table 14.2.10.4.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus History Type
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable Pemphigus History Type (Newly Diagnosed vs Relapsed).
Add footnote: Newly diagnosed subjects are defined as diagnostic date being less than 6 months to Screening date, otherwise a subject is defined as relapsed.

Repeat for the following populations.

Table 14.2.10.4.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus History Type
mITT Population

Table 14.2.10.4.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus History Type
Per Protocol Population

Table 14.2.10.5.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable anti-dsg1 positive vs. anti-dsg1 positive anti-dag3 negative vs. neither positive.

Repeat for the following populations.

Table 14.2.10.5.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
mITT Population

Table 14.2.10.5.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
Per Protocol Population

Table 14.2.10.6.1
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
ITT Population

Prog Note: Repeat Table 14.2.2.4.5 with the addition of page variable Pemphigus Severity: mild vs moderate. Add footnote: Mild Pemphigus subject is defined as screening PDAI < 15, otherwise moderate.

Repeat for the following populations.

Table 14.2.10.6.2
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
mITT Population

Table 14.2.10.6.3
Cumulative Incidents of Control of Disease Activity (CDA) without Predniso(lo)ne > 0.5mg/kg by Visit by Pemphigus Anti-DSG Profile
Per Protocol Population

Table 14.3.1.1
Overall Summary of Treatment-Emergent Adverse Events
Safety Analysis Population

	400 mg (N=xx) n (%) [E]	500 mg (N=xx) n (%) [E]	600 mg (N=xx) n (%) [E]	Total (N=xx) n (%) [E]
ANY TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx
ANY SERIOUS TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx
ANY STUDY DRUG-RELATED TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx
ANY STUDY DRUG-RELATED TREATMENT-EMERGENT SERIOUS ADVERSE EVENT	xx (xx.x) xxx	xx (xx.x) xxx	xx (xx.x) xxx	xx (xx.x) xxx
ANY TREATMENT-EMERGENT ADVERSE EVENT LEADING TO TREATMENT DISCONTINUATION	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx
ANY TREATMENT-EMERGENT ADVERSE EVENT LEADING TO DEATH	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx	xx (xx.x) xx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
A treatment-emergent AE (TEAE) is defined as an AE that begins on or after the first study drug taken.
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Source Data: Listing 16.2.5.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.3.1.2
Treatment-Emergent Adverse Events by System Organ Class and Preferred Term
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) n (%) [E]	500 mg (N=xx) n (%) [E]	600 mg (N=xx) n (%) [E]	Total (N=xx) n (%) [E]
ALL TREATMENT-EMERGENT ADVERSE EVENTS	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				
SYSTEM ORGAN CLASS #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events.
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.1.3
Treatment-Emergent Adverse Events by Relationship to Study Drug
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) Not Related n (%) [E]	 Related n (%) [E]	 Total n (%) [E]
ALL TREATMENT-EMERGENT ADVERSE EVENTS	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #3	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…			
…			

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events. If the relationship of an AE is missing, the AE is imputed as "Related".
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: Include 500 mg, 600 mg, Total on the subsequent pages. AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.1.4
Treatment-Emergent Adverse Events by Severity
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) Grade 1 n (%)	Grade 2 n (%)	Grade 3 n (%)	Grade 4 n (%)	Grade 5 n (%)	Total n (%)
TOTAL NUMBER OF TREATMENT-EMERGENT ADVERSE EVENTS	xx	xx	xx	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…						

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once for the most severe event if the patient reported one or more events. If the severity of an AE is missing, the AE is imputed as "Grade 3".
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Include 500 mg, 600 mg and Total on the subsequent pages AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.1.5
Treatment Related Treatment-Emergent Adverse Events by Severity
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) Grade 1 n (%)	 Grade 2 n (%)	 Grade 3 n (%)	 Grade 4 n (%)	 Grade 5 n (%)	 Total n (%)
TOTAL NUMBER OF TREATMENT RELATED TREATMENT-EMERGENT ADVERSE EVENTS	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NUMBER OF PATIENTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…						

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once for the most severe event if the patient reported one or more events. If the severity of an AE is missing, the AE is imputed as "Grade 3".
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Include 500 mg, 600 mg and Total on the subsequent pages AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.2.1
Serious Treatment-Emergent Adverse Events by System Organ Class and Preferred Term
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) n (%) [E]	500 mg (N=xx) n (%) [E]	600 mg (N=xx) n (%)[E]	Total (N=xx) n (%)[E]
ALL SERIOUS TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				
SYSTEM ORGAN CLASS #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events.
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.2.2
Serious Treatment-Emergent Adverse Events by Relationship to Study Drug
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) Not Related n (%) [E]	 Related n (%) [E]	 Total n (%) [E]
ALL SERIOUS TREATMENT-EMERGENT ADVERSE EVENTS	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #3	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…			
…			

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events. If the relationship of an AE is missing, the AE is imputed as "Related".
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: Include 600 mg, Total on the subsequent pages. AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.2.3
Treatment-Emergent Adverse Events Leading to Treatment Discontinuation
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) n (%) [E]	500 mg (N=xx) n (%) [E]	600 mg (N=xx) n (%) [E]	Total (N=xx) n (%) [E]
ALL TREATMENT-EMERGENT ADVERSE EVENTS LEADING TO TREATMENT DISCONTINUATION	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				
SYSTEM ORGAN CLASS #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events.
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.2.4
Treatment-Emergent Adverse Events Leading to Death
Safety Analysis Population

System Organ Class Preferred Term	400 mg (N=xx) n (%) [E]	500 mg (N=xx) n (%) [E]	600 mg (N=xx) n (%) [E]	Total (N=xx) n (%) [E]
ALL TREATMENT-EMERGENT ADVERSE EVENTS LEADING TO DEATH	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
SYSTEM ORGAN CLASS #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				
SYSTEM ORGAN CLASS #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #1	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
PREFERRED TERM #2	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]	xx (xx.x) [xx]
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
At each level of patient summarization, a patient is counted once if the patient reported one or more events.
n: number of patients at each level of summarization.
[E]: number of events at each level of summarization.
Adverse Events were coded using MedDRA, Version 18.0.
Source Data: Listing 16.2.5.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: AEs will be sorted in descending order of frequency of SOC based on the total of all treatment groups. Within each SOC, Preferred terms will also be sorted in descending order.

Table 14.3.3.1
Summary of Actual Value and Change from Baseline in Chemistry Tests by Visit
Safety Analysis Population

Parameter: LAB TEST PARAMETER #1 (UNIT)

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE				
AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE				
AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Programming Note: Please include “Unscheduled” as the last visit if there are records with unscheduled visit as a visit label.

Table 14.3.3.2
Summary of Actual Value and Change from Baseline in Clinical Laboratory Tests Monitored for Drug Induced Liver Injury (DILI) by Visit
Safety Analysis Population

Parameter: LAB TEST PARAMETER #1 (UNIT)

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.

Clinical laboratory tests monitored for drug induced liver injury (DILI) include ALT (alanine aminotransferase or SGPT), AST (aspartate transaminase or SGOT), TBL (total bilirubin), and ALP (alkaline phosphatase).

Source Data: Listing 16.2.6.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programming Note: Please include "Unscheduled" as the last visit if there are records with unscheduled visit as a visit label.

Table 14.3.3.3
Summary of Actual Value and Change from Baseline in Hematology Tests by Visit
Safety Analysis Population

Parameter: LAB TEST PARAMETER #1 (UNIT)

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programming Note: Please include “Unscheduled” as the last visit if there are records with unscheduled visit as a visit label.

Table 14.3.3.4
Summary of Actual Value and Change from Baseline in Coagulation Tests by Visit
Safety Analysis Population

Parameter: LAB TEST PARAMETER #1 (UNIT)

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK3 D15				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
…				
CHANGE FROM BASELINE AT WEEK5 D29				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programming Note: Please include “Unscheduled” as the last visit if there are records with unscheduled visit as a visit label.

Table 14.3.3.5
Summary of Urinalysis Tests at Screening
Safety Analysis Population

Laboratory Test	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
LAB TEST PARAMETER #1				
CATEGORY 1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…				
LAB TEST PARAMETER #2 (UNIT)				
CATEGORY 1	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 2	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…				
LAB TEST PARAMETER #3 (UNIT)				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Percentages are based on the number of patients with data at each visit of the respective dose levels.
Source Data: Listing 16.2.6.5

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Programming Note: Please include “Unscheduled” as the last visit if there are records with unscheduled visit as a visit label.

Table 14.3.3.6
Shift from Baseline to Post-baseline Visits in Normality Status of Chemistry Tests
Safety Analysis Population

Parameter: LAB TEST #1 (UNIT)

	400 mg (N=xx)				500 mg (N=xx)			
	Baseline				Baseline			
Visit	Low n (%)	Normal n (%)	High n (%)	Total n (%)	Low n (%)	Normal n (%)	High n (%)	Total n (%)
WEEK3 D15								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx
WEEK5 D29								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx

…

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Only patients with non-missing values at both baseline and respective post-baseline visit will be counted in the summary table.
Percentages are based on the number of patients with data at each post-baseline visit of the respective dose levels.
Source Data: Listing 16.2.6.1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMONYYYY hh:mm

Prog Note: Include 600 mg, Total on the subsequent pages. Lab test parameters will be displayed in the same order as the lab test listing.
Programming Note: Please include “Unscheduled” as the last visit if there are records with unscheduled visit as a visit label.
In the case where Lower or higher upper ranges are not available for a specific parameter, the lower and higher categories will be populated with N/A.

Table 14.3.3.7
Shift from Baseline to Post-baseline Visits in Normality Status of Clinical Laboratory Tests Monitored for Drug Induced Liver Injury (DILI)
Safety Analysis Population

Parameter: LAB TEST #1 (UNIT)

	400 mg (N=xx)				500 mg (N=xx)			
	Baseline				Baseline			
Visit	Low n (%)	Normal n (%)	High n (%)	Total n (%)	Low n (%)	Normal n (%)	High n (%)	Total n (%)
WEEK3 D15								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx
WEEK5 D29								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx

…

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Only patients with non-missing values at both baseline and respective post-baseline visit will be counted in the summary table.
Clinical laboratory tests monitored for drug induced liver injury (DILI) include ALT (alanine aminotransferase or SGPT), AST (aspartate transaminase or SGOT), TBL (total bilirubin), and ALP (alkaline phosphatase).
Percentages are based on the number of patients with data at each post-baseline visit of the respective dose levels.
Source Data: Listing 16.2.6.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMONYYYY hh:mm
Prog Note: Include 600 mg, Total on the subsequent pages. Lab test parameters will be displayed in the same order as the lab test listing.

In the case where Lower or higher upper ranges are not available for a specific parameter, the lower and higher categories will be populated with N/A.

Table 14.3.3.8
Shift from Baseline to Post-baseline Visits in Normality Status of Hematology Tests
Safety Analysis Population

Parameter: LAB TEST #1 (UNIT)

	400 mg (N=xx)				500 mg (N=xx)			
	Baseline				Baseline			
Visit	Low n (%)	Normal n (%)	High n (%)	Total n (%)	Low n (%)	Normal n (%)	High n (%)	Total n (%)
WEEK3 D15								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx
WEEK5 D29								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx

…

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Only patients with non-missing values at both baseline and respective post-baseline visit will be counted in the summary table.
Percentages are based on the number of patients with data at each post-baseline visit of the respective dose levels.
Source Data: Listing 16.2.6.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMONYYYY hh:mm

Prog Note: Include 600 mg, Total on the subsequent pages. Lab test parameters will be displayed in the same order as the lab test listing.
Please include "Unscheduled" as the last visit if there are records with unscheduled visit as a visit label.
In the case where Lower or higher upper ranges are not available for a specific parameter, the lower and higher categories will be populated with N/A.

Table 14.3.3.9
Shift from Baseline to Post-baseline Visits in Normality Status of Coagulation Tests
Safety Analysis Population

Parameter: LAB TEST #1 (UNIT)

	400 mg (N=xx)				500 mg (N=xx)			
	Baseline				Baseline			
Visit	Low n (%)	Normal n (%)	High n (%)	Total n (%)	Low n (%)	Normal n (%)	High n (%)	Total n (%)
WEEK3 D15								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx
WEEK5 D29								
LOW	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
HIGH	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
TOTAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx

…

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Only patients with non-missing values at both baseline and respective post-baseline visit will be counted in the summary table.
Percentages are based on the number of patients with data at each post-baseline visit of the respective dose levels.
Source Data: Listing 16.2.6.4

<DIRECTORY PATH>PROGRAM NAME Executed: DDMONYYYY hh:mm

Prog Note: Include 600 mg, Total on the subsequent pages. Lab test parameters will be displayed in the same order as the lab test listing.
In the case where Lower or higher upper ranges are not available for a specific parameter, the lower and higher categories will be populated with N/A.

Table 14.3.4.1
Summary of Actual Value and Change from Baseline in Vital Signs by Visit
Safety Analysis Population

Parameter: VITAL SIGN PARAMETER #1 (UNIT)

Visit	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
BASELINE				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK1 D2				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE				
AT WEEK1 D2				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK 3 D15				
…				
CHANGE FROM BASELINE				
AT WEEK3 D15				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.7

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Programming Note: Please include "Unscheduled" as the last visit if there are records with unscheduled visit as a visit label.

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Table 14.3.4.2
Summary of Blood Pressure and Heart Rate Outside Normal Range
Safety Analysis Population

Parameter: VITAL SIGN PARAMETER #1 (UNIT)

	400 mg (N=xx)				500 mg (N=xx)			
Visit	n	Low	Normal	High	n	Low	Normal	High
BASELINE	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK1 D2	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK3 D15	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK5 D29	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK9 D57	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK13 D85	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK17 D113	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK21 D141	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK25 D169	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Systolic blood pressure values <90 or >140 mmHg, diastolic blood pressure values <60 or >80 mmHg, heart rate <40 or >100 beats per minute will be counted as Low or High, respectively.
Percentages are based on number of patients with non-missing values in each visit.
Source Data: Listing 16.2.6.7

<DIRECTORY PATH>PROGRAM NAME Executed: DDMONYYYY hh:mm

Prog Note: Include 600 mg and Total on the subseqent pages.

Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Table 14.3.5.1
Summary of Physical Examination at Screening Visit
Safety Analysis Population

Body System	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
CRITERIA #1				
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT DONE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
CRITERIA #2				
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT DONE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
CRITERIA #3				
…				
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.8

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: physical examination at screening visit include: general appearance; skin; eyes, ears, nose, throat; heart; chest/breast; abdomen; neurologycal; lymphnodes; skeletal; other.

Table 14.3.5.2
Summary of Physical Examination in Post Screening Visit
Safety Analysis Population

Body System: CRITERIA #1

Visit	400 mg (N=xx) n (%)	500 mg (N=xx) n (%)	600 mg (N=xx) n (%)	Total (N=xx) n (%)
BASELINE				
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT DONE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
WEEK3 D15				
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT DONE	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…				

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.8

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: physical examination after screening visit include: general appearance; abdomen; cardiorespiratory; other.
Prog Note: For all efficacy and safety by visit summaries, add "unscheduled" visit after other visits if such data exist in the database.

Table 14.3.6.1
Summary of Electrocardiogram at Screening Visit
Safety Analysis Population

Parameter	400 mg (N=xx)	500 mg (N=xx)	600 mg (N=xx)	Total (N=xx)
Ventricular Rate (BEATS/MIN)				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
PR Interval (msec)				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
QRS Duration (msec)				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
QT Interval (msec)				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
QTcF Interval (msec)				
n	xxx	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
QTcB Interval (msec)				
n	xxx	xxx	xxx	xxx

MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
Normal Sinus Rhythm, n (%)				
YES	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
NO	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
Interpretation, n (%)				
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL NOT CLINICALLY SIGNIFICANT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL CLINICALLY SIGNIFICANT	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Source Data: Listing 16.2.6.9

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Table 14.4.1.1
Summary of Actual Value and Change from Baseline in Anti-DSG by Visit
Safety Analysis Population

Repeat table 14.3.3.1, for Anti-DSG 1, Anti-DSG 3 and Total Anti-DSG, which is defined as Anti-DSG 1+Anti-DSG 3. The unit is to be shown as "unit(s)".
If either Anti-DSG 1 or 3 is missing, then total Anti-DSG is set as missing.
Missing values are not imputed and not included.
Only include visits where Anti-DSG are supposed to be measured per protocol, i.e. day 1, week 3, 5, 9, 13, 17, 21 and 25.
Percentage is based on the number of subject with non-missing data for both baseline and post-baseline visit in the parameter of interest.
This analysis will be performed only using Anti-DSG 1 and Anti-DSG 3 data above the limit of quantification, and for Total Anti-DSG, only use samples where at least one of Anti-DSG 1 or 3 is above the limit of quantification at baseline Anti-DSG value is available for each given visit.
Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Total Anti-DSG is defined as the sum of Anti-DSG 1 and 3, and is set to missing if either is missing.
Source Data: Listing 16.2.7.3

Table 14.4.2.1
Change from Baseline in Anti-DSG Status by CDA status at Week 5 without Prednis(ol)one >0.5 mg/kg
ITT Population

CDA BY WEEK 5: YES

ANTI-DSG PARAMETER TIME POINT Statistics	400mg (N=xx)	500mg (N=xx)	600mg (N=xx)	Total (N=xx)
Anti-DSG 1 (unit)				
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK5 D29				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK5 D29				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Patients who achieved CDA at the Week 5 visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg before the the first CDA date during the study treatment will be counted as responders.
Total Anti-DSG is defined as the sum of Anti-DSG 1 and 3, and is set to missing if either is missing.
Source Data: Listing 16.2.4.1.1, 16.2.7.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: repeat for Anti-DSG 3 and Total Anti-DSG.
PROG NOTE: Repeat for CDA BY WEEK 5 VISIT: No AND OVERALL.

This analysis will be performed only using Anti-DSG 1 and Anti-DSG 3 data above the limit of quantification, and for Total Anti-DSG, only use samples where at least one of Anti-DSG 1 or 3 is above the limit of quantification at baseline and the Week 5 visit Anti-DSG value is available.

Table 14.4.2.2
Change from Baseline in Anti-DSG Status by CR status at Week 13 without Prednis(ol)one >0.5 mg/kg
ITT Population

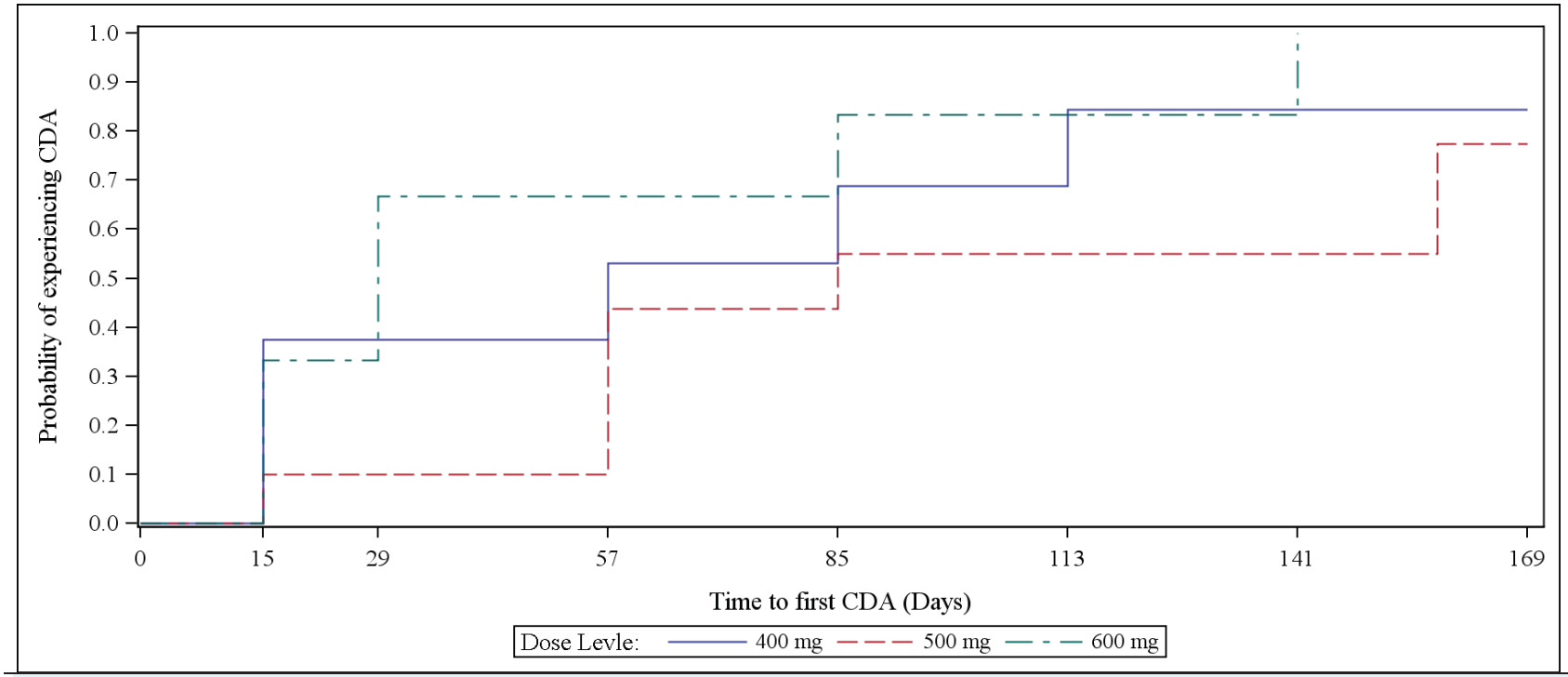
CR STATUS BY WEEK 13: YES

ANTI-DSG PARAMETER TIME POINT Statistics	400mg (N=xx)	500mg (N=xx)	600mg (N=xx)	Total (N=xx)
Anti-DSG 1 (unit)				
BASELINE				
n	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
WEEK13 D85				
N	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx
CHANGE FROM BASELINE AT WEEK13 D85				
N	xx	xx	xx	xx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx	xxx, xxx

Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Patients who achieved CR at the Week 13 visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg on or before the CR date during the study treatment will be counted as responders.
Total Anti-DSG is defined as the sum of Anti-DSG 1 and 3, and is set to missing if either is missing.
Source Data: Listing 16.2.4.1.1, 16.2.7.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm
Prog Note: repeat for Anti-DSG 3 and Total Anti-DSG. This analysis will be performed only using Anti-DSG 1 and Anti-DSG 3 data above the limit of quantification, and for Total Anti-DSG, only use samples where at least one of Anti-DSG 1 or 3 is above the limit of quantification at baseline and the Week 5 visit Anti-DSG value is available.

Figure 14.2.3.1.1
Kaplan Meier Plots for Time to First Control of Disease Activity (CDA)
ITT Population



Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Time to first CDA = Date of first CDA confirmed – Date of first dose + 1.
Patients who have not achieved CDA by the date of study completion or discontinuation will be censored at the date of completion or discontinuation.
Source Data: Table 14.2.3.1

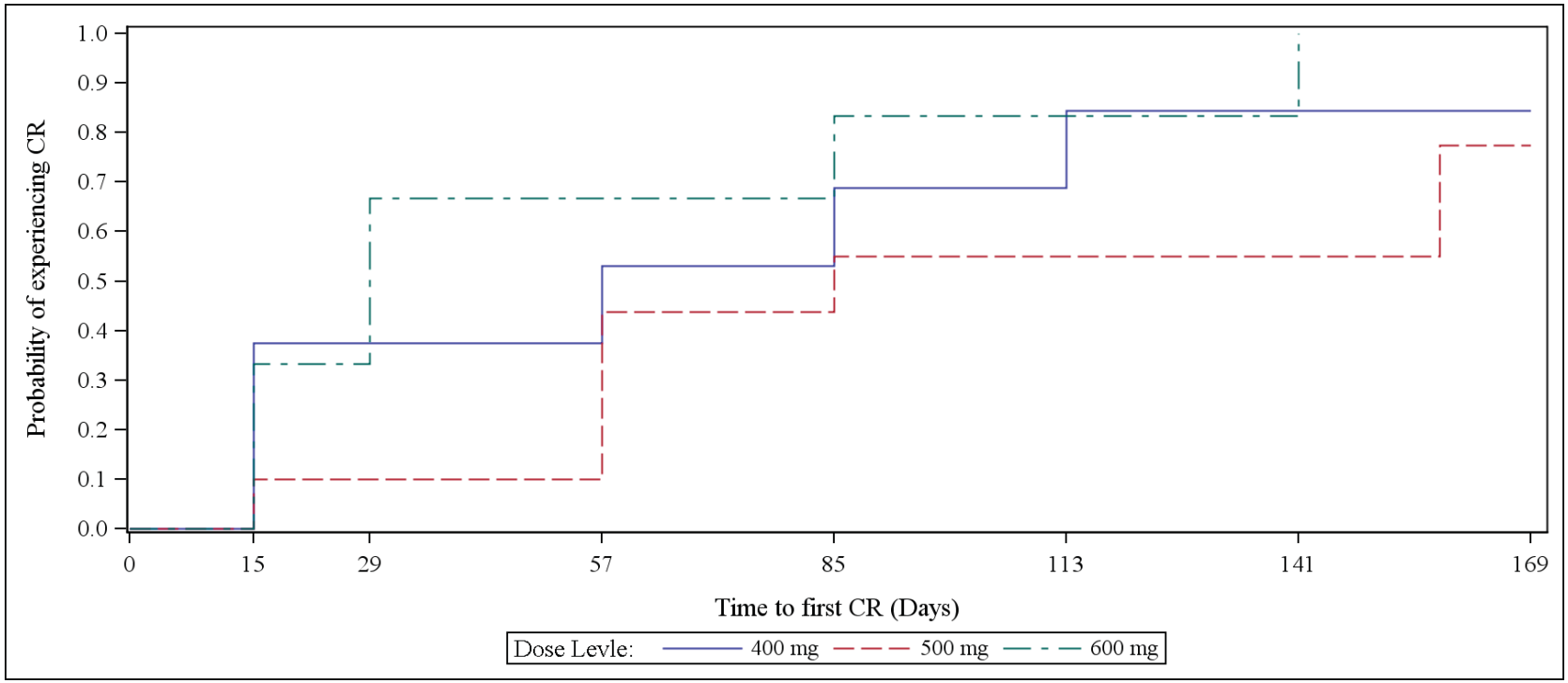
<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog NOTE: Please also add "all dose levels" in this plot.
Repeat for the following populations

Figure 14.2.3.1.2
Kaplan Meier Plots for Time to First Control of Disease Activity (CDA)
mITT Population

Figure 14.2.3.1.3
Kaplan Meier Plots for Time to First Control of Disease Activity (CDA)
PP Population

Figure 14.2.3.2.1
Kaplan Meier Plots for Time to First Complete Response (CR)
ITT Population



Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. Complete response (CR) is defined as the absence of new or established lesions while the patient is Receiving minimal or no non-PRN 1008 immunotherapy.
Time to first CR = Date of first CR confirmed – Date of first dose + 1.
Patients who have not achieved CR by the date of study completion or discontinuation will be censored at the date of completion or discontinuation.
Source Data: Table 14.2.3.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

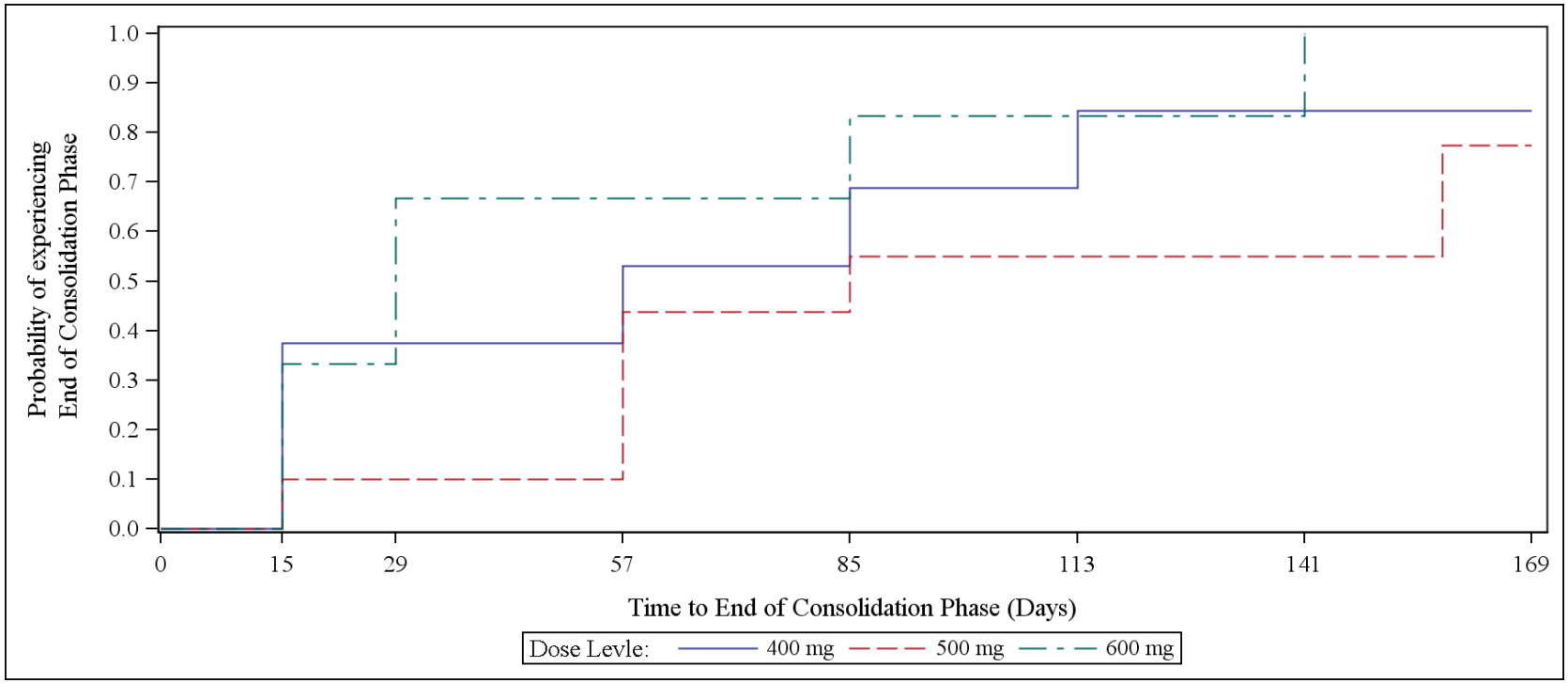
Prog NOTE: Please also add "all dose levels" in this plot.

Repeat for the following populations

Figure 14.2.3.2.2
Kaplan Meier Plots for Time to First Complete Response (CR)
mITT Population

Figure 14.2.3.2.3
Kaplan Meier Plots for Time to First Complete Response (CR)
PP Population

Figure 14.2.3.3.1
Kaplan Meier Plots for Time to First End of Consolidation Phase
ITT Population



Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period. End of consolidation phase: The time at which no new lesions have developed for minimum of 2 weeks, approximately 80% of lesions have healed, and when most clinicians start to taper steroids.
Time to first end of consolidation phase = Date of end of consolidation phase confirmed – Date of first dose + 1.
Patients who have not achieved End of Consolidation Phase by the date of study completion or discontinuation will be censored at the date of completion or discontinuation.
Source Data: Table 14.2.3.3

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

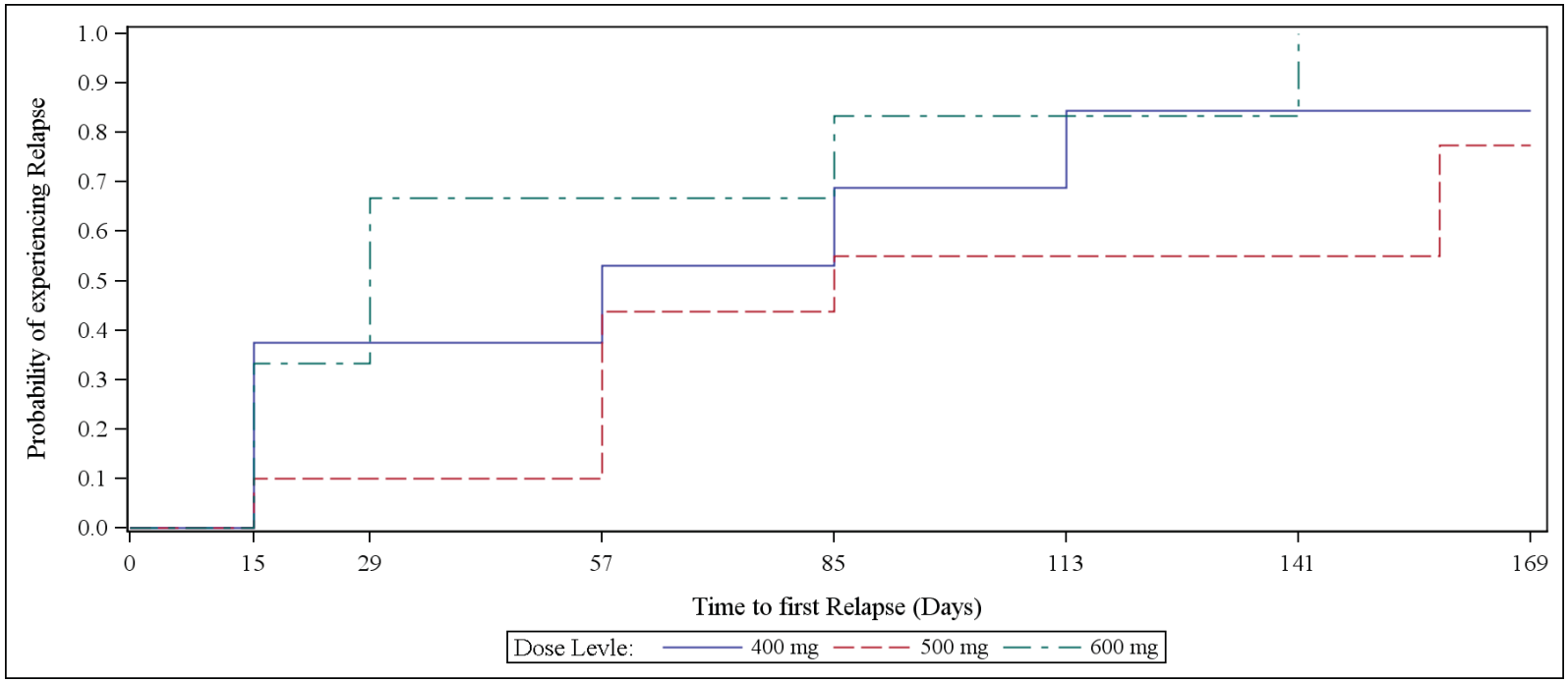
Prog NOTE: Please also add "all dose levels" in this plot.

Repeat for the following population

Figure 14.2.3.3.2
Kaplan Meier Plots for Time to First End of Consolidation Phase
mITT Population

Figure 14.2.3.3.3
Kaplan Meier Plots for Time to First End of Consolidation Phase
PP Population

Figure 14.2.3.4.1
Kaplan Meier Plots for Time to First Relapse after PRN1008 Treatment Completion or Discontinuation
ITT Population



Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
Relapse/flare: Appearance of >=3 new lesions/month that do not heal spontaneously within 1 week, or by extension of established lesions, in a patient who has achieved disease control.
Only patients with confirmed CDA before treatment completion or discontinuation will be included into this analysis.
Time to first relapse = Date of first relapse confirmed – Date of last dose + 1.
Patients who have not achieved Relapse by the date of study completion or discontinuation will be censored at the date of completion or discontinuation.
Source Data: Table 14.2.3.4

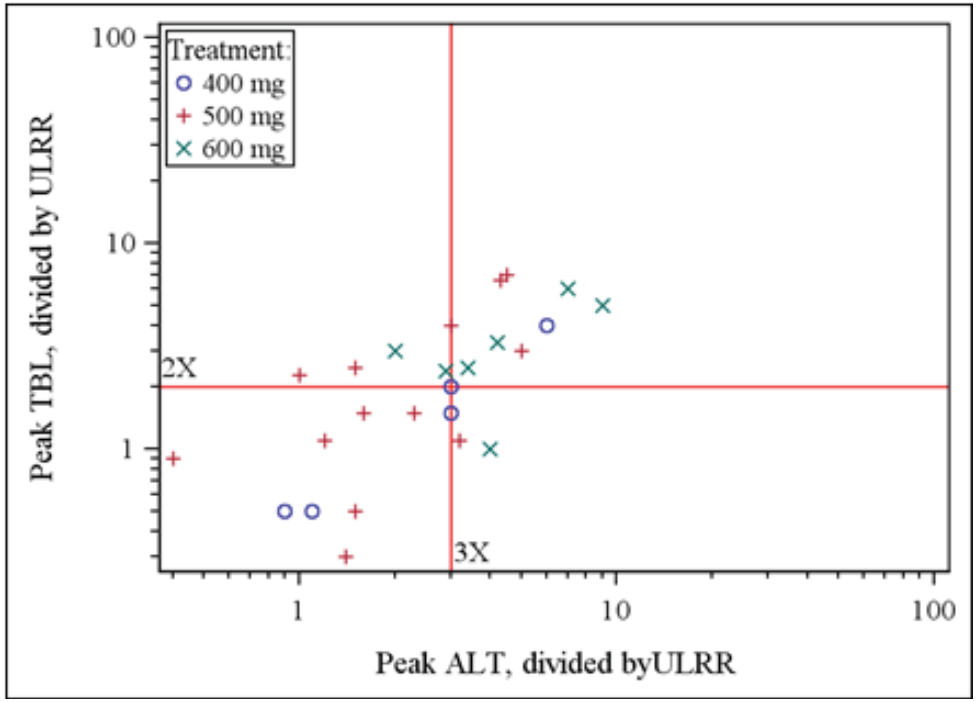
<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog NOTE: Please also add "all dose levels" in this plot.
Repeat for the following populations.

Figure 14.2.3.4.2
Kaplan Meier Plots for Time to First Relapse after PRN1008 Treatment Completion or Discontinuation
mITT Population

Figure 14.2.3.4.3
Kaplan Meier Plots for Time to First Relapse after PRN1008 Treatment Completion or Discontinuation
PP Population

Figure 14.3.3.2
Scatter Plots of Peak TBL vs Peak ALT for Identification of Drug Induced Liver Injury (DILI)
Safety Analysis Population



Note: Patients are classified into different dose levels according to the maximum dose they received during the 12 week treatment period.
TBL = total bilirubin, ALT = alanine aminotransferase or SGPT
The maximum measurement value observed during the 12 week treatment will be defined as the Peak value.
Source Data: Listing 16.2.6.2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Listing 16.2.1.1
Patient Disposition
Screening Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Site	Informed Consent Date Time	Analysis Set	First Dose Date/ Last Dose Date(Day)	Study Completed	Completion or Early Withdrawl Date(Day)	If Early Withdrawl, Reason
XXXXXX	65/F/W	XXXXXX	DDMMMYYYY XX:XX	Safety/ ITT/mITT/ PP	DDMMMYYYY/ DDMMMYYYY (XX)	Yes	DDMMMYYYY(XX)	
XXXXXX	50/M/W	XXXXXX	DDMMMYYYY XX:XX	Safety/ ITT/mITT/ PP	DDMMMYYYY/ DDMMMYYYY(XX)	No	DDMMMYYYY(XX)	Lost of Follow-up
XXXXXX	50/M/W	XXXXXX	DDMMMYYYY XX:XX	Safety/ ITT/mITT/ PP	DDMMMYYYY/ DDMMMYYYY(XX)	No	DDMMMYYYY(XX)	Physician Decision: XXXXXXXXXXXX XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID.

Listing 16.2.1.2
Protocol Deviations
Screening Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Date of Deviation	Significant Deviations	Category	Protocol Deviation
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY	Yes	XXXXXXXXXXXXXX	XXXXXXXXXXXXXX
XXXXXX	65/F/BL	Safety/ Efficacy	DDMMMYYYY	No	XXXXXXXXXXXXXX	XXXXXXXXXXXXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Date of Deviation.

Listing 16.2.2.1
Demographics and Baseline Characteristics
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Ethnicity	Skin Biopsy Confirmed PV/ Date of Biopsy	Pemphigus Type	Height (cm)	Weight (kg)	BMI (kg/m2)
XXXXXX	50/M/W	Safety/ Efficacy	NOT HISPANIC OR LATINO	Yes/ DDMMMYYYY	Pemphigus Follaceus	XXX.X	XXX.X	XX.XX
XXXXXX	65/F/BL	Safety/ Efficacy	HISPANIC OR LATINO	No/ DDMMMYYYY	Pemphigus Vulgaris	XXX.X	XXX.X	XX.XX
XXXXXX	60/F/W	Safety/ Efficacy	HISPANIC OR LATINO	Not Done	Linear IgA Bullous Dermatoses	XXX.X	XXX.X	XX.XX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected; PV= Pemhigus Vulgaris
BMI=(Baseline body weight in kilograms)/(Baseline height in meters)^2

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID.

Listing 16.2.2.2
Medical History
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Body System/ Medical Condition(s) Reported	Start Date	End Date	Ongoing
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXXXXX	DDMMMYYYY	DDMMMYYYY	No
XXXXXX	65/F/BL	Safety/ Efficacy	XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXXXXX	DDMMMYYYY	DDMMMYYYY	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Body System (in order presented on CRF), and alphabetically by Medical Condition Reported, then start date. Body System will be taken directly from the CRF page. Only those body systems with non-missing results will be displayed.

Listing 16.2.2.3
Inclusion/Exclusion Criteria
Screening Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Protocol Version	Met all Eligibility Criteria?	Inclusion Criteria Not Met	Exclusion Criteria Met
XXXXXX	50/M/W	Safety/ Efficacy	1.1	No	Ix, Ix, Ix	Ex, Ex, Ex
XXXXXX	65/F/BL	Safety/ Efficacy	1.0	Yes		

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID.

Listing 16.2.3.1
Prior and Concomitant Corticosteroid Medications
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	ATC Level 2/ Preferred Term/ Medication Reported/ Indication	Flag	Start Date (Day)/ Stop Date (Day)	On-going	Dose	Unit	Route	Freq
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX	P	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	Yes	XXX	XXXX	XXXXX	XXXX
XXXXXX	32/F/W	Safety/ Efficacy	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX	P/C	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	No	XXX	XXXX	XXXXX	XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Flag: P=Prior medication, C=Concomitant medication; Freq=Frequency
A prior medication is defined as any medication that has a stop date before the date of first dose of study drug. A Concomitant medication is defined as any medication that has a stop date on or after the date of first dose of study drug.
Medications with missing stop dates will be classified as both prior and concomitant.
Prior and Concomitant medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Start Date, and alphabetically by ATC Level 2, Preferred Term, and Medication Reported.

Listing 16.2.3.2
Prior and Concomitant Non-Corticosteroid Medications
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	ATC Level 2/ Preferred Term/ Medication Reported/ Indication	Flag	Start Date (Day)/ Stop Date (Day)	On-going	Dose	Unit	Route	Freq
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX	P	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	Yes	XXX	XXXX	XXXXX	XXXX
XXXXXX	32/F/W	Safety/ Efficacy	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX	P/C	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	No	XXX	XXXX	XXXXX	XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Flag: P=Prior medication, C=Concomitant medication; Freq=Frequency
A prior medication is defined as any medication that has a stop date before the date of first dose of study drug. A Concomitant medication is defined as any medication that has a stop date on or after the date of first dose of study drug.
Medications with missing stop dates will be classified as both prior and concomitant.
Prior and Concomitant medications were coded with the WHO Drug dictionary (WHO Drug DDE June 2015).

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Start Date, and alphabetically by ATC Level 2, Preferred Term, and Medication Reported.

Listing 16.2.3.3
Study Drug Accountability
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	First Dose Date	Visit Name	Formulation (mg)	Dispensed		Returned	
					Date(Day)	No.	Date(Day)	No.
XXXXXX	50/M/W	DDMMMYYYY	WEEK1 D1	100	DDMMMYYYY (XXX)	XXX	DDMMMYYYY (XXX)	XXX
			WEEK1 D1	300	DDMMMYYYY (XXX)	XXX	DDMMMYYYY (XXX)	XXX
			XXXXXXXXXXX	XXX	DDMMMYYYY (XXX)	XXX	DDMMMYYYY (XXX)	XXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg), Patient ID, Visit.

Listing 16.2.3.4
Study Drug Administration
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	First Dose Date	Visit Name	Dose (mg)	Start Date (Day)	Stop Date (Day)	Duration (Days) [1]	Dose Adjusted/ Reason
XXXXXX	50/M/W	DDMMMYYYY	WEEK1 D1	400	DDMMMYYYY(XX)	DDMMMYYYY(XX)	XXX	No
			WEEK5 D29	XXX	DDMMMYYYY(XX)	DDMMMYYYY(XX)	XXX	Yes/ XXXXXXXXX XXXXX
			WEEK9 D57	XXX	DDMMMYYYY(XX)	DDMMMYYYY(XX)	XXX	No
XXXXXX	32/F/W	DDMMMYYYY	WEEK1 D1	400	DDMMMYYYY(XX)	DDMMMYYYY(XX)	XXX	No
			XXXXXXXXX	XXX	DDMMMYYYY(XX)	DDMMMYYYY(XX)	XXX	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] Duration (Days) = Stop Date – Start Date +1

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg), Patient ID, Visit, Start Date.

Listing 16.2.3.5
Study Drug Exposure
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	First Dose Date	Last Dose Date	Overall Duration (Days) [1]	Cumulative Dose (mg) [2]	Average Daily Dose (mg/day) [3]	Minimum Dose Level (mg)	No. of Dose Adjustments
XXXXXX	50/M/W	DDMMMYYYY	DDMMMYYYY	XXX	XXX	XXX	XXX	XXX
XXXXXX	32/F/W	DDMMMYYYY	DDMMMYYYY	XXX	XXX	XXX	XXX	XXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] Overall Duration (Days) = Last Dose Date – First Dose Date +1
[2] Cumulative Dose is defined as the sum of the doses taken by patients during the treatment
[3] Average Daily Dose = Cumulative Dose / Overall Duration

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg), Patient ID.

Listing 16.2.3.6
Study Drug Compliance
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	First Dose Date	Last Dose Date	Number of Tablets Taken [1]	Number of Tablets Planned [2]	Overall Compliance (%) [3]
XXXXXX	50/M/W	DDMMMYYYY	DDMMMYYYY	XXX	XXX	XX.X
XXXXXX	32/F/W	DDMMMYYYY	DDMMMYYYY	XXX	XXX	XX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] Number of tablets taken is calculated by subtracting the number of tablets returned from the number of tablets dispensed
[2] Number of tablets planned is calculated by multiplying the number of planned exposure days by number of tablets planned per day.
[3] Overall Compliance (%) = Total number of tablets taken / Total number of tablets planned * 100

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg), Patient ID.

Listing 16.2.4.1.1
Control of Disease Activity (CDA) Status and Predni(ol)one Use Condition Within 4 Weeks
IIT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Achieved CDA at the Week 5 Visit	Predni(ol)one >0.5 mg/kg on or before the Week 5 Visit	Responder [1]
XXXXXX	50/M/W	Safety/ Efficacy	Yes	No	Yes
XXXXXX	32/F/W	Safety/ Efficacy	Yes	Yes	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
[1] Patients who achieved CDA at the Week 5 visit and had no occurrence of daily prednis(ol)one >0.5 mg/kg on or before the Week 5 visit during the study treatment will be considered as responders.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.2
Control of Disease Activity (CDA) Status and Corticosteroids Use Condition Within 4 Weeks
IIT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Achieved CDA at the Week 5 Visit	Any Corticosteroid Used on or before the Week 5 Visit	Responder [1]
XXXXXX	50/M/W	Safety/ Efficacy	Yes	No	Yes
XXXXXX	32/F/W	Safety/ Efficacy	Yes	Yes	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
[1] Patients who achieved CDA at the Week 5 visit and had no occurrence of any corticosteroid on or before the Week 5 visit during the study treatment will be considered as responders.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.3
Complete Response (CR) Status and Corticosteroids Use Condition Within 12 Weeks
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Achieved CR at the Week 13 Visit	Any Corticosteroid Used on or before the Week 13 Visit	Responder [1]
XXXXXX	50/M/W	Safety/ Efficacy	Yes	No	Yes
XXXXXX	32/F/W	Safety/ Efficacy	Yes	Yes	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
Complete response (CR) is defined as the absence of new or established lesions while the patient is Receiving minimal or no non-PRN 1008 immunotherapy.
[1] Patients who achieved CR at the Week 13 visit and had no occurrence of any corticosteroid on or before the Week 13 visit during the study treatment will be considered as responders.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.4
Complete Response (CR) Status and Predni(ol)one Use Condition Within 12 Weeks
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Achieved CR on or before the Week 13 Visit	Predni(ol)one >0.5 mg/kg on or before the Fist CR	Responder [1]
XXXXXX	50/M/W	Safety/ Efficacy	Yes	No	Yes
XXXXXX	32/F/W	Safety/ Efficacy	Yes	Yes	No

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Complete response (CR) is defined as the absence of new or established lesions while the patient is Receiving minimal or no non-PRN 1008 immunotherapy.
[1] Patients who achieved CR at any visit on or before the Week 13 visit during the study treatment and had no occurrence of daily prednis(ol)one >0.5 mg/kg between the first dose date of PRN1008 treatment and the date of the first CR will be considered as responders.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.5
Disease Outcome Status
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit Name	Prednis(ol)one >0.5 mg/kg [1]	Disease Status	Achieved?	Achieved Date	CS Dose at Time of First CR (mg) [2]
XXXXXX	50/M/W	Safety/ Efficacy	Screen	No	Control of Disease Activity	No		
					Complete Response	No		
					End of Consolidated Phase	No		
					Relapse	Yes	DDMMMYYYY	XX.X
			WEEK1 D1	No	…	…		
			…		…	…		

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] Daily prednis(ol)one >0.5 mg/kg on or before the specific visit during the study treatment.
[2] Dose of CS combined from all recorded CS on the day before date of the first CR for this subject.
<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.
For CS dose at time of achievement, please note that if a record of CS is ongoing and does not have an end date, the end date is assumed to be the date of last follow up.

Listing 16.2.4.1.6
Time to First Control of Disease Activity (CDA)
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	First Dose Date/ Last Dose Date	Date of First CDA or Censored Date	Time to First CDA (Days)	Censored
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY/ DDMMMYYYY	DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	No
					…	
					…	

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Control of disease activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal.
Time to first CDA = Date of first CDA confirmed – Date of first dose + 1. Patients who did not achieve CDA by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.7
Time to First Complete Response (CR)
IIT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	First Dose Date/ Last Dose Date	Date of First CR or Censored Date	Time to First CR (Days)	Censored
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY/ DDMMMYYYY	DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	No
					…	
					…	

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Complete response (CR) is defined as the absence of new or established lesions while the patient is receiving minimal or no non-PRN 1008 immunotherapy.
Time to first CR = Date of first CR confirmed – Date of first dose + 1. Patients who did not achieve CR by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.8
Time to First End of Consolidation Phase
IIT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	First Dose Date/ Last Dose Date	Date of First End of Consolidation Phase or Censored Date	Time to First End of Consolidation Phase (Days)	Censored
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY/ DDMMMYYYY	DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	Yes
				DDMMMYYYY	xx	No
					…	
					…	

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
End of consolidation phase: The time at which no new lesions have developed for minimum of 2 weeks, approximately 80% of lesions have healed, and when most clinicians start to taper steroids.
Time to first end of consolidation phase = Date of first end of consolidation phase confirmed – Date of first dose + 1. Patients who did not achieve end of consolidation phase by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.9
Time to First Relapse after Week 13 Visit
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	First Dose Date/ Last Dose Date	Date of First Relapse After Week 13 or Censored Date	CDA at Week 13 Yes/No	CR at Week 13 Yes/No	Time to First Relapse From Week 13 (Days)	Censored
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY/ DDMMMYYYY	DDMMMYYYY	Yes/No	Yes/No	xx	Yes
				DDMMMYYYY	Yes/No	Yes/No	xx	Yes
				DDMMMYYYY	Yes/No	Yes/No	xx	Yes
				DDMMMYYYY	Yes/No	Yes/No	xx	No
							…	
							…	

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Relapse/flare: Appearance of >=3 new lesions/month that do not heal spontaneously within 1 week, or by extension of established lesions, in a patient who has achieved disease control. Only patients with confirmed CDA before treatment completion or discontinuation will be included into this listing.
Time to first relapse = Date of first relapse confirmed after week 13 – Date of week 13 visit + 1.
Patients who did not achieve relapse by the date of study completion or discontinuation were censored at the date of completion or discontinuation respectively.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.1.10
Duration of Complete Response (CR) Status Among CR Subjects
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	CR Start Date	Last Known CR Date	Last Follow up Date	Duration [1]
XXXXXX	50/M/W	Safety/ Efficacy	DDMMMYYYY	DDMMMYYYY	DDMMMYYYY	Xx
XXXXXX	32/F/W	Safety/ Efficacy	DDMMMYYYY	DDMMMYYYY	DDMMMYYYY	xx

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Complete response (CR) is defined as the absence of new or established lesions while the patient is Receiving minimal or no non-PRN 1008 immunotherapy.
[1] Duration is defined as the earlier of 1) Last known CR date and 2) Last follow up date –CR start date + 1. Only CR after start of treatment is counted in this listing.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Only include subjects with CR, Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit.

Listing 16.2.4.2.1
Pemphigus Disease Area Index (PDAI) Score
IIT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	Body Part	Anatomical Location	Parameter	Result
XXXXXX	50/M/W	SCREEN	DDMMMYYY	Skin	EARS	xxxxxxxxxxx	x
					NOSE	xxxxxxxxxxx	x
					REST OF THE FACE	xxxxxxxxxxx	x
					NECK	xxxxxxxxxxx	x
					CHEST	xxxxxxxxxxx	x
					ABDOMEN	xxxxxxxxxxx	x
					BACK, BUTTOCKS	xxxxxxxxxxx	x
					ARMS	xxxxxxxxxxx	x
					HANDS	xxxxxxxxxxx	x
					LEGS	xxxxxxxxxxx	x
					FEET	xxxxxxxxxxx	x
					GENITALS	xxxxxxxxxxx	x
				Scalp	SCALP	xxxxxxxxxxx	x
				Mucousa	EYES	xxxxxxxxxxx	x
					NOSE	xxxxxxxxxxx	x
					BUCCAL MUCOSA	xxxxxxxxxxx	x
					HARD PALATE	xxxxxxxxxxx	x
					SOFT PALATE	xxxxxxxxxxx	x
					UPPER GINGIVA	xxxxxxxxxxx	x
					LOWER GINGIVA	xxxxxxxxxxx	x
					TOGUE	xxxxxxxxxxx	x
					FLOOR OF MOUTH	xxxxxxxxxxx	x
					LABIAL MUCOSA	xxxxxxxxxxx	x
					POSTERIOR PHARYNX	xxxxxxxxxxx	x
					ANOGENITAL	xxxxxxxxxxx	x
		WEEK1 D1	DDMMMYYYY	…	…	…	…
		…	…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

[1] Activity Score for Skin: 0=absent; 1=1-3 lesions up to one >2 cm in any diameter, none >6 cm; 2=2-3 lesions, at least two >2 cm diameter, none >6 cm; 3=>3 lesions, none >6 cm diameter; 5=>3 lesions, and/or at least one >6 cm; 10=>3 lesions, and/or at least one lesion > 16 cm diameter or entire area.
Activity Score for Scalp: 0=absent; 1=in one quadrant; 2=two quadrants; 3=three quadrants; 4 =affects whole skull; 10=at least one lesion > 6cm.
Activity Score for Mucosa: 0= absent; 1=1 lesion; 2=2-3 lesions; 5=>3 lesions or 2 lesions >2 cm; 10=entire area.
[2] Damage Score for Skin and Scalp: 0=absent; 1=present.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date, Body Part, Anatomical Location.

Listing 16.2.4.2.2
Pemphigus Disease Area Index (PDAI) Total Scores
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	PDAI Scores	Score
XXXXXX	50/M/W	SCREEN	DDMMMYYY	Total Skin Activity Score	XXX
				Total Scalp Activity Score	XX
				Total Mucosa Activity Score	XXX
				Total Skin Damage Score	XX
				Total Scalp Damage Score	X
				Total Activity Score	XXX
				Total Damage Score	XX
		WEEK1 D1	DDMMMYYY	…	…
		…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Total Activity Score = Total Skin Activity Score + Total Scalp Activity Score + Total Mucosa Activity Score
Total Damage Score = Total Skin Damage Score + Total Scalp Damage Score
Total PDAI Score = Total Activity Score + Total Damage Score

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date, PDAI Score.

Listing 16.2.4.3.1
Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)—Skin Involvement
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	Skin Involvement (Max BSA)	Patient's BSA (%)	Weighting Factor [1]	Skin Involvement Total Score [2]
XXXXXX	50/M/W	SCREEN	DDMMMYYY	HEAD & NECK (9%)	XX	X.X	XX.X
				L ARM INCLUDING HAND (9%)	XX	X.X	XX.X
				R ARM INCLUDING HAND (9%)	XX	X.X	XX.X
				TRUNK (FRONT & BACK)(36%)	XX	X.X	XX.X
				L LEG (18%)	XX	X.X	XX.X
				R LEG (18%)	XX	X.X	XX.X
				GENITALS (1%)	XX	X.X	XX.X
		WEEK1 D1	DDMMMYYYY	…	…	…	…
		…	…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected; BSA=Body Surface Area
[1] 1.5 = Erosive, exudative lesions; 1 = Erosive, dry lesions; 0.5 = reepithelialized lesions
[2] • Skin involvement total score = sum of (%BSA * Weighting factor)

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.3.2
Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)—Oral Involvement
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	Oral Involvement	Extent [1]
XXXXXX	50/M/W	SCREEN	DDMMMYYY	UPPER GINGIVAL MUCOSA	X
				LOWER GINGIVAL MUCOSA	X
				UPPER LIP MUCOSA	X
				LOWER LIP MUCOSA	X
				LEFT BUCCAL MUCOSA	X
				RIGHT BUCCAL MUCOSA	X
				TONGUE	X
				FLOOR OF THE MOUTH	X
				HARD PALATE	X
				SOFT PALATE	X
				PHARYNX	X
		WEEK1 D1	DDMMMYYYY	…	…
		…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] 1=presence of lesions; 0=absence of lesion

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.3.3
Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)—Severity
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	Food	Level	Factor of Discomfort [1]	Severity Score [2]
XXXXXX	50/M/W	SCREEN	DDMMMYYY	WATER	1	X.X	X.X
				SOUP	2	X.X	X.X
				YOGURT	3	X.X	X.X
				CUSTARD	4	X.X	X.X
				MASHED POTATOES / SCRAMBLED EGG	5	X.X	X.X
				BAKED FISH	6	X.X	X.X
				WHITE BREAD	7	X.X	X.X
				APPLE / RAW CARROT	8	X.X	X.X
				FRIED STEAK / WHOLE-GRAIN BREAD	9	X.X	X.X
		WEEK1 D1	DDMMMYYYY	…	…	…	…
		…	…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] 1=Pain/bleeding occurred always; 0.5=Pain/bleeding occurred sometimes; 0=Never experienced problems
[2] Severity Score = Level * Factor of Discomfort

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.3.4
Autoimmune Bullous Skin Disorder Intensity Score (ABSIS)
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	Sub-Category	ABSIS Score
XXXXXX	50/M/W	SCREEN	DDMMMYYY	Skin Involvement Total Score	XXX
				Oral Involvement Total Score	XX
				Severity Total Score	XX
				Total Activity Score	
				Total ABSIS Score	
		WEEK1 D1	DDMMMYYY	…	…
		…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Skin involvement total score = sum of %BSA * Weighting factor
Oral involvement total score = sum score from each oral part
Severity total score = sum of Level * Factor of discomfort
ABSIS total activity score = Skin involvement total score + Oral involvement total score
Total ABSIS score = Skin involvement total score + Oral involvement total score + Severity total score

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.4.1
Autoimmune Bullous Diseases Quality of Life (ABQOL) Questionnaire
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	ABQOL Question	ABQOL Answer	ABQOL Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	In regards to your blistering disease, does your skin burn, sting or hurt in any way?	All the time	1
				In regards to your blistering disease, does you skin itch?	Sometimes	2
				Have you had to change your clothing because of your blistering disease?	I have had to change most of the things I wear	2
				Do you notice your skin heals slowly?	I notice this occasionally	3
				Do you have difficulty bathing or showering because of your blistering disease?	Occasionally	3
				In regards to your blistering disease, does your mouth have erosions which are painful?	Sometimes	2
				In regards to your blistering disease, do your gums bleed easily?	All the time	1
				Does your blistering disease results in you having to avoid food or drinks that you enjoy?	I can eat some of the foods I enjoy	2
				As a result of your blistering disease, are you embarrassed about your appearance?	Sometimes	2
				Do you feel depressed or angry because of your blistering disease?	Occasionally	3
				Do you feel anxious or cannot relax as a result of your blistering	Not at all	4

			disease?		
			Do you worry that friends and family find your blistering skin condition tiresome?	Not at all	4
			Is your blistering disease causing sexual difficulties?	Sometimes	2
			Do you worry that friends and family find your blistering skin condition tiresome?	Relationships are very difficult	2
			Does your blistering disease affect your social life?	I can go to most social events	3
			Does your blistering disease affect your work or study?	Yes, I find it difficult to work or study	2
			Do employers discriminate against you because of your blistering disease?	My employers are completely understanding OR not Applicable (N/A)	4
	WEEK3 D15	DDMMMYYY	…	…	…
	…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.4.2
Autoimmune Bullous Diseases Quality of Life (ABQOL) Score
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	ABQOL Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	XX
		WEEK3 D15	DDMMMYYY	XX
		XXXX	DDMMMYYY	XX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.5.1
Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) Questionnaire
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	TABQOL Question	TABQOL Answer	TABQOL Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	As a result of your blistering disease treatment, do you notice you bruise or bleed easily?	I notice this all the time	1
				As a result of your blistering disease treatment, can you still tolerate hot or cold temperatures?	I am sometimes sensitive to changes in temperature	2
				Do you have to take your medications for your blistering disease at a specific time?	Yes: it is a little annoying	2
				Do you take many medications for your blistering disease?	Yes but I do not mind	3
				Does the treatment for your blistering disease result in you feeling bloated?	Some of the time	3
				Does the treatment for your blistering disease make it difficult to walk?	A lot of the time	2
				As a result of your blistering disease treatment, can you think as quickly or as clearly as you used to?	No it is very frustrating	1
				Do you find your blistering disease treatment very time consuming?	Yes it is quite annoying	2
				Do you mind the needles or blood tests involved in the treatment of your blistering disease?	Yes I dislike needles	2
				Do you worry your blistering disease will get worse when you drop the dosage of your medications?	Yes I sometimes worry about it	3

			Do you worry about your blistering disease being dangerous?	I never worry about it	4
			Do you feel tired and lethargic as a result of the treatment for your blistering disease?	Not at all	4
			Do you worry about getting sick (with the flu, etc.) due to your depressed immunity because of the treatment for your blistering disease?	Yes I worry about it a lot	2
			As a result of your blistering disease treatment, have you stopped doing many activities to avoid getting sick?	Yes I cannot do many of the activities I enjoy	2
			Do you have nightmares or bad memories as a result of your blistering disease treatments?	Some of the time	3
			Does your blistering disease treatment affect your holidays?	Does your Blistering disease treatment affect your holidays?	2
			Is your blistering disease treatment giving you financial difficulties?	No	4
	WEEK3 D15	DDMMMYYY	…	…	…
	…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.5.2
Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) Score
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	TABQOL Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	XX
		WEEK3 D15	DDMMMYYY	XX
		XXXX	DDMMMYYY	XX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.6.1
Simplified Nutritional Appetite Questionnaire (SNAQ) Questionnaire
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	SNAQ Question	SNAQ Answer	SNAQ Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	My appetite is	average	3
				When I eat	I feel full after eating about a third of a meal	2
				Food tastes	bad	2
				Normally I eat	more than three meals a day	5
		WEEK3 D15	DDMMMYYY	…	…	…
		…	…	…	…	…

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.4.6.2
Simplified Nutritional Appetite Questionnaire (SNAQ) Score
ITT Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Visit Name	Assessment Date	SNAQ Score
XXXXXX	50/M/W	WEEK1 D1	DDMMMYYY	XX
		WEEK3 D15	DDMMMYYY	XX
		XXXX	DDMMMYYY	XX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level(400 mg, 500 mg, 600 mg, missing), Patient ID, Visit, Assessment Date.

Listing 16.2.5.1
Adverse Events
Safety Analysis Population

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
*=Treatment-emergent AE
Dur=Duration (Days = Stop date - Start date + 1)
Pat of AE=Pattern of AE: Int=intermittent, Cont=continuous
Outcome: 1=Recovered/Resolved, 2=Recovering/Resolving, 3=Recovered/Resolved with sequelae, 4=Not recovered/not resolved, 5=Fatal, 6=Unknown
C/A=Concomitant or Additional Treatment Given?
Sev=Severity: 1=Grade 1, 2=Grade 2, 3=Grade 3, 4=Grade 4, 5=Grade 5
Act=Action taken with study treatment: 1=Dose increased, 2=Dose not changed, 3=Dose reduced, 4=Drug interrupted, 5=Drug Withdrawn, 6=Not Applicable, 7=Unknown
Rel=Relationship to study treatment: 1=Not related, 2=Related
Study Disc=Caused study discontinuation?
Ser=Serious?
Adverse Events were coded using MedDRA, Version 18.0.
<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Listing 16.2.5.1
Adverse Events
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date Time (Day)/ Stop Date Time (Day)	Dur (Days)	Pat of AE	Out- come	C/ A	Sev	Act/ Rel	Study Disc/ Ser
XXXXXX	65/F/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXX	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	126	Cont	4	Y	2	5/1	Y/Y
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXX *	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	45	Int	2	Y	1	2/2	Y/Y

Notes are listed on page 1.

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, AE Start Date/Time, and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.

Listing 16.2.5.2
Serious Adverse Events
Safety Analysis Population

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
*=Treatment-emergent AE
Dur=Duration (Days = Stop date - Start date + 1)
Pat of AE=Pattern of AE: Int=intermittent, Cont=continuous
Outcome: 1=Recovered/Resolved, 2=Recovering/Resolving, 3=Recovered/Resolved with sequelae, 4=Not recovered/not resolved, 5=Fatal, 6=Unknown
C/A=Concomitant or Additional Treatment Given?
Sev=Severity: 1=Grade 1, 2=Grade 2, 3=Grade 3, 4=Grade 4, 5=Grade 5
Act=Action taken with study treatment: 1=Dose increased, 2=Dose not changed, 3=Dose reduced, 4=Drug interrupted, 5=Drug Withdrawn, 6=Not Applicable, 7=Unknown
Rel=Relationship to study treatment: 1=Not related, 2=Related
Study Disc=Caused study discontinuation?
Adverse Events were coded using MedDRA, Version 18.0.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Listing 16.2.5.2
Serious Adverse Events
Safety **Analysis** Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date Time (Day)/ Stop Date Time (Day)	Dur (Days)	Pat of AE	Out-come	C/ A	Sev	Act/ Rel	Study Disc/ Ser
XXXXXX	65/F/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	126	Cont	4	Y	2	5/1	Y/Y
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX *	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	45	Int	2	Y	1	2/2	Y/Y

Notes are listed on page 1.

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, AE Start Date/Time, and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.

Listing 16.2.5.3
Adverse Events Leading to Treatment Discontinuation
Safety Analysis Population

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
*=Treatment-emergent AE
Dur=Duration (Days = Stop date - Start date + 1)
Pat of AE=Pattern of AE: Int=intermittent, Cont=continuous
Outcome: 1=Recovered/Resolved, 2=Recovering/Resolving, 3=Recovered/Resolved with sequelae, 4=Not recovered/not resolved, 5=Fatal, 6=Unknown
C/A=Concomitant or Additional Treatment Given?
Sev=Severity: 1=Grade 1, 2=Grade 2, 3=Grade 3, 4=Grade 4, 5=Grade 5
Rel=Relationship to study treatment: 1=Not related, 2=Related
Study Disc=Caused study discontinuation?
Ser=Serious?
Adverse Events were coded using MedDRA, Version 18.0.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Listing 16.2.5.3
Adverse Events Leading to Treatment Discontinuation
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date Time (Day)/ Stop Date Time (Day)	Dur (Days)	Pat of AE	Out-come	C/ A	Sev	Rel	Study Disc/ Ser
XXXXXX	65/F/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	126	Cont	4	Y	2	1	Y/Y
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX *	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	45	Int	2	Y	1	2	Y/Y

Notes are listed on page 1.

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg), Patient ID, AE Start Date/Time, and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.

Listing 16.2.5.4
Adverse Events Leading to Death
Safety Analysis Population

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
*=Treatment-emergent AE
Dur=Duration (Days = Stop date - Start date + 1)
Pat of AE=Pattern of AE: Int=intermittent, Cont=continuous
Outcome: 1=Recovered/Resolved, 2=Recovering/Resolving, 3=Recovered/Resolved with sequelae, 4=Not recovered/not resolved, 5=Fatal, 6=Unknown
C/A=Concomitant or Additional Treatment Given?
Sev=Severity: 1=Grade 1, 2=Grade 2, 3=Grade 3, 4=Grade 4, 5=Grade 5
Act=Action taken with study treatment: 1=Dose increased, 2=Dose not changed, 3=Dose reduced, 4=Drug interrupted, 5=Drug Withdrawn, 6=Not Applicable, 7=Unknown
Rel=Relationship to study treatment: 1=Not related, 2=Related
Study Disc=Caused study discontinuation?
Ser=Serious?
Adverse Events were coded using MedDRA, Version 18.0.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Listing 16.2.5.4
Adverse Events Leading to Death
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date Time (Day)/ Stop Date Time (Day)	Dur (Days)	Pat of AE	Out-come	C/ A	Sev	Act/ Rel	Study Disc/ Ser
XXXXXX	65/F/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	126	Cont	5	Y	2	5/1	Y/Y
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX *	DDMMMYYYY XX:XX (XX)/ DDMMMYYYY XX:XX (XX)	45	Int	5	Y	1	2/2	Y/Y

Notes are listed on page 1.

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, AE Start Date/Time, and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.

Listing 16.2.6.1
Clinical Laboratory Results - Chemistry Tests
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Flag[1]	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	L	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X	H	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	H*	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
[1] L=Low, H=High, *= Newly Notable if the patient did not meet the abnormality criteria at baseline

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.2
Clinical Laboratory Results – Drug Induced Liver Injury
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Flag[1]	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	L	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X	H	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	H*	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Clinical laboratory tests related to drug induced liver injury (DILI) include ALT (alanine aminotransferase or SGPT), AST (aspartate transaminase or SGOT), TBL (total bilirubin), and ALP (alkaline phosphatase).
[1] L=Low, H=High, *= Newly Notable abnormality if the patient did not meet the abnormality criteria at baseline

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.3
Clinical Laboratory Results – Hematology
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Flag[1]	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	L	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X	H	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	H*	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] L=Low, H=High, *= Newly Notable abnormality if the patient did not meet the abnormality criteria at baseline

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.4
Clinical Laboratory Results – Coagulation
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Flag[1]	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	L	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXX.X	H	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X	H*	XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X
					XXXXXXXXXXXXXXX	XXX.X		XXX.X-XXX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] L=Low, H=High, *= Newly Notable abnormality if the patient did not meet the abnormality criteria at baseline

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.5
Clinical Laboratory Results – Urinalysis
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX-XXXX
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX-XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.6
Clinical Laboratory Results – Serology
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time(Day)	Laboratory Test (Unit)	Result	Normal Range
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXXXX	DDMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
				DMMMYYYY XX:XX (XX)	XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX
					XXXXXXXXXXXXXXX	XXXXXX	XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Lab Category, Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.6.7
Vital Sign Results
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit/ Date(Day)	Vital Sign Parameter	Result [1]
XXX	50/M/W	Safety/ Efficacy	XXXX/ DDMMMYYYY(XXX)	Systolic Blood Pressure (mmHg)	XXX (L)
				Diastolic Blood Pressure (mmHg)	XX (H*)
				Heart Rate (BEATS/MIN)	XXX
				Respiratory Rate (BREATHS/MIN)	XX
				Temperature (C)	XX.X
				Height (cm)	XXX.X
				Weight (kg)	XXX.X

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
[1] L= Low (systolic blood pressure value <90 mmHg, diastolic blood pressure value <60 mmHg, heart rate <40 BEATS/MIN); H=High (systolic blood pressure value >140 mmHg, diastolic blood pressure value >80 mmHg, heart rate >100 BEATS/MIN); *=Newly notable abnormality if the patient did not meet the abnormality criteria at baseline

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Date.

Listing 16.2.6.8
Physical Examination
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit/ Exam Date Time(Day)	Assessment	Status	Abnormality Comment(s)
XXXXXX	50/M/W	Safety/ Efficacy	XXXX/ DDMMMYYYY XX:XX(XX)	SKIN	Abnormal	XXXXXXXXXXXXXXXXXXXX
				HEAD, EYES, EARS, NOSE, THROAT	Normal	
				…		

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, Exam Date, Assessment. All assessments will be displayed for each patient.

Listing 16.2.6.9
Electrocardiogram Results at Screening Visit
Safety Analysis Population

Maximum Dose Level: XXX mg

Patient ID	Age(yrs) / Sex/ Race	Analysis Set	Visit/ ECG Date Time(Day)	Normal Sinus Rhythm	ECG Parameter	Result	Status	Interpretation [1]	Abnormality Detail
XXXXXX	50/M/W	Safety/ Efficacy	XXXX/ DDMMMYYYY XX:XX(XX)	Yes	Ventricular Rate BEATS/MINUTE)			NCS	XXXXXXXXXX XXXXXXX
							Not Done		
					PR Interval (msec)	XX		CS	XXXXXXXXXX XXXXXXX
					QRS Duration (msec)	XXX		N	
					QT Interval (msec)		Not Done	NCS	XXXXXXXXXX XXXXXXX
					QTc*F* Interval (msec)	XXX		N	
					QTc*B* Interval (msec)	XXX		N	

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
[1] NCS=Not Clinically Significant, CS=Clinically Significant, N=Normal

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Maximum Dose Level (400 mg, 500 mg, 600 mg, missing), Patient ID, ECG Date, Time.

Listing 16.2.6.10
Female Reproductive Status and Pregnancy Test Results
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Fertility Status	Method of Birth Control	Pregnancy Test				
					Visit	Test	Performed	Collection Date	Results
XXXXXXX X	33/ F/ W	Safety/ Efficacy	XXXXXX	XXXXXXXX	XXXXXX	Urine Pregnancy	Yes	DDDMMYYY	XXXXXXX
						Serum Pregnancy	Yes	DDDMMYYY	XXXXXXX
						FSH	Yes	DDDMMYYY	XXX.X
					XXXXXX	Urine Pregnancy	No	DDDMMYYY	
						Serum Pregnancy	Yes	DDDMMYYY	XXXXXXX
						FSH	Yes	DDDMMYYY	XXX.X
XXXXXXX X	56/ F/ W	Safety/ Efficacy	XXXXXX	XXXXXXXX	XXXXXX	Urine Pregnancy	Yes	DDDMMYYY	XXXXXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Visit, Test.

Listing 16.2.6.11
Food Intake Status
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Food Intake Date Time	Food Intake
XXXXXX	50/M/W	Safety/ Efficacy	WEEK1 D1	DDMMMYYYY XX:XX	Liquid
			WEEK1 D2	DDMMMYYYY XX:XX	Liquid
			XXXXX	DDMMMYYYY XX:XX	Liquid
			XXXXX	DDMMMYYYY XX:XX	Solid such as toast
			XXXXX	DDMMMYYYY XX:XX	Not Applicable
			XXXXX	DDMMMYYYY XX:XX	Solid such as toast

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Date.

Listing 16.2.6.12
Brain MRI Results
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Allergy to MRI Macrocyclic Contrast	Date of Scan	Result
XXXXXX	50/M/W	Safety/ Efficacy	SCREEN	No	DDMMMYYYY	Normal
			WEEK1 D15	No	DDMMMYYYY	Abnormal clinically significant
XXXXXX	56/F/W	Safety/ Efficacy	XXXXXX	No	DDMMMYYYY	XXXXX
			XXXXXX	No	DDMMMYYYY	XXXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Only patients from French sites had this measurement.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Date.

Listing 16.2.6.13
Neurological Symptoms
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Headache	Nausea	Vomiting	Visual Disturbance	AE Number
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXX	No	Yes	No	Yes	XXXX
			XXXXXX	No	No	No	No	
XXXXXX	56/F/W	Safety/ Efficacy	XXXXXX	Yes	Yes	Yes	No	XXXX
			XXXXXX	No	Yes	No	Yes	XXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only - Not Collected
Only patients from French sites had this measurement.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Visit.

Listing 16.2.6.14
Neurological Examination
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Date of Assessment	Neurological Assessment	Result
XXXXXX	50/M/W	Safety/ Efficacy	XXXXXX	DDMMMYYYY	Cranial Nerve 1	Normal
					XXXXXX	Abnormal, XXXXX
			XXXXXX	DDMMMYYYY	XXXXXX	XXXXX
					XXXXXX	XXXXX

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
Only patients from French sites had this measurement.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Date.

Listing 16.2.7.1
Plasma Concentrations of PRN1008
Safety Analysis Population

Patient ID	Age(yrs)/ Sex/ Race	Visit	Sample Collected	Collection Date Time	Collection Timepoint	Sample Performed	Concentration (ng/mL)
XXXXXX	50/M/W	WEEK1 D1	Yes	DDMMMYYYY XX:XX	Pre-Dose	Yes	LLOQ (<0.100)
					2 Hours Post Dose	Yes	XX.X
		WEEK1 D2	Yes	DDMMMYYYY XX:XX		Yes	XX.X
		WEEK3 D15	No			No	
		…					

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
LLOQ = Lower Limit of quantitation

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Visit.

Listing 16.2.7.2
Percentage BTK Occupancy
ITT Population

Patient ID	Age(yrs)/ Sex/ Race	Visit	Sample Collected	Collection Date Time	Collection Timepoint	Date Time of Last Dose	Time Since Last Dose (Hr)	BTK Occupancy (%)
XXXXXX	50/M/W	WEEK1 D1	Yes	DDMMMYYYY XX:XX	Pre-Dose	DDMMMYYYY XX:XX	X.X	XX.X
					2 Hours Post Dose			XX.X
				DDMMMYYYY		DDMMMYYYY	X.X	
		WEEK1 D2	Yes	XX:XX		XX:XX		XX.X
		WEEK3 D15	No					
		…						

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Visit.

Listing 16.2.7.3
Anti-DSG 1-3 Autoantibody Levels
ITT Population

Patient ID	Age(yrs)/ Sex/ Race	Analysis Set	Visit	Collection Date Time	Anti-DSG 1	Anti-DSG 3
XXXXXX	50/M/W	Safety/ITT/mITT/PP	WEEK1 D1	DDMMMYYYY XX:XX	XX	XX
			WEEK5 D29	DDMMMYYYY XX:XX	XX	XX
			WEEK9 D57			
		…	…			

Note: Sex: M=Male, F=Female; Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, FSNC=French Sites Only – Not Collected
The limit of quantification for Anti-DSG 1 and 3 are 14 and 9 respectively.
Collection time is not available beyond Week 13/Day 85.

<DIRECTORY PATH>PROGRAM NAME Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Patient ID, Visit.
Values are presented as collected, disregarding limit of quantification.

NCT02704429

STATISTICAL ANALYSIS PLAN

Protocol PRN1008-005 (Part B)

An Open-Label, Phase 2, Pilot Study Investigating the Safety, Clinical Activity, Pharmacokinetics, and Pharmacodynamics of Oral Treatment with the BTK Inhibitor PRN1008 in Patients with Newly Diagnosed or Relapsing Pemphigus Vulgaris

Development Phase: Phase 2

Analysis Stage: Final

Sponsor

Principia Biopharma

Document Version

Final, 24-Feb-2020

Prepared by

[Filename: PRN1008-005B - SAP final 24Feb2020.docx]

SIGNATURES

Statistical Analysis Plan

Version: Final

24-Feb-2020

for PRN1008-005 (Part B) -- Final

Prepared by

[redacted]

Approval

[redacted]

[redacted]

VERSION HISTORY

Version No.	Version Date (dd-MMM-yyyy)	Description of Changes
1	24-Feb-2020	Original version

ABBREVIATIONS

Abbreviation	Definition
ABSIS	Autoimmune Bullous Skin Disorder Intensity Score
ABQOL	Autoimmune Bullous Diseases Quality of Life (assessment)
ADaM	Analysis Data Model
AE	Adverse Event
ALP	Alkaline Phosphatase
ALT	Alanine Aminotransferase
Anti-DSG	Anti-Desmoglein
AST	Aspartate Aminotransferase
BID	bis in die (twice a day)
BMI	Body Mass Index
BP	Blood Pressures
bpm	Beats per Minute
BTK	Bruton's Tyrosine Kinase
CDISC	Clinical Data Interchange Standards consortium
CDA	Control of Disease Activity
CI	Confidence Interval
CPK	Creatinine phosphokinase
CR	Complete Response
CRF	Case Report Form
CS	Clinically Significant
CTCAE	Common Terminology Criteria for Adverse Events
DBP	Diastolic Blood Pressure
DILI	Drug Induced Liver Injury
DSG	Desmoglein
ECG	Electrocardiogram
EOT	End of Treatment
ELISA	enzyme-linked immunosorbent assay
EQ-5D VAS	Euro-QoL 5-Dimension Visual Analog Scale
EOT	End of Treatment
FSH	Follicle Stimulating Hormone
GLP	Good Laboratory Practice

Abbreviation	Definition
Hep	Hepatitis
HIV	Human Immunodeficiency Viruses
IDSM	Independent Data Safety Monitor
IP	Investigational Product
ITT	Intent-to-Treat
MedDRA	Medical Dictionary for Regulatory Activities
NCS	Not Clinically Significant
PD	Pharmacodynamic
PDAI	Pemphigus Disease Area Index
PF	Pemphigus foliaceus
PK	Pharmacokinetic
PT	Preferred Term
PV	Pemphigus vulgaris
QD	quaque die (once a day)
QTc	QT interval corrected for heart rate
QOL	Quality of Life
SAE	Serious adverse event
SAP	Statistical Analysis Plan
SBP	Systolic Blood Pressure
SD	Standard Deviation
SDTM	Study Data Tabulation Model
SI	Système international d'unités (International system of units)
SMC	Safety Monitoring Committee
SNAQ	Simplified Nutritional Appetite Questionnaire
SOC	System Organ Class
TABQOL	Treatment of Autoimmune Bullous Diseases Quality of Life (assessment)
TBL	Total Bilirubin
TEAE	Treatment Emergent Adverse Event
TLFs	Tables, Listings and Figures
TPO	Thrombopoietin
TSH	Thyroid stimulating hormone
ULN	Upper Limit of Normal
WHODRUG	World Health Organization Drug

Abbreviation	Definition
WHO DDE	World Health Organization Drug Dictionary

TABLE OF CONTENTS

1 INTRODUCTION

The purpose of this document is to describe the final statistical analysis for Part B of the PRN1008-005 study. This statistical analysis plan (SAP) is based on protocol version 7.0 dated 19-Mar-2019 and case report form dated 08-Aug-2018.

The last Part A patient completed the study on 20-Jun-2019. The final Part A study SAP is version 2.0 dated 06-Jan-2019.

2 SYNOPSIS OF STUDY DESIGN & PROCEDURES

2.1 Study Objectives

2.1.1 Primary Objectives

- To evaluate the clinical safety of PRN1008 in patients with PV over a 24-week (Part B) treatment period
- To evaluate the clinical activity of PRN1008 in patients with PV

2.1.2 Secondary Objective

- To evaluate the pharmacokinetics (PK) and the pharmacodynamics (PD) of PRN1008 in patients with PV

2.1.3 Exploratory Objectives

- To evaluate the relationship of PK and PD to each other and to efficacy and safety in this patient populationTo explore effect of PRN1008 on markers of hemolysis

2.2 Study Design

This is a Phase 2, open-label, pilot cohort study to evaluate the safety, clinical activity, pharmacodynamics, and pharmacodynamics of PRN1008 in patients with newly diagnosed or relapsing PV. An open-label design was chosen for several reasons: PV does not spontaneously remit so there is no placebo effect to account for; clinicians need to know when to use rescue CSs, if necessary, in a timely manner; and lastly, the pilot nature of the investigation itself is best explored in an open-label fashion.

Up to 28 days before enrollment in the study, patients will be required to sign an informed consent form, after which screening assessments will be carried out. Patients must fulfill all entry criteria to be enrolled into the study. Patients who fail to meet the entry criteria may be rescreened at the discretion of the Investigator. Up to 25 male or female patients with newly diagnosed (i.e., naïve to an effective induction treatment regimen) or relapsing, biopsy-proven, mild or moderate PV patients (Pemphigus Disease Activity Index [PDAI] 8 to 45) to enrolled into each parts of the study.

In Part A, the initial dosing was 400 mg twice daily (bid). The duration of study therapy was 12 weeks starting on Day 1 and ending on study Day 84 (total duration of individual subject participation is 28 weeks), with follow-up visits (off-treatment) until Week 29.

In Part B, the initial dosing is 400 mg once daily (qd), with intra-patient dose escalation to 400 mg bid allowed at or after the Week 3 visit for insufficient clinical response (and then again to 600 mg bid if necessary at or after the Week 5 visit). The duration of study therapy is 24 weeks starting on Day 1 and ending on study Day 169, with a follow-up visit (off-treatment) on Day 197 (Week 29). The total duration of individual subject participation is approximately 32 weeks.

Dose Escalation Rules Part B

Current Dose	Inadequate Clinical Response* Dose-Escalation Rules
400 mg qd	Increase to 400 mg bid (allowed at Week 3 visit or later)**
400 mg bid	Increase to 600 mg bid (allowed at Week 5 visit or later)**
600 mg bid	No dose increase possible.

* Investigator discretion: Generally, clinical response is shown by some improvement seen in first 2 weeks with CDA achieved by the Week 5 visit
** Unless tolerability issues preclude dose-escalation

The first dose on Day 1 of Week 1 is supervised in the clinic and the patient observed for approximately 2 hours, at which time a PK/PD sample will be drawn. Day 2 study medication is withheld in the morning in order to gain the trough BTK occupancy approximately 12 (when bid dosing) or approximately 24 hours (when qd dosing) after the prior dose.

Where follow-up is not feasible the next day, another day in the first week of treatment may be used to get through occupancy instead, again withholding dose on the morning of that day to get an occupancy measurement close to 12 (when bid dosing) or approximately 24 (when qd dosing) hours post dose. At other follow-up visits, BTK occupancy can be measured at any time after the usual morning dose of PRN1008.

Part B patients will complete the visit procedures per the Schedule of Assessment (Table 1).

The maximum dose of PRN1008 in both parts of this study, after dose adjustment, will be 600 mg bid.

2.3 Expected Sample Size

Sample size for this pilot study was determined pragmatically by the number of subjects required to determine a preliminary safety profile and a point estimate for the primary endpoint of efficacy. If the clinical response rate is 50%, 25 subjects in each Part will result in an 80% CI of ± 13%. Results from this pilot study will be used to design confirmatory clinical trials.

2.4 Randomization and Blinding

This is an open-label study.

2.5 Protocol Versions

Study enrollment started on protocol version v3.0 04NOV2015. The current protocol version is version 6.0 dated 30 Jul 2018.

2.6 Protocol Amendments

There are 7 versions of the protocol:

Protocol Version	Version Date
Version 1 – Original protocol	25SEP2015
Version 2	05OCT2015
Version 3	13OCT2015
Version 3.1	04APR2016
Version 4	20APR2016
Version 4.1 – French Protocol Amendment	12MAY2017
Version 5	06APR2018
Version 6	30JUL2018
Version 7	19MAR2019

2.7 Schedule of Assessments

Table 1 Schedule of Assessments – Part B (24-weeks Treatment/ 4-weeks Follow Up)

	Screen	Day 1, Week 1 Pre-dose	Day 1 Week 1 Post-dose [a]	Day 2, Week 1[g]	Day 15, Week 3 +/- 3 days	Day 29, Week 5 +/- 3 days	Day 57, Week 9 +/- 7 days	Day 85, Week 13 +/- 7 days	Day 113, Week 17 +/- 7 days	Day 141, Week 21 +/- 7 days	Day 169, Week 25 +/- 7 days	Day 197, Week 29 +/- 7 days	Unscheduled Visit
Informed Consent[k]	X												
Inclusion/Exclusion Criteria	X	X											
Height	X												
Weight	X	X			X	X	X	X	X	X	X	X	X
Physical exam/med. History PDAI/ABSIS	X												
Abbreviated physical exam, PDAI, ABSIS		X			X	X	X	X	X	X	X	X	X
ECG (12-lead)	X												(X)[b]
Vital Signs[j]	X	X		X	X	X	X	X	X	X	X	X	X
Urinalysis	X												
Hep B &C, HIV, T-spot TB Test, QuantiFERON®-TB Gold, QuantiFERON®-TB Gold Plus (QFT Plus)	X												
Pregnancy test[c]	X	X				X	X	X	X	X	X	X	
Skin biopsy[d]	X												
Hem, Coag, Chem	X[e]	X			X	X	X	X	X	X	X	X	X
FSH[f]	X												
BTK occupancy sample		X[g]	X[h]	X	X	X	X	X	X	X	X		(X)[b]
PK sample		X	X[h]	X[a]	X	X	X	X	X	X	X		(X)[b]
Anti-DSG antibodies·		X				X	X	X	X	X	X	X	

	Screen	Day 1, Week 1 Pre-dose	Day 1 Week 1 Post-dose [a]	Day 2, Week 1[g]	Day 15, Week 3 +/- 3 days	Day 29, Week 5 +/- 3 days	Day 57, Week 9 +/- 7 days	Day 85, Week 13 +/- 7 days	Day 113, Week 17 +/- 7 days	Day 141, Week 21 +/- 7 days	Day 169, Week 25 +/- 7 days	Day 197, Week 29 +/- 7 days	Unscheduled Visit
Photography (Optional)[i]		X			X	X	X	X	X	X	X	X	X
ABQOL & TABQOL		X			X	X	X	X	X	X	X	X	X
SNAQ questionnaire		X				X	X	X	X	X	X	X	X
AEs		X		X	X	X	X	X	X	X	X	X	X
Concomitant Medications	X	X		X	X	X	X	X	X	X	X	X	X
Drug dispensed		X				X	X	X	X	X			
Drug reconciliation					X	X	X	X	X	X	X	X	

a. Withhold PRN1008 on morning of Day 2 until PK/PD measurement has been taken as close to 12 hours (when *bid* dosing) or 24 hours (when q*d* dosing) after the prior dose as possible. Where follow-up on Day 2 is not possible, PK/PD samples may be taken on another day in the first week of treatment. On all other days, instruct patient to take PRN1008 in the morning as usual prior to clinic and take note of the time taken. Extra PK/PD sample intended for 1 to 5 days after dose adjustment or to replace missing samples–not required for other extra visits.
b. Only if clinically indicated.
c. For women of childbearing potential only. Serum pregnancy test done at screening, urine dip test done at other time points.
d. Performed only if no suitable prior biopsy.
e. TSH and CPK taken as part of chemistry panel.
f. To confirm postmenopausal status for women who are not surgically sterile only.
g. Two-8mL pre-dose blood tubes to be collected at baseline to ensure sufficient samples for later time point assay calculations.
h. 2 hours post-dose (+/- 15 mins)
i. Photography is used to document skin disease changes; ideally in most patients. Strict masking of patient identity is required.
j. Vital sign include blood pressure (BP), pulse rate, body temperature and respiratory rate and are recorded at the time points specified
k. The Schedule of Assessments has been revised to be consistent with the 24 week treatment duration and 4 week safety follow up assessments described in this protocol Version 5, Part B. Patients enrolled in an earlier protocol version (Part A) who are in the active12-week treatment period are eligible to continue treatment, initially at their current dose, for up to a total of 24 weeks active treatment upon review and signature of the Part B EC approved Patient Informed Consent Form, with procedures and tests per this Schedule. Patients that have completed **Part A** of the trial are also eligible to be screened as a **Part B** patient, upon review and signature of the *Part B* EC-approved Patient Informed Consent Form.

3 ANALYSIS CONSIDERATIONS

3.1 Type of Analyses

The analyses and documentation described in this SAP are for the final analysis of Part B of the study.

3.2 Analysis Populations

3.2.1 Screening Population

All patients who provide informed consent and have screening assessments in Part B evaluated for study participation will be included in the Screening Population.

3.2.2 Safety Population

All patients who received at least one dose of PRN1008 in Part B will be included in the safety population. The Safety population will be used for all safety analyses.

3.2.3 Efficacy Analysis/Intent-to-Treat (ITT) Population

All patients who received at least one dose of PRN1008 in Part B will be included in the Efficacy/ITT analysis population.

3.2.4 Modified Intent-to-Treat (mITT) Population

Patients treated up to Week 5 and had a Week 5 disease assessment will be included in the mITT population.

3.2.5 Completer Population

Patients treated up to Week 25 and had a Week 29 disease assessment will be included in the Completer population.

3.2.6 Per-Protocol (PP) Population

The PP population will include patients who had:

1) No major protocol deviations relevant to data integrity per Medical Monitor review
2) At least 80% compliance based on drug accountability
3) Completed Week 29 visit.

3.2.7 Pharmacokinetic Analysis Population

The Pharmacokinetic Analysis Population will be described in the PK SAP, a separate document.

3.2.8 Patients from Part A

Two patients who were enrolled in Part A of the study also participated in Part B of the study.

Patient [redacted] relapsed in Part A, was then requalified and entered into Part B. In Part B, the patient's number was [redacted]. The date of last dose in Part A is 13MAR2018 and the date of first dose in Part B is 06DEC2018. In the Part B statistical documentation, the baseline values for the patient will be the measurements made prior to date of first study drug dosing in Part B.

Patient [redacted] with Part A starting dose of 400 mg BID rolled over into the Part B study due to timing of protocol amendment version 5. This patient is included in the Part B analyses with starting dose of 400 mg BID.

3.3 Treatment Groups

3.3.1 Treatment Group Labels

Patient disposition, demographics and other baseline data will be summarized by starting dose. Efficacy and safety data will be summarized by the dose the patient received at or prior to the assessment or at or prior to the time of event (i.e. AE start date). As a result, a given patient can be accounted for in multiple dose level groups.

The following treatment group labels will be used in the tables, listings and figures:

- Starting Dose 400 mg QD
- Starting Dose 400 mg BID
- 400 mg QD
- 400 mg BID
- 600 mg BID
- Overall
- Post Last Dose

One dosing error has been reported in the study. Between the Week 13/Day 85 and Week 17/Day 113 visits, patient [redacted] received 600 mg QD instead of 400 mg BID for 30 days; from 28MAY2019 to 29JUN2019. The assessments or events reported under the 600 mg QD dose will be summarized in the Overall group only.

3.4 Analysis Timepoints and Definitions

The study visits in Part B are:

- SCREENED
- WEEK 1 DAY 1
- WEEK 1 DAY 2
- WEEK 3 DAY 15
- WEEK 5 DAY 29

- WEEK 9 DAY 57
- WEEK 13 DAY 85
- WEEK 17 DAY 113
- WEEK 21 DAY 141
- WEEK 25 DAY 141
- WEEK 25 DAY 169
- UNSCHEDULED

All scheduled visits will be summarized. 'Last Visit' will be summarized for selected parameters and is defined as the last non-missing post-baseline visit, including unscheduled visits.

3.4.1 Baseline for PDAI, ABSIS, ABQOL, TABQOL and SNAQ scores

The value measured at Week 1 Day 1 pre-dose will be used as the Baseline value. If the Week 1 Day 1 Pre-dose result is missing and the screening result is available, then the screening result will be used as Baseline.

3.4.2 Baseline for Lab and Vital Signs

The value measured at Week 1 Day 1 pre-dose will be used as the Baseline value. If the Week 1 Day 1 pre-dose result is missing and the screening result is available, then the screening result will be used as Baseline.

3.4.3 Pre-treatment vs. treatment emergent vs. post last dose adverse events

Pre-treatment events are the events that started prior to the first study drug dosing.

Treatment emergent adverse events (TEAEs) are the events started on or after the first study drug.

Post last dose adverse events are the events that started after the last date of study drug dosing.

3.4.4 Prior medications vs. concomitant medications vs. post last dose medications

Prior medications are the medications stopped before the date of first study drug dosing.

Concomitant medications are the medications that are ongoing or stopped after the date of first study drug dosing and started on or prior to the last date of study drug dosing.

Post last dose medications are medications that started after the last date of study drug dosing.

3.4.5 Study Day

The date of first study drug dosing is considered as Study Day 1.

- Study Day = (Date of assessment – date of first dose in Part B + 1), if the assessment date is on or after the date of first dose in Part B.
- Study Day = (Date of assessment – date of first dose in Part B), if the assessment date is prior to the date of first dose in Part B.

3.4.6 Time from Pemphigus Diagnosis (years)

Time from event (years) = (Date of screening – First date of Pemphigus reported in Medical Hisotry + 1)/365.25

3.4.7 Control of Disease Activity (CDA), Complete Remission (CR), End of Consolidation Phase (ECP) and Relapse

Control of Disease Activity (CDA) is defined as the time at which new lesions cease to form and established lesions begin to heal. This is also considered the beginning of the consolidation phase. Date of CDA should be the first date CDA occurred i.e. a visit date or earlier date reported by the patient. If there is a relapse and a subsequent second CDA, visits from that second CDA visit forward would record the date of the second CDA. If CDA is yes, relapse must be no.

Complete remission (CR) in this study is defined as the complete healing of all lesions and absence of new lesions on any dose of corticosteroid or no steroids. Date of CR should be the first date CR occurred i.e. a visit date or earlier date reported by the patient. The duration of time in CR e.g. 8 weeks is not included in the definition. If CR is yes, CDA, ECP must be yes also, and relapse must be no.

End of Consolidation Phase (ECP) is defined as the time at which no new lesions have developed for a minimum of 2 weeks and the majority (approximately 80%) of established lesions have healed. It is at this point that most clinicians begin to taper corticosteroid doses. Date of ECP should be the first date ECP occurred i.e. a visit date or earlier date reported by the patient. If ECP is yes, CDA must be yes also, and relapse must be no.

Relapse (i.e. flare) is defined by the appearance of 3 or more new lesions in a month that do not heal spontaneously within 1 week, or by the extension of established lesions, in a patient who has achieved control of disease (i.e. CDA). Date of relapse should be the first date relapse occurred i.e. a visit date or earlier date reported by the patient. If relapse is yes, CDA, ECP and CR must be no.

3.5 Data Handling

3.5.1 Partial dates

Imputation of day, month and/or year for partial or missing AE onset dates will be performed as described below.

- Day, month and year missing
  - Leave as missing, no imputation. In this case, the adverse event will be considered as treatment emergent.
- Day and month missing
  - If year of start date is the same as the year of the 1st dose of PRN1008, then impute day and month as the day and month of 1st dose of PRN1008, ELSE
  - Impute day and month as 31-DEC, if the year is before the year of the 1st dose of PRN1008, ELSE

- Impute day and month as 01-JAN, if the year is after the year of the 1st dose of PRN1008

- Only day missing
  - If the year is the same as the year of the 1st dose of PRN1008
    - If the month is before the month of the 1st dose of PRN1008, then the last day of the month will be assigned to the missing day.
    - If the month is the same as the month of the 1st dose of PRN1008, then the day of the 1st dose of PRN1008 will be assigned to the missing day.
    - If the month is after the month of the 1st dose of PRN1008, then "01" will be assigned to the missing day.
  - If the year is before the year of the first dose of study drug, then the last day of the month will be assigned to the missing day.
  - If the year is after the year of the first dose of study drug, then "01" will be assigned to the missing day.

Imputation of day, month and/or year for partial or missing medication stop dates will be performed as described below if the medication is not flagged as "Ongoing".

- Day, month and year missing
  - Leave as missing, no imputation. In this case, the medication will be considered as concomitant.
- Day and month missing
  - If year of stop date is the same as the year of the 1st dose of PRN1008, then impute day and month as the day and month of 1st dose of PRN1008, ELSE
  - Impute day and month as 31-DEC.
- Only day missing
  - If month and year of start date are prior to the month and year of the 1st dose of PRN1008, then impute day as 01, ELSE
  - Impute day as day of 1st dose of PRN1008.

Imputation of day and month for partial Date of PV Diagnosis (years) will be performed as described below.

- If only day is missing, impute as 01
- If day and month are missing, impute as 01-JAN

3.5.2 Missing data imputation

For AE data, missing causality will be imputed to "Related" and missing intensity grade will be imputed to "Grade 3 – Severe".

Other missing data will not be imputed unless other specified.

3.5.3 Lab values below or above a threshold

Lab results below or above a threshold, e.g. ">= 50 mg/dL, will be excluded from descriptive statistics. The number of patients with results below or above the threshold will be presented for each lab parameter.

3.6 Interim Analyses

No interim analysis was planned for Part B of the study.

3.7 Handling of Study Center Effects

Study center effects will not be assessed.

3.8 Documentation & Other Considerations

Data are entered electronically into a clinical database built by Medidata RAVE and exported as SAS® datasets. SDTM and ADaM datasets will be generated following the Clinical Data Interchange Standards consortium (CDISC) conventions (SDTMIG v3.2 and ADaMIG v1.1). Summary tables and figures will be produced based on the ADaM datasets and patient data listings will be produced based on the SDTM datasets or raw clinical database. All tables, figures and listings will be produced using SAS® version 9.4.

AEs are coded using the Medical Dictionary for Regulatory Activities (MedDRA) Version 18.0, to assign a system organ class (SOC) and preferred term (PT) to each AE. Prior and concomitant medications are coded to preferred drug names using the World Health Organization Drug Dictionary (WHODRUG) dictionary WHO-DDE-B2-June 2015.

Continuous data will be summarized with the following descriptive statistics unless otherwise noted: number of observations, mean, standard deviation (SD), median, minimum, and maximum. Categorical data will be summarized with frequencies and percentages. Patient data listings will be listed patient ID and visit, unscheduled visits will be placed in chronological order.

No hypothesis testing will be performed.

For presentation of data, the mean and median will be presented to 1 decimal place greater than the original data, SD will be to 2 decimal places greater than the original data, and the minimum and maximum will have the same number of decimal places as the original data. Change from screening and change from baseline will be 1 decimal place greater than the observed statistics. The format for minimum and maximum will be "Min, Max". Standard deviation will be abbreviated as "SD". Percentage will be reported to 1 decimal place.

For the shift tables, the Normal to Abnormal or Normal to Out-of-Range shifts will be highlighted in yellow. Zero (0) count and percentage will not be displayed.

For the abnormal results listings, the abnormal or out-of-range results will be highlighted in yellow and clinically significant values will be in highlighted in a different color with bold font.

4 STATISTICAL METHODS

4.1 Safety Endpoints

The incidence of treatment-emergent AEs (TEAEs), including clinically significant changes in physical examination, laboratory tests, and vital signs.

4.2 Primary Efficacy Endpoint

The primary efficacy endpoint is the proportion of patients who are able to achieve control of disease activity (CDA) within 4 weeks of starting PRN1008 treatment without the need for doses of prednis(ol)one >0.5 mg/kg on or before the Week 5 visit.

CDA is defined as the time at which new lesions cease to form and established lesions begin to heal.

4.3 Secondary Efficacy Endpoints

The secondary efficacy endpoints are:

1) Cumulative incidence of patients able to achieve CDA by each visit
2) Cumulative incidence of patients able to achieve CDA by each visit on zero steroids.
3) Cumulative incidence of patients able to achieve CDA by each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
4) Cumulative incidence of patients able to achieve CDA by each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
5) Proportion of patients able to achieve CDA at each visit
6) Proportion of patients able to achieve CDA at each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
7) Proportion of patients able to achieve CDA at each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
8) Cumulative incidence of patients able to achieve CR by each visit
9) Cumulative incidence of patients able to achieve CR by each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
10) Cumulative incidence of patients able to achieve CR by each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
11) Proportion of patients able to achieve CR at each visit
12) Proportion of patients able to achieve CR at each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
13) Proportion of patients able to achieve CR at each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
14) Proportion of patients able to achieve a complete CR **with** the need for dose of prednis(ol)one of greater than 0.5 mg/kg within 24 weeks, i.e. on or prior to Week 25 visit
15) Proportion of patients able to achieve a complete response (CR) **without** prednis(ol)one of greater than 0.5 mg/kg within 24 weeks, i.e. on or prior to Week 25 visit

16) Time to first CDA
17) Time to first CR
18) Time to first end of consolidation phase
19) Time to first relapse (after CDA has been achieved, as per relapse definition)
20) Time to first increase in PDAI
21) Cumulative CS usage over 24 weeks
22) Change from baseline in Pemphigus Disease Area Index (PDAI) and Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) scores at each visit
23) Change from baseline in Autoimmune Bullous Diseases Quality of Life (ABQOL) and Treatment of Autoimmune Bullous Diseases Quality of Life (TABQOL) scores at each visit
24) Change from baseline in appetite (SNAQ score) at each visit

CR, End of consolidation phase, and Relapse definitions are listed below and noted where they differ listed below:

- Complete response (CR): the absence of new or established lesions while the patient is receiving low dose CS, defined in this study as < or = 0.5mg/kg or 0, as stated in the specific endpoint. The duration of CR is not part of the definition for this study.

- End of consolidation phase: The time at which no new lesions have developed for minimum of 2 weeks, approximately 80% of lesions have healed, and when most clinicians start to taper steroids.

- Relapse/flare: Appearance of ≥3 new lesions/month that do not heal spontaneously within 1 week, or by extension of established lesions, in a patient who has achieved disease control

4.4 Pharmacokinetic Outcome Measure

Plasma concentrations of PRN1008 will be measured at approximately the time of maximum concentration on Day 1 and at various subsequent times during outpatient dosing.

A separate SAP will describe the PK outcome measures and analyses. PK results will be summarized in an independent report.

4.5 Pharmacodynamic Outcome Measures

- Percentage BTK occupancy for individuals in PBMCs will be determined at 2 & 24 hours after the first PRN1008 dose and at varied subsequent times during outpatient dosing

- Actual result, change and percent change from screening and change and percent change from baseline in Anti-DSG-1-3 autoantibody levels by ELISA at various time points for all patients and by baseline Anti-DSG-1-3 values of ≥ 100 unit vs. < 100 unit.

A separate SAP will be created for the BTK occupancy analysis and the results will be summarized in a separate report.

4.6 Exploratory Measures

- Exploratory PK/PD analysis will examine the effects, if any, of covariates on PK and/or PD, and the relationship between PK, PD and efficacy in this population

A separate SAP will be created for the PK/PD analysis and the results will be summarized in a separate report.

4.7 Changes to Planned Analyses from Protocol

Change	**Description**	**See SAP section**
Analysis populations	The following analysis populations are added: ▪ Modified Intent-to-Treat (mITT) Population ▪ Completer Population ▪ Per-Protocol (PP) Population	3.2.4, 3.2.5, 3.2.6
Secondary endpoints	The secondary endpoints are re-ordered to group all the CDA endpoints together and then followed by the CR endpoints. Some of the endpoints are re-worded for clarity and consistency within the SAP but the meaning and interpretation remained the same as defined in the protocol.	4.3, 5.4.2.1
Additional secondary endpoints	The following secondary efficacy endpoints were not listed in the protocol but included in the Part A SAP. They will be also analyzed for Part B of the study. • Proportion of patients able to achieve CDA at each visit • Proportion of patients able to achieve CDA at each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg • Cumulative incidence of patients ever achieved CDA by each visit • Cumulative incidence of patients able to achieve CDA by each visit on zero steroids. • Cumulative incidence of patients ever achieved CDA by each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg • Proportion of patients able to achieve CR at each visit • Proportion of patients able to achieve CR at each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg • Cumulative incidence of patients ever achieved CR by each visit	4.3

	• Cumulative incidence of patients ever achieved CR by each visit with or without the need for doses of prednis(ol)one of greater than 0.5 mg/kg • Time to first increase in PDAI	

5 ANALYSIS DETAILS

5.1 Patient Disposition, Demographics and Baseline Characteristics

The Screening population will be used for patient disposition and reason for screen fails. The ITT population will be used for demographics and baseline characteristics. Descriptive statistics will be done by starting dose and overall, unless specified otherwise. Patient data listings will be listed by starting dose and patient and start date of event, if applicable.

5.1.1 Patient Disposition

The number and percentage of patients will be tabulated for patients who:

- Enrolled into the study (Screening population)
- Had at least one dose of PRN1008 (Safety and ITT populations)
- Completed treatment up to Week 5 and had a Week 5 disease assessment (mITT population)
- Had no major protocol deviations, completed Week 29/Day 197 visit and had at least 80% study drug compliance (PP population)
- Completed the study
- Early discontinued
- Reason for early discontinuation for patients who early discontinued
    - Adverse Event
    - Death
    - Lack of Efficacy
    - Lost to Follow-up
    - Non-Compliance with Study Drug
    - Physician Decision
    - Pregnancy
    - Progressive Disease
    - Recovery
    - Trial Screen Failure
    - Study Terminated by Sponsor
    - Technical Problem
    - Withdrawal by Subject
    - Other

The number and percentage of patients who attended each visit and the number of percentages of patients enrolled by country and study site will also be tabulated.

Detailed disposition, study enrollment and analysis population information will be listed.

Screen failures will be listed with date and reason for screen failure.

5.1.2 Demographics and Baseline Characteristics

Demographics variables including age, gender, fertility status and method of birth control for female patients, race, ethnicity, height at screening (cm), weight at screening (kg) and body mass index (BMI) (kg/m^2) at screening will be summarized by descriptive statistics and listed in a patient data listing.

5.1.3 Disease History

Disease history data including pemphigus subtype at screening, time from pemphigus diagnosis (by the earliest medical history record) date to screen date (years), Pemphigus history type (Newly diagnosed vs. Relapsed), baseline Anti-DSG 1 and Anti-DSG 3 antibody status/profile, Total Anti-DSG at baseline (< 100 vs ≥100) PDAI total activity score, Pemphigus severity at baseline (PDAI < 15 (mild-moderate) or PDAI ≥ 15 (moderate-severe)), PDAI mucous membrane activity (% >0), PDAI scalp activity (% >0), PDAI skin activity (% >0) and ABSIS oral activity (% >0) will be summarized in a frequency table and listed.

Newly diagnosed pemphigus is defined as subjects who were diagnosed at most 180 days prior to date of screening, otherwise the patient is defined as relapsed.

Baseline Anti-DSG status will be determined by the central lab results and presented in the following categories:

- Anti-DSG 1 positive, Anti-DSG 3 negative;
- Anti-DSG 1 negative, Anti-DSG 3 positive;
- Anti-DSG 1 positive, Anti-DSG 3 positive;
- Anti-DSG 1 negative, Anti-DSG 3 negative

Ranges for Anti-DSG status:

	Status	Range
Anti-DSG 1	positive	≥ 14
	negative	< 14
Anti-DSG 3	positive	≥ 9
	negative	< 9

5.1.4 Medical History

Medical history events are reported for the following Body Systems: Skin, Head-Eyes-Ears-Nose-Throat, Respiratory, Cardiovascular, Gastrointestinal, Endocrine/Metabolic, Genitourinary, Neurological, Blood/Lymphatic, Musculoskeletal, Hepatic, Allergies, Psychological/Psychiatric and Other. The medical history events will be summarized by Body System, Starting dose and overall.

Medical history data will be listed in a patient data listing.

5.1.5 *Prior and Concomitant Medications*

All medications will be coded according to the WHODRUG dictionary WHO-DDE-B2-June 2015.

The following prior medications will be summarized by Level 2 Classification and Preferred Name for each starting dose and overall:

- Previous corticosteroid medications taken and stopped prior to date of first study drug dosing
- Other medications taken and stopped prior to date of first study drug dosing

The following concomitant medications will be summarized by Level 2 Classification and Preferred Name for each dose level and overall:

- Corticosteroid medications taken during treatment
- Corticosteroid medications started after date of last study drug dosing
- Other medications taken on or after date of first study drug dosing

Medications will be attributed to the dose level where the medication is taken during the dose level. The same medication may be attributed to more than one dose level. Corticosteroid medication started after the date of last study drug dosing will be summarized separately.

Separate listings will be provided for prior corticosteroid treatment, concomitant corticosteroid medications, post last dose corticosteroid medications, other prior medications and other concomitant medications.

5.2 Protocol Deviations

Protocol deviations will be identified and classified into major vs. non-major by the Sponsor prior to database lock.

The major protocol deviations will be tabulated by the deviation categories, starting dose and overall for patients who are in the ITT population.

A patient data listing with deviation category and deviation details for both will be provided for the major protocol deviations and the non-major protocol deviations.

5.3 Study Drug Treatment Exposure and Compliance

Study drug treatment information will be summarized and documented in the ITT and mITT Population. Number of dose adjustments and maximum dose will be tabulated by starting dose. Descriptive statistics for study drug exposure and compliance will be done by dose level and overall. Patient data listings will be listed by starting dose and patient and study visit.

5.3.1 Study Drug Exposure

Study drug exposure will be summarized by dose level.

- Duration of exposure for Dose level i (days) =
  (date of last dose for dose level i – date of first dose for dose level i) + 1.

- Dose Taken for Dose level i (mg) = (Number of tablets dispensed – Number of tablets returned)* Current Dose Level (mg)
- Calculated Dose for Dose level i (mg/day) =
  (Dose taken for Dose level i)/Duration of exposure for Dose level i.

5.3.2 Study Drug Compliance

Compliance with study drug at each dose level and overall will be calculated as the actual number of doses taken divided by the expected number of doses to be taken (taking into account the # tablets for each dose), expressed as a percentage. Expected number of doses will be determined by the number of days in between visits and the frequency of dosing (QD or BID).

Compliance will be summarized by descriptive statistics for each dose level and overall. The number and percentage of patients with 100% compliance, greater than 80% but less than 100% compliance and less than 80% compliance will be tabulated for each dose level.

5.4 Efficacy Analyses

The mITT population will be used for all efficacy analyses. For analyses at a particular study drug dose, all patients who received that dose at least once will be included. In addition, for selected analyses, where specified, the Completer and PP population will be used.

The descriptive statistics will be done by dose level and overall for all study visits, unless specified otherwise. All binomial confidence intervals for efficacy analyses will use the Exact (Clopper-Pearson) method. Patient data listings will be listed by starting dose, patient, dose level and study visit.

5.4.1 Primary Efficacy Endpoint

The primary efficacy endpoint is:

- The proportion of patients who are able to achieve CDA within 4 weeks (on or prior to Week 5 visit) of starting PRN1008 treatment **without** the need for doses of prednis(ol)one > 0.5 mg/kg

The Control of Disease Activity (CDA) responses are assessed at visits specified in the Schedule of assessments (Table 1). Types of CS other than prednis(ol)one are converted to dose-equivalent prednis(ol)one (see Section 5.4.2.3).

This is operationalized as meaning the first CDA is achieved on or before the Week 5 visit but after first dose of PRN1008, and will be summarized, by dose level to the Week 5 visit and overall in a table, using frequencies and percentages. In addition, two-sided 80% and 95% CIs (Confidence Intervals) of the response rate will be provided for each dose level and overall.

Patients who are able to achieve CDA between first dose of study drug and the Week 5 visit without prednis(ol)one >0.5 mg/kg on the date prior to the CDA will be considered as responders, even if the CDA status was not maintained or higher doses of prednis(ol)one were used after the first CDA. Patients who are not able to achieve CDA prior to or at the Week 5 visit or had prednis(ol)one >0.5 mg/kg on the date prior to CDA will be considered as non-responders. Patients whose CDA status is unknown at the Week 5 visit for whatever reason (including early termination before Week 5 visit) will be also considered as non-responders. Only systemic CSs (as defined by medication taken orally or intravenously) are to be used to evaluate the efficacy endpoints, topical CSs (including buccal CS) will not be counted.

The number and percentage of patients who meet the primary efficacy endpoint criteria will be tabulated by dose level and overall (all patients).

A sensitivity analysis using the Completer and PP population will be provided.

Panel plots will be provided for PDAI score over the study period for each patient. Dose level (mg/day) will also be plotted on the same figure. Lines for the steroid equivalent dose in mg/kg will be added to the plots. A total of 9 plots (3 x 3) will be fitted on one page.

5.4.1.1 Subset Analyses

The robustness of the primary endpoint results will be explored by calculating the number and percent of patients meeting the primary efficacy endpoint overall (i.e. across all doses) within the following patient subsets:

1) Age (< 50 years, ≥ 50 years)
2) Gender
3) Total anti-desmoglein (DSG) antibody (≥ 100, < 100 units) at baseline.
4) Pemphigus History Type: Newly diagnosed (≤ 6 months from screening) vs. relapsed (> 6 months from screening)
5) Pemphigus anti-DSG Profile at baseline: Pemphigus Vulgaris (PV) vs. Pemphigus foliaceus (PF) vs. negative anti-DSG profile, defined as follows:
    - PV: anti-DSG3 positive with anti-DSG1 negative or anti-DSG3 positive with anti-DSG1 positive.
    - PF: anti-DSG1 positive only
    - Negative anti-DSG profile: neither detectable.
6) Pemphigus Severity: PDAI < 15 (mild-moderate) vs PDAI ≥ 15 (moderate-severe) at baseline If screening PDAI is not available use Week 1 Day 1 Pre-dose.

A sensitivity analysis using the Completer and PP populations will be provided for each subset analysis.

5.4.2 Secondary Efficacy Endpoints

5.4.2.1 CDA Responses

The number and percentage of patients who meet the criteria below will be tabulated by dose level and overall where the response first occurred and overall. 80% and 95% CIs will be calculated for the proportions using the Exact (Clopper-Pearson) method .

1) Cumulative incidence of patients able to achieve CDA by each visit
2) Cumulative incidence of patients able to achieve CDA by each visit on zero steroids.
3) Cumulative incidence of patients able to achieve CDA by each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
4) Cumulative incidence of patients able to achieve CDA by each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
5) Proportion of patients able to achieve CDA at each visit
6) Proportion of patients able to achieve CDA at each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
7) Proportion of patients able to achieve CDA at each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
8) Cumulative incidence of patients able to achieve CR by each visit
9) Cumulative incidence of patients able to achieve CR by each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
10) Cumulative incidence of patients able to achieve CR by each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
11) Proportion of patients able to achieve CR at each visit
12) Proportion of patients able to achieve CR at each visit **with** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
13) Proportion of patients able to achieve CR at each visit **without** the need for doses of prednis(ol)one of greater than 0.5 mg/kg
14) Proportion of patients able to achieve a complete CR **with** the need for dose of prednis(ol)one of greater than 0.5 mg/kg within 24 weeks, i.e. on or prior to Week 25 visit
15) Proportion of patients able to achieve a complete response (CR) **without** prednis(ol)one of greater than 0.5 mg/kg within 24 weeks, i.e. on or prior to Week 25 visit

The CDA status, CR status, ECP status, and Relapse status by visit, by patient will be presented in a listing.

Duration of CR will be summarized and listed, and is defined by the longest period of consecutive visits in CR (last known CR date – first known CR date + 1). Each subject will only have one CR duration as a result.

A sensitivity analysis using the Completer and PP populations will be provided for all analyses of CDA response.

5.4.2.2 Time-to-Event

The following time-to-event analyses will be performed by dose level and overall for events occurred after date of first study drug dosing:

a) Time to first CDA
b) Time to first CR
c) Time to first ECP
d) Time to first relapse (after CDA has been achieved, as per the definition of relapse) after first dose of study drug
e) Time to first increase in PDAI

For each event listed above, the number of days to the event will be calculated as:

(Date of first occurrence of event – Date of first study drug dosing of the dose level where the event is observed) + 1

The number of days to first occurrence of each event (a to d) will be summarized by descriptive statistics for each dose level and overall.

Patients who did not achieve the specific event are censored at the last day in a dose level or date of study completion/discontinuation.

Kaplan-Meier estimates of quartiles in days and their associated 80% and 95% CI will be calculated by using the LOGLOG transformation. Minimum and maximum survival times in days as well as number of the patients and the probability of experiencing the event on or before specific date will be provided in a table.

A sensitivity analysis using the Completer and PP populations will be provided.

5.4.2.3 Cumulative Corticosteroid (CS) Usage

Cumulative corticosteroid (CS) usage per patient (mg) will be presented by treatment period (up to first 12 weeks, after first 12 weeks, entire treatment period and follow-up period), by dose level and overall in a table. To calculate this endpoint, the following steps will be followed:

Step 1: Select CSs with route = “ORAL” or “INTRAVENOUS”. The CSs will be converted into prednisolone/prednisone dosage equivalent using the below conversion chart.

CS medication	Conversion factor
Prednisone, Prednisolone	1
Hydrocortisone	0.25
Methylprednisolone, Methylprednisolone Sodium Succinate	1.25

Dexamethasone, Betamethasone	6.67

Step 2: The CSs usage will be converted to standard frequency using the following conversion chart.

Frequency	Unit	Conversion factor
QD, Q24H, ONCE, CONTINUOUS, BD PRN, PRN	mg	1
5 TIME A DAY	mg	5
QID	mg	4
TID	mg	3
2-3 DAILY	mg	2.5
BID	mg	2
QOD, EVERY 2 DAYS	mg	1/2
BIS	mg	2/7
QS, EVERY WEEK	mg	1/7
Any value	mg/day	1

Once the prednisolone/prednisone dosage equivalent and frequency are converted as above, each record of CSs taken will be converted to prednisolone with standard frequency (mg/day).

Step 3: The duration for each record of CS taken will be calculated using the formula: Duration = CS end date – CS start date + 1, in days. Any partial start or end date of CSs taken will be imputed using the algorithm specified in Section 3.5.1. If the CS start date is completely missing, then the date will be imputed as Week 1 Day 1, i.e. it is assumed that the medication is taken at the study entry. If the CS stop date is completely missing, then the date will be imputed as Date of End of Study, i.e. it is assumed that the medication is taken up to the end of study.

If the CS start date is before the Week 1 Day, then the CS start date will be replaced by the Week 1 Day 1; if the CS start date is after the date of Week 29 (Day 197) - 1, this record will not be used to summarize cumulative dose for CSs.

If the CS end date is after the date of Week 29 (Day 197) - 1, then the CS end date will be replaced by the date of Week 29 (Day 197) - 1; if the CS end date is before the study treatment start date, this record will not be used to summarize cumulative dose for CSs.

Note that above date adjustments only apply to cumulative CS dose calculation. Other analyses, where CS dose is needed, will use CS start and end dates as reported.

Step 4: The “record” level cumulative CSs usage will be calculated as: record level cumulative dose = prednisolone/prednisone dosage equivalent * conversion factor for frequency * duration (days).

Step 5: The patient level cumulative CSs usage will be calculated as the summation of all the non-missing record level cumulative doses. If a patient had no CS records that meet the above criteria, the CS

cumulative dose is set as 0. The average daily CS usage in mg/day will be calculated as the cumulative doses dividual the number of days within a study period.

All CS usage will be presented in a listing showing record level prednisone dosage equivalent (mg), dose, frequency, route, start and stop dates. The total prednisone dosage equivalent in mg/kg will also be calculated by using the last measured weight prior to the current visit.

5.4.2.4 CS Dose on the Day Before Each Visit

For each patient on the day before each scheduled visit, dosage level of CS dose in mg/day and mg/kg/day will be summarized using summary statistics by dose level and overall. Types of CS other than prednis(ol)one are converted to dose-equivalent prednis(ol)one (see Section 5.4.2.3).

For Screening and Week 1/Day 1 visits, however, the CS dose is set as the day of, rather than the day before.

If a patient did not report any CS, then the dosage level is set as 0. If a subject is terminated prior to a visit, then this subject is not included in the calculation for visit.

The same table will be repeated for: 1) patients achieved at least one CDA and 2) to included patients achieved at least one.

A listing of the CS dose in my/day and mg/kg/day will be listed with the CDA and CR response at each visit.Change from Baseline in PDAI ABSIS, ABQOL and TABQOL Scores

Quantitative assessment of patient response and disease progression will be determined using the following scores at visits specified in the Schedule of assessments (Table 1):

1) Pemphigus Disease Area Index (PDAI) score – Total Activity Score
2) Pemphigus Disease Area Index (PDAI) score – Total Damage Score
3) Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) score
4) Autoimmune Bullous Disease Quality of Life (ABQOL) score
5) Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) score

The PDAI questionnaire has two components including activity and damage. The activity component consists of skin, scalp and mucosa parts and the damage component consists of skin and scalp parts. Actual values, change from screening, percent change from screening, change from baseline and percent change from baseline in PDAI total activity, total damage scores will be summarized by dose level and overall, respectively.

The PDAI activity or damage total scores per body part will be calculated as the sum of the scores on that body part as follows. PDAI total activity and total damage scores will be calculated as follows:

- PDAI total activity score = Total skin activity + Total scalp activity + Total mucosa activity
- PDAI total damage score = Total skin damage + Total scalp damage

The ABSIS questionnaire has three components, including skin involvement, oral involvement and (oral) severity. The ABSIS scores will be calculated separately for each component. The ABSIS total activity score and total ABSIS score will also be calculated as follows:

- Skin involvement total score = sum of (%BSA * Weighting factor for each skin involvement area)
- Oral involvement total score = sum of (score from each oral part)
- (Oral) Severity total score = sum of (Level * Factor of discomfort for each food item)
- ABSIS total activity score = Skin involvement total score + Oral involvement total score
- Total ABSIS score = Skin involvement total score + Oral involvement total score + Severity total score

Actual values and change from baseline in ABSIS skin involvement total score, ABSIS oral involvement total score and ABSIS total activity score will be summarized for all patients, and for the two sub-groups:

- baseline oral involvement total score = 0 vs. >0
- baseline skin involvement score = 0 vs >0.

When calculating skin involvement total score, if %BSA = 0, then skin involvement score component is 0. If %BSA > 0, then weighting factor needs to be considered, and if weighting factor is missing in this circumstance, then the skin involvement total score and ABSIS total score are considered missing.

The ABQOL and TABQOL questionnaires both contains 17 questions with numerical scores. The ABQOL and TABQOL scores will be calculated as the sum of scores from each question. Actual values and change from baseline in ABQOL and TABQOL scores will also be summarized by dose level and overall in a table. If individual scores are missing, then the total scores are set as missing.

PDAI, ABSIS, ABQOL and TABQOL scores and sub-scores (for PDAI and ABSIS) by patient and visit will be presented in separate listings.

5.4.2.5 Change from Baseline in Simple Nutritional Appetite Questionnaire (SNAQ) score

Simple Nutritional Appetite Questionnaire (SNAQ) contains 4 questions with numerical scores and is assessed at visits specified in the Schedule of assessments (Table 1). The SNAQ score will be calculated as the sum of scores from each question. The SNAQ score ranges from 4 to 20. If individual scores are missing, then the total score is set as missing.

Actual values and change from baseline in SNAQ score at each visit will be summarized by dose level and overall.

The SNAQ score will be presented in a listing by patient and visit.

5.4.2.1 Change from Baseline and Percent Change from Baseline in Total Anti-DSG, Anti-DSG 1 and Anti-DSG

The Anti-DSG 1 and Anti-DSG 3 results are measured at visits specified in the Schedule of assessments (Table 1). Total Anti-DSG is the sum of Anti-DSG 1 and Anti-DSG3, where at least one of Anti-DSG 1 or 3 is positive per Section 5.1.3, i.e. Anti-DSG 1 ≥ 14 and/or Anti-DSG 3 ≥ 9.

Actual result, change from baseline and percent change from baseline in Total Anti-DSG, positive Anti-DSG1, i.e. ≥ 14, and positive Anti-DSG 3, i.e. ≥ 9, at each visit will be summarized by dose level and overall. The same descriptive statistics will be produced by baseline Anti-DSG value (>= 100 units vs. < 100 unit).

The Total Anti-DSG, Anti-DSG 1 and Anti-DSG 3 values will be presented in a listing by patient and visit.

5.5 Safety Analyses

The Safety Population will be used for the safety analyses. The summary statistics will be done by dose level and overall for all study visits, unless specified otherwise. Patient data listings will be listed by starting dose, patient, dose level and study visit or start date of event if applicable.

5.5.1 Adverse Events

TEAEs will be attributed to the dose level where the adverse event start date is on or after the start date of that dose level and before the next dose level if applicable.

Adverse events with preferred term (PT) = “Pemphigus” will be excluded from the TEAE tables. All pemphigus PT are Disease Under Study and will be documented in a separate summary table and a separate patient data listing labeled as Disease Under Study Events.

A summary of adverse events by dose level and overall will include:

- The number of pre-treatment AEs and the number/percentage of patients reporting at least one pre-treatment AE
- The number of pre-treatment SAEs and the number/percentage of patients reporting at least one pre-treatment SAE
- The number of TEAEs and the number/percentage of patients reporting at least one TEAE
- The number of post last dose adverse events and the number/percentage of patients reporting at least one post last dose adverse event
- The number of related TEAEs and the number/percentage of patients reporting at least one related TEAE
- The number of serious TEAEs and the number/percentage of patients reporting at least one serious TEAE
- The number of related serious TEAEs and the number/percentage of patients reporting at least one related serious TEAE

- The number of TEAEs leading to study discontinuation and the number/percentage of patients reporting at least one TEAE leading to study discontinuation
- The number of deaths and the number/percentage of patients reporting at least one TEAE death
- The number/percentage of patients reporting TEAEs by Action Taken with Study Drug
- The number/percentage of patients reporting TEAEs by the maximum grade

TEAEs will be summarized by SOC and PT by dose level and overall. Patients with multiple events within a particular SOC or PT within each dose level will be counted once within that dose level. The number of episodes of events for each SOC and PT will also be displayed. The same tabulation will be performed for related TEAEs, SAEs and AE leading to study drug discontinuation.

TEAEs will also be summarized by the maximum intensity grade (Grade 1 to 5), SOC and PT. Patients with multiple events within a particular SOC or PT will be counted once under the maximum intensity grade. The same tabulation will be performed for related TEAEs.

Patient data listings will be generated for:

- Prior adverse events
- Prior SAE
- TEAEs
- Related TEAEs
- SAEs
- TEAE leading to study drug discontinuation
- TEAE leading to death (AE with intensity = Grade 5 – Death related AE)
- Post last dose TEAEs
- Disease Under Study (Pemphigus PT events)

5.5.2 Laboratory Data

The following laboratory parameters are measured at visits specified in the Schedule of Assessments (Table 1).

Laboratory Panel	Test Parameters
Hematology	hemoglobin, hematocrit, erythrocyte count (RBCs), thrombocyte count (platelets), leukocyte count (WBCs) with differential in absolute counts (including neutrophils, eosinophils, basophils, lymphocytes, and monocytes)
Coagulation	PT/INR, thrombin time, aPTT, fibrinogen level.
Serum Chemistry	aspartate aminotransferase (AST), slanine aminotransferase (ALT), total, direct, and indirect bilirubin levels, alkaline phosphatase (ALP), albumin, creatinine, urea, total protein, sodium, chloride, calcium, phosphate, potassium, glucose (random), creatine phosphokinase (CPK) and thyroid stimulating hormone (TSH), chloride, calcium, phosphate, potassium, glucose (random), creatinine phosphokinase (CPK) and serum pregnancy
Urinalysis	pH, specific gravity, protein, glucose, ketones, bilirubin, blood, nitrites, urobilinogen and leukocytes measured by dipstick

Laboratory Panel	Test Parameters
Serology	HIV, hepatitis B and hepatitis C and QuantiFERON® TB testing at screening only

Serology tests are only measured at screening. These results will be presented in a patient data listing.

For other laboratory panels, the actual values and change from baseline values in numerical results will be summarized by dose level and overall. For categorical results, the frequency counts and percentages will be tabulated by each dose level and overall.

Shifts from baseline tables will be provided for all laboratory parameters where low/normal/high can be determined by the normal ranges or can be classified as normal/abnormal.

A special group of clinical laboratory tests, including ALT, AST, TBL and ALP, which are believed to be the indicators for drug induced liver injury (DILI) will be assessed separately. Frequency tables will be used to summarize elevations in these parameters. The following criteria will be documented:

- AST > 3x, 5x, 10x and 20x Upper Limit of Normal (ULN)
- ALT > 3x, 5x, 10x, and 20x ULN
- TBL > 1.5x and 2x ULN
- ALP > 1.5x ULN
- ALT or AST > 3x ULN and Total Bilirubin > 1.5x ULN
- ALT or AST > 3x ULN and Total Bilirubin > 2x ULN

Scatter plots of the peak TBL divided by the ULN will be plotted on a log scale against the peak ALT divided by the ULN. Reference lines will be plotted for the ULN for TBL and ALT, 2x ULN for TBL and 3x ULN for ALT. Any patients in the upper right quadrant, which is defined by 2x ULN for TBL and 3x ULN for ALT, would represent cases to be investigate for potential DILI.

Patient data listings will be provided for each laboratory panel and the DILI parameters. Separate listings will be provided for patients with out of range results or abnormal results at any visits.

5.5.3 Vital Signs and Body Weight

Vital signs include blood pressures (BP), pulse, temperature and respiratory rate and body weight are measured at visits specified in the Schedule of assessments (Table 1).

The actual values and change from baseline values will be summarized by dose level and overall for each visit. A separate table will be generated to include only the baseline, Day 85, Day 169 and Day 197 visits.

The following ranges will be used to classified the BP and pulse measurements as Low/Normal/High:

- Systolic blood pressure (SBP): 90 to 140 mmHg, inclusive
- Diastolic blood pressure (DBP): 60 to 80 mmHg, inclusive
- Pulse: 40 to 100 bpm, inclusive

Shift from baseline tables will be produced by using the above criteria.

Patient data listings will be provided for the vital sign parameters and body weight. Separate listings will be provided for patients with out of range SBP, DBP or pulse results at any visits.

5.5.4 12-Lead ECG

ECG assessment is performed at the screening visit and unscheduled visit, if clinically indicated.

The ECG parameters include: normal sinus rhythm? (yes or no), ventricular rate, P-R interval, QRS interval, QT duration, QTcF, QTcB interval and overall ECG interpretation (normal, abnormal – not clinically significant (NCS) or abnormal – clinically significant (CS)).

For the numerical values, actual values will be summarized by descriptive statistics for each dose level and overall. For normal sinus rhythm and ECG interpretation, the results will be tabulated by each dose level and overall in a shift from baseline table.

A patient data listing will be provided for the ECG results.

5.5.5 Physical Examinations

Physical examinations are performed as specified in the Schedule of Assessments (Table 1).

At the screening visit, a complete physical examination consists of checking the normality or abnormality of the following body systems: general appearance, skin, eyes, ears, nose, throat, heart, chest/breast, abdomen, neurological system, lymph nodes, skeletal and other.

At other visits, an abbreviated physical examination consists of checking the normality or abnormality of the following body systems: general appearance, abdomen, cardiorespiratory and other.

The number and percentage of patients with assessments of normal, abnormal NCS, abnormal CS or Not Done at all scheduled visits, will be summarized in a frequency table for each body system by dose level and overall.

Patient data listings will be provided for all physical examinations results. Separate listings will be provided for patients with abnormal NCS or abnormal CS findings at any visits.

5.5.6 Female Reproductive Status and Pregnancy Test Results

The fertility status and method of birth control used for women is reported at the screening visit. The pregnancy test is performed for women of childbearing potential only. A pregnancy serum test is done at the screening visit and urine dipstick tests are done at other time points. The reproductive status as well as serum and urine pregnancy test results will be presented.

5.5.7 Food Intake Status

Date and time of food intake and type of food intake (liquid, solid such as toast or not applicable) are reported at each visit. A listing of patient food intake status will be presented.

6 MOCK SHELLS FOR TABLES, LISTINGS AND FIGURES (TLFS)

The mock shells define the layout of the TFLs and may be adjusted to incorporate the actual data collected. The decimals shown on the shells are examples, the actual decimals to be presented will be based on the precision of the data collected.

TABLE OF CONTENTS

Table B.14.1.1.1 Patient Disposition – Completion Status (Screening Population)

	Number of patients (%)		
	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Screening population – Enrolled	n	n	n
ITT population – Had at least one dose of PRN1008 [1]	x (x.x)	x (x.x)	x (x.x)
Safety population – Had at least one dose of PRN1008 [1]	x (x.x)	x (x.x)	x (x.x)
mITT population – Treated up to Week 5 and had a Week 5 CDA assessment [1]	x (x.x)	x (x.x)	x (x.x)
Completer population – Treated up to Week 25 andhad a Week 29 CDA assessment [1]	x (x.x)	x (x.x)	x (x.x)
PP population – Had no major protocol deviations, completed Week 13/Day 85 visit and had at least 80% study drug compliance [1]	x (x.x)	x (x.x)	x (x.x)
Completion status [2]			
Completed	x (x.x)	x (x.x)	x (x.x)
Early discontinuation	x (x.x)	x (x.x)	x (x.x)
Primary reason(s) for early discontinuation [3]			
Adverse Event	x (x.x)	x (x.x)	x (x.x)
Death	x (x.x)	x (x.x)	x (x.x)
Lack of Efficacy	x (x.x)	x (x.x)	x (x.x)
Lost to Follow-up	x (x.x)	x (x.x)	x (x.x)
Non-Compliance with Study Drug	x (x.x)	x (x.x)	x (x.x)
Physician Decision	x (x.x)	x (x.x)	x (x.x)
Pregnancy	x (x.x)	x (x.x)	x (x.x)
Progressive Disease	x (x.x)	x (x.x)	x (x.x)

Table B.14.1.1.1 Patient Disposition – Completion Status (Screening Population)

	Number of patients (%)		
	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Recovery	x (x.x)	x (x.x)	x (x.x)
Trial Screen Failure	x (x.x)	x (x.x)	x (x.x)
Study Terminated by Sponsor	x (x.x)	x (x.x)	x (x.x)
Technical Problem	x (x.x)	x (x.x)	x (x.x)
Withdrawal by Subject	x (x.x)	x (x.x)	x (x.x)
Other	x (x.x)	x (x.x)	x (x.x)

[1] Percentages are based on the number of patients enrolled into the study.
[2] Percentages are based on the number of patients who received at least one dose of PRN1008.
[3] Percentages are based on the number of patients who withdrew early.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.1.2 Patient Disposition by Study Visit (ITT Population)

	Number of patients (%)		
Attended	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
SCREENED	x (x.x)	x (x.x)	x (x.x)
WEEK 1 DAY 1	x (x.x)	x (x.x)	x (x.x)
WEEK 1 DAY 2	x (x.x)	x (x.x)	x (x.x)
WEEK 3 DAY 15	x (x.x)	x (x.x)	x (x.x)
WEEK 5 DAY 29	x (x.x)	x (x.x)	x (x.x)
WEEK 9 DAY 25	x (x.x)	x (x.x)	x (x.x)
WEEK 13 DAY 85	x (x.x)	x (x.x)	x (x.x)
WEEK 17 DAY 113	x (x.x)	x (x.x)	x (x.x)
WEEK 21 DAY 141	x (x.x)	x (x.x)	x (x.x)
WEEK 25 DAY 169	x (x.x)	x (x.x)	x (x.x)
WEEK 29 DAY 197	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.1.3 Patient Disposition by Country and Study Site (ITT Population)

	Number of patients (%)		
	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Country xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)
Country xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)
Country xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)
Country xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
Site xx	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.1.4 Analysis Populations (ITT Population)

	Number of patients (%)		
	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
ITT/Safety Population – PRN Dose Level	x	x	x
400 mg QD	x (x.x)	x (x.x)	x (x.x)
400 mg QD	x (x.x)	x (x.x)	x (x.x)
600 mg BID	x (x.x)	x (x.x)	x (x.x)
mITT Population – PRN Dose Level	x (x.x)	x (x.x)	x (x.x)
400 mg QD	x (x.x)	x (x.x)	x (x.x)
400 mg QD	x (x.x)	x (x.x)	x (x.x)
600 mg BID	x (x.x)	x (x.x)	x (x.x)
Completer Population – PRN Dose Level	x (x.x)	x (x.x)	x (x.x)
400 mg QD	x (x.x)	x (x.x)	x (x.x)
400 mg BID	x (x.x)	x (x.x)	x (x.x)
600 mg BID	x (x.x)	x (x.x)	x (x.x)
PP Population – PRN Dose Level	x (x.x)	x (x.x)	x (x.x)
400 mg QD	x (x.x)	x (x.x)	x (x.x)
400 mg BID	x (x.x)	x (x.x)	x (x.x)
600 mg BID	x (x.x)	x (x.x)	x (x.x)

ITT/Safety population – Had at least one dose of PRN1008
mITT population – Treated up to Week 5 and had a Week 5 CDA assessment
Completer population – Treated up to Week 25 andhad a Week 29 CDA assessment
PP population – Had no major protocol deviations, completed Week 13/Day 85 visit and had at least 80% study drug compliance
Percentages are based on the number of patients in each starting dose and overall.

Program: <program name (date, time)>, Path: <path>

Table B.14.1.2.1 Demographics and Baseline Characteristics (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Age (years)			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x
Gender n (%)			
Male	x (x.x)	x (x.x)	x (x.x)
Female	x (x.x)	x (x.x)	x (x.x)
If Female – Fertility Status n (%)			
Sterile	x (x.x)	x (x.x)	x (x.x)
Post-Menopausal	x (x.x)	x (x.x)	x (x.x)
Potentially Able to Bear Children	x (x.x)	x (x.x)	x (x.x)
If Female – Method of birth control n (%)			
Effective Methods of Contraception	x (x.x)	x (x.x)	x (x.x)
Post-menopausal	x (x.x)	x (x.x)	x (x.x)
Surgically sterilized	x (x.x)	x (x.x)	x (x.x)
Complete abstinence	x (x.x)	x (x.x)	x (x.x)
Race n (%)			
American Indian or Alaska Native	x (x.x)	x (x.x)	x (x.x)

Table B.14.1.2.1 Demographics and Baseline Characteristics (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Asian	x (x.x)	x (x.x)	x (x.x)
Black or African American	x (x.x)	x (x.x)	x (x.x)
Native Hawaiian or Other Pacific Islander	x (x.x)	x (x.x)	x (x.x)
White	x (x.x)	x (x.x)	x (x.x)
Other	x (x.x)	x (x.x)	x (x.x)
Not Reported	x (x.x)	x (x.x)	x (x.x)
Ethnicity n (%)			
Hispanic or Latino	x (x.x)	x (x.x)	x (x.x)
Not Hispanic or Latino	x (x.x)	x (x.x)	x (x.x)
Not Reported	x (x.x)	x (x.x)	x (x.x)
Height (cm) at Screening			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x
Weight (kg) at Screening			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x

Table B.14.1.2.1 Demographics and Baseline Characteristics (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Body Mass Index (BMI) (kg/m^2)			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x

Percentages are based on the number of patients in each starting dose and overall.
Calculated Body Mass Index (BMI) = (Weight (kg) at Screening)/(Height (m) at Screening)^2
Program: <program name (date, time)>, Path: <path>

Table B.14.1.3.1 Disease History (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Pemphigus subtype at Week 1 Day 1 n (%)			
Pemphigus Vulgaris	x (x.x)	x (x.x)	x (x.x)
Bullous Pemphigoid	x (x.x)	x (x.x)	x (x.x)
Pemphigus Follaceus	x (x.x)	x (x.x)	x (x.x)
Epidermolysis Bullosa Aquisita	x (x.x)	x (x.x)	x (x.x)
Linear IgA Bullous Dermatoses	x (x.x)	x (x.x)	x (x.x)
Mucous Membrane Pemphigoid	x (x.x)	x (x.x)	x (x.x)
Other	x (x.x)	x (x.x)	x (x.x)
Time from Pemphigus Diagnosis [1] (years)			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x
Pemphigus history type [2] n (%)			
Newly Diagnosed	x (x.x)	x (x.x)	x (x.x)
Relapsed	x (x.x)	x (x.x)	x (x.x)
Anti-DSG status [3] n (%)			
Anti-DSG 1 positive, Anti-DSG 3 negative	x (x.x)	x (x.x)	x (x.x)
Anti-DSG 1 negative, Anti-DSG 3 positive	x (x.x)	x (x.x)	x (x.x)
Anti-DSG 1 positive, Anti-DSG 3 positive	x (x.x)	x (x.x)	x (x.x)

Table B.14.1.3.1 Disease History (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Anti-DSG 1 negative, Anti-DSG 3 negative	x (x.x)	x (x.x)	x (x.x)
Total Anti-DSG [3][4]			
< 100 unit	x (x.x)	x (x.x)	x (x.x)
≥ 100 unit	x (x.x)	x (x.x)	x (x.x)
PDAI total activity score [3]			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x
Pemphigus Severity [3]			
PDAI < 15 (mild-moderate)	x (x.x)	x (x.x)	x (x.x)
PDAI ≥ 15 (moderate-severe)	x (x.x)	x (x.x)	x (x.x)
PDAI Skin Activity > 0 n (%) [3]	x (x.x)	x (x.x)	x (x.x)
PDAI Scalp Activity > 0 n (%)[3]	x (x.x)	x (x.x)	x (x.x)
PDAI Mucous Membrane Activity > 0 n (%)[3]	x (x.x)	x (x.x)	x (x.x)
ABSIS Oral Involvement > 0 n (%) [3]	x (x.x)	x (x.x)	x (x.x)

[1] Based on date of first pemphigus reported in Medical History and date of screening
[2] Newly diagnosed is defined as subjects who were diagnosed at most 180 days prior to date of screening, otherwise the subject is relapsed.
[3] Result at Week 1 Day 1. If Week 1 Day 1 is missing, then the Screening result is used.
[4] Total Anti-DSG is the sum of Anti-DSG 1 and Anti-DSG3, where at least one of Anti-DSG 1 or 3 is positive, i.e. Anti-DSG 1 ≥ 14 and/or Anti-DSG 3 ≥ 9.

Percentages are based on the number of patients in each starting dose and overall.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.4.1 Medical History (ITT Population)

	Number of patients (%)		
Body System Code	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Number of patients reporting any medical history	x (x.x)	x (x.x)	x (x.x)
Skin	x (x.x)	x (x.x)	x (x.x)
Head, Eyes, Ears, Nose, Throat	x (x.x)	x (x.x)	x (x.x)
Respiratory	x (x.x)	x (x.x)	x (x.x)
Cardiovascular	x (x.x)	x (x.x)	x (x.x)
Gastrointestinal	x (x.x)	x (x.x)	x (x.x)
Endocrine/Metabolic	x (x.x)	x (x.x)	x (x.x)
Genitourinary	x (x.x)	x (x.x)	x (x.x)
Neurological	x (x.x)	x (x.x)	x (x.x)
Blood/Lymphatic	x (x.x)	x (x.x)	x (x.x)
Musculoskeletal	x (x.x)	x (x.x)	x (x.x)
Hepatic	x (x.x)	x (x.x)	x (x.x)
Allergies	x (x.x)	x (x.x)	x (x.x)
Psychological/Psychiatric	x (x.x)	x (x.x)	x (x.x)
Other	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
If a medical history event of the same Body System Code is reported multiple times for a patient, it is counted only once.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.5.1 Major Protocol Deviations (ITT Population)

	Number of patients (%)		
Protocol Deviation Categories	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Number of patients reporting major protocol deviations	x (x.x)	x (x.x)	x (x.x)
Category 1	x (x.x)	x (x.x)	x (x.x)
Category 2	x (x.x)	x (x.x)	x (x.x)
Category 3	x (x.x)	x (x.x)	x (x.x)
Category 4	x (x.x)	x (x.x)	x (x.x)
Category 5	x (x.x)	x (x.x)	x (x.x)
Category 6	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
If the same protocol deviation category is reported multiple times for a patient, it is counted only once.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.6.1 Prior Corticosteroid Medications (ITT Population)

	Number of patients (%)		
ATC Level 2 Class Preferred Name	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Number of patients reporting prior corticosteroid medications	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.3	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.3	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
Prior corticosteroid medications are those corticosteroid mediations that have stopped prior to the first dose of study drug.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.6.2 Concomitant Corticosteroid Medications (ITT Population)

	Number of patients (%)			
ATC Level 2 Class Preferred Name	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Number of patients reporting concomitant corticosteroid medications	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.3	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.3	x (x.x)	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)	x (x.x)

Patients are classified into different dose levels according to the dose they received during the time the medication was taken.
Percentages are based on the number of patients in each dose group and overall.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
This table includes concomitant corticosteroid medications that are ongoing or stopped on or after the date of first dose of study drug and started prior to date of last dose of study drug.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.6.3 Concomitant Corticosteroid Medications Started Post Last Dose (ITT Population)

	Number of patients (%)
ATC Level 2 Class Preferred Name	Overall (N = n)
Number of patients reported post last dose corticosteroid medications	x (x.x)
ATC Level 2 Class 1	x (x.x)
Preferred Name 1.1	x (x.x)
Preferred Name 1.2	x (x.x)
Preferred Name 1.3	x (x.x)
ATC Level 2 Class 2	x (x.x)
Preferred Name 2.1	x (x.x)
Preferred Name 2.2	x (x.x)
Preferred Name 2.3	x (x.x)
…etc…	x (x.x)

Percentages are based on the number of patients in the ITT population.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
This table includes corticosteroid medications started after the date of last dose of study drug.
Program: <program name (date, time)>, Path: <path>

Programming note for "Post Last Dose" column: N for Post Last dose column is the number of patients reported a Date of Last Dose.

Table B.14.1.7.1 Prior Medications other than Corticosteroid Medications (ITT Population)

	Number of patients (%)		
ATC Level 2 Class Preferred Name	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Number of patients reported prior medications other than corticosteroid medications	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.3	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.1	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.2	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.3	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)

Percentages are based on the number of patients in each starting dose and overall.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
Prior medications are those non-corticosteroid mediations that have stopped prior to the first dose of study drug.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.7.2 Concomitant Medications other than Corticosteroid Medications (ITT Population)

	Number of patients (%)			
ATC Level 2 Class Preferred Name	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Number of patients reported concomitant medications other than corticosteroid medications	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 1.3	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ATC Level 2 Class 2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.1	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.2	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Preferred Name 2.3	x (x.x)	x (x.x)	x (x.x)	x (x.x)
…etc…	x (x.x)	x (x.x)	x (x.x)	x (x.x)

Patients are classified into different dose levels according to the dose they received during the time the medication was taken.
Percentages are based on the number of patients in each dose group and overall.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
Concomitant medications are those non- corticosteroid mediations that are ongoing or stopped on or after the date of first dose of study drug.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.7.3 Concomitant Medications other than Corticosteroid Medications Started Post Last Dose (ITT Population)

	Number of patients (%)
ATC Level 2 Class Preferred Name	Overall (N = n)
Number of patients reported post last dose non-CS medications	x (x.x)
ATC Level 2 Class 1	x (x.x)
Preferred Name 1.1	x (x.x)
Preferred Name 1.2	x (x.x)
Preferred Name 1.3	x (x.x)
ATC Level 2 Class 2	x (x.x)
Preferred Name 2.1	x (x.x)
Preferred Name 2.2	x (x.x)
Preferred Name 2.3	x (x.x)
…etc…	x (x.x)

Percentages are based on the number of patients in the ITT population.
Medications are coded using WHODD, version 01SEP2015.
ATC Level 2 Class is sorted in descending order in the Overall group. Within each ATC Level 2 Class, Preferred Name is sorted in descending order in the Overall group.
If a medication of the same ATC Level 2 Class and Preferred Name is reported multiple times for a patient, it is counted only once.
This table includes corticosteroid medications started after the date of last dose of study drug.
Program: <program name (date, time)>, Path: <path>

Table B.14.1.8.1 Number of Dose Adjustments and Maximum Dose (ITT Population)

	Starting Dose 400 mg QD (N = n)	Starting Dose 400 mg BID (N = n)	Overall (N = n)
Number of Dose Adjustments			
n	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x
Maximum Dose			
400 mg QD	x (x.x)	x (x.x)	x (x.x)
400 mg BID	x (x.x)	x (x.x)	x (x.x)
600 mg BID	x (x.x)	x (x.x)	x (x.x)

Program: <program name (date, time)>, Path: <path>

Table B.14.1.8.2 Study Drug Exposure and Compliance (ITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Duration of Exposure (day)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
Calculated Dose (mg/day)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
Compliance (%)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
Compliance (%) n (%)				
100 %	x (x.x)	x (x.x)	x (x.x)	x (x.x)
>=80 % and < 100 %	x (x.x)	x (x.x)	x (x.x)	x (x.x)
< 80%	x (x.x)	x (x.x)	x (x.x)	x (x.x)

The duration of exposure at each dose level is the date of last dose in a given dose level – date of first dose in the same dose level + 1.
The duration of exposure for "Overall" is date of last dose – date of first dose + 1.

Calculated dose in mg/day at each dose level = [Dose level (mg/day) x (Duration of exposure (x2 if BID) – doses missed)]/Duration of Exposure.
Compliance with study drug = the actual number of doses taken divided by the expected number of doses take, expressed as a percentage.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.1.1.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg (mITT Population)

Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CDA.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Primary efficacy endpoint: Proportion of patients who achieved CDA within 4 weeks of staring PRN1008 treatment, i.e. At or prior to Week 5, without prednis(ol)one equivalent > 0.5 mg/kg on the day pror to the assessment.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.1.1.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg (Completer Population)
Table B.14.2.1.1.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg (PP Population)

Table B.14.2.1.2.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Age (mITT Population)

Subgroup	Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Age < 50	Number of patients	n	n	n	n
	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
Age ≥ 50	Number of patients	n	n	n	n
	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx

Percentages are based on the number of patients in each subgroup, dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CDA.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Primary efficacy endpoint: Proportion of patients who achieved CDA within 4 weeks of staring PRN1008 treatment, i.e. At or prior to Week 5, without prednis(ol)one equivalent > 0.5 mg/kg on the day pror to the assessment..
Program: <program name (date, time)>, Path: <path>

Table B.14.2.1.2.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Age (Completer Population)
Table B.14.2.1.2.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Age (PP Population)
Note: Subgroups are: 'Age < 50 years' and 'Age ≥ 50 years'

Table B.14.2.1.3.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Gender (mITT Population)
Table B.14.2.1.3.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Gender (Completer Population)
Table B.14.2.1.3.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Gender (PP Population)
Note: Subgroups are: 'Male' and 'Female'

Table B.14.2.1.4.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Total AntiDSG (mITT Population)
Table B.14.2.1.4. Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Total Anti-DSG (Completer Population)
Table B.14.2.1.4.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Total Anti-DSG (PP Population)

Note: Subgroups are: 'Baseline Total DSG antibody ≥ 100 units' and 'Baseline Total DSG antibody < 100 units'

Table B.14.2.1.5.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Pemphigus History (mITT Population)
Table B.14.2.1.5.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Pemphigus History (Completer Population)
Table B.14.2.1.5.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Pemphigus History (PP Population)

Note: Subgroups are: 'Newly diagnosed (≤ 6 months from screening)', 'Relapsed (>6 months from screening)
Additional footnote: Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.

Table B.14.2.1.6.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Pemphigus Anti-DSG Profile (mITT Population)
Table B.14.2.1.6.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Pemphigus Anti-DSG Profile (Completer Population)
Table B.14.2.1.6.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup – Baseline Pemphigus Anti-DSG Profile (PP Population)

Note: Subgroups are: 'Phemphigus Vularis (PV)', 'Pemphigus foliaceus (PF)' and 'Negative anti-DSG profile'

Table B.14.2.1.7.1 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup Analysis – Baseline Pemphigus Severity (mITT Population)
Table B.14.2.1.7.2 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup Analysis – Baseline Pemphigus Severity (Completer Population)
Table B.14.2.1.7.3 Proportion of patients Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg – Subgroup Analysis – Baseline Pemphigus Severity (PP Population)

Note: Subgroups are: 'Mild at Baseline', 'Moderate at Baseline'
Additional footnote: Based on PDAI total activity score. For newly diagnosed patients: Mild is 0 to 14 and Moderate is 15 to 44. For relapsed patients: Mild is 0 to 8 and Moderate is 9 to 24.

Table B.14.2.2.1.1 Cumulative Incidence of Patients Achieved CDA by Visit – Cumulative Incidence (mITT Population)

Visit	Achieved CDA	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 1	Cumulative N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 2	Cumulative N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 3 DAY 15	…etc…				
…etc..					
WEEK 29 DAY 197	Cumulative N achieved (%)				x (x.x)
	- 80% CI				x.xx, x.xx
	- 95% CI				x.xx, x.xx

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CDA.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Secondary efficacy endpoint: Proportion of patients who achieved CDA at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.5.1 Proportion of patients Achieved CDA by Visit (mITT Population)

Visit	Achieved CDA	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 1	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 5					
…etc..					
WEEK 29 DAY 197					

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CDA.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Secondary efficacy endpoint: Proportion of patients who achieved CDA at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.5.2 Proportion of patients Achieved CDA by Visit (Completer Population)
Table B.14.2.2.5.3 Proportion of patients Achieved CDA by Visit (PP Population)

Table B.14.2.2.6.1 Proportion of patients Achieved CDA with prednis(ol)one >0.5 mg/kg by Visit (mITT Population)
Table B.14.2.2.6.2 Proportion of patients Achieved CDA with prednis(ol)one >0.5 mg/kg by Visit (Completer Population)
Table B.14.2.2.6.3 Proportion of patients Achieved CDA with prednis(ol)one >0.5 mg/kg by Visit (PP Population)

Table B.14.2.2.7.1 Proportion of patients Achieved CDA without prednis(ol)one >0.5 mg/kg by Visit (mITT Population)
Table B.14.2.2.7.2 Proportion of patients Achieved CDA without prednis(ol)one >0.5 mg/kg by Visit (Completer Population)
Table B.14.2.2.7.3 Proportion of patients Achieved CDA without prednis(ol)one >0.5 mg/kg by Visit (PP Population)

Table B.14.2.2.8.1 Cumulative Incidence of Patients Achieved CR by Visit – Cumulative Incidence (mITT Population)

Visit	Achieved CR	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 1	Cumulative N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 2	Cumulative N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 3 DAY 15	…etc…				
…etc..					
WEEK 29 DAY 197	Cumulative N achieved (%)				x (x.x)
	- 80% CI				x.xx, x.xx
	- 95% CI				x.xx, x.xx

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CR.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Secondary efficacy endpoint: Proportion of patients who achieved CDA at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.8.2 Cumulative Incidence of Patients Achieved CR by Visit – Cumulative Incidence (Completer Population)
Table B.14.2.2.8.3 Cumulative Incidence of Patients Achieved CR by Visit – Cumulative Incidence (PP Population)

Table B.14.2.2.9.1 Cumulative Incidence of Patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (mITT Population)
Table B.14.2.2.9.2 Cumulative Incidence of Patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (Completer Population)
Table B.14.2.2.9.3 Cumulative Incidence of Patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (PP Population)

Table B.14.2.2.10.1 Cumulative Incidence of Patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (mITT Population)
Table B.14.2.2.10.2 Cumulative Incidence of Patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (Completer Population)
Table B.14.2.2.10.3 Cumulative Incidence of Patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit – Cumulative Incidence (PP Population)

Table B.14.2.2.11.1 Proportion of patients Achieved CR by Visit (mITT Population)

Visit	Achieved CR	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 1	N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
	- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
WEEK 1 DAY 2					
…etc..					
WEEK 29 DAY 197					

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CDA.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Secondary efficacy endpoint: Proportion of patients who achieved CDA at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.11.2 Proportion of patients Achieved CR by Visit (Completer Population)
Table B.14.2.2.11.3 Proportion of patients Achieved CR by Visit (PP Population)

Table B.14.2.2.12.1 Proportion of patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit (mITT Population)
Table B.14.2.2.12.2 Proportion of patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit (Completer Population)
Table B.14.2.2.12.3 Proportion of patients Achieved CR with prednis(ol)one >0.5 mg/kg by Visit (PP Population)

Table B.14.2.2.13.1 Proportion of patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit (mITT Population)
Table B.14.2.2.13.2 Proportion of patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit (Completer Population)
Table B.14.2.2.13.3 Proportion of patients Achieved CR without prednis(ol)one >0.5 mg/kg by Visit (PP Population)

Table B.14.2.2.14.1 Proportion of patients Achieved CR at or Prior to Week 25 with prednis(ol)one >0.5 mg/kg (mITT Population)

Achieved CDA at or Prior to Week 5 without prednis(ol)one >0.5 mg/kg	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
N achieved (%)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
- 80% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx
- 95% CI	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx	x.xx, x.xx

Percentages are based on the number of patients in each dose group and overall.
Patients are classified into different dose levels according to the dose they received prior to achieving CR.
Confidence Intervals are based on the Exact (Clopper-Pearson) method.
Primary efficacy endpoint: Proportion of patients who achieved CR within 24 weeks of staring PRN1008 treatment, i.e. At or prior to Week 5, without prednis(ol)one equivalent > 0.5 mg/kg on the day pror to the assessment.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.14.2 Proportion of patients Achieved CR at or Prior to Week 25 with prednis(ol)one >0.5 mg/kg (Completer Population)
Table B.14.2.2.14.3 Proportion of patients Achieved CR at or Prior to Week 25 with prednis(ol)one >0.5 mg/kg (PP Population)

Table B.14.2.2.15.1 Proportion of patients Achieved CR at or Prior to Week 25 without prednis(ol)one >0.5 mg/kg (mITT Population)
Table B.14.2.2.15.2 Proportion of patients Achieved CR at or Prior to Week 25 without prednis(ol)one >0.5 mg/kg (Completer Population)
Table B.14.2.2.15.3 Proportion of patients Achieved CR at or Prior to Week 25 without prednis(ol)one >0.5 mg/kg (PP Population)

Table B.14.2.2.16.1 Duration of Complete Remission (CR) (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Duration of CR	x	x	x	x
n	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Mean (SD)	x.xx	x.xx	x.xx	x.xx
Median	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
Min, Max				

Duration of CR = Last Known CR Date – First Known CR Date + 1.
Patients are classified into different dose levels according to the dose they received prior to achieving CR.

Table B.14.2.2.16.2 Duration of Complete Remission (CR) (Completer Population)
Table B.14.2.2.16.3 Duration of Complete Remission (CR) (PP Population)

Table B.14.2.2.17.1 Time to First CDA: Kaplan Meier Estimates (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
Time to first CDA (days) [1]				
n	x	x	x	x
25% Quartile	x.x	x.x	x.x	x.x
- 80% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
- 95% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
Median	x.x	x.x	x.x	x.x
- 80% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
- 95% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
75% Quartile	x.x	x.x	x.x	x.x
- 80% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
- 95% CI	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)	(x.xx, x.xx)
Min, Max [2]	x , x	x , x	x , x*	x , x*
Kaplan-Meier estimates % (n) [3]				
15 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
29 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
57 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
85 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
113 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
141 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)
169 Days	x.x (xx)	x.x (xx)	x.x (xx)	x.x (xx)

Patients are classified into different dose levels according to the dose they received at a given visit.

[1] Time to first CDA in days will be calculated as: Date of first occurrence of CDA – Date of first study drug dosing of the dose level where the CDA is observed + 1.
Patients who did not achieve CDA are censored at the last day in a dose level or date of study completion/discontinuation. 80% and 95% confidence intervals are calculated using the LOGLOG transformation.

[2] * indicates that this value is from a censored data.

[3] Kaplan-Meier estimate of the probability of experiencing the event on or before the specified timepoint (n = number of patients remaining at risk beyond the specified timepoint.)
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.17.2 Time to First CDA: Kaplan Meier Estimates (Completer Population)
Table B.14.2.2.17.3 Time to First CDA: Kaplan Meier Estimates (PP Population)

Table B.14.2.2.18.1 Time to First CR: Kaplan Meier Estimates (mITT Population)
Table B.14.2.2.18.2 Time to First CR: Kaplan Meier Estimates (Completer Population)
Table B.14.2.2.18.3 Time to First CR: Kaplan Meier Estimates (PP Population)

Table B.14.2.2.19.1 Time to First ECP: Kaplan Meier Estimates (mITT Population)
Table B.14.2.2.19.2 Time to First ECP: Kaplan Meier Estimates (Completer Population)
Table B.14.2.2.19.3 Time to First ECP: Kaplan Meier Estimates (PP Population)

Table B.14.2.2.20.1 Time to First Relapse: Kaplan Meier Estimates (mITT Population)
Table B.14.2.2.20.2 Time to First Relapse: Kaplan Meier Estimates (Completer Population)
Table B.14.2.2.20.3 Time to First Relapse: Kaplan Meier Estimates (PP Population)

Table B.14.2.2.21.1 Time to First Increase in PDAI: Kaplan Meier Estimates (mITT Population)
Table B.14.2.2.21.2 Time to First Increase in PDAI: Kaplan Meier Estimates (Completer Population)
Table B.14.2.2.21.3 Time to First Increase in PDAI: Kaplan Meier Estimates (PP Population)

Table B.14.2.2.22.1 Cumulative and Daily Average Corticosteroid Usage by Treatment Period (mITT Population)

	Overall (N = n)	
Period	Cumulative CS Usage (mg)	Daily Average CS Usage (mg/day)
Treatment Period 1 (Up to Week 12) [1]		
n	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x
Treatment Period 2 (from Week 13 to Week 24) [2]		
…etc..		
Entire Treatment Period (from Week 1 to Week 24) [3]		
…etc..		
Follow Up Period [2]		
…etc..		

All corticosteroids were converted into prednisolone. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.
[1] Period Start Date = Week 1 Day 1. Period End Date = Date of Week 13 Visit -1 or Date of Last Study Drug if patient discontinued prior to Week 13.
[2] Period Start Date = Date of Week 13 Visit; Period End Date = Date of Week 24 Visit – 1 or Date of Last Study Drug if patient discontinued prior to Week 24.
[3] Period Start Date = Week 1 Day 1; Period End Date = Date of Week 24 Visit – 1 or Date of Last Study Drug if patient discontinued prior to Week 24..
[4] Period Start Date = Date of Last Study Drug + 1. Period End Date = Date of Week 29/End of Study.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.22.2 Cumulative and Daily Average Corticosteroid Usage by Treatment Period – (Completer Population)
Table B.14.2.2.22.3 Cumulative and Daily Average Corticosteroid Usage by Treatment Period – (PP Population)

Table B.14.2.2.23.1 Corticosteroid Dose (mg/day) on the Day or at the Day Prior to Visit by Study Visit – (mITT Population)

Study Visit	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED (on the Day of)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 1 (On the Day of) - BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 1 (On the Day of) - CHANGE FROM SCREENED				
WEEK 3 DAY 15 (the day prior)				
WEEK 3 DAY 15 (the day prior) - CHANGE FROM SCREENED				
WEEK 3 DAY 15 (the day prior) - CHANGE FROM BASELINE				
..etc..				

All corticosteroids were converted into prednisolone. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.
For SCREENED and WEEK 1 DAY1 (BASELIEN): The dosage level on the day of the visit date.
For the post-baseline visit: The dosage level at each visit is defined as the dose level the patient is on the day before the visit date.
Week 1 Day 2 and unscheduled assessments are not included.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.22.2 Corticosteroid Dose (mg/day) on the Day or at the Day Prior by Study Visit – (Completer Population)
Table B.14.2.2.22.3 Corticosteroid Dose (mg/day) on the Day or at the Day Prior by Study Visit – (PP Population)

Table B.14.2.2.24.1 Corticosteroid Dose (mg/kg/day) on the Day or at the Day Prior by Study Visit – (mITT Population)
Table B.14.2.2.24.2 Corticosteroid Dose (mg/kg/day) on the Day or at the Day Prior by Study Visit – (Completer Population)
Table B.14.2.2.24.3 Corticosteroid Dose (mg/kg/day) on the Day or at the Day Prior by Study Visit – (PP Population)

Table B.14.2.2.25.1 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CDA – (mITT Population)

Study Visit	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 3 DAY 15 (the day prior)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 (the day prior) - Change from Baseline				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 5 DAY 29				
WEEK 5 DAY 29 – Cshange from Baseline				

All corticosteroids were converted into prednisolone. Each 10 mg of prednisolone is equivalent to 10 mg of prednisone, 8 mg of methylprednisolone, 1.5 mg dexamethasone, 40 mg hydrocortisone or 1.5 mg betamethasone.
The dosage level at each visit is defined as the dose level the patient is on the day before the CDA reponse visit date.
Week 1 Day 2 and unscheduled assessments are not included.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.25.2 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CDA – (Completer Population)
Table B.14.2.2.25.3 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CDA – (PP Population)

Table B.14.2.2.26.1 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CDA – (mITT Population)
Table B.14.2.2.26.2 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CDA – (Completer Population)
Table B.14.2.2.26.3 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CDA – (PP Population

Table B.14.2.2.27.1 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CR – (mITT Population)
Table B.14.2.2.27.2 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CR – (Completer Population)
Table B.14.2.2.27.3 Corticosteroid Dose (mg/day) by Study Visit in Patients who achieved at least one CR – (PP Population)

Table B.14.2.2.28.1 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CR – (mITT Population)
Table B.14.2.2.28.2 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CR – (Completer Population)
Table B.14.2.2.28.3 Corticosteroid Dose (mg/kg/day) by Study Visit in Patients who achieved at least one CR – (PP Population)

Table B.14.2.2.29.1 PDAI – Total Activity Score – Actual Values, Change and Percent Change from Screening and from Baseline (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 1 (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 – CHANGE FROM SCREENED				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x

Table B.14.2.2.29.1 PDAI – Total Activity Score – Actual Values, Change and Percent Change from Screening and from Baseline (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 3 DAY 15 – PERCENT CHANGE FROM SCREENED				
WEEK 3 DAY 15 – CHANGE FROM BASELINE				
WEEK 3 DAY 15 – PERCENT CHANGE FROM BASELINE				
…etc..				

Patients are classified into different dose levels according to the dose they received at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.29.2 PDAI – Total Activity Score – Actual Values, Change and Percent Change from Screening and from Baseline (Completer Population)
Table B.14.2.2.29.3 PDAI – Total Activity Score – Actual Values, Change and Percent Change from Screening and from Baseline (PP Population)

Table B.14.2.2.30.1 PDAI – Total Damage Score – Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.30.2 PDAI – Total Damage Score – Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.30.3 PDAI – Total Damage Score – Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.31.1 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 1 DAY 1 (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 – CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 5 DAY 29				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x

Table B.14.2.2.31.1 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline (mITT Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 5 DAY 29 – CHANGE FROM BASELINE				
…etc..				

Patients are classified into different dose levels according to the dose they received at each visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.2.2.31.2 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.29.3 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.32.1 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.32.2 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.32.3 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.33.1 ABSIS – Severity Total Score –Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.33.2 ABSIS – Severity Total Score –Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.33.3 ABSIS – Severity Total Score –Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.34.1 ABSIS – Total Activity Score –Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.34.2 ABSIS – Total Activity Score –Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.34.3 ABSIS – Total Activity Score –Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.35.1 ABSIS – Total Score –Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.35.2 ABSIS – Total Score –Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.35.3 ABSIS – Total Score –Actual Values and Change from Baseline (PP Population)

Note: For Tables B.14.2.2.36.1 to B.14.2.2.45.3, Baseline Oral Involvement = 'Baseline Oral Involvement = 0' vs 'Baseline Oral Involvement > 0', start each subgroup on a new page.

Table B.14.2.2.36.1 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Oral Involvement (mITT Population)
Table B.14.2.2.36.2 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Oral Involvement (Completer Population)
Table B.14.2.2.36.3 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Oral Involvement (PP Population)

Table B.14.2.2.37.1 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (mITT Population)
Table B.14.2.2.37.2 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (Completer Population)
Table B.14.2.2.37.3 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (PP Population)

Table B.14.2.2.38.1 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Oral Involvement (mITT Population)
Table B.14.2.2.38.2 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Oral Involvement (Completer Population)
Table B.14.2.2.38.3 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Oral Involvement (PP Population)

Table B.14.2.2.39.1 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (mITT Population)
Table B.14.2.2.39.2 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (Completer Population)
Table B.14.2.2.39.3 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (PP Population)

Table B.14.2.2.40.1 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (mITT Population)
Table B.14.2.2.40.2 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (Completer Population)
Table B.14.2.2.40.3 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Oral Involvement (PP Population)

Table B.14.2.2.41.1 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Skin Involvement (mITT Population)
Table B.14.2.2.41.2 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Skin Involvement (Completer Population)
Table B.14.2.2.41.3 ABSIS – Skin Involvement Total Score – Actual Values and Change from Baseline – by Baseline Skin Involvement (PP Population)

Table B.14.2.2.42.1 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (mITT Population)
Table B.14.2.2.42.2 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (Completer Population)
Table B.14.2.2.42.3 ABSIS – Oral Involvement Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (PP Population)

Table B.14.2.2.43.1 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Skin Involvement (mITT Population)
Table B.14.2.2.43.2 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Skin Involvement (Completer Population)
Table B.14.2.2.43.3 ABSIS – Severity Total Score –Actual Values and Change from Baseline– by Baseline Skin Involvement (PP Population)

Table B.14.2.2.44.1 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (mITT Population)
Table B.14.2.2.44.2 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (Completer Population)
Table B.14.2.2.44.3 ABSIS – Total Activity Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (PP Population)

Table B.14.2.2.45.1 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (mITT Population)
Table B.14.2.2.45.2 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (Completer Population)
Table B.14.2.2.45.3 ABSIS – Total Score –Actual Values and Change from Baseline – by Baseline Skin Involvement (PP Population)

Table B.14.2.2.46.1 ABQOL – Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.46.2 ABQOL – Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.46.3 ABQOL – Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.47.1 TABQOL – Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.47.2 TABQOL – Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.47.3 TABQOL – Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.48.1 SNAQ – Actual Values and Change from Baseline (mITT Population)
Table B.14.2.2.48.2 SNAQ – Actual Values and Change from Baseline (Completer Population)
Table B.14.2.2.48.3 SNAQ – Actual Values and Change from Baseline (PP Population)

Table B.14.2.2.49.1 Total Anti-DSG – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (mITT Population)

Group: All patients

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 1 DAY 1 (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 – CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 – PERCENT CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x

Table B.14.2.2.49.1 Total Anti-DSG – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (mITT Population)

Group: All patients

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
WEEK 5 DAY 29				
…etc..				

Patients are classified into different dose levels according to the dose they received at each visit.

Total Anti-DSG is the sum of Anti-DSG 1 and Anti-DSG 3 if at least one of them is positive (Anti-DSG 1 ≥ 14 and/or Anti-DSG 3 ≥ 9).
Program: <program name (date, time)>, Path: <path>

Repeat for "Group: Baseline < 100" units and "Group: Baseline ≥ 100 units"

Table B.14.2.2.49.2 Total Anti-DSG – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (Completer Population)
Table B.14.2.2.49.3 Total Anti-DSG – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (PP Population)

Table B.14.2.2.50.1 Anti-DSG 1 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (mITT Population)
Footnote: Anti-DSG 1 data above the limit of quantification, i.e. ≥ 14, are included.

Table B.14.2.2.50.2 Anti-DSG 1 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (Completer Population)
Table B.14.2.2.50.3 Anti-DSG 1 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (PP Population)

Table B.14.2.2.51.1 Anti-DSG 3 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (mITT Population)
Footnote: Anti-DSG 3 data above the limit of quantification, i.e. ≥ 9, are included.

Table B.14.2.2.51.2 Anti-DSG 3 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (Completer Population)
Table B.14.2.2.51.3 Anti-DSG 3 – Actual Values, Change and Percent Change from Baseline – Overall and by Baseline Value (PP Population)

Table B.14.3.1.1 Summary of Adverse Events (Safety Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Post Last Dose (N =n)	Overall (N = n)
Number of Pre-treatment AEs					x
Number (%) of patients with at least one Pre-treatment AE					x (x.x)
Number of Pre-treatment SAEs					x
Number (%) of patients with at least one Pre-treatment SAE					x (x.x)
Number of Treatment Emergent Adverse Events (TEAE)	x	x	x	x	x
Number (%) of patients with at least one TEAE	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Number of Related TEAEs	x	x	x	x	x
Number (%) of patients with at least one Related TEAE	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Number of Treatment Emergent SAEs	x	x	x	x	x
Number (%) of patients with at least one Treatment Emergent SAE	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Number of Related Treatment Emergent SAEs	x	x	x	x	x
Number (%) of patients with at least one Related Treatment Emergent SAE	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
TEAE - Action taken with study drug – Number (%) of patients*					
Dose not changed	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Dose reduced	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Study drug interrupted	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Study drug discontinued	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)

Table B.14.3.1.1 Summary of Adverse Events (Safety Population)

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Post Last Dose (N =n)	Overall (N = n)
Not applicable	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Unknown	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
TEAE - Maximum Intensity of TEAE – Number (%) of patients					
Grade 1	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Grade 2	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Grade 3	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Grade 4	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Grade 5	x (x.x)	x (x.x)	x (x.x)	x (x.x)	x (x.x)

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Pre-treatment adverse events are the events that started prior to the first dose.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Post last dose adverse events are the events started after the last dose of study drug.
* Patient is counted for each reported Action taken, therefore patient may be counted more than once.
Program: <program name (date, time)>, Path: <path>

Programming note for all AE tables with "Post Last Dose" column: N for Post Last dose column is the number of patients reported a Date of Last Dose.

Table B.14.3.1.2 Treatment Emergent Adverse Events by SOC and PT (Safety Population)

System Organ Class (SOC) / Preferred Term (PT)	400 mg QD (N = n)		400 mg BID (N = n)		600 mg BID (N = n)		Post Last Dose (N = n)		Overall (N = n)	
	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one TEAE	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.1.3 Related Treatment Emergent Adverse Events by SOC and PT (Safety Population)

System Organ Class (SOC)	400 mg QD (N = n)		400 mg BID (N = n)		600 mg BID (N = n)		Post Last Dose (N = n)		Overall (N = n)	
Preferred Term (PT)	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one related TEAE	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.1.4 Treatment Emergent Adverse Events by Maximum Intensity, SOC and PT (Safety Population)

	Overall (N = n)									
System Organ Class (SOC)	Grade 1		Grade 2		Grade 3		Grade 4		Grade 5	
Preferred Term (PT)	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one TEAE	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Patients with multiple events within a particular SOC or PT will be counted once under the maximum intensity grade.
Program: <program name (date, time)>, Path: <path>

Programming note: Repeat table for the 400 mg QD, 400 mg BID and 600 mg BID, Post Last Dose groups.

Table B.14.3.1.5 Related Treatment Emergent Adverse Events by Maximum Intensity, SOC and PT (Safety Population)

	Overall (N = n)									
System Organ Class (SOC)	Grade 1		Grade 2		Grade 3		Grade 4		Grade 5	
Preferred Term (PT)	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one related TEAE	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Patients with multiple events within a particular SOC or PT will be counted once under the maximum intensity grade.
Program: <program name (date, time)>, Path: <path>

Programming note: Repeat table for the 400 mg QD, 400 mg BID and 600 mg BID, Post Last Dose groups.

Table B.14.3.1.6 Serious Treatment Emergent Adverse Events by SOC and PT (Safety Population)

System Organ Class (SOC)	400 mg QD (N = n)		400 mg BID (N = n)		400 mg BID (N = n)		Post Last Doe (N = n)		Overall (N = n)	
Preferred Term (PT)	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one SAE	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Serious adverse events where PT='Pemphigus' are included in this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.1.7 Treatment Emergent Adverse Events Leading to Study Drug Discontinuation by SOC and PT (Safety Population)

System Organ Class (SOC)	400 mg QD (N = n)		400 mg BID (N = n)		600 mg BID (N = n)		Post Last Dose (N = n)		Overall (N = n)	
Preferred Term (PT)	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]	n (%)	[E]
Patients with at least one TEAE leading to study drug discontinuation	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT1.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
SOC2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.1	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.2	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
PT2.3	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x	x (x.x)	x
…etc…										

Patients are classified into different dose levels according to the dose they received at the start of AE.
Percentages are based on the number of patients in each dose group and overall.
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this table.
Adverse events are attributed to the dose level where the AE start date is on or after the start date of the dose level, but before the next dose level, if applicable.
Treatment emergent adverse events are the events started on or after the first dose of study drug and prior to the date of last dose of study drug.
Adverse events are sorted by overall descending frequency of SOC and within each SOC, by overall descending frequency of PT.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.2.1 Hematology – Actual Values and Change from Baseline by Study Visit (Safety Population)

Parameter = Parameter x [unit]

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK1 DAY 1 (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 3 DAY 15 – CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx

Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
… etc…				

Patients are classified into different dose levels according to the dose they received at a given visit.
BASELINE: If Week 1 Day 1 is missing, then screening value is used.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.2.2 Hematology – Shift from Baseline by Study Visit (Safety Population)

Parameter = Parameter x [unit]

	Overall (N = n)			
	WEEK DAY 1 (BASELINE)			
Post-baseline	N assessed	Low	Normal	High
WEEK 3 DAY 15	n	x (x.x)	x (x.x)	x (x.x)
Low	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
High	x (x.x)	x (x.x)	x (x.x)	x (x.x)
WEEK 5 DAY 29	n	x (x.x)	x (x.x)	x (x.x)
Low	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
High	x (x.x)	x (x.x)	x (x.x)	x (x.x)
… etc…				

BASELINE: If Week 1 Day 1 is missing, then screening value is used.
Program: <program name (date, time)>, Path: <path>

Programming notes:

1) ***Repeat table for the 400 mg QD, 400 mg BID and 600 mg BID groups.***
2) ***Highlight Normal to Low/High shifts in yellow. Do not display 0 (0.0)% for zero count for shift.***

Mock shells for Table B.14.3.2.1 to Table B.14.3.2.2 will be used for the following tables:

Table B.14.3.3.1 Coagulation – Actual Values and Change from Baseline by Study Visit (Safety Population)
Table B.14.3.3.2 Coagulation – Shift from Baseline by Study Visit (Safety Population)

Table B.14.3.4.1 Serum Chemistry – Actual Values and Change from Baseline by Study Visit (Safety Population)
Table B.14.3.4.2 Serum Chemistry – Shift from Baseline by Study Visit (Safety Population)

Table B.14.3.4.3 Serum Chemistry – Drug Induced Liver Injury (DILI) Criteria (Safety Population)

Patient who met the DILI criteria during treatment period	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
AST				
> 3x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 5x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 10x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 20x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ALT				
> 3x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 5x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 10x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 20x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
TBL				
> 1.5x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
> 2x ULN	x (x.x)	x (x.x)	x (x.x)	x (x.x)
ALP > 1.5x ULN	…etc..			
ALT or AST > 3x ULN and Total Bilirubin > 1.5x ULN	…etc..			
ALT or AST > 3x ULN and Total Bilirubin > 2x ULN	…etc..			

ULN = Upper Limit of Normal
Patients are classified into different dose levels according to the dose they received at the time a given DILI criteria was met.
Program: <program name (date, time)>, Path: <path>

Mock shells for Table B.14.3.2.1 to Table B.14.3.2.2 will be used for the following tables:

Table B.14.3.5.1 Urinalysis – Actual Values and Change from Baseline by Study Visit (Safety Population)
Table B.14.3.5.2 Urinalysis – Shift from Baseline by Study Visit (Safety Population)

Table B.14.3.6.1 Vital Signs and Body Weight – Actual Values and Change from Baseline by Study Visit (Safety Population)

Parameter = Parameter x [unit]

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 1 (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 2				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
WEEK 1 DAY 2 – CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x

… etc…

Patients are classified into different dose levels according to the dose they received at a given visit.
BASELINE: If Week 1 Day 1 is missing, then screening value is used.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.6.2 Vital Signs – BP and Pulse – Shift from Baseline by Study Visit (Safety Population)

Parameter = Parameter x [unit]

	Overall (N = n)			
	WEEK 1 DAY 1 (BASELINE)			
Post-baseline	N assessed	Low	Normal	High
WEEK 1 DAY 2	n	x (x.x)	x (x.x)	x (x.x)
Low	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
High	x (x.x)	x (x.x)	x (x.x)	x (x.x)
WEEK 3 DAY 15	n	x (x.x)	x (x.x)	x (x.x)
Low	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
High	x (x.x)	x (x.x)	x (x.x)	x (x.x)
… etc…				

Patients are classified into different dose levels according to the dose they received at a given visit.
BASELINE: If Week 1 Day 1 is missing, then screening value is used.
Program: <program name (date, time)>, Path: <path>

Programming notes:

- ***Repeat table for the 400 mg QD,400 mg BID and 600 mg BID groups.***
- ***Highlight Normal to Low/High shifts in yellow. Do not display 0 (0.0)% for zero count for shifts.***

Table B.14.3.7.1 12-Lead ECG Results – Actual Values and Change from Baseline (Safety Population)

Parameter = Parameter x [unit]

	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
SCREENED (BASELINE)				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
LAST VISIT*				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x
LAST VISIT* – CHANGE FROM BASELINE				
n	x	x	x	x
Mean (SD)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)	x.xx (x.xxx)
Median	x.xx	x.xx	x.xx	x.xx
Min, Max	x.x, x.x	x.x, x.x	x.x, x.x	x.x, x.x

Patients are classified into different dose levels according to the dose they received at a given visit.
*LAST VISIT is the last post-baseline visit.
Program: <program name (date, time)>, Path: <path>

Table B.14.3.8.3 12-Lead ECG – Overall Assessment – Shift from Baseline to Last Post-Baseline Visit (Safety Population)

Parameter = Parameter x [unit]

	Overall (N = n) SCREENED (BASELINE)			
Post-baseline	N assessed	Low	Normal	High
LAST VISIT	n	x (x.x)	x (x.x)	x (x.x)
Low	x (x.x)	x (x.x)	x (x.x)	x (x.x)
Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
High	x (x.x)	x (x.x)	x (x.x)	x (x.x)

Patients are classified into different dose levels according to the dose they received at a given visit.
Program: <program name (date, time)>, Path: <path>

Programming notes:

- ***Repeat table for the 400 mg QD, 400 mg BID and 600 mg BID.***
- ***Highlight Normal to Low/High shifts in yellow. Do not display 0 (0.0)% for zero count for shifts.***

Table B.14.3.9.1 Abbreviated Physical Examination by Study Visit (Safety Population)

Body System	Study Visit	N attended visit/ Result	400 mg QD (N = n)	400 mg BID (N = n)	600 mg BID (N = n)	Overall (N = n)
General Appearance	SCREENED	N attended visit	x	x	x	x
		Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal NCS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal CS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Not None	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	WEEK 1 DAY 1	N attended visit	x	x	x	x
		Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal NCS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal CS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Not None	x (x.x)	x (x.x)	x (x.x)	x (x.x)
	WEEK 3 DAY 15	N attended visit	x	x	x	x
		Normal	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal NCS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Abnormal CS	x (x.x)	x (x.x)	x (x.x)	x (x.x)
		Not None	x (x.x)	x (x.x)	x (x.x)	x (x.x)
…etc…						

The full physical examination was done at Screening visit. Abbreviated Physical Examination was consists of checking the normality or abnormality of the following body systems: general appearance, abdomen, cardiorespiratory and other was done at Day 1, Week 1 and at each visit from Day 15, Week 3 onward.
Patients are classified into different dose levels according to the dose they received at a given visit.
Percentages are based on the number of patients in each dose group and overall.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.1 Patient Disposition (Screening Population)

Starting Dose	Country	Patient ID	Age/ Gender	Date of First Dose (ddMMMyyyy)	Initial Dose	Maximum Dose	Completed Study?	Date of Completion/ Withdrawal (ddMMMyyyy)	Last Visit	Reason for Study Discontinuation
400 mg QD	xxx	xxxxxxx	xx/F	ddMMMyyyy	400 mg QD	400 mg BID	No	ddMMMyyyy	DXWX	xxxx
400 mg BID	xxx	xxxxxxx	xx/M	ddMMMyyyy	400 mg BID	600 mg BID	Yes	ddMMMyyyy		

F=Female; M=Male

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.2 Patient Enrollment (Screening Population)

									French sites only		
Starting Dose	Country	Patient ID	Age/Gender	Date and time of Informed Consent (ddMMMyyyy hh:mm 24hr)	Protocol Version	Does the subject response to the question for opting in or out allow for the use of their clinical data in future research?	Does the subject response to the question for opting in or out of having pictures taken for the purpose of the study and use by the sponsor in future research?	Is the patient consenting to enroll into A New Protocol Amendment?	Is the patient enrolling into a French site under French Protocol amendment V4.1-12 May 2017?	Is the patient consenting to enroll into a French site under A New Protocol Amendment?	If the patient is enrolling into a French site are additional MRI and Neurological forms required?
400 mg QD	xxx	xxxxxxx	xx/F	ddMMMyyyy hh:mm	Version x.x	Y/N	Y/N	Y/N	Y/N	Y/N	Y/N
400 mg BID	xxx	xxxxxxx	xx/M	ddMMMyyyy hh:mm	Version x.x	Y/N	Y/N	Y/N	Y/N	Y/N	Y/N

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.3 Screen Failure (All Screen Failures)

Country	Patient ID	Age/Gender	Did the subject meet all eligibility criteria?	Category of failed criteria	Inclusion/Exclusion Criterion Short Name
xxx	xxxxxxx	xx/F	No	Inclusion Criteria	INCxxx
xxx	xxxxxxx	xx/M	No	Exclusion Criteria	EXCxxx

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.4 Analysis Population (Screening Population)

				Analysis Populations					
Starting Dose	Country	Patient ID	Age/Gender	Screening	Safety	Efficacy Analysis (Intent-to-Treat) (ITT)	Modified Intent-to-Treat (mITT)	Completer	Per-Protocol (PP)
400 mg QD	xxx	xxxxxxx	xx/F	Yes	Yes	Yes	Yes	Yes	Yes
400 mg BID	xxx	xxxxxxx	xx/M	Yes	No	Yes	Yes	No	No

F=Female; M=Male
The Screening population consists of all patients who provided informed consent and have screening assessments in Part B evaluated for study participation.
The Safety population consists of all patients who have received at least one dose of PRN1008 in Part B.
The ITT population consists of all patients who have received at least one dose of PRN1008 in Part B.
The mITT population consists of all patients treated up to Week 5 and had a Week 5 disease assessment.
The Completer population consists of all patients treated up to Week 25and had a Week 29 disease assessment.
The PP population consists of all patients who have: 1) No major protocol deviations relevant to data integrity per Medical Monitor review; 2) At least 80% compliance based on drug accountability; and 3) Completed Week 29visit.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.5 Demographics and Baseline Characteristics (ITT Population)

						If female				At Screening		
Starting Dose	Country	Patient ID	Birth Date (ddMMMyyyy)	Age (years)	Sex	Fertility status	Method of birth control	Ethnicity	Race	Height (cm)	Weight (kg)	BMI (kg/m^2)
400 mg QD	xxx	xxxxxxx	xxxx	xx	Female	xxxx	Other - xxx	xxx	xxx	xxx	xx.x	xx.x
400 mg BID	xxx	xxxxxxx	xxxx	xx	Male			xxx	xxx	xxx	xx.x	xx.

Body Mass Index (BMI) = (Weight (kg) at Screening)/(Height (m) at Screening)^2
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.6 Disease History – Pemphigus history and Anti-DSG at Screening (ITT Population)

				Pemphigus Vulgaris			
Starting Dose	Patient ID	Age/Gender	Pemphigus subtype	Date Confirmed	Time to Screening (months)	Pemphigus history type [1]	Anti-DSG status
400 mg QD	xxxxxxx	xx/F	xxx	ddmmmyyyy	x.x months	Newly diagnosed / Relapsed	Anti-DSG 1 xxx, Anti-DSG 3 xxx

F=Female; M=Male
[1] Newly diagnosed is defined as subjects who were diagnosed at most 6 months prior to date of screening, otherwise the subject is relapsed.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.7 Disease History – Baseline PDAI and ABSIS Score (ITT Population)

Starting Dose	Patient ID	Age/Gender	Total Activity Score	Total Damage Score	Pemphigus Severity*	PDAI Skin Activity	PDAI Scalp Activity	PDAI Mucous Membrane Activity	ABSIS Oral Activity	ABSIS Skin Involvement	ABSIS Oral Involvement
400 mg QD	xxxxxxx	xx/F	xx	xx	xx	xx	xx	xx	xx	xx	xx

*Mild = PDAI Activity < 15; Moderate = PDAI Activity ≥ 15
F=Female; M=Male

Listing B.16.2.1.8 Medical History (ITT Population)

Starting Dose	Patient ID	Age/Gender	Any medical History to report?	Body System	Verbatim Term	Start Date (ddMMMyyyy)	End Date (ddMMMyyyy) /Ongoing
400 mg QD	xxxxxxx	xx/F	Yes	xxx	xxx	ddMMMyyyy	ddMMMyyyy
400 mg BID	xxxxxxx	xx/M	No				

F=Female; M=Male

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.9 Prior Corticosteroid Medications (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any Prior corticosteroid medication?	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:	Rescue criteria
400 mg QD	xxxxxxx	xx/F	Yes	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date ddMMMyyyy(Day x)	Indication: xxx AE or MH No: AE #1 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Not applicable

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
Prior corticosteroid medications are those corticosteroid mediations that have stopped prior to the first dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.10 Concomitant Corticosteroid Medications (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any corticosteroid medication?	PRN Dose Level at Medication Start	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:	Rescue criteria
400 mg QD	xxxxxxx	xx/F	Yes	400 mg QD	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: ddMMMyyyy (Day x)	Indication: xxx AE or MH No: AE #1 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Not applicable
				600 mg BID	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: Ongoing	Indication: xxx Treatment for an AE?: No Dose: xx mg Frequency: xxx Route: xxx	xxx

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
This table includes concomitant corticosteroid medications that are ongoing or stopped on or after the date of first dose of study drug and started prior to date of last dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.11 Concomitant Corticosteroid Medications Started Post Last Dose (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any Post Last Dose Corticosteroid med?	PRN Dose Level Prior to Last Dose of study drug	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:	Rescue criteria
400 mg QD	xxxxxxx	xx/F		600 mg BID	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: ddMMMyyyy (Day x)	Indication: xxx AE or MH No: AE #1 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Not applicable
				600 mg BID	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: Ongoing	Indication: xxx AE or MH No: MH #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	No

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
This table includes patients entered into follow up period and corticosteroid medications started after the date of last dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.11 Rescue Medications Taken During Study (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any corticosteroid medication?	PRN Dose Level at Medication Start	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:	Rescue criteria
400 mg QD	xxxxxxx	xx/F	Yes	400 mg QD	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day) Stop Date: ddMMMyyyy (Day x)	Indication: xxx AE or MH No: AE #1 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Rescue Criteria #1
400 mg BID	xxxxxxx	xx/M	Yes	600 mg BID	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: Ongoing	Indication: xxx AE or MH No: MH #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Rescue Criteria #2

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
This listing includes rescued corticosteroid medications reported as “Ongoing” or stopped on or after the date of first study drug dosing.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.12 Prior Medications other than Corticosteroid Medications (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any medication taken?	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:
400 mg QD	xxxxxxx	xx/F	Yes	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: ddMMMyyyy (Day x)	Indication: xxx AE or MH No: MH #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx
				R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: Ongoing	Indication: xxx AE or MH No: AE #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
Prior medications are those non-corticosteroid mediations that have stopped prior to the first dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.13 Concomitant Medications other than Corticosteroid Medications (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Any medication taken?	PRN Dose Level at Medication Start	Reported Medication (R) ATC Level 2 Class (Level 2) Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: Dose unit: Frequency Route:
400 mg QD	xxxxxxx	xx/F	Yes	400 mg QD	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: ddMMMyyyy (Day x)	Indication: xxx AE or MH No: MH #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx
				600 mg BID	R: xxx Level 2: xxx PT: xxxx	Start Date: ddMMMyyyy (Day x) Stop Date: Ongoing	Indication: xxx AE or MH No: AE #3 Dose: xx Dose unit: mg Frequency: xxx Route: xxx

F=Female; M=Male
Medications are coded using WHODD, version 01SEP2015.
Concomitant medications are those non-corticosteroid mediations that are ongoing or stopped on or after the date of first dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.1.14 Protocol Deviations (ITT Population)

Starting Dose	Patient ID	Age/Gender	Major or Minor	Protocol Deviation Category	Details
400 mg QD	xxxxxxx	xx/F	Minor	Visit Schedule	xxx
400 mg QD	xxxxxxx	xx/M	Major	Concomitant Medication	xxx

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.5.1 Study Drug Administration (ITT Population)

Starting Dose	Patient ID Age/ Gender	Visit	Dose Start Date and Time (ddMMMyyyy hh:mm 24hr)	Was dose admin on PK sample day?	Date and Time of current dose received (ddMMMyyyy hh:mm 24hr)	Dose: Route: Frequency	Dose Missed?	Date and Time Dose Missed (ddMMMyyyy hh:mm 24hr)	Reason Dose Missed	Dose Restarted?	Date Dose Restarted (ddMMMyyyy)	Reason if dose adjusted	Adjusted dose	Dose Stop date (ddMMMyyyy)
400 mg QD	xxxxxxx xx/F	Dx, Wx	ddMMMyyyy hh:mm	Yes or No	ddMMMyyyy hh:mm									
		Dx, Wx												
		Dx, Wx												
		Dx, Wx												

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.5.2 Study Drug Exposure and Compliance (ITT Population)

Starting Dose	Patient ID	Age/Gender	PRN Dose Level	Date of First Dose (ddMMMyyyy)	Date of Last Dose (ddMMMyyyy)	Duration of Exposure (day)	Number of Missed doses	Calculated dose (mg/day)	Compliance (%)
400 mg QD	xxxxxxx	xx/F	400 mg QD	ddMMMyyyy	ddMMMyyyy	x days	x	xxx mg/day	xxx %
			400 mg QD	ddMMMyyyy	ddMMMyyyy	x days	x	xxx mg/day	xxx %
			Overall	ddMMMyyyy	ddMMMyyyy	x days	x	xxx mg/day	xxx %

F=Female; M=Male
Calculated dose in mg/day at each dose level = [Dose level (mg/day) x (Duration of exposure (x2 if BID) – doses missed)]/Duration of Exposure.
Compliance with study drug = the actual number of doses taken divided by the expected number of doses take, expressed as a percentage.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.1 Disease Assessment (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Control of Disease Activity (CDA) Status Achieved at this visit If Yes, Date (ddMMMyyyy)	Complete Remission (CR) Status Achieved at this visit If Yes, Date (ddMMMyyyy)	End of Consolidated Phase Status Achieved at this visit If Yes, Date (ddMMMyyyy)	Relapse Status Achieved at this visit If Yes, Date (ddMMMyyyy)
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	No	No	No	No
			xxx	400 mg QD	Yes – ddMMMyyyy	No	No	No

F=Female; M=Male

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.2 Pemphigus Disease Area Index (PDAI) - Skin (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current Dose	Date if PDAI (ddMMMMyyyy)	Total Skin (/120) – Activity	Total Skin (/12) – Damage	Anatomical Location	Erosion /blister or new erythema	Number Lesion if <=3	Post-inflammatory hyperpigmentation or erythema from resolving lesions
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	xxxx	xx	xx	EARS	xx	xx	Absent
								NOSE	xx	xx	Absent
								REST OF THE FACE	xx	xx	Present
								…etc..			
	…etc..										

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.3 Pemphigus Disease Area Index (PDAI) - Scalp (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Date if PDAI (ddMMMMyyyy)	Total Scalp (/10) – Activity	Total Scalp (/1) – Damage	Anatomical Location	Erosion /blister or new erythema	Number Lesion if <=3	Post-inflammatory hyperpigmentation or erythema from resolving lesions
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	xxxx	xx	xx	SCALP	xx	xx	Absent
			xxx	400 mg QD	xxxx	xx	xx	SCALP	xx	xx	Absent
			xxx	400 mg QD	xxxx	xx	xx	SCALP	xx	xx	Present
	…etc..										

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.4 Pemphigus Disease Area Index (PDAI) – Mucous Membrane (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Date if PDAI (ddMMMMyyyy)	Total Mucosa (/120) – Activity	Anatomical Location	Erosion/blister	Number Lesion if <=3
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	xxxx	xx	EYES	xx	xx
							NOSE	xx	xx
							BUCCAL MUCOSA	xx	xx
	…etc..								

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.5 Pemphigus Disease Area Index (PDAI) – Total Scores (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	PDAI Total Activity Score	PDAI Total Damage Score	Total Skin (/120) – Activity	Total Skin (/12) – Damage	Total Scalp (/10) – Activity	Total Scalp (/1) – Damage	Total Mucosa (/120) – Activity
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	xx	xx	xx	xx	xx	xx	xx
			xxx	400 mg QD	xx	xx	xx	xx	xx	xx	xx
			xxx	400 mg QD	xx	xx	xx	xx	xx	xx	xx
	…etc..										

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.6 Autoimmune Bullous Skin Disorder Intensity Score (ABSIS) - Skin and Oral Involvement (ITT Population)

						Skin				Oral	
Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Date (ddMMMyyyy)	Patient's weight (unit)	Skin Involvement (Max BSA)	Patient's BSA	Weighting factor	Oral Involvement	Lesion Presences or Absence
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	ddMMMyyyy	xx	xx	xx	xx	xx	xx
			xxx	400 mg QD	xx	xx	xx	xx	xx	xx	xx
			xxx	400 mg QD	xx	xx	xx	xx	xx	xx	xx
	…etc..										

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.7 Treatment of Autoimmune Bullous Disease Quality of Life (TABQOL) (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Date completed (ddMMMyyyy)	Time take to finish the survey	Total Score	Question	Answer
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	ddMMMyyyy	x min x secs	xxx	1. As a result of your blistering disease treatment, do you notice your bruise or bleed easily	I notice this all the time
								2. As a result of your blistering disease treatment, can you still tolerate hot or cold temperatures	I have not had this problem
								3. xxx	xxxx
								…etc..	
	…etc..								

F=Female; M=Male

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.8 Simple Nutritional Appetite Questionnaire (SNAQ) (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Date completed (ddMMMyyyy)	Total Score	Question	Answer
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	ddMMMyyyy	xxx	My appetite is	Good
							When I eat	I hardly ever feel full
							xxx	xxxx
							…etc..	
	…etc..							

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.9 Corticosteroid (CS) Usage (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Total Prednisone dosage equivalent (mg/kg)	Weight (kg)	Total Prednisone dosage equivalent (mg)	Reported Medication (R) ATC Level 2 Class Preferred Name/Term (PT)	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) /Ongoing	Indication: AE or MH No.: Dose: (Pred. equiv. dose) Dose unit: Frequency Route:	Rescue criteria
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	xx.xx	xx.x	xx	xxx xxx xxx	ddMMMyyyy ddMMMyyyy	Indication: xxx AE or MH No: AE #1 Dose: 10 (12.5) Dose unit: mg Frequency: xxx Route: xxx	Not applicable
							xx	xxx xxx xxx	ddMMMyyyy Ongoing	Indication: xxx AE or MH No: AE #1 Dose: xx Dose unit: mg Frequency: xxx Route: xxx	Not applicable
							xx	xxx xxx xxx	ddMMMyyyy Ongoing	…etc…	

etc.

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.6.10 Corticosteroid (CS) Usage on the Day prior to each Visit and CDA/CR Response (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Previous PRN Dose	Prednisone dosage equivalent (mg/day)*	Prednisone dosage equivalent (mg/kg/day)*	CDA?	CR?
400 mg QD	xxxxxxx	xx/F	SCREENED	---	---	Xx	xx	No	No
			Week 1 DAY 1	400 mg QD	---	Xx	xx	No	No
			WEEK 3 DAY 15	400 mg BID	400 mg QD	Xx	xx	Yes	No
			etc.						

F=Female; M=Male
*For SCREENED and WEEK 1 DAY1: The dosage level on the day of the visit date. For the post-baseline visits: The dosage level at each visit is defined as the dose level the patient is on the day before the visit date.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.7.1 Pre-treatment Adverse Events (ITT Population)

Starting Dose	Patient ID	Age/ Gender	SOC: System Organ Class PT: Preferred Term V: Verbatim	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) or Ongoing Duration (days) (if not ongoing) Pattern of AE (Continuous or Intermittent)	SAE? (Y/N)	CTCAE Grade	Relationship to Study Drug	Details
400 mg QD	xxxxxxx	xx/F	SOC: xxx PT: xxx V: xxx	Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days Pattern of AE: xxx	Y	Grade x	xxxx	Action Taken to study drug: xxx Outcome: xxx Has the Cause of the AE been established: Y - xxx Caused study discontinuation?: N or Y Treated for this AE?: Y or N (AE description)
			SOC: xxx PT: xxx V: xxx	Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days Pattern of AE: xxx	N	Grade x	xxxx	Action Taken to study drug: xxx Outcome: xxx Has the Cause of the AE been established: Y - xxx Caused study discontinuation?: N or Y Treated for this AE?: Y or N (AE description)

F=Female; M=Male
Adverse Events were coded using MedDRA, Version 18.0.

Adverse events where PT='Pemphigus' are excluded from this listing.
Pre-treatment adverse events are the events that started prior to the first dose.
Study Day: If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Programming note: For all AE listings, sort by cohort, patient, start date, stop date, SOC and PT.

Same listing shell will be used for the following listing:

Listing B.16.2.7.2 Pre-treatment Serious Adverse Events (ITT Population)

Programming note: The following variables will be added to the "SAE" column for the SAE listing: Start Date (ddMMMyyyy) (Study Day), Stop Date (ddMMMyyyy) (Study Day) or Ongoing, Duration (if not ongoing), SAE Criteria

Listing B.16.2.7.3 Treatment Emergent Adverse Events (Safety Population)

Starting Dose	Patient ID	Age/ Gender	PRN Dose Level at AE start	SOC: System Organ Class PT: Preferred Term V: Verbatim	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) or Ongoing Duration (if not ongoing) Pattern of AE (Continuous or Intermittent)	SAE? (Y/N)	CTCAE Grade	Relationship to Study Drug	Details
400 mg QD	xxxxxxx	xx/F	400 mg QD	SOC: xxx PT: xxx V: xxx	Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days Pattern of AE: xxx	Y	Grade x	xxxx	Action Taken to study drug: xxx Outcome: xxx Has the Cause of the AE been established: Y - xxx Caused study discontinuation?: N or Y Treated for this AE?: Y or N (AE description)
				SOC: xxx PT: xxx V: xxx	Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days Pattern of AE: xxx	N	Grade x	xxxx	Action Taken to study drug: xxx Outcome: xxx Has the Cause of the AE been established: Y - xxx Caused study discontinuation?: N or Y Treated for this AE?: Y or N (AE description)

F=Female; M=Male
Adverse Events were coded using MedDRA, Version 18.0.
Adverse events where PT='Pemphigus' are excluded from this listing.
Treatment emergent adverse events are the events started on or after the first dose and prior to the date of last dose of study drug.
If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).
Program: <program name (date, time)>, Path: <path>

Programming note for all TEAE listings: Display "Post last dose" if AE started after date of last study drug dosing

Same listing shell will be used for the following listing:

Listing B.16.2.7.4 Related Treatment Emergent Adverse Events (Safety Population)
Listing B.16.2.7.5 Serious Adverse Events (Safety Population)

Programming note: The following variables will be added to the "SAE" column for the SAE listing: Start Date (ddMMMyyyy) (Study Day), Stop Date (ddMMMyyyy) (Study Day) or Ongoing, Duration (if not ongoing), SAE Criteria

Listing B.16.2.7.6 Treatment Emergent Adverse Events Leading to Study Drug Discontinuation (Safety Population)

Listing B.16.2.7.7 Serious Treatment Emergent Adverse Events Leading to Study Drug Discontinuation (Safety Population)

Starting Dose	Patient ID	Age/ Gender	PRN Dose Level at AE start	SOC: System Organ Class PT: Preferred Term V: Verbatim	Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) or Ongoing Duration (if not ongoing) Pattern of AE (Continuous or Intermittent)	SAE? (Y/N) If yes: Start Date (ddMMMyyyy) (Study Day) Stop Date (ddMMMyyyy) (Study Day) or Ongoing Duration (if not ongoing) SAE Criteria Details	CTCAE Grade	Relationship to Study Drug	Details
400 mg QD	xxxxxxx	xx/F	400 mg QD	SOC: xxx PT: xxx V: xxx	Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days Pattern: xxx	Y Start: ddMMMyyyy (Day X) Stop: ddMMMyyyy (Day X) Duration: x days SAE Criteria: xxx Hospitalization: Initial or Prolongation Admission Date: Discharge Date: Date of Death: Cause of Death: Autopsy performed?: Death certificate completed?:	Grade x	xxxx	Action Taken to study drug: xxx Outcome: xxx Has the Cause of the AE been established: Y - xxx Caused study discontinuation?: N or Y Treated for this AE?: Y or N (AE description)

	Description of SAE: Did the SAE abate after use of study treatment stopped? Did the SAE reoccur after reintroduction of study treatment? Interruption of study treatment: Date stopped: Date restarted: Type of Sequelae: PI rationale for "Not Related":

F=Female; M=Male

Adverse Events were coded using MedDRA, Version 18.0.

Serious Adverse events where PT='Pemphigus' are included in this listing.

Treatment emergent adverse events are the events started on or after the first dose and prior to the date of last dose of study drug.

If Date >= first dose date, then Study Day = (Date - Date of first dose +1). If Date < first dose date, then Study Day = (Date - Date of first dose).

Program: <program name (date, time)>, Path: <path>

Programming note for all SAE listing: Display 'Hospitalization' rows only if patient was hospitalized and display Death rows only If death is reported.

Shell for Listing B.16.2.7.3 will be used for the following listing:

Listing B.16.2.7.8 Treatment Emergent Adverse Events Leading to Death (Safety Population)
Listing B.16.2.7.9 Treatment Emergent Adverse Events Started Post Last Dose (Safety Population)
Listing B.16.2.7.10 Disease under Study Events (Safety Population)

Programming note: For Listings B.16.2.7.10 replace the footnote regarding 'Pemphigus' with the following footnote: Only include adverse events where PT='Pemphigus'.

Listing B.16.2.8.1 Hematology Results (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Parameter	Current PRN Dose	Visit	Date / Time of Sample (ddMMMyyyy / hh:mm 24 hr)	Lab Result	Unit	Normal Range	Low or High
400 mg QD	xxxxxxx	xx/F	xxxxx	---	xxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	L
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	H
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				…etc..						

F=Female; M=Male
Abnormal results are highlighted in yellow.
Program: <program name (date, time)>, Path: <path>

Programming note for all safety listings: Display "Post last dose" if sample date is after date of last study drug dosing.

Listing B.16.2.8.2 Hematology Results – Patients with Abnormal Results (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Parameter	Current PRN Dose	Visit	Date / Time of Sample (ddMMMyyyy / hh:mm 24 hr)	Lab Result	Unit	Normal Range	Low or High
400 mg QD	xxxxxxx	xx/F	xxxxx	---	xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	L
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	H
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
					xxxx	ddMMMyyyy / hh:mm	x.x	xx	x.x to x.x	
				…etc..						

F=Female; M=Male
Abnormal results are highlighted in yellow
Program: <program name (date, time)>, Path: <path>

Programming note: This listing excludes patients/parameters with normal results for all visits. Abnormal results and the Low/High flag will be highlighted in yellow.

Mock shells for Listing B.16.2.8.1 and Listing B.16.2.8.2 will be used for the following listings:

Listing B.16.2.8.3	**Coagulation Results (Safety Population)**
Listing B.16.2.8.4	**Coagulation Results – Patients with Abnormal Results (Safety Population)**
Listing B.16.2.8.5	**Serum Chemistry Results (Safety Population)**
Listing B.16.2.8.6	**Serum Chemistry Results – Patients with Abnormal Results (Safety Population)**
Listing B.16.2.8.7	**Urinalysis Results (Safety Population)**
Listing B.16.2.8.8	**Urinalysis Results – Patients with Abnormal Results (Safety Population)**
Listing B.16.2.8.9	**Drug Induced Liver Injury (DILI) Test Results (Safety Population)**
Listing B.16.2.8.10	**Drug Induced Liver Injury (DILI) Test Results – Patients with Abnormal Results (Safety Population)**

Listing B.16.2.8.11 Anti-DSG Antibodies Results (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Was the sample collected ?	Collection Date (ddMMMyyyy)	Collection Time (hh:mm 24h)	Lab Test	Result (unit)
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	Yes	ddMMMyyyy	Hh:mm	Anti-DSG 1	xxx unit
								Anti-DSG 3	Xxx unit
	…etc..								

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.12 PK and BTK Sample Collection (ITT Population)

Starting Dose	Patient ID	Age/ Gender	Visit	Current PRN Dose	Was the sample collected?	Collection Date (ddMMMyyyy)	Collection Timepoint	Collection Time (hh:mm 24h)	Was PK sample performed for this visit?	Date of most recent prior dose received (ddMMMyyyy)	Time of most recent prior dose received (Hh:mm 24h)
400 mg QD	xxxxxxx	xx/F	xxx	400 mg QD	Yes	ddMMMyyyy	xxx	Hh:mm	Yes	ddMMMyyyy	Hh:mm
	…etc..										

F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.13 Vital Signs and Body Weight (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Parameter	Current PRN Dose	Visit	Date / Time (ddMMMyyyy / hh:mm 24 hr)	Result	Normal Range	Low or High
400 mg QD	xxxxxxx	xx/F	SBP (mmHg)	---	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	L
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	H
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				…etc..					

F=Female; M=Male
Low or High results are highlighted in yellow.
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.14 Vital Signs – Blood Pressure and Pulse – Patient with Abnormal Results (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Parameter	Current PRN Dose	Visit	Date / Time (ddMMMyyyy / hh:mm 24 hr)	Result	Normal Range	Low or High
400 mg QD	xxxxxxx	xx/F	SBP (mmHg)	---	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	L
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				400 mg QD	xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	H
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
					xxxx	ddMMMyyyy / hh:mm	x.x	90 to 140	
				…etc..					

F=Female; M=Male
Low or High results are highlighted in yellow.
Program: <program name (date, time)>, Path: <path>

Programming note: This listing excludes patients/parameters with normal results for all visits. Abnormal results and the Low/High flag will be highlighted in yellow.

Listing B.16.2.8.15 12-Lead ECG Results (Safety Population)

Starting Dose	Patient ID	Age/Gender	Current PRN Dose	Visit	Assessment #	Date / Time (ddMMMyyyy / hh:mm 24 hr)	Heart Rate (bpm)	QT Interval (msec)	PR Interval (mesc)	RR Interval (msec)	QRS Duration (mesc)	QTcF Interval (msec)	ECG Assessment	If Abnormal, details AE #, if abnormal CS
400 mg QD	xxxxx xx	xx/F	---	Screening		ddMMMyyyy	x.x	x.x	x.x	x.x	x.x	x.x	Normal	
			400 mg QD	xxxx	#1	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal	
					#2	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal	
					#3	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Abn, NCS	
					Average/Worst finding		x.x	x.x	x.x	x.x	x.x	x.x	Normal	
					Additional	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal	
					…etc…									
					Average/Worst finding		x.x	x.x	x.x	x.x	x.x	x.x	Normal	
				xxxx	…etc..									

F=Female; M=Male

Abnormal NCS assessments are highlighted in yellow. Abnormal CS assessments are highlighted in pink.

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.16 12-Lead ECG Results – Patient with Abnormal Results (Safety Population)

Cohort	Patient ID	Age/Gender	Current PRN Dose	Visit	Assessment #	Date / Time (ddMMMyyyy / hh:mm 24 hr)	Heart Rate (bpm)	QT Interval (msec)	PR Interval (mesc)	RR Interval (msec)	QRS Duration (mesc)	QTcF Interval (msec)	ECG Assessment	If Abnormal, details	AE #, if abnormal CS
400 mg QD	xxxxx xx	xx/F	---	xxxx		ddMMMyyyy	x.x	x.x	x.x	x.x	x.x	x.x	Normal		
			400 mg QD	xxxx	#1	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal		
					#2	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Abn, NCS	xxx	x
					#3	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal		
					Average/Worst finding		x.x	x.x	x.x	x.x	x.x	x.x	Abn, CS	xxx	x
					Additional	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal		
					Additional	ddMMMyyyy / hh:mm	x.x	x.x	x.x	x.x	x.x	x.x	Normal		
					…etc..										

F=Female; M=Male

Abnormal NCS assessments are highlighted in yellow. Abnormal CS assessments are highlighted in pink.

Program: <program name (date, time)>, Path: <path>

Programming note: This listing excludes patients with normal assessments for all visits. Results for abnormal assessments will be highlighted in yellow.

Listing B.16.2.8.17 Physical Examination (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Body System	Current PRN Dose	Visit	Result	If abnormal, briefly describe
400 mg QD	xxxxxxx	xx/F	General Appearance	---	xxxx	Normal	
				400 mg QD	xxxx	Abnormal	xxxx
					xxxx	Not Done	
					xxxx	xxx	
					xxxx	Abnormal	xxx
				400 mg QD	xxxx	xxx	
					xxxx	xxx	
					xxxx	xxx	
					xxxx	xxx	
				…etc..			

F=Female; M=Male

Abnormal assessments are highlighted in yellow

Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.18 Physical Examination – Patients with Abnormal Results (Safety Population)

Starting Dose	Patient ID	Age/ Gender	Body System	Current PRN Dose	Visit	Exam Date and Time (ddMMMMyyyy hh:mm 24h)	Result	If abnormal, briefly describe
400 mg QD	xxxxxxx	xx/F	General Appearance	---	xxxx	dddMMMMyyyy hh:mm	Normal	
				400 mg QD	xxxx	xxxx	Abnormal	xxxx
					xxxx	xxxx	Not Done	
					xxxx	xxxx	xxx	
					xxxx	xxxx	Abnormal	xxx
				400 mg QD	xxxx	xxxx	xxx	
					xxxx	xxxx	xxx	
					xxxx	xxxx	xxx	
					xxxx	xxxx	xxx	
				…etc..				

F=Female; M=Male
Abnormal assessments are highlighted in yellow.
Program: <program name (date, time)>, Path: <path>

Programming note: This listing excludes patients with normal results for all body systems and all visits. Abnormal results will be highlighted in yellow.

Listing B.16.2.8.19 Pregnancy Test Results (Safety Population)

Starting Dose	Patient ID	Age/Gender	Current PRN Dose	Visit	Pregnancy sample collected?	Collection Date (ddMMMyyyy)	Collection Time (hh:mm 24h)	Pregnancy Lab Test	Result
400 mg QD	xxxxxxx	xx/F	400 mg QD	xxx	Yes	ddMMMyyyy	hh:mm	FSH	Negative
			400 mg QD	xxx	Yes	ddMMMyyyy	hh:mm	Serum Pregnancy	Negative
	..etc..								

F=Female; M=Male

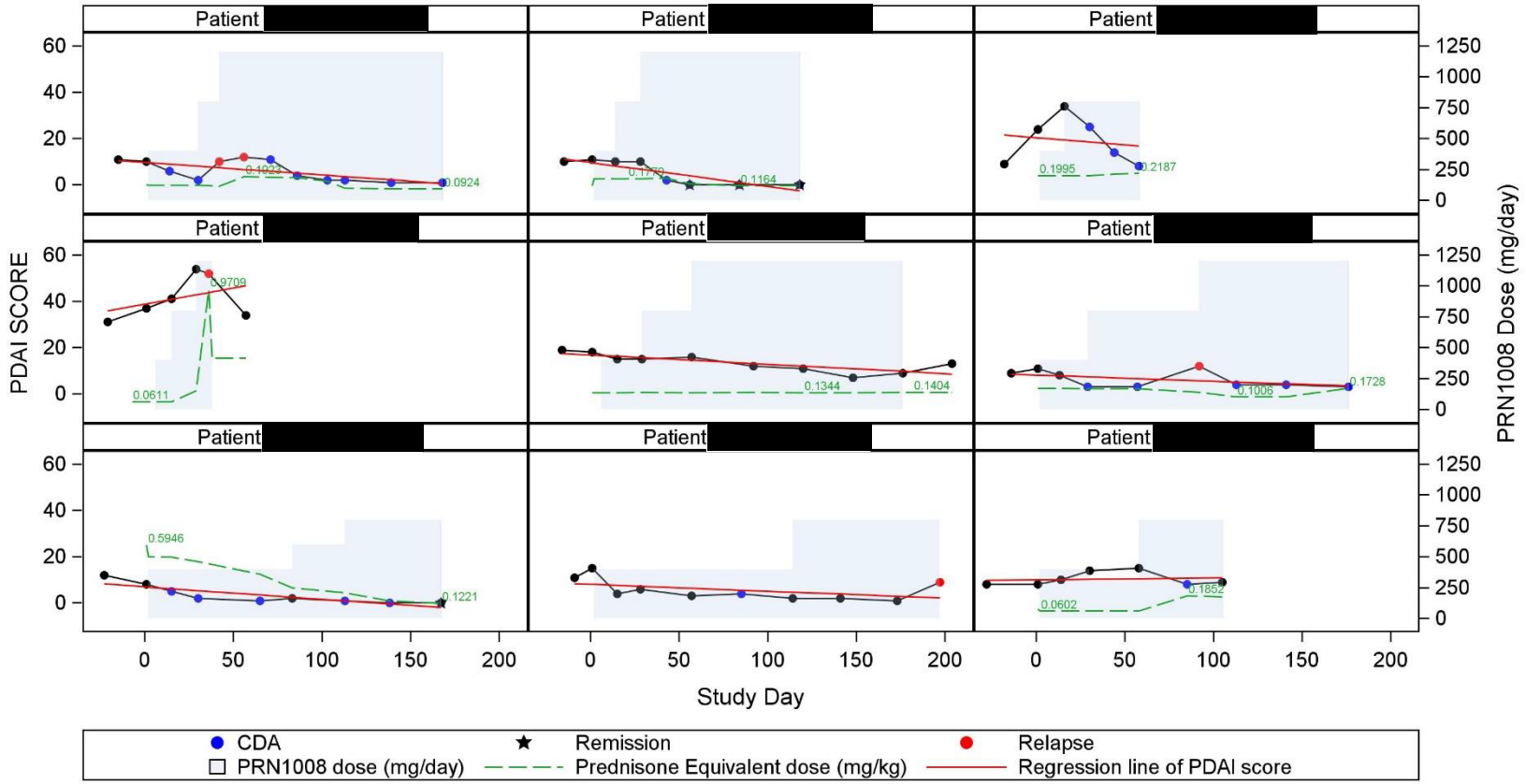
Program: <program name (date, time)>, Path: <path>

Listing B.16.2.8.20 Food Intake (Safety Population)

Starting Dose	Patient ID	Age/Gender	Current PRN Dose	Visit	Date of Food Intake (ddMMMyyyy)	Time of Food Intake (hh:mm 24h)	Food Intake
400 mg QD	xxxxxxx	xx/F	400 mg QD	xxx	ddMMMyyyyy	Hh:mm	xxxx
	..etc..						

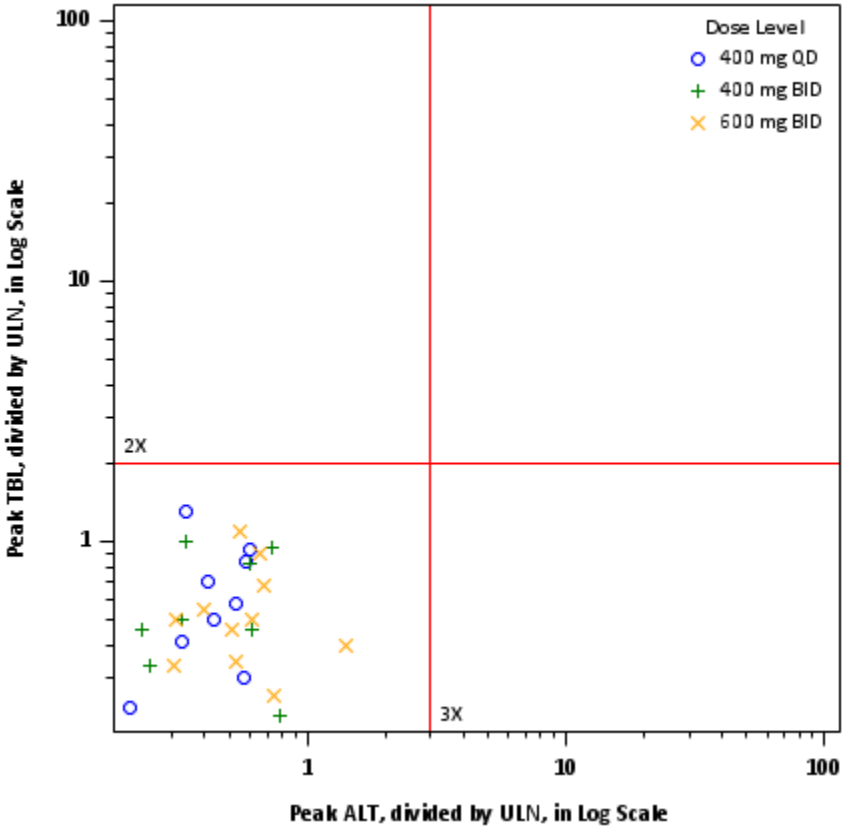
F=Female; M=Male
Program: <program name (date, time)>, Path: <path>

Figure B.14.1.1.1 PDAI Score and PRN Dose Level By Study Day – Panel Plots (mITT Population)



DB Version: 06SEP2019.
Prednisone equivalent medication doses have been multiplied by 1000 and displayed using the 'PRN1008 Dose (mg/day)' axis. The true prednisone equivalent medication absolute minimum and maximum have been displayed on the graph for each patient.

Figure B.14.3.2.1 Scatter Plots of Peak TBL vs Peak ALT for Identification of Drug Induced Liver Injury (DILI) (Safety Population)



Patients are classified into different dose levels according to the dose they received at a given visit.
TBL = total bilirubin, ALT = alanine aminotransferase or SGPT, ULN = Upper Limit of Normal.
The maximum measurement value observed while on a given dose is defined as the peak value.
Program: <program name (date, time)>, Path: <path>