A Phase 3, Multi-center, Randomized, Double-Blind, Placebo Controlled Study of the Efficacy and Safety of SD-101 Cream in Patients with Epidermolysis Bullosa (ESSENCE Study)

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PROTOCOL SD-005

A Phase 3, Multi-center, Randomized, Double-Blind, Placebo Controlled Study of the Efficacy and Safety of SD-101 Cream in Patients with Epidermolysis Bullosa
ESSENCE Study

Statistical Analysis Plan

Final Version 6.0

Date 12 May 2017

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CONFIDENTIAL

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Revision History

Version	Date	Prepared By	Revision Author	Comments
Draft 1.0	17Nov2014	Chiltern	PPD	First external draft
Draft 2.0	15Dec2014	Chiltern		Second external draft based on the Final Protocol, Version 2.0 dated 12Nov2014
Draft 3.0	20Jan2015	Chiltern		Third external draft after SAP meeting on 19Dec2014
Final 1.0	05Feb2015	Chiltern		Final
Final 2.0	12May2015	Chiltern		Update with sensitivity analysis recommended by Regulatory; add primary confirmatory analysis; adjust windowing table
Final 3.0	06Aug2015	Chiltern		Update sensitivity analysis to have primary endpoint stratified by region tested using CMH; change secondary endpoint for change from lesional skin at Month 2 compared to Baseline and move Month 3 to exploratory
Draft 4.0	23Mar2016	Scioderm		Section Changes to Analyses Planned in the Protocol: Changes were introduced to align with the changes to the protocol: elevated the secondary endpoint of time to complete target wound closure within 3 months to a one of two primary endpoints, introduction of group-sequential procedure with two interim analyses IA1 and IA2, the time duration for the existing primary endpoint (proportion of patients experiencing complete wound closure of their target wound within two months) was changed to “within 3 months”, the primary missing data handling approach changed to multiple imputations, the primary analysis method for proportions was changed from Fisher’s exact test to logistic regression, proportion of patients experiencing complete target wound closure at 2 months was demoted to the first secondary efficacy endpoint, proportion of patients experiencing complete target wound closure at 1 month efficacy endpoint was moved from an exploratory to a secondary efficacy endpoint.
Final 4.0	19Sep2016	Scioderm		Section Changes to Analyses Planned in the Protocol: general clarifications and edits
Draft 5.0	16Feb2017	Scioderm		Update from co-primary to two primary endpoints. Remove references related to interim analyses. Rename secondary endpoints as key secondary endpoints and exploratory endpoints as other secondary endpoints. Update adjustment for covariates.

Final 6.0	12May2017	Scioderm	PPD	Update safety analysis to describe details. Add subgroup analyses. Clarify study success criteria. Clarify efficacy analyses where needed by updating statistical methodology Table 3.
Amendment 1 of Final 6.0	01Mar2018	Scioderm		Addition of subgroup analysis, removal of some sensitivity analyses, and update to the safety section 8.7. The amendment also lists pre-unblinding clarifications recorded in the Note to File of 08Aug2017.

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1. LIST OF ABBREVIATIONS AND DEFINITIONS OF TERMS

AE	Adverse Event
ANCOVA	Analysis of Covariance
BMI	Body Mass Index
BSA	Body Surface Area
BSAI	Body Surface Area Index
^{o}C	Degrees Celsius
CI	Confidence Interval
cm	centimeters
CRF	Case Report Form
d/c	discontinuation
EB	Epidermolysis Bullosa
EBS	Epidermolysis Bullosa Simplex
FDA	Food and Drug Administration
FLACC	Face, Legs, Activity, Cry, Consolability
HR	Hazard Ratio
ITT	Intent-to-Treat
IWRS	Interactive Web Response Services
JEB	Junctional non-Herlitz EB
K-M	Kaplan-Meier
MAR	Missing at Random
MCMC	Markov Chain Monte Carlo
MI	Multiple Imputation
MMRM	Mixed Model Repeated Measures
MNAR	Missing Not at Random
MedDRA	Medical Dictionary for Regulatory Activities
NTF	Note to File
PP	Per Protocol
PT	Preferred Term
RDEB	Recessive Dystrophic Epidermolysis Bullosa
ROW	Rest of the World

SAE	Serious Adverse Event
SAF	Safety analysis population
SAP	Statistical analysis Plan
SOC	System Organ Class
TBWB	Total Body Wound Burden
TEAE	Treatment-Emergent Adverse Event
TESAE	Treatment-Emergent Serious Adverse Event
WHO	World Health Organization

2. INTRODUCTION

The SAP (Statistical Analysis Plan) was finalized prior to database lock (submitted to the Food and Drug Administration (FDA) on 16 May 2017 in Serial No. 0093) and is consistent with the Amendment 3, version 4.0 dated 10 March 2017 of the protocol. This amendment incorporates clarifications presented in the pre-database lock Note to File (NTF) of 8 August 2017, as well as post-database lock addition of further subgroup analyses and removal of some sensitivity analyses.

3. STUDY OBJECTIVE

The primary objective is to evaluate the efficacy and safety of SD-101-6% vs. Placebo in patients with Epidermolysis Bullosa Simplex (EBS), Recessive Dystrophic Epidermolysis Bullosa (RDEB), or Junctional non-Herlitz Epidermolysis Bullosa (JEB). SD-101-6% is the treatment referred to in the study protocol as SD-101-6.0. Placebo is the treatment referred to in the study protocol as SD-101-0.0.

4. STUDY DESIGN

4.1. General Design

This is a Phase 3, multi-center, randomized, double-blind, placebo controlled study to assess the efficacy and safety of SD-101-6% cream vs. Placebo on lesions in approximately 150 patients with Simplex, Recessive Dystrophic, or Junctional non-Herlitz Epidermolysis Bullosa.

Patients will be randomized on a 1:1 basis to either SD-101-6% cream or Placebo cream. SD-101-6% or Placebo will be applied topically, once a day to the entire body for a period of 90 days.

Patients will have 1 target wound selected at Baseline by the investigator. The investigator should identify a target wound per the ARANZ SilhouetteStar™ system manuals and training provided. At screening, multiple wounds on the subject can be assessed against study inclusion/exclusion criteria. The selected target wound must be at least 21 days old (size 10 to 50 cm^2). Photographic confirmation of the target wound location will be collected at Baseline, and the picture saved from the first visit will be used to confirm location of the target wound at subsequent visits. Once the target wound is identified, it should be followed during the subsequent study visits 2, 3, 4, and 5. It should be ensured that only 1 wound (captured as wound A in the ARANZ system) is identified for the study.

Patients who have an eligible target wound and meet all other inclusion/exclusion criteria will be randomized. Patients who screen fail may be rescreened. The first dose of treatment will be administered during the office visit. Patients randomized will initially be given one-month supply of study drug.

The patient will return for scheduled visits in accordance with the protocol: Visit 2 (14 days ± 5 days from Baseline), Visit 3 (30 days ± 7 days from Baseline), Visit 4 (60 days ± 7 days from Baseline), and Visit 5 (90 days ±7 days from Baseline) to have the target wound, previously identified at Baseline, re-assessed for the level of healing. In addition, itching, pain, body surface

area index (BSAI), and scarring of healed target wound will also be assessed at each visit. The ARANZ SilhouetteStar will be used to measure the target wound area at all visits.

All females of childbearing potential must have a negative urine pregnancy test prior to enrolling in the study and must agree to use some form of birth control or agree to remain abstinent until the study is completed. In addition, a urine pregnancy test will be performed at the screening visit and every 30 days until the final visit.

Safety and tolerability assessments will include monitoring of AEs (Adverse Events) and physical examinations.

Patients who complete the study will be eligible to enroll into an open-label study (SD-006).

4.2. Method of Assignment of Patients to Treatment Groups

Approximately 150 patients will be randomized using Interactive Web Response System (IWRS) to 1 of 2 treatment groups: Placebo or SD-101-6%. Patients are randomized using a 1:1 scheme.

4.3. Blinding

The blinding code will be produced by an independent unblinded team designated by Scioderm, who will provide safety oversight for the study. A patient will be unblinded only if the Investigator feels that knowledge of the specific treatment is necessary to determine how to treat the patient. In an emergency situation, the Investigator may need to break the code immediately, or as quickly as possible, if he/she finds it is in the best interest of the patient. The Investigator will be able to unblind using the IWRS system. If the blinding is prematurely broken, it is the responsibility of the Investigator to promptly document and explain any unblinding to the Sponsor. Any unblinding will be monitored for prompt documentation.

4.4. Determination of Sample Size

The two primary efficacy endpoints for this study are:

- Time to complete target wound closure within 3 months
- The proportion of patients experiencing complete closure of their target wound within 3 months

Complete target wound closure is defined as skin re-epithelialization without drainage.

These endpoints will be compared between SD-101-6% and Placebo, and tested via a step-down procedure beginning with the time to complete target wound closure. This will address multiplicity and control the overall study type 1 error level at 0.025 1-sided (refer to Section 7.5 for more detail).

Assumptions underlying the sample size estimation are that 35% of Placebo patients will experience complete closure of their target wound at or before the 3-month follow-up visit and at least 60% of patients treated with SD-101-6% will experience complete closure of their target wound by this time (hazard ratio approximately 2.127, assuming exponential hazards over time). Note that ALL wounds are open at Baseline; hence, the survival endpoint is an open wound. It is expected that fewer wounds will remain as open on test treatment than on placebo; hence, the expected proportions of open wounds at 3 months are 65% on placebo and 40% on test

treatment, which corresponds to a hazard ratio HR=2.127. These yield the 35% expected wound closures for placebo (35% = 100% - 65% surviving open), and 60% for SD-101-6% (60% = 100% - 40% surviving open). Approximately 150 patients are required (75 in each group) to provide at least 86% power for the time to complete target wound closure. There is estimated 85% power for the proportion of patients with complete target wound closure.

Therefore approximately 150 patients are to be enrolled.

5. CHANGES IN THE CONDUCT OF THE STUDY OR PLANNED ANALYSES

5.1. Changes in the Conduct of the Study

There were no changes in the conduct of the study at the time of preparing this statistical analysis plan.

5.2. Changes from the Analyses Planned in the Statistical Analysis Plan

The following changes were made to the analyses planned in the Version 6.0 of the SAP dated 12 May 2017:

- Wound age subgroup analysis is only conducted for ≤30 days and >30 days subgroups.
- Subgroups of interest were defined post-database lock. These are;
  - Age of 2 years to <12 years old
  - Wound age >30 days
  - Total Body Wound Burden (TBWB) ≥ 5% at Baseline
  - Age of 2 years to <12 years old plus TBWB ≥ 5% at Baseline
  - Age of 2 years to <12 years old at Baseline plus RDEB.
- Subgroup analyses for gender, race, and region were not performed except for AEs and Serious Adverse Events (SAEs) leading to d/c (discontinuation).
- In general, subgroup analyses were performed only for the 6 "subgroups of interest" (ie, 2 to <12 years, target wound age >30 days, TBWB ≥ 5%, and the 2 combined groups as specified above with exception of demographics and some efficacy analyses. Details are provided in the appendices.
- Addition of time to event analysis truncated at Day 44.
- Addition of wound size reduction to efficacy analysis.
- Removal of some sensitivity analyses.
- Removal of subgroup analyses for itching and pain based on who was an evaluator.
- Addition of skin infection analysis.

The detailed list of tables and figures is provided in the appendices.

6. BASELINE, EFFICACY AND SAFETY EVALUATIONS

6.1. Schedule of Evaluations

The assessments to be conducted at each scheduled visit are displayed in the protocol.

6.2. Time Point Algorithms

6.2.1. Relative Day

The date of randomization or Visit 1, will be considered study Day 0. Days prior to Day 0 will be negative. Day of randomization will be used to establish visit windows. Study days will be calculated as f:

date of assessment – date of randomization.

For relative day on study drug, the date of first study drug administration will be used. The first date of drug administration will be either the randomization day or the first day of study drug administration recorded on diary, if the study drug was not administered during the randomization office visit.

The relative day on study drug will be calculated as:

date of assessment – date of the first study drug administration + 1.

6.2.2. Analysis Windows

For analysis purposes, the visit numbers will be allotted into windowed visits, as illustrated in the following table.

Table 1: Analysis Windows

Week	Visit	Scheduled Study Day	Visit Window for Analysis (Days)
Screening/Baseline	Visit 1	-7 to 0	Day -7 – Day 0
Randomization	Visit 2	0	Day 0
Week 2	Visit 3	14	Day 1 – Day 21
Month 1	Visit 4	30	Day 22 – Day 45
Month 2	Visit 5	60	Day 46 – Day 75
Month 3 / Final Visit	Visit 6	90	Day 76 – Completion

As per the NTF, for the first primary endpoint (time to wound closure), the time to the first wound closure will be utilized irrespective of visit windows. For the second primary endpoint (proportion of subjects experiencing complete closure of their target wound), if there are more than one visit within the assigned visit window and the complete target wound closure occurred at any of them, it would count as wound closure at that visit. In case of the Month 3 / Final Visit analysis, even if the visit falls prior to Day 76 it will be utilized (example: there are two visits in the Month 2 visit window - Day 46 and Day 75. Day 75 visit is the last one. Day 46 visit will be utilized as Month 2 visit and Day 75 will be utilized as Month 3/ Final Visit. For the secondary

endpoints, the following approach will be used. The visit closest to the scheduled study day will be used for analysis. If two visits are the same distance from the scheduled study day, the first one will be used. In case of the Month 3 /Final Visit analysis, even if the visit falls prior to Day 76 it will be utilized (example: there are two visits in the Month 2 visit window- Day 46 and Day 75. Day 75 visit is the last one. Day 46 visit will be utilized as Month 2 visit and Day 75 will be utilized as Month 3 / Final Visit. If an unscheduled visit comprises only of dispensing or returning of study drug, it will not count for determination of the study visit used for the analysis.

This way all data available will be utilized.

6.3. Efficacy Endpoints

6.3.1. Primary Efficacy Endpoints

The two primary efficacy endpoints for this study are:

- Time to complete target wound closure within 3 months.
- Proportion of patients experiencing complete closure of their target wound within 3 months. Time to complete wound closure is based on the date of the first administration of the study drug.

  As the clarification in the NTF, if the target wound closes at a given visit, it will be considered closed at all subsequent visits.

They will be tested in a step-down procedure with the time to complete target wound closure being the first tested. If the time to complete target wound closure endpoint is statistically significant at the alpha level 0.025 one-sided, the endpoint ‘proportion of patients experiencing complete target wound closure within 3 months’ will be formally tested. The study will be considered a success if the first primary endpoint achieves statistical significance (refer to Section 7.5 for more detail).

As per clarifications in the NTF, the first primary efficacy analysis will be done on the ITT (Intent-To-Treat) population defined as all patients who were randomized. For clarification, the time to first wound closure analysis being conducted in the ITT population will inherently include patients with at least one post-Baseline assessment of target wound.

For the second primary endpoint of wound closure within 3 months, missing values imputed using the values observed at previous visits in multiple imputations, will be used in a logistic regression model to compare treatment groups while adjusting for baseline factors. In addition, for the second primary endpoint of wound closure within 3 months, a sensitivity analysis will be performed to assess the robustness of the imputation method: patients who do not experience wound closure will be considered as treatment failures; all patients who are randomized are analyzed (ITT).

As defined post database lock, primary efficacy analyses will be performed only on one subgroup based on the actual target wound age at baseline: ≤30 days, and >30 days.

In addition, the analyses will be also performed for subgroups of interest.

6.3.2. Key Secondary Efficacy Endpoints

The secondary efficacy endpoints are:

- Proportion of patients experiencing complete closure of their target wound within 2 months.
- Proportion of patients experiencing complete closure of their target wound within 1 month.
- Change in lesional skin based on BSAI at Month 3 compared to Baseline.

  Lesional skin for assessment consists of area(s) that could contain any of the following: blisters, erosions, ulcerations, scabbing, bullae, and eschars, as well as areas that are weeping, sloughing, oozing, crusted, and denuded. BSAI is the percent of this area recorded for each defined body region (number from 0-100% assigned for each region). Other areas categorized as skin that is either healed or scarred are not considered lesional skin. The BSAI will be assessed by a study physician for each patient visit. Complete calculations are listed in Appendix 11.1.
- Change in TBWB at Month 3, compared to Baseline.

  A wound is defined as an open area on the skin (epidermal covering is disrupted). Complete calculations are listed in Appendix 11.2.
- Change in itching assessed at Day 7, compared to Baseline, where change is defined as Day 7 itching value minus the Baseline value.

  If the assessment on Day 7 is missing, then an assessment window of ±2 days will be utilized. The assessments closest toDay 7 will be used and if two assessment are same distance from Day 7, then the latest would be used (examples: Day 6 present, Day 7 missing, Day 8 missing, Day 9 present - Day 6 will be used; Day 6 present, Day 7 missing, Day 8 present – Day 8 will be used).

  Change in itching assessed at Day 7, compared to Baseline will be measured using the “Itch Man Pruritus Assessment Tool”. For patients 1 month to 5 years of age itching will be assessed using caretaker’s response and patients 6 years of age and older will self-report their itching assessments. The “Itch Man Pruritus Tool” rates itching on a scale from 0 to 4, where larger values indicate more itching.
- Change in pain assessed at Day 7, compared to Baseline, where change is defined as Day 7 pain value minus the Baseline value.

  If the assessment on Day 7 is missing, then an assessment window of ± 2 days will be utilized. The assessments closest to Day 7 will be used and if two assessment are same distance from Day 7 then the latest would be used (examples: Day 6 present, Day 7 missing, Day 8 missing, Day 9 present - Day 6 will be used; Day 6 present, Day 7 missing, Day 8 present – Day 8 will be used).

  Change in pain assessed at Day 7, compared to Baseline will be measured using the “FLACC Behavioral Scale” for patients 1 month to 3 years of age, and for patients 4 years of age and older the “Wong-Baker FACES Pain Rating Scale” will be utilized. The FLACC Behavioral Scale (Face, Legs, Activity, Cry, Consolability) is a

measurement to assess pain in children or persons unable to communicate pain. Each of the five categories are scored from 0 to 2, resulting in a total score from 0 to 10. The "Wong-Baker FACES Pain Rating Scale" is a scale that shows a series of faces ranging from a happy face at 0 to a crying face at 10. For both scales, larger scores equate to more pain.

The analysis will be performed on the ITT population and subgroups of interest.

6.3.3. Other Secondary Efficacy Endpoints

The additional efficacy endpoints are:

- Change in Total Body Wound Burden based on BSAI at Week 2, Months 1, and 2, compared to Baseline.
- Percent change from Baseline in Total Body Wound Burden based on BSAI at 2 Week 2 weeks, Months 1, 2, and 3.
- Change in lesional skin based on BSAI at Week 2,Months 1, and 2, compared to Baseline.
- Percent change in lesional skin based on BSAI at Week 2, Months 1, 2, and 3, compared to Baseline.
- Presence of scarring of healed target wound at the visit where the complete closure is documented.
- Change in target wound characteristics (ie:. inflammation, blistering, granulation tissue, erythema, exudate) at Week 2, Months 1, 2, and 3, compared to Baseline.
- Changes in itching and pain at Days 1 to 6, Week 2, and Months 1, 2, and 3 compared to Baseline.
- Proportion of patients experiencing target wound closure within Week 2.

The analysis will be performed on the ITT population and subgroups of interest.

6.4. Safety Endpoints

6.4.1. Extent of Exposure and Compliance to Study Drug

SD-101-6% or Placebo creams will be supplied in 8 ounce plastic tubes, to be reclosed after use and stored at room temperature. SD-101-6% or Placebo will be applied topically once a day to the entire body for a period of 90 days. The first dose of study drug will be administered during the first study visit after randomization and enrollment visit assessments as described in Section 6.1.

The patient diary will be returned at visits 3, 4, and 5 to evaluate compliance.

Compliance with study drug is based on drug usage recorded in the patient diary. It will be calculated as follows:

Compliance (%) = 100*(number of days cream applied / days on study)

6.4.2. Treatment-emergent Adverse Events

Treatment-emergent AEs (TEAEs) are undesirable experiences, signs, or symptoms that begin or change in severity or relationship to treatment at the time of or after the first administration of study drug prior to the end of study.

The investigator's verbatim term of both serious and non-serious AEs will be mapped to system organ class (SOC) and preferred terms (PTs) using the 19.1 version of the Medical Dictionary for Regulatory Activities (MedDRA). Partial dates will be imputed as the following:

1. If year is not missing and is after the year of first application of study drug:
    a. If Month is missing, then Month will be imputed as January
    b. If Day is missing, then Day will be imputed as the first of the month
2. If year is not missing and is the same as the year of the first application of study drug:
    a. If Month is missing, then impute the Month as the month of the first application of study drug
    b. If Day is missing but Month is on or after the month of first application of study drug, then impute Day as the first day of study drug application
3. If year is missing, then impute the year as the year of the first study drug application:
    a. If Month is missing, then impute the Month as the Month of the first drug application
    b. If Day is missing, then impute the Day as the day of the first study drug application.
4. If the start date is completely missing, but the AE is either ongoing or the stop date is after the first date of drug application, then impute the start date as the first date of drug application.
5. If using the above rules, the stop date is before the start date, then leave the stop date missing and assume that AE is treatment emergent for the purpose of the analysis.

6.4.3. Vital Signs

Vital signs (temperature, height/length, and weight) are a part of the physical examination, which will be performed at the Screening visit and Month 3/ Final visit. All vital signs will be reported in metric units.

6.4.4. Physical Examination

Physical examinations will be done by a physician at the Screening visit and Month 3 /Final visit. The following sites will be examined: head, eyes, ears, nose, throat, neck, chest, lungs, heart, abdomen, skin, and lymph nodes; and the following systems will be assessed: musculoskeletal and neurological.

7. STATISTICAL METHODS

7.1. General Methodology

For statistical analysis purposes, Baseline is defined as the last measurement prior to the first application of the study drug.

In general, no formal inferential analyses are planned for the Baseline and safety data, and only descriptive statistics will be provided.

For the descriptive statistics, the categorical variables will be summarized by frequency and percentage for each response category (number of patients, %) and the continuous variables will be summarized using means, medians, minimum, maximum, and standard deviations.

Data will be summarized by treatment groups, and overall (where appropriate).

7.2. Adjustments for Covariates

Epidermolysis Bullosa (EB) type and Baseline characteristics of the target wound at Baseline, (ie., target wound size and target wound age) are considered to be potential effect modifiers. Their effects will be considered for the two primary efficacy endpoints (Month 3) as covariates.

Wound age is based on the wound start date provided by the subject from recall and as clarified in the NTF, it is calculated as follows: calculated wound age = Baseline date-wound start date. If the calculated wound age is ≤365 days then wound age used as a covariate in analysis is the calculated wound age. If the calculated wound age is >365 days, then the wound age used as covariate in analysis is replaced by 365 days. The reason for the definition is that it is difficult for the subject to provide an exact wound start date for old wounds.

Same covariates will be used in analysis of wound closure endpoints at other time points.

For other endpoints, EB type and the Baseline value for a given endpoint will be used.

7.3. Handling of Dropouts or Missing Data

For the time to wound closure endpoint, data will be right censored at 3 months or at the time of patient's withdrawal from the study. For the second primary endpoint and for the binary secondary endpoints of wound closure, the first method of imputation for missing data will be Multiple Imputation (MI) using the Missing At Random (MAR) assumption. As clarified in the NTF, if the target wound closure occurred at a prior visit, the target wound is considered closed at all subsequent visits that took place. In such a case, no imputation is required for subsequent visits.

For the first MAR based multiple imputation, the MI procedure of the SAS® system will be used to generate sets of data with missing values imputed from observed data. It is expected that the pattern of missing data will be monotonic, with slight deviations being corrected by the Markov Chain Monte Carlo (MCMC) method of the MI procedure.

As per clarification in the NTF, after the MCMC correction, the rounding will be performed for the binary endpoint. If imputed values are greater than or equal to 0.5, the value is set to 1 and anything else is set to 0.

Linear regression will be employed to model the missing continuous data and a logistic regression model will be used for the binary data, with the following covariates included in the imputation model: treatment, EB type, Baseline characteristics of a given endpoint (as described in Section 7.2), and non-missing data from earlier time points. As per clarification in the NTF, if the Week 2 data for wound closure are missing. there are no previous visit data available to utilize in the imputation procedure. Therefor,e patients with missing Week 2 wound closure data will be excluded from analysis. The imputed datasets will be analyzed using the methodology

described in Section 8. The results from the analysis of the multiple imputed datasets will be combined by the MIANALYZE procedure of the SAS system. The seed number to be used will be 010005 and the number of imputations is planned to be 5.

The final p-value, estimate of regression coefficient for treatment effect, and the 95% CI of the estimate of the treatment effect on each multiply-imputed dataset will be generated from the MIANALYZE procedure of SAS.

In addition, for the proportion of complete wound healing within Month 3, the final difference in proportions between treatment groups (SD-101-6% - Placebo) and the 95% CI of the final difference from each multiply imputed dataset will be generated using the method by Bohdana Ratitch et al. 2013.

To assess the robustness of the first missing data imputation result for the second primary endpoint, the following sensitivity analyses will also be conducted:

a. The same multiple imputation method as above will be repeated but based on the Pattern-Mixture Model discussed by Bohdana Ratitch et al. 2013 under the Missing Not At Random (MNAR) assumption, by using the profiles from the placebo subjects with observed data to imput missing data
b. Subjects with missing wound healing data within 3 months will be considered as 'failures' for the wound healing rate

7.4. Multi-center Studies and Pooling of Centers

All study sites will be included in the statistical analyses. However sites will be pooled according to geographic regions (US, Europe, and Rest of the World [ROW]).

7.5. Multiple Comparisons/Multiplicity

The two primary efficacy endpoints for this study are time to complete target wound closure within 3 months and the proportion of patients experiencing complete closure of their target wound within 3 months. These variables will be compared between SD-101-6% and Placebo, and tested via a step-down procedure beginning with the time to complete target wound closure. This will address multiplicity and control overall type 1 error level at 0.0-5 2-sided. Note that if the first primary endpoint to be tested (time to complete wound closure within 3 months) meets the significance criterion of $p<0.05$ 2-sided the study will be declared a success. If the first primary endpoint does not meet the significance criterion, the second primary endpoint (proportion of patients experiencing complete closure of the target wound within 3 months) will not be formally tested.Formal testing of the first key secondary endpoint at 0.05 2-sided will proceed (which is equivalent to 0.025 1-sided) if both primary endpoints are significant. If the first key secondary endpoint is statistically significant, then the second endpoint on the list of the key secondary endpoints will be tested at the two-sided 0.05 alpha level. Formal statistical testing will not proceed to the next endpoint if the current endpoint does not reach statistically significant level of 0.05 (two-sided). While the formal statistical testing will not proceed, nominal p-values will be generated for all endpoints.

Following the evaluation of the two primary endpoints, the ranking of the key secondary endpoints is as follows:

1. Proportion of patients experiencing complete closure of their target wound within 2 months
2. Proportion of patients experiencing complete closure of their target wound within 1 month
3. Change in lesional skin based on BSAI at Month 3, compared to Baseline
4. Change in Total Body Wound Burden at Month 3, compared to Baseline
5. Change in itching assessed at Day 7, compared to Baseline
6. Change in pain assessed at Day 7, compared to Baseline

The inclusion of other secondary endpoints listed in Section 6.3.3 is intended to yield supportive evidence related to the primary and key secondary objectives. Statistical tests of these variables are considered exploratory and no adjustment for multiplicity for these other secondary endpoints will be made.

7.6. Examination of Subgroups

All subgroup analyses are exploratory in nature and serve the purpose of numerical assessment of consistency of treatment effects across subgroups.

Subgroups if interest have been defined post-database lock. These are

- Age of 2 years to <12 years old
- Wound age >30 days
- Total Body Wound Burden (TBWB) ≥5 % at Baseline
- Age of 2 years to <12 years old plus TBWB ≥ 5% at Baseline
- Age of 2 years to <12 years old at baseline plus RDEB.

In general, subgroup analyses are performed only for the 6 "subgroups of interest" (ie, 2 to < 12, target wound age >30 days, TBWB ≥ 5%, and the 2 combined groups as specified above with exception of demographics and some efficacy analyses. Details are provided in the appendices.

Subgroups for subject's age are defined based on the subject's age at Baseline. For the purpose of subgroup analysis, race will be classified as White and Non-White.

For subgroup analysis of of the two primary and key secondary endpoints, -treatment effects, nominal p-values and nominal 95% CI by category of the classification variables listed above will be presented.

Based on the medical review of ARANZ SilhouetteStar system data prior to unblinding, the location of target wound will be determined to be in the flexure (eg. elbow, knee) or non-flexure region. Proportion of patients experiencing complete closure of their target wound within each visit, as well as itching and pain assessment at Day 7 are to be performed for these subgroups.

Subgroup analyses for gender, race, and region were performed for AEs and SAEs leading to d/c.

8. STATISTICAL ANALYSIS

8.1. Disposition of Patients

The number and percentage of patients who were screened, randomized, treated, completed, discontinued, and the primary reason for discontinuation based on the CRF (Case Report Form) termination page will be displayed. In addition, the number and percentage of subjects in each study population (ie., Intent-to-Treat population [ITT], Safety population [SAF] and Per Protocol population [PP]) as defined in Section 8.4 will be summarized by treatment group.

Same summaries will be produced for each investigational site, by country, and by region.

8.2. Demographic and Other Baseline Characteristics

Descriptive summaries of demographic and Baseline characteristics will be presented for the ITT and SAF populations. If the ITT and the SAF populations are identical, then the summaries will be based on ITT population only.

Continuous variables such as patient age, weight, height/length, body mass index (BMI), body surface area (BSA) calculated by Mosseler formula, body surface area index BSAI (for lesion and wound burden), age of target wound, target wound size, and temperature will be summarized as described previously.

Categorical variables such as patient gender, race, and age (0 to 27 days, 28 days to < 2 years, 2 years to < 12 years, 12 years to < 18 years, ≥ 18 years), age of target wound (≤ Median, > Median; and 0 ≤ 30 days, 31≤ 60 days, 61 ≤ 90 days, > 90 days), will be summarized using number of observations and percentages for each category.

Demographics is also presented for subgroups of interest.

Medical history, including disease history and EB type, will be summarized and listed.

Age will be defined as follows:

- If age was collected instead of the birth date then, the collected value will be used,
- If the birth date was collected, then

  Age = (date of informed consent date - date of birth + 1) / 365.25 and truncated to completed years.

Weight will be displayed in kg, height/length will be displayed in centimeters (cm), and temperature will be displayed in degrees Celsius (ºC).

8.3. Protocol Deviations

Number and percentage of subjects with major protocol deviations will be summarized for each treatment group in the ITT population and for subgroups as outlined in detail in the appendices. A listing of subjects with major protocol deviations will also be provided; major protocol deviations are further described in Section 8.4.3.

8.4. Analysis Populations

ITT population will be used for all efficacy analyses. SAF population will be used for all safety analyses. PP population will be used for supportive analyses of the efficacy endpoints.

If the SAF and ITT populations are identical, then the efficacy and safety analyses will be performed using the ITT population only.

8.4.1. Intent-to-Treat (ITT) Population

ITT population will be defined as all patients who have been randomized. These patients will be analyzed according to the assigned treatment.

8.4.2. Safety (SAF) Population

SAF population is defined as all randomized patients who applied/were administered the study drug at least once. These patients will be analyzed according to the treatment actually received.

8.4.3. Per Protocol (PP) Population

PP population will be defined as the ITT patients who have no major protocol deviations and have target wound data at Baseline and from ≥1 post-Baseline assessment.

Major protocol deviations include but are not limited to:

1. Entry criterion deviations that impact primary endpoint analyses
2. Non-compliance to study drug (as defined by compliance to study drug <70% as perpatient diary)
3. Administrative dispensing errors

A final list of patients who have any major protocol deviations will be documented before final database lock. A listing will also be created.

8.5. Prior and Concomitant Therapy

The World Health Organization (WHO) Drug Dictionary Version of March 2017 will be used to classify medications. Any medication taken before and continuing after randomization, or initiated after randomization, is considered a concomitant medication. Any medication given before randomization but discontinued prior to randomization is considered a prior medication. A medication can be considered both prior and concomitant. Medications missing both start and stop dates, or having a start date prior to the last dose of study drug and missing the stop date, or having a stop date after the start of study drug and missing the start date, will be considered concomitant. Medications will also be considered concomitant if partial start and stop dates are present but it cannot be determined if the medication ended prior to start of study drug.

A summary table will be provided for each of the following in the safety population:

1. Number and percentage of subjects who had previous therapies/medications by Anatomical Therapeutic Chemical **(**ATC) code text and WHO drug name
2. Number and percentage of subjects who had concomitant therapies/medications by ATC text and WHO drug name

The summary of use will also be provided for the groups of drug classes of concomitant medications listed in the table below:

Table 2: Concomitant Medications of Interest

SD-101-6%	Placebo	Timepoint or duration	Drug Class
All subjects Subgroups of interest	All subjects Subgroups of interest	Entire study	A06A: drugs for constipation A11: vitamins A12: mineral supplements
All subjects Subgroups of interest	All subjects Subgroups of interest	Entire study	B03: antianemic drugs B05: blood substitutes and perfusion solutions J01: antibacterial drugs J02: antimycotic drugs
All subjects Subgroups of interest	All subjects Subgroups of interest	Entire study	N02: analgesic drugs N05: psycholeptic drugs N06: psychoanaleptics
All subjects Subgroups of interest	All subjects Subgroups of interest	Entire study	R06: antihistamines for systemic use
All subjects Subgroups of interest	All subjects Subgroups of interest	Entire study	V20: surgical dressings D09: medicated dressings D08: antiseptics and disinfectant drugs D06: antibiotics for derm use D03: treatment of wounds and ulcers
All subjects Subgroups of interest	All subjects Subgroups of interest	At Baseline	D07: topical dermatological corticosteroids H02: corticosteroid systemic L04A: immunosuppressant drugs

8.6. Analysis of Efficacy Endpoints

Table 3 in Section 8.6.2 summarizes the efficacy endpoints analyses.

8.6.1. Analysis of the Two Primary Efficacy Endpoints

8.6.1.1. Statistical Methods

The two primary efficacy endpoints for this study are time to complete target wound closure within 3 months and the proportion of patients experiencing complete closure of their target wound within 3 months. These variables will be compared between SD-101-6% and Placebo, and tested via a step-down procedure beginning with the time to complete target wound closure.

Each endpoint might have analysis performed by primary and supportive methods.

The statistical methodology is as follows:

- Time to complete target wound closure

  As clarified in the NTF, time to event analyses will be summarized using a Kaplan-Meier approach, where the event is complete target wound closure measured from the date of the first administration of the study drug to the date of wound closure. Patients will be censored if they did not have a response within 3 months, or withdraw early, before the confirmation of their target wound closing. Comparison of the treatment groups will be performed using the Cox-proportional model with Baseline target wound size, target wound age, and EB type as covariates. The p-value, hazard ratio (HR), and 95% CI (Confidence Interval) of the hazard ratio (HR) from the Cox model will be presented.

  Only patients who have the Week 2 wound closure data will be analyzed.

  In addition, a sensitivity analysis will be performed using the log-rank test for the comparison of the two treatment groups.

  The proportional hazards assumption will be assessed by adding the treatment by time interaction into the Cox-proportional hazards model. If that interaction term approaches or reaches statistical significance, and if the Kaplan-Meier (K-M) survival curves indicate substantially varying treatment differences in cumulative proportion of patients with total wound closure, then the cumulative proportions may be compared between treatments for appropriate time periods suggested by the K-M plot. These between-treatment comparisons per time period would be via logistic regression or exact binomial tests.

  The consistency of the treatment effect across each of the subgroups (see Section 7.6) will be evaluated using descriptive statistics.

  As decided post-database lock, same analysis using K-M approach and Cox-proportional hazards model will be performed assuming 45 day cutoff for target wound closure. It means that any time to wound closure that is 44 days or more is going to be right censored.

- Proportion of patients experiencing complete closure of their target wound within 3 months

  Complete closure of the target wound will be determined at each visit. A patient will be considered a “responder” if they experience complete wound closure at the Week 2, Months 1, 2, or 3 visits.

  The proportions of “responders” (patients experiencing complete target wound closure within 3 months) in the two treatment groups will be compared using the following hypothesis testing where the null and alternate hypotheses are as follows:

  H_0: $P_0 = P_6$ H_a: $P_0 < P_6$,

  where P_0 is the proportion of patients receiving Placebo experiencing the second primary endpoint and P_6 is the proportion of patients receiving SD-101-6% experiencing the second primary endpoint.

  The proportion of “responders” (proportion of patients exhibiting complete closure of their target wound within 3 months of randomization) will be analyzed using the

logistic regression model with Baseline target wound size, target wound age, and EB type as covariates. The first analysis will be performed using the ITT population based on the Multiple Imputation methodology (MI) as the first imputation method for missing values, see Section 7.3.

In the case that complete separation and quasi-complete separation occurs from the logistic regression model as specified above, the explanatory variable causing the situation will be identified and excluded from the model. If the treatment factor is that variable, then the treatment effect will be tested using the chi-square test.

The p-value for the treatment comparison, odds ratio of response between the two treatment groups, and the 95% CI of the odds ratio will be presented from the logistic regression model. In addition, the unadjusted response rates for each group, the difference of unadjusted response rates between the two treatment group,s and the 95% CI of the difference using the Miettinen-Nurminen method will also be provided.

The consistency of the treatment effect across each of the subgroups (see Section 7.6) will be evaluated using descriptive statistics.

8.6.1.2. Sensitivity Analyses

The above primary efficacy analysis will be repeated using the PP population. Sensitivity analyses are considered supportive.

To assess the robustness of the primary efficacy result, the following sensitivity analyses will be conducted using the ITT population:

a. The same imputation method above will be repeated based on the Pattern-Mixture Model as discussed by Ratitch et al.[(2)] under the Missing Not at Random (MNAR) assumption, by using the profiles from placebo patients with observed data to impute missing data.
b. Patients who withdraw early, before the confirmation of their target wound closing or their 3-month visit (without target wound closure), will be considered treatment failures with respect to the second primary endpoint.

The treatment-by-covariate interaction will be tested in a separate model for each endpoint and each covariate. Sensitivity analyses will not be done for subgroups.

8.6.2. Analysis of Key Secondary Efficacy Endpoints

Formal statistical testing of the secondary efficacy endpoints will be performed after the two primary efficacy endpoints achieve statistical significance. To control overall Type I error rate at 5%, the step-down strategy will be applied as described in Section 7.5.

The covariates ie., EB type and Baseline characteristics (of the secondary endpoint being analyzed), will be used for the secondary analyses as they are for the primary analyses. Target wound age and target wound size will be used for:

- Proportion of patients experiencing complete closure of their target wound within 2 months

- Proportion of patients experiencing complete closure of their target wound within 1 month.

For other secondary endpoints, target wound age and target wound size will not be used as covariates because they are not considered effect modifiers.

Table 3: Analysis Strategy for Efficacy Endpoints

Endpoint	Primary vs. Supportive Approach	Statistical Method	Analysis Population	Missing Data Approach
Two primary endpoints				
Time to complete target wound closure within 3 months	P	Cox Model	ITT	Censoring
	S	Log-rank Test	ITT	Censoring
	S	Cox Model	PP	Censoring
	S	Log-rank Test	PP	Censoring
	S	Descriptive Statistics	ITT	As observed
Complete closure of target wound within 3 months	P	Logistic Regression	ITT	MI
	S	Logistic Regression	PP	MI
	S	Logistic Regression	ITT	MI (MNAR)
	S	Logistic Regression	ITT	Missing as Failures
	S	Descriptive Statistics	ITT	As observed
Key Secondary Endpoints				
Complete closure of target wound within 2 months	P	Logistic Regression	ITT	MI
	S	Descriptive Statistics	ITT	As observed
Complete closure of target wound within 1 month	P	Logistic Regression	ITT	MI
	S	Descriptive Statistics	ITT	As observed

Table 3: Analysis Strategy for Efficacy Endpoints (Continued)

Endpoint	Primary vs. Supportive Approach	Statistical Method	Analysis Population	Missing Data Approach
Change in BSAI of lesional skin at Month 3, compared to Baseline	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed
Change in BSAI of Total Body Wound Burden at Month 3, compared to Baseline	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed
Change in Itching assessment at Day 7, compared to Baseline	P	Logistic Regression	ITT	As observed
	S	Logistic Regression	ITT	As observed excluding missing
	S	Descriptive Statistics	ITT	As observed
Change in Pain assessment at Day 7, compared to Baseline	P	Logistic Regression	ITT	As observed
	S	Logistic Regression	ITT	As observed excluding missing
	S	Descriptive Statistics	ITT	As observed

Table 3: Analysis Strategy for Efficacy Endpoints (Continued)

Endpoint	Primary vs. Supportive Approach	Statistical Method	Analysis Population	Missing Data Approach
Other Secondary Endpoints				
Change in BSAI of Total Body Wound Burden at Weeks 2, Months 1 and 2, compared to Baseline	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed
Percent change from Baseline in BSAI of Total Body Wound Burden at Week 2Months 1, 2, and 3	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed
Percent change in BSAI of lesional skin at Week 2, Months 1, 2, and 3, compared to Baseline	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed

Table 3: Analysis Strategy for Efficacy Endpoints (Continued)

Endpoint	Primary vs. Supportive Approach	Statistical Method	Analysis Population	Missing Data Approach
Change in BSAI of lesional skin at Week 2, Month 1 and Month 2, compared to Baseline	P	MMRM	ITT	MI
	S	MMRM	ITT	As observed
	S	ANCOVA	ITT	MI As observed
	S	ANCOVA on transformed data	ITT	MI As observed
	S	Descriptive Statistics	ITT	As observed
Presence of scarring of healed target wound at the visit where the complete closure is documented	P	Descriptive Statistics	ITT with wound closure	As observed
Change in target wound characteristics (ie. milia, inflammation, blistering, granulation tissue, erythema, exudate) at Week 2, Months 1, 2, and 3, compared to Baseline	P	Logistic regression	ITT	As observed
	S	Descriptive Statistics	ITT	As observed
Change in Itching and Pain at Days 1 to 6, Week 2, and Months 1, 2 and, 3, compared to Baseline	P	Logistic Regression	ITT	As observed
	S	Logistic Regression	ITT	As observed (excluding missing)
	S	Descriptive Statistics	ITT	As observed
Target Wound Closure within Week 2, compared to Baseline	P	Logistic Regression	ITT	MI
	S	Descriptive Statistics	ITT	As observed

Abbreviations: MMRM=Mixed model for repeated measures with treatment, visit, and visit-treatment interaction as fixed effects; P=Primary approach; S=Supportive approach

The analysis of key secondary efficacy variables will be as follows:

- Proportion of patients experiencing complete target wound closure at Month 2

  This assessment will be analyzed using the same approach as the second primary endpoint.

- Proportion of patients experiencing complete target wound closure at Month 1

This assessment will be analyzed using the same approach as the second primary endpoint.

- Change in lesional skin based on BSAI at Month 3 compared to Baseline

  For analysis, a mixed model repeated measures approach (using Restricted Maximum Likelihood (REML) estimation) will be used on each multiply imputed data set. The model will include treatment, Baseline BSAI, EB type, visit, and visit-treatment interaction as the fixed effects. Visit will be used as a repeated measure and an unstructured covariance approach will be applied. If the fit of the unstructured covariance structure fails to converge, the following covariance structures will be tried in order until convergence is reached: Toeplitz with heterogeneity, autogressive with heterogeneity, Toeplitz, and autoregressive.

  Means and least squares mean difference between SD-101-6% and Placebo, along with a two-sided 95% CI and p-value for the difference between treatments will also be provided.

  The following provides sample code for implementing the MMRM analysis:

  ```
  proc mixed method = reml;
                  class TRT VISIT SUBJID;
                  model <CHG>=TRT VISIT TRT*VISIT BASELINE
                  EBTYPE/s ddfm=kr;
                  repeated VISIT/type=UN subject=SUBJID;
                  lsmeans TRT*VISIT /slice=VISIT pdiff diff alpha=0.05 cl;
                  /*testing difference in treatment group level for each visit*/;
  run;
  ```

  where TRT is the assigned treatment, VISIT is the visit based on the window mapping, CHG is the change from Baseline in the BSAI measurements, EBTYPE is the EB type, and BASELINE is the Baseline BSAI measurements.

  In addition, the difference in changes will be presented across each of the subgroups (see Section 7.6) using descriptive statistics. MMRM with a subgroup factor in the model will be applied. In case the effects are not estimable, a simple ANCOVA analysis of covariance) model with EB type and Baseline BSAI measurement as covariates will be used with treatment and subgroup as fixed effect in the model.

  As decided post database lock, for target wound age, the following categorization will be used: $0 \leq 30$ days, >30 days.

  As clarified in the NTF, if any of covariates are missing, then a subject with a missing covariate will be excluded from the ANCOVA model.

  The above analyses will be conducted on the data after MI imputation. Analysis on the observed data will be conducted as a sensitivity analysis.

  A graph of the mean BSAI of lesional skin for each treatment group over time will also be presented.

- Change in Total Body Wound Burden at Month 3, compared to Baseline

The analysis will be based on the same statistical method used for the change in BSAI at Month 3 of lesional skin.

A graph of the mean Total Body Wound Burden for each treatment group over time will also be presented.

- Change in itching assessed at Day 7, compared to Baseline

  As clarified in the NTF, itching scores will be categorized into three groups based on improvement. These groups include Improved or No Itching, Not Improved, and Missing, where Improved or No Itching is an itching score reduction from Baseline greater than or equal to 1 point on the scale or itching score of 0 at both Baseline and post-Baseline. The proportion of patients experiencing improvement in itching or no presence of itching versus non-improvement (including missing) will be compared between the two treatment groups for Day 7 using the same method as the second primary endpoint, but only with Baseline itching score, and EB type as covariates.

  It is anticipated that no patients will withdraw from the study prior to Day 7. To provide an analysis of the sensitivity of the conclusions resulting from the analysis of difference in itching scores from Baseline to Day 7, an additional analysis will be performed on the ITT population, but will not consider missing itching scores and will only compare the proportions of patients categorized as Improved or Not Improved.

  Descriptive statistics will be presented for two categories defined above and for the actual scores at Baseline and Day 7, as well as the change in itching scores from Baseline to Day 7.

  In addition, the consistency of the treatment effect across each of the subgroups (see Section 7.6) will be evaluated using descriptive statistics.

- Change in pain assessed at Day 7, compared to Baseline

  As clarified in the NTF, similar to itching scores, pain scores will be categorized into three groups based on improvement. These groups include Improved, Not Improved or No Pain, and Missing, where Improved or No Pain is a pain score reduction from Baseline greater than or equal to 2 points on the scale or pain score of 0 at both Baseline and post-Baseline.The proportion of patients experiencing improvement in pain versus non-improvement (including missing) will be compared between the two treatment groups for Day 7 using the same method as the second primary endpoint,but only with Baseline pain score, and EB type as covariates.

  It is anticipated that no patients will withdraw from the study prior to Day 7. To provide an analysis of the sensitivity of the conclusions resulting from the analysis of pain at Day 7, an additional analysis will be performed on the ITT population, but will not consider missing pain scores and will only compare the proportions of patients categorized as Improved or Not Improved.

  Descriptive statistics will be presented for two categories defined above and for the actual scores at Baseline and Day 7, as well as the change in pain scores from Baseline to Day 7.

The treatment-by-covariate interaction will be tested in a separate model for each endpoint and each covariate. Subgroup means and 95% CI's will be presented as derived from this model. The plot of individual patient values on y-axis and covariate on x-axis, using different symbols for each treatment and a regression line for each treatment will be presented for continuous data.

In addition, same analysis as above will be conducted for subgroups factors listed in Section 7.6 keeping continuous factors as continuous (eg. patient's age).

In addition, the consistency of the treatment effect across each of the subgroups (see Section 7.6) will be evaluated using descriptive statistics.

For continuous secondary endpoints, normality will be assessed via Shapiro-Wilk test statistics' values and graphically. If suggestion of substantial departure from normality is detected, then the data will be transformed using log or rank transformations to yield analysis scale closest to normality. ANCOVA with EB type and Baseline value for the endpoint under analysis will be performed on transformed values.

Supportive analyses (Table 3) will not be done for subgroups.

8.6.3. Analyses of Other Secondary Endpoints

The analysis of other secondary efficacy endpoints will be as follows:

- Change in Total Body Wound Burden at Week 2, Months 1, and 2, compared to Baseline.
- Percent change from Baseline in Total Body Wound Burden at Week 2, Months 1, 2, and 3.
- Percent change in lesional skin based on BSAI at Week 2, Months 1, 2, and 3, compared to Baseline
- Change in lesional skin based on BSAI at Week 2, and Months 1 and 2, compared to Baseline.
- The four endpoints listed above will be analyzed using the same approach as the change at Month 3 for both change from Baseline and the percentage change from Baseline. Although the comparison of Month 3 against Baseline is a key secondary endpoint, these other time points will be included in the exploration of a temporal pattern. Presence of scarring of healed target wound at visit of documented complete wound closure.

  The proportion of patients experiencing scarring will be presented for each of the two treatment groups only for those patients who experienced complete closure of target wound.
- Change in target wound characteristics (ie. milia,inflammation, blistering, granulation tissue, erythema, exudate) atWeek 2, Months 1, 2, and 3, compared to Baseline.

  As clarified in the NTF, the presence of each characteristic was recorded as Yes on the CRF. The blank value will be considered the absence of a characteristics. The same approach used in the evaluation of itching and pain as key secondary endpoints

on observed data will be employed in the analysis of these endpoints, but will be performed at each of the specified visits. The categorization will be as follows:

Baseline	Post-baseline	Category
Absent	Absent	Improved or Absent
Present	Absent	Improved or Absent
Absent	Present	Not Improved
Present	Present	Not Improved

Assessments will be summarized using descriptive statistics at each visit by treatment group. A plot comparing the two treatments over time will also be presented for each endpoint.

- Change in itching and pain at Days 1 to 6, Week 2, and Months 1, 2, and 3, compared to Baseline.

  The same approaches used in the evaluation of itching and pain as key secondary endpoints will be employed in the analysis of these endpoints, but will be performed at each of the specified visits. Assessments will be summarized using descriptive statistics at each visit by treatment group. A plot comparing the two treatments over time will also be presented for each endpoint.

- Proportion of patients experiencing target wound closure within Week 2.

  This assessment will be analyzed independently at each visit using the same approach as the Month 3 primary endpoint.

Supportive analyses will not be done for subgroups.

8.7. Analysis of Safety

8.7.1. Extent of Exposure and Compliance of Study Drug

8.7.1.1. Extent of Exposure

The number of days that cream is applied will be summarized using descriptive statistics by treatment group and overall. Descriptive statistics will also be provided for duration of exposure days (number of days cream applied) into categorical summary of < 14, ≥ 14 to < 30, ≥ 30 to < 60, ≥ 60 to < 90, ≥ 90).

Extent of exposure will also be presented for subgroups of interest.

8.7.1.2. Measurements of Treatment Compliance

Percent compliance will be summarized using descriptive statistics (n, mean, standard deviation, median, minimum, maximum) for $< 70\%$, $\geq 70\%$, and overall by treatment group.

8.7.2. Adverse Events by Preferred Term and System Organ Class

Adverse events will be classified as treatment-emergent or non treatment-emergent.

A non-treatment emergent AE is defined as an AE that originated prior to the first dose of study drug and did not change in severity or relationship to treatment on or after the date of the first dose of study drug. If the severity or relationship to treatment changed on or after the first dose of study drug prior to the end of study, then that AE is considered a TEAE.

For subjects entering directly into extension study SD-006, there is no follow-up period after the last day on study. The safety information will be entered into SD-006 database. For subjects not entering the extension study the SD-006, the follow-up information will be captured in the source documents and will not be collected in the clinical database. Serious Adverse Events (SAEs) will be entered into the safety database.

8.7.3. Summaries of Adverse Event Incidence Rates for All Subjects

The number and percentage of subjects who experienced TEAEs will be presented by system organ class (SOC) and by preferred term (PT) within SOC for each treatment group. TEAEs will be similarly presented by severity (mild, moderate, severe), by relationship to study drug (related would include definite, probable, and possible) and by outcome of events. Additionally, the number of TEAEs (as opposed to the number and percentage of subjects) will be presented by treatment group.

Analysis will also be done for subgroups of interest. Subgroups for age are defined based on the subject's age at Baseline. Details are provided in the appendices.

To count the number of subjects who experienced each TEAE, a subject experiencing the same TEAE multiple times will only be counted once for the corresponding PT. Similarly, if a subject experiences multiple TEAEs within the same SOC, the subject will be counted only once for that SOC. If a subject experiencing more than one TEAE within different severity or relationship categories within the same SOC/PT, only the worst case (worst severity and related TEAE) will be reported. TEAEs will be sorted alphabetically by SOC and within each SOC the PT will be presented by decreasing order of total frequency.

For TEAE summary by outcome, if a subject experienced more than one TEAE, the worst outcome will be counted under that subject. If a subject experienced more than one outcome within a SOC (or PT), the subject is only counted once under worst outcome in that SOC (or PT).

In addition, all adverse events will be provided by study site and by treatment group, in a listing, which will include the subject identifier, the PT, the reported term, the severity, the seriousness, the action taken, the outcome, the causality, the date onset, date of onset relative to the date of the first study drug administration, date of resolution, date of resolution related to the first study drug administration, and the TEAE duration (resolution date –onset date + 1). Separate listings will be provided for non-treatment emergent AEs, if any.

8.7.4. Missing AE Onset, Severity, and Relationship

8.7.4.1. Missing or Partial AE Dates

The following list describes how partially missing date information will be handled as it relates to partial or missing start dates. Partial dates will be imputed as follows:

1. If year is not missing and is after the year of first application of study drug:

a. If Month is missing, then Month will be imputed as January
b. If Day is missing, then Day will be imputed as the first of the month

2. If year is not missing and is the same as the year of the first application of study drug:
    a. If Month is missing, then impute the Month as the month of the first application of study drug
    b. If Day is missing but Month is on or after the month of first application of study drug, then impute Day as the first day of study drug application
    c. If Day is missing but Month is on or after the month of first application of study drug, then impute Day as the first day of - the month.
    d. If the Day and Month are missing, then impute Day and Month as the Day and Month of the first study drug application.
3. If year is missing, then impute the year as the year of the first study drug application:
    a. If Month is missing, then impute the Month as the Month of the first study drug application
    b. If Day is missing, then impute the Day as the day of the first study drug application
4. If the start date is completely missing, but the AE is either ongoing or the stop date is after the first date of drug application, then impute the start date as the first date of study drug application.
5. If using the above rules, the stop date is before the start date, then leave the stop date missing and assume that AE is treatment emergent for the purpose of the analysis.

Completely missing stop dates will not be imputed. The partial stop dates will be imputed as follows:

1. If the year is missing, the stop date will not be imputed
2. If the month is missing, then the month will be imputed as December
3. If the day is missing, then the day will be imputed as the last day of the month

Imputed dates will be flagged in the individual supportive subject listings.

8.7.4.2. Missing Severity

If severity is missing for any AE, then its severity will be classified as missing in the summary tables.

8.7.4.3. Missing Relationship

If the assessment of relationship of the AE to study drug is missing for any AE, then it will be presented as missing in the listings and in the summary tables.

8.7.5. Summaries of Adverse Event Incidence Rates for Serious Adverse Events (SAE), Adverse Event Dropouts, and Death

During the treatment period, the number and percentage of subjects who experienced TESAEs (treatment emergent serious AEs) and separately TEAEs leading to withdrawal from the study will be presented by SOC and by PT within each SOC for each treatment. TESAEs and

separately TEAEs resulting in withdrawal from the study will be similarly presented by severity (mild, moderate, severe), by relationship to study drug (related would include definite, probable, and possible) and by outcome of events. Additionally, the number of TESAEs (as opposed to the number and percentage of subjects) and separately TEAEs leading to withdrawal will be presented.

Subgroup analyses for gender, race, and region are performed for AEs and SAEs leading to d/c.

Analysis will be also done for subgroups of interest. Subgroups for age are defined based on the subject's age at Baseline. Details are provided in appendices.

In case that there are non-treatment emergent adverse events observed, the number and percentage of patients with all SAEs will be summarized by SOC and PT within each SOC. Additionally, the number of SAEs (as opposed to the number and percentage of subjects) will be presented.

A listing of all SAEs and discontinuations due to AEs will be included. Subject with deaths, SAEs, or subjects who withdraw due to AE will be listed separately and discussed with subject narratives.

8.7.6. Discontinuation from the Study

Number of subjects who discontinued from the study and reasons for discontinuation will be summarized by treatment group.

8.7.7. Clinical Laboratory Evaluations

8.7.7.1. Pregnancy Test

Pregnancy test results will be listed for all female patients of child bearing potential only.

8.7.8. Other Observations Related to Safety

8.7.8.1. Vital Signs

Vital signs (weight, height/length, and temperature) conducted under the physical examination will be summarized using descriptive statistics for both actual results and change from Baseline for each treatment group. Vital Signs' assessments will also be presented in a listing. All vital signs will be presented using metric units.

8.7.8.2. Physical Examination Findings

Physical examination findings will be summarized in shift tables that will be presented to display the shift in the normal range categories (normal vs abnormal) from Baseline to the final evaluation.

8.7.8.3. Skin Infections

Skin infections are to be compared between treatment groups. Skin infection cluster comprises of the following PTs:

- Skin infection

- Wound infection
- Staphylococcal skin infection
- Skin bacterial infection
- Wound infection staphylococcal
- Folliculitis
- Wound infection bacterial
- Cellulitis
- Cellulitis staphylococcal
- Impetigo
- Infected skin ulcer
- Postoperative wound infection
- Rash pustular

9. COMPUTER SOFTWARE

All analyses will be performed by the designee of Scioderm using Version 9.2 or later of SAS software. All summary tables, data listings, and figures will be prepared utilizing SAS software.

10. REFERENCES

B. Ratitch, I. Lipkovich, and M. O'Kelly. "Combining Analysis Results from Multiply Imputed Categorical Data", 2013, PharmaSUG Proceedings, Paper SP-03.

B. Ratitch, M. O'Kelly, and R. Tosiello, "Missing Data in Clnical Trials: From Clinical Assumptions to Statistical Analysis Using Pattern Mixture Models", January 2013, Pharmaceutical Statistics.

11. APPENDICES

11.1. Body Surface Area Index (BSAI) of Lesional Skin

(Check only one box and complete the appropriate sections for each region)

1 ☐ Ages 1 month to 7 years

Column 1	Column 2	Column 3	Column 4	Column 5
Region Number	**Region Description**	**Regional BSA % that is affected***	**Weighting Factor**	**Total BSA % that is affected ****
1	Head / Neck		x .2	
2	Upper Limbs		x .2	
3	Trunk (includes groin)		x .3	
4	Lower Limbs		x .3	
			TOTAL (BSAI)	

2 ☐ Age 8 years or greater

Column 1	Column 2	Column 3	Column 4	Column 5
Region Number	**Region Description**	**Regional BSA % that is affected***	**Weighting Factor**	**Total BSA % that is affected ****
1	Head / Neck		x .1	
2	Upper Limbs		x .2	
3	Trunk (includes groin)		x .3	
4	Lower Limbs		x .4	
			TOTAL (BSAI)	

* For each region, enter the % of BSA that is affected with lesional skin. Score each region separately from 0% - 100%.

** Multiply the value in column 3 by the factor in column 4. The Total value at the bottom of the table is the sum of Column 5 values for each region.

11.2. Body Surface Area Index (BSAI) of Total Wound Burden

(Check only one box and complete the appropriate sections for each region)

1 ☐ Ages 1 month to 7 years

Column 1	Column 2	Column 3	Column 4	Column 5
Region Number	**Region Description**	**Regional BSA % that is affected***	**Weighting Factor**	**Total BSA % that is affected ****
1	Head / Neck		x .2	
2	Upper Limbs		x .2	
3	Trunk (includes groin)		x .3	
4	Lower Limbs		x .3	
			TOTAL (BSAI)	

2 ☐ Age 8 years or greater

Column 1	Column 2	Column 3	Column 4	Column 5
Region Number	**Region Description**	**Regional BSA % that is affected***	**Weighting Factor**	**Total BSA % that is affected ****
1	Head / Neck		x .1	
2	Upper Limbs		x .2	
3	Trunk (includes groin)		x .3	
4	Lower Limbs		x .4	
			TOTAL (BSAI)	

* For each region, enter the % of BSA that is affected with open wounds. Score each region separately from 0% - 100%.

** Multiply the value in column 3 by the factor in column 4. The Total value at the bottom of the table is the sum of Column 5 values for each region.

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Figure Number	Figure Title
Ad-hoc Figure 14.2.16.2.1.3	Improvement in Pain over Time (Baseline Target Wound Age >30 Days) (Day 1 to Day 7) (ITT Population)
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Ad-hoc Figure 14.2.16.2.1.5	Improvement in Pain over Time (Baseline BSAI of Total Body Wound Burden >=5%) (Day 1 to Day 7) (ITT Population)
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11.5 List of Listings

Listing Number	Listing Title
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Listing 16.2.2	Protocol Deviations (ITT Population)
Listing 16.2.4.1	Demographics and Baseline Data (ITT Population)
Listing 16.2.4.2	Medical History (Safety Population)
Listing 16.2.6.1	Body Surface Area Index of Lesional Skin by Visit (ITT Population)
Listing 16.2.6.2	Body Surface Area Index of Total Body Wound Burden by Visit (ITT Population)
Listing 16.2.6.3	Itch Man Pruritus Assessment Tool (ITT Population)
Listing 16.2.6.4	FLACC Pain Scale (ITT Population)

Listing Number	Listing Title
Listing 16.2.6.5	Wong-Baker Faces Pain Scale (ITT Population)
Listing 16.2.7.1	Treatment-Emergent Adverse Events (Safety Population)
Listing 16.2.7.2	Non-Treatment Emergent Adverse Events (Safety Population)
Listing 16.2.7.3	All Serious Adverse Events (Safety Population)
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Listing 16.2.8	Pregnancy Test (All Female Patients of Childbearing Potential)
Listing 16.2.9	Vital Signs (Safety Population)
Listing 16.2.10	Target Wound Closure (ITT Population)
Listing 16.2.11	Prior and Concomitant Medications (Safety Population)
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Ad-hoc Listing 16.2.13	Physical Examination (Safety Population)
Ad-hoc Listing 16.2.14	Exposure (Safety Population)
Ad-hoc Listing 16.2.15	Target Wound Characteristics Assessment (ITT Population)
Ad-hoc Listing 16.2.16	Target Wound Assessment of Scarring (ITT Population)