Official Title: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of the Efficacy and Safety of Parsaclisib in Participants With Primary Warm Autoimmune Hemolytic Anemia (PATHWAY)

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Statistical Analysis Plan

INCB 50465-309

A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of the Efficacy and Safety of Parsaclisib in Participants With Primary Warm Autoimmune Hemolytic Anemia (PATHWAY)

IND Number:	**147,208**
EudraCT Number:	**2021-002844-66**
Phase of Study:	**3**
Sponsor:	**Incyte Corporation** **1801 Augustine Cut-Off** **Wilmington, DE 19803** **United States**
Protocol Version:	**Protocol Amendment 2 dated 10 MAY 2023**
CRF Approval Date:	**27 APR 2023**
SAP Version:	**Original**
SAP Author:	[redacted] **Biostatistics**
Date of Plan:	**17 JAN 2024**

This study is being conducted in compliance with Good Clinical Practice, including the archiving of essential documents.

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LIST OF ABBREVIATIONS

Abbreviation	Term
[redacted]	[redacted]
6MWT	6-minute walk test
AE	adverse event
AIHA	autoimmune hemolytic anemia
BMI	body mass index
[redacted]	[redacted]
CRF	case report form
CTCAE	Common Terminology Criteria for Adverse Events
ECG	electrocardiogram
eCRF	electronic case report form
FACIT-F	Functional Assessment of Chronic Illness Therapy - Fatigue
MedDRA	Medical Dictionary for Regulatory Activities
PD	pharmacodynamic
[redacted]	[redacted]
[redacted]	[redacted]
Pi3K	phosphoinositide 3-kinase
[redacted]	[redacted]
PT	preferred term
QD	once daily
QTcF	Fridericia's correction
SAP	statistical analysis plan
SOC	system organ class
TEAE	treatment-emergent adverse event
ULN	upper limit of normal
[redacted]	[redacted]
wAIHA	warm autoimmune hemolytic anemia
[redacted]	[redacted]
WHO	World Health Organization

1. INTRODUCTION

This is a Phase 3, multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of parsaclisib 2.5 mg QD compared with placebo over a 24-week double-blind treatment period followed by a 24-week open-label treatment period of parsaclisib. Participants may then continue to receive parsaclisib in a long-term extension period.

On 17 APR 2023, Incyte decided to terminate this Phase 3 study. The decision was based on the change in the regulatory landscape for Pi3k-delta inhibitors combined with enrollment challenges. At the time of this decision, 13 participants had been randomized and received study drug in this study.

The purpose of this SAP is to provide details of the statistical analyses that have been outlined in the INCB 50465-309 Protocol. The scope of this plan includes the final analyses, which will be executed by the Department of Biostatistics or designee.

2. STUDY INFORMATION, OBJECTIVES, AND ENDPOINTS

2.1. Protocol and Case Report Form Version

This SAP is based on INCB 50465-309 Protocol Amendment 2 dated 10 MAY 2023 and CRF approved 27 APR 2023. Unless superseded by an amendment, this SAP will be effective for all subsequent Protocol amendments and eCRF versions.

2.2. Objectives and Endpoints

Table 1 presents the objectives and endpoints.

Table 1: Objectives and Endpoints

Objectives	Endpoints
Primary	
To evaluate the efficacy of parsaclisib in the treatment of participants with wAIHA.	Proportion of participants attaining a durable hemoglobin response, defined as hemoglobin ≥ 10 g/dL with an increase from baseline of ≥ 2 g/dL not attributed to rescue therapy at ≥ 3 of the 4 available visits at Week 12 and/or later during the 24-week double-blind treatment period.
Key Secondary	
To further evaluate the efficacy of parsaclisib in the treatment of participants with wAIHA.	Proportion of participants with a ≥ 3-point increase from baseline in FACIT-F score at Week 24.

Table 1: Objectives and Endpoints (Continued)

Objectives	Endpoints
Other Secondary	
To further evaluate the efficacy of parsaclisib in the treatment of participants with wAIHA.	• Proportion of participants with a 50 m increase from baseline to Week 24 in a 6MWT. • Change and percent change from baseline in FACIT-F score at each postbaseline visit. • Change and percentage change from baseline in hemoglobin at each postbaseline visit. • Proportion of participants who received transfusions from Week 6 to Week 24 and from Week 24 to Week 48. • Change and percentage change from baseline in daily corticosteroid dose at Week 24. • Proportion of participants who required rescue therapy at any visit from Week 6 through Week 24 and from Week 24 to Week 48.
To evaluate the safety and tolerability of parsaclisib in participants with wAIHA.	Frequency and severity of AEs, including the evaluation of clinical laboratory results, vital signs, ECGs, and the results of physical examinations.
[redacted]	

Table 1: Objectives and Endpoints (Continued)

3. STUDY DESIGN

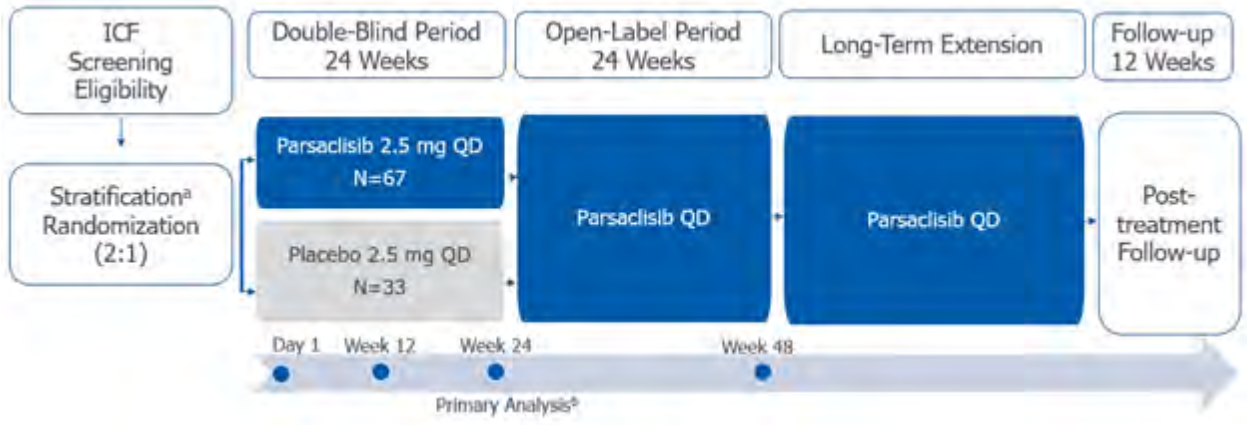
This is a Phase 3, multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of parsaclisib 2.5 mg QD compared with placebo over a 24-week double-blind treatment period followed by a 24-week open-label treatment period of parsaclisib. Participants may then continue to receive parsaclisib in a long-term extension period.

Participants will receive parsaclisib 2.5 mg QD or matching placebo QD for 24 weeks during the double-blind treatment period. All participants who complete the double-blind treatment period, tolerate study treatment, and (per investigator's opinion) may benefit from treatment, may remain in the study for an additional 24-week, open-label treatment period in which they will receive parsaclisib administered at the same dose and schedule as in the double-blind treatment period.

All participants who complete the open-label treatment period, tolerate study treatment, and (per investigator's opinion) may benefit from continued treatment, may enter into the long-term extension. Parsaclisib will be administered at the same dose and schedule as in the open-label treatment period. During the long-term extension, participants may take a drug holiday one time with the option for re-treatment, should they experience worsening of wAIHA during the drug holiday.

The study schema is shown below in Figure 1. All participants will have follow-up assessments 12 weeks after the last dose of study drug.

Figure 1: Study Design Schema



ICF = informed consent form.
[a] Hemoglobin, at screening, as determined by local laboratory (< 9 g/dL or ≥ 9 g/dL) and corticosteroid (prednisone or equivalent) dose at screening (≤ 20 mg/day vs > 20 mg/day).
[b] The primary analysis will be conducted when the last randomized participant has completed the 24-week, double-blind treatment period.

3.1. Randomization

Approximately 100 participants will be randomized 2:1 to receive initial double-blind study treatment (parsaclisib:matched placebo) for 24 weeks. Participants will be stratified by the hemoglobin value at screening as determined by local laboratory (hemoglobin < 9 g/dL or ≥ 9 g/dL) and corticosteroid (prednisone or equivalent) dose at screening (≤ 20 mg/day vs > 20 mg/day).

3.2. Control of Type I Error

For the primary and key secondary endpoints, the overall 2-sided Type I error is 0.05.

The gatekeeping testing strategy for the primary and key secondary analyses will be implemented to control the overall Type I error rate, 2-sided $\alpha = 0.05$. The p-value for between-treatment group testing for the primary endpoint will be compared at 2-sided $\alpha = 0.05$ level. The key secondary endpoint will be tested at 2-sided $\alpha = 0.05$ level only if the null hypothesis of the primary endpoint is rejected.

3.3. Sample Size Consideration

Approximately 100 participants will be randomized 2:1 (approximately 67:33) to receive either parsaclisib 2.5 mg QD or matching placebo using a fixed block size within each stratum. For the statistical comparison on the binary primary efficacy endpoint, the sample size calculation will be based on a Chi-square test.

Based on the results of the Phase 2 study (INCB 50465-206), the response rate for the primary endpoint is assumed to be 70% for parsaclisib 2.5 mg QD. With limited data, such response rates in the placebo group are not reported in historical studies. The response rate for the placebo group can only be assumed to around 25% from the literature reviewed (Barcellini et al 2014, Birgens et al 2013, Lechner and Jäger 2010). Based on these assumptions, the sample size of 100 will provide enough power (over 90%) to detect such a difference with a 2-sided alpha of 0.05.

3.4. Schedule of Assessments

Refer to Protocol Amendment 2 dated 10 MAY 2023 for a full description of all procedures and assessment schedules for this study.

4. DATA HANDLING DEFINITIONS AND CONVENTIONS

4.1. Scheduled Study Evaluations

4.1.1. Day 1

Day 1 is the date that the first dose of study drug is administered to the participants.

4.1.2. Study Day

If a visit/reporting date is on or after Day 1, then the study day at the visit/reporting date will be calculated as follows:

Day # = (visit/reporting date – Day 1 date + 1)

If the visit/reporting date is before Day 1, then the study day at the visit/reporting date will be calculated as follows:

Day # = (visit/reporting date – Day 1 date)

A study day of −1 indicates 1 day before Day 1.

4.1.3. Baseline Value

Baseline is the last nonmissing measurement obtained before the first administration of study drug.

4.1.4. Handling of Missing and Incomplete Data

In general, values for missing data will not be imputed unless methods for handling missing data are specified in this section or relevant sections.

4.2. Variable Definitions

4.2.1. Body Mass Index

Body mass index will be calculated as follows:

BMI (kg/m^2) = [weight (kg)] / [height (m)]^2

4.2.2. Prior and Concomitant Medication

Prior medication is defined as any nonstudy medication started before the first dose of study drug.

Concomitant medication is defined as any nonstudy medication that is started accordingly:

- Before the date of first dose of study drug and is ongoing throughout the study or ends on/after the date of first study drug administration.
- On/after the date of first dose of study drug and is ongoing or ends during the course of study treatment.

A prior medication could also be classified as "both prior and concomitant medication," if the end date is on or after the first dose of parsaclisib. In the listing, it will be indicated whether a medication is prior-only, concomitant-only, or both prior and concomitant.

For the purposes of analysis, all medications will be considered concomitant medications unless the medications can unequivocally be defined as not concomitant.

5. STATISTICAL METHODOLOGY

5.1. General Methodology

Unless otherwise noted, SAS software (SAS Institute Inc, Cary, NC; v9.1 or later) will be used for the generation of all tables, graphs, and statistical analyses. Descriptive summaries for continuous variables will include, but not be limited to, the number of observations, mean, standard deviation, median, minimum, maximum, first quartile, third quartile, and 95% confidence interval. Descriptive summaries for categorical variables will include the number and percentage of participants in each category.

5.2. Treatment Groups

This is a Phase 3, multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of parsaclisib 2.5 mg QD. Data will be summarized based on the actual treatment that the participant received at Day 1.

Unless other specified, the treatment groups will be parsaclisib 2.5 mg QD and placebo QD. For exposure, compliance, and adverse events, the treatment groups will be placebo QD, placebo QD to parsaclisib 2.5 mg QD, and parsaclisib 2.5 mg QD.

5.3. Analysis Populations

Upon implementation of Protocol Amendment 2, enrollment closed and Protocol-required procedures have been reduced for ongoing participants. With limited participants enrolled in each treatment group, as well as the option for early crossover to parsaclisib, the statistical analyses, as defined by the Protocol, will not be applicable; as a result, only the safety population [redacted] will be used for analysis.

5.3.1. Safety Population

All randomized participants who received at least 1 dose of study drug will constitute the safety population. Treatment groups for this population will be determined according to the actual treatment the participant received on Day 1 regardless of assigned study treatment.

All safety and efficacy analyses will be conducted using the safety population.

[redacted]

6. BASELINE, EXPOSURE, AND DISPOSITION VARIABLES AND ANALYSES

Appendix A provides a list of planned tables and listings. Sample data displays are included in a separate document.

6.1. Demographics, Baseline Characteristics, and Disease History

6.1.1. Demographics and Baseline Characteristics

The following demographics will be summarized for the safety population: age, sex, race, ethnicity, weight, height, and BMI.

6.1.2. Baseline Disease Characteristics and Disease History

The following baseline disease characteristics and disease history will be summarized for the safety population and will include, but will not be limited to the following:

- Years since first onset (primary) wAIHA (< 2 years, 2-5 years, or > 5 years)
- Serological evidence
- Symptoms of wAIHA
- Number of hospitalizations due to hemolytic anemia in the year prior to screening
- Number of transfusions in the year prior to screening
- Failed therapies (no/yes)
- Any therapies contraindicated for the participant (no/yes)

6.1.3. Prior Therapy

Prior medication information for wAIHA will be used to identify other nonpredefined medication received by participants before enrollment into the study. Prior medication for wAIHA will be summarized by treatment group.

6.1.4. Medical History

Medical history will be summarized by treatment group. This summary will include the number and percentage of participants with medical history for each body SOC/PT as documented in the eCRF.

6.2. Disposition of Participant

The number and percentage of participants who were enrolled, who were randomized (double-blind period only), who were treated, and who completed treatment for each period will be summarized for the safety populations.

6.3. Protocol Deviations

Protocol deviations will be summarized and listed.

6.4. Exposure

For participants in the safety population, exposure will be summarized descriptively for duration of treatment, average daily dose, and total dose. The treatment groups will be provided based on the actual treatment received (see Section 5.2).

Duration of treatment with study drug is defined as the number of days from Day 1 to the date of last record of study drug administration. In addition, daily usage of prednisone (or equivalent) will be summarized using descriptive statistics.

6.5. Study Drug Compliance

For the participants in the safety population, overall compliance (%) for study drug administration will be calculated as follows:

Compliance (%) = 100 × [total dose taken] / [total intended dose]

The total number of intended doses defined as the number of dose prescribed.

6.6. Prior and Concomitant Medication

Prior medications and concomitant medications will be coded using the WHO Drug Dictionary. The number and percentage of participants in the safety population for each prior and concomitant medication will be summarized by WHO drug class and WHO drug preferred name.

Prior and concomitant medications will also be summarized (and listed) by treatment group.

7. EFFICACY

With limited participants enrolled in each treatment group, as well as the option for early crossover to parsaclisib, the statistical analyses, as related to efficacy and defined by the Protocol, will not be applicable; as a result, no p-values will be provided and no multiple adjustment will be made.

7.1. Efficacy Hypotheses

Not applicable.

7.2. Analysis of the Primary Efficacy Parameters

The primary endpoint will be defined as a success if the proportion of participants attain a durable hemoglobin response ≥ 10 g/dL with an increase from baseline of ≥ 2 g/dL. The key secondary endpoint will be considered a success if the proportion of participants have a ≥ 3-point increase from baseline in FACIT-F score at Week 24.

For primary and key secondary endpoints, postbaseline visits will be summarized by treatment received on Day 1 of the study; the visits will use descriptive statistics.

[redacted]

9. SAFETY AND TOLERABILITY

Appendix A provides a list of data displays. Sample data displays will be included in a separate document.

9.1. General Considerations

Analyses in this section will be provided for the safety population. Treatment groups will be provided based on the actual treatment received (see Section 5.2). For participants who cross over treatments, the date of first dose is treatment-specific; the end date will be either 30 days after the date of the last dose of this treatment or the date of the first dose in the next treatment, whichever comes first. Analysis of AEs will be limited to TEAEs, but data listings will include all AEs regardless of their timing in relation to study drug administration. Summary tables may be replaced with listings when appropriate. For instance, an AE frequency table may be replaced with a listing if it only contains a few unique PTs reported on relatively few participants.

9.2. Adverse Events

9.2.1. Adverse Event Definitions

A TEAE is any AE either reported for the first time or worsening of a pre-existing event after first dose of study drug and within 30 days of the last dose of study drug. Analysis of AEs (as discussed below) will be limited to TEAEs, but data listings will include all AEs regardless of their timing in relation to study drug dose administration.

Adverse events will be tabulated by MedDRA PT and SOC. Severity of AEs will be graded using the National Cancer Institute CTCAE v5. The CTCAE reporting guidelines and grading details are available on the Cancer Therapy Evaluation Program website.

The subset of AEs considered by the investigator to be related to study drug will be considered treatment-related AEs. If the investigator does not specify the relationship of the AE to study drug, the AE will be considered to be treatment-related. The incidence of AEs and treatment-related AEs will be tabulated. Serious AEs will also be tabulated.

Any missing onset date, causality, or severity must be queried for resolution. Unresolved missing causality and severity will be handled according to the following rules:

- An unresolved missing causality will be considered treatment-related.
- An unresolved missing severity will be identified as an unknown severity.

For purposes of analysis, all AEs will be considered TEAEs unless the AE can unequivocally be defined as not treatment-emergent.

9.2.2. Adverse Event Summaries

An overall summary of AEs by treatment group will include the following:

- Number (%) of participants reporting any TEAEs
- Number (%) of participants reporting any serious TEAEs
- Number (%) of participants reporting any Grade 3 or higher TEAEs
- Number (%) of participants reporting any treatment-related TEAEs
- Number (%) of participants who temporarily interrupted study drug because of TEAEs
- Number (%) of participants who permanently discontinued study drug because of TEAEs
- Number (%) of participants who had any fatal TEAE

The following summaries will be produced by MedDRA term (if 2 or fewer participants appear in a table, a listing may be appropriate):

- Summary of TEAEs by SOC and PT
- Summary of TEAEs by SOC and PT
- Summary of TEAEs by PT in decreasing order of frequency
- Summary of Grade 3 or higher TEAEs by SOC and PT
- Summary of serious TEAEs by SOC and PT
- Summary of treatment-related TEAEs by SOC and PT
- Summary of TEAEs with a fatal outcome by SOC and PT
- Summary of TEAEs leading to dose interruption of study drug by SOC and PT
- Summary of TEAEs leading to discontinuation of study drug by SOC and PT

9.3. Clinical Laboratory Tests

9.3.1. Laboratory Value Definitions

Laboratory values and change from baseline will be summarized descriptively by central laboratory. Baseline will be determined according to Section 4.1.3, with exceptions listed below, in which case the investigative site laboratory or an accredited local laboratory may be used:

- Hematology panels (ie, complete blood count and differential count), will be sent to a local laboratory for analysis.
- Participant management, which includes dose modification, serious adverse event, or COVID-19 restrictions (eg, travel), for local laboratory assessments deemed necessary by the investigator for participant management. Note: Such assessment data and reference ranges must be recorded in the participant's eCRF.

In the event both local and central laboratory assessments are available, local laboratory assessment will be used for analysis. Laboratory values, change from baseline values, and percent change from baseline values will be summarized descriptively by visit. Baseline values will be determined using the nonmissing values collected before the first administration, prioritizing scheduled assessments over unscheduled visits. For baseline laboratory values with the same date and time in the same priority category, additional rules may be provided following consultation with the medical monitor to delineate the value that will be defined as baseline.

9.3.2. Laboratory Value Summaries

Clinical laboratory tests, including hematology, serum, and chemistry, will be performed for each participant per the schedule of assessments. If specific safety issues arise, additional unscheduled laboratory tests/analyses will be performed at the discretion of the investigator.

All test results and associated normal ranges from central laboratories will be reported in SI units. All tests with numeric values will have a unique unit per test.

For test results that will be summarized with available normal ranges, the number and percentage of participants with the laboratory values being low, normal, high, and missing will be tabulated for each test and each visit.

9.4. Vital Signs

Descriptive statistics and mean change from baseline will be determined for vital signs (blood pressure, pulse, respiratory rate, and body temperature) at each assessment time.

Criteria for clinically notable vital sign abnormalities are defined in Table 2. The abnormal values for participants exhibiting clinically notable vital sign abnormalities will be listed along with their assigned treatment group. Alert vital signs are defined as an absolute value outside the defined range and percentage change > 25%. The abnormal values for participants exhibiting alert vital sign abnormalities will be listed.

Table 2: Criteria for Clinically Notable Vital Sign Abnormalities for ≥ 18 Years Old

Parameter	High Threshold	Low Threshold
Systolic blood pressure	> 155 mmHg	< 85 mmHg
Diastolic blood pressure	> 100 mmHg	< 40 mmHg
Pulse	> 100 bpm	< 45 bpm
Temperature	> 38ºC	< 35.5ºC
Respiratory rate	> 20 breaths/min	< 8 breaths/min

9.5. Electrocardiograms

Descriptive statistics and mean change from baseline will be summarized for each ECG parameter at each assessment time. Electrocardiogram results will be reviewed for clinically notable abnormalities according to predefined criteria (see Table 3). Participants exhibiting clinically notable ECG abnormalities will be listed.

Table 3: Criteria for Clinically Notable Electrocardiogram Abnormalities

Parameter	High Threshold	Low Threshold
QTcF	> 460 ms	< 295 ms
PR	> 220 ms	< 75 ms
QRS	> 120 ms	< 50 ms
QT	> 500 ms	< 300 ms
RR	> 1330 ms	< 600 ms

10. INTERIM ANALYSES

No formal interim analysis is planned in this study.

11. CHANGES AND MODIFICATIONS TO THE ANALYSIS PLAN

All versions of the SAP are listed in Table 4.

Table 4: Statistical Analysis Plan Versions

SAP Version	Date
Original	17 JAN 2024

11.1. Changes to Protocol-Defined Analyses

On 17 APR 2023, Incyte decided to terminate this Phase 3 study. The decision was based on change in the regulatory landscape for Pi3k-delta inhibitors combined with enrollment challenges.

With limited enrollment in each treatment group, as well as participant option for early crossover (to parsaclisib), most of the Protocol-defined statistical analyses on efficacy endpoints will not be applicable; therefore, summary statistics will only be provided for hemoglobin response and FACIT-F scores and listings will only be provided for all other efficacy endpoints. Additionally, no p-values will be provided and no statistical comparisons will be made.

11.2. Changes to the Statistical Analysis Plan

Not applicable.

12. REFERENCES

Barcellini W, Fattizzo B, Zaninoni A, et al. Clinical heterogeneity and predictors of outcome in primary autoimmune hemolytic anemia: a GIMEMA study of 308 patients. Blood 2014;124:2930-2936.

Birgens H, Frederiksen H, Hasselbalch HC, et al. A Phase III randomized trial comparing glucocorticoid monotherapy versus glucocorticoid and rituximab in patients with autoimmune haemolytic anaemia. Br J Haematol 2013;163:393-399.

Lechner K, Jäger U. How I treat autoimmune hemolytic anemias in adults. Blood 2010;116:1831-1838.

APPENDIX A. PLANNED TABLES AND LISTINGS

This appendix provides a list of the planned tables and listings for the Clinical Study Report. Shells are provided in a separate document for tables that are not in the most current Standard Safety Tables v1.13.

The list of tables and listings are to be used as guidelines. Modifications of the lists that do not otherwise affect the nature of the analysis will not warrant an amendment to the SAP.

Tables

Table No.	Title	Population	Standard
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1.5.2	Summary of wAIHA History	Safety	X
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3.2.2	Summary of Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term	Safety	X
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Table No.	Title	Population	Standard
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3.2.6	Summary of Treatment-Emergent Adverse Events With a Fatal Outcome by MedDRA System Organ Class and Preferred Term	Safety	X
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3.4.5	Summary of Body Temperature	Safety	X
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3.5.1	Summary of PR Interval (ms) From 12-Lead ECG	Safety	X
3.5.2	Summary of QRS Interval (ms) From 12-Lead ECG	Safety	X
3.5.3	Summary of QT Interval (ms) From 12-Lead ECG	Safety	X
3.5.4	Summary of QTcF Interval (ms) From 12-Lead ECG	Safety	X
3.5.5	Summary of RR Interval (ms) From 12-Lead ECG	Safety	X
3.5.6	Summary of Clinically Significant ECG Abnormality	Safety	X

Listings

Listing No.	Title
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1.1.3	Protocol Deviations
1.2.1	Demographic
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2.2	6MWT

Listing No.	Title
2.3	Transfusion
2.4	Daily Corticosteroid Dose
2.5	Rescue Therapy
[redacted]	[redacted]
[redacted]	[redacted]
[redacted]	[redacted]
[redacted]	[redacted]
[redacted]	[redacted]
[redacted]	[redacted]
[redacted]	[redacted]
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