Clinical Study Report

NGAM-10

16.1.9 Documentation of Statistical Methods

16.1.9.1 NGAM-10 Statistical Analysis Plan – Version 1.0 (31 Jan 2024)

Octapharma USA.

STATISTICAL ANALYSIS PLAN

Protocol Title:	Post-Marketing Study to Evaluate the Efficacy and Safety of PANZYGA in Pediatric Patients with Chronic Immune Thrombocytopenia (ITP)
Protocol Number:	NGAM-10
Phase:	Phase 4
Sponsor:	Octapharma USA, Inc. 117 West Century Road Paramus, NJ 07652
Author:	Summit Analytical, LLC 8354 Northfield Blvd., Bldg. G Suite 3700 Denver, CO 80238
SAP Date:	2024-01-31
Status:	FINAL V1.0

SUMMITANALYTICAL

Statistical Analysis Plan Approval Form

Sponsor: Octapharma USA
Protocol: NGAM-10
Protocol Title: A Post-Marketing Study to Evaluate the Efficacy and Safety of PANZYGA in Pediatric Patients with Chronic Immune Thrombocytopenia (ITP)
SAP Version: Final v1.0
SAP Date: 2024-01-31

The statistical analysis plan has been reviewed and approved.

Signed: [redacted]
Octapharma

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Signature Date

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Summit Analytical, LLC

[redacted]
Signature Date

1. TABLE OF CONTENTS

2. ABBREVIATIONS AND DEFINITION OF TERMS

Only abbreviations and terms relevant to the SAP are repeated herein. The reader is referred to the protocol for the complete and comprehensive list of abbreviations and definitions of terms for the study.

Abbreviation/Term	Definition
AE	Adverse event
ALT	Alanine aminotransferase
AST	Aspartate aminotransferase
ATC	Anatomical Therapeutic Chemical (Classification)
CBC	Complete blood count
CRO	Contract Research Organization
eCRF	Electronic Case Report Form
g	Gram
ICF	Informed Consent Form
ICH	International Council for Harmonization
IDMC	Independent Data Monitoring Committee
IMP	Investigational Medicinal Product
IRB	Institutional Review Board
MedDRA	Medical Dictionary for Regulatory Activities
SAP	Statistical Analysis Plan
TEAE	Treatment Emergent Adverse Event
WHODDE	WHO Drug Dictionary Enhanced

3. INTRODUCTION

3.1. Preface

Study NGAM-10 was terminated early after inclusion of only 6 patients. Since such a small sample size renders statistical summaries of limited value, this SAP deviates from the planned analyses outlined in the protocol. Please refer to the protocol version history for more information on the initially planned analyses.
This document provides a statistical analysis plan (SAP) for Octapharma USA. Protocol NGAM-10 *(Post-Marketing Study to Evaluate the Efficacy and Safety of PANZYGA in Pediatric Patients with Chronic Immune Thrombocytopenia (ITP)*. This SAP provides details on the reporting of the subject characteristics, efficacy, and safety information, and will be finalized and approved prior to database lock.

Reference Documents Used for SAP

Study Document	Approval Date
Protocol Version 04	27 April 2020
CRF Version 03	15 July 2020

3.2. Purpose of Analyses

The purpose of the planned analyses described in this SAP is to facilitate assessing the efficacy and safety profiles of PANZYGA infusion administration. Results from the analyses completed will be included in the final clinical study report, and may also be utilized for regulatory submissions, manuscripts, or other clinical development activities.

Post-hoc exploratory analyses not identified in this SAP may be performed to further examine the study data. Such analyses will be clearly identified in the final clinical study report.

Additional analyses not prospectively identified in this SAP may also be completed for publications, or regulatory or funding inquiries. These analyses, if performed, may not be reported in the clinical study report, but will be fully documented in the document containing the additional analyses.

3.3. Summary of Statistical Analysis Changes to the Protocol

The analyses described in this analysis plan have been revised when compared to the analyses originally described in the study protocol (version 04 27-Apr-2020). This occurred due to the early termination of the study, and includes the following items:

- Populations for Analysis now includes only Safety Analysis Set (SAF).

- For the primary endpoint parameter, only individual profile platelet counts over time will be presented as Trellis plots. Not presented will be the number and proportion of responders together with the associated exact 95% confidence intervals.

- Time to platelet response as well as maximum platelet count and duration of response will be listed individually and will not be summarized in statistical tables. Vital signs will be listed only. Descriptive tables on the sampling statistics of these parameters at each time point and their changes from baseline will not be provided.

- Laboratory parameters will be listed only. Change from baseline will not be presented.

4. STUDY OBJECTIVES AND ENDPOINTS

Study objectives and endpoints defined in the protocol include efficacy and safety endpoints. Objectives and pre-specified endpoints are as follows:

4.1. Study Objectives

4.1.1. Primary Objective

- To evaluate efficacy of PANZYGA in increasing the platelet count in pediatric patients with chronic ITP.

4.1.2. Secondary Objectives

- Assess safety of up to 2 g/kg PANZYGA (1 g/kg PANZYGA on Day 1 and an optional dose of 1 g/kg PANZYGA on Day 3) infused in the pediatric population within the scope of the clinical study.
- Determine the time to reach a platelet count of $\geq 50x10^9$/L.
- Determine duration of time during which the platelet count is maintained at the level $\geq 50 \times 10^9$/L.
- Determine the maximum platelet count during the study.

4.2. Study Endpoints

Table 1 Primary and Secondary Endpoints

Primary Endpoints	**Parameters**
Efficacy	The number of patients with an elevation of platelet count at least once to $\geq 50x10^9$/L within 7 days [ie, Day 8] after the first infusion
Secondary Endpoint	**Parameters**
Efficacy	• Time to reach a platelet count of $\geq 50x10^9$/L • Duration of platelet response (number of days platelet count remains above $\geq 50x10^9$/L) • Maximum platelet count during the study
Adverse events	• TEAEs • Adverse drug reaction • Infusion AE
Vital signs	Blood pressure, heart rate, temperature, respiratory rate
Physical findings	General appearance, skin, heart, lungs, abdomen, extremities
Laboratory parameters	• Hematology: CBC with white blood cell, (WBC) differential, hematocrit, hemoglobin, platelet count, reticulocytes • Serum chemistry: ALT, AST, creatinine, Na, Ca, K, BUN, LDH • Hemolysis: total, direct, and indirect bilirubin.

5. STUDY METHODS

5.1. Overall Study Design

As background for the statistical methods presented below, this section provides an overview of the study design and plan of study execution. The protocol is the definitive reference for all matters discussed in what follows.

This is a prospective, open-label, single-arm, multicenter, Phase 4 study evaluating the efficacy and safety of PANZYGA in pediatric patients with chronic ITP.

The initial plan of this study was to enroll at least 20 patients ≥1 year to <18 years old, not stratified by age group. A patient is considered enrolled into the study after successfully completing all baseline assessments and receiving at least a partial dose of PANZYGA.

Patients with a confirmed diagnosis of chronic ITP, without evidence of active major bleeding, may be enrolled in the study after written approval of the patient by the Sponsor has been obtained. Study procedures will only begin after written informed consent (from parent or guardian) and assent (from the patient, as age appropriate per Institutional Review Board [IRB] requirements) have been obtained. Patients who meet all the inclusion and none of the exclusion criteria may receive the first infusion of PANZYGA within 2 days after Baseline investigations have been completed.

Each patient will be administered PANZYGA by intravenous infusion at a dose of 1 g/kg body weight PANZYGA on Day 1 and optionally another dose of 1 g/kg PANZYGA on Day 3; body-weight dependent doses will be based on each patient's weight collected at Baseline. Total PANZYGA doses will be 1 g/kg for patients receiving only 1 dose on Day 1 and 2g/kg for those receiving the optional Day 3 dose. Prior to the Day 3 infusion, Investigators will assess each patient's pre-infusion platelet count, hematocrit, and hemoglobin. If the Day 3 platelet count is $>50x10^9$/L, the Day 3 infusion will not be administered. If the Baseline platelet count is still $\leq 50x10^9$/L, the Day 3 infusion will be administered. If any parameters indicate a clinically relevant change such that, in the Investigator's opinion, it is not safe to administer the second infusion, the patient should be withdrawn from study treatment; however, the patient will be followed for safety through Day 32.

The study was planned to begin screening procedures Q3 2019 at up to 8 sites in the USA, with recruitment lasting approximately 30 months, and was initially anticipated to complete in Q1 2022. In Q4 2023 the study was terminated early with 6 patients enrolled. The study duration for a single patient was planned to be approximately 39 days, including up to a 1-week Screening period.

The schedule for assessments and timing of events is presented in Table 2.

Table 2 Flow Chart of Assessments

ASSESSMENTS	Scr / BL[1]	Day 1	Day 3	Day 5[12]	Day 8[12]	Day 15[12]	Day 22[12]	Day 32[13]
Visit Window (Days)	-7	0	0	±1	±1	±3	±3	±5
Informed Consent	X							
Inclusion / Exclusion Criteria Review	X	X						
Demographics	X							
Medical and Surgical History[2]	X							
Body Weight and Height	X							
Physical Examination	X[3]	X[3]	X[3]	X[4]	X[4]	X[4]	X[4]	X[3]
Urine or Blood Pregnancy Test (females of child-bearing potential)	X							X
Hematology: CBC with white blood cell (WBC) differential, hematocrit, hemoglobin, platelet counts[1], reticulocytes	X[1]	X[5,6]	X[5,6,7]	X	X	X	X	X
Hemolysis: total, direct, and indirect bilirubin	X	X	X	X	X	X	X	X
Serum Chemistry: ALT, AST, creatinine, Na, Ca, K, BUN, LDH	X	X	X	X	X			X
Viral markers (HIV, HCV, HBV nucleic acid test [NAT])	X[7]							
Receive Sponsor written approval prior to enrollment		X						
PANZYGA INFUSIONS		**X**	X[8]					
Vital Signs: heart rate, blood pressure, temperature, respiratory rate	X	X[5]	X[9]					X
AE Monitoring[10]		X	X	X	X	X	X	X
Prior and Concomitant Therapy (drug and non-drug)[11]		X	X	X	X	X	X	X

Abbreviations: BL = Baseline, Scr = Screening
1) All Screening Period/Baseline evaluations must be completed within 7 days of Day 1 with the following exception: Baseline samples for platelet count must be drawn and evaluated by the Investigator no more than 2 days before the Day 1 infusion. Note: If Screening/Baseline and Day 1 Visits occur on the same day, the Day 1 investigations need not be repeated.
2) Medical History will be collected for the previous year and will include the onset date of ITP. Surgical history will include the date of splenectomy (if applicable), and any other surgical procedures in the previous year.
3) Brief Physical Examination: general appearance, skin, heart, lungs, abdomen, extremities.
4) Targeted Physical Examination limited to targeted body systems per Investigator discretion.
5) Pre-infusion
6) Platelet count, hematocrit, and hemoglobin results must be available and reviewed by the Investigator prior to start of the infusion.
7) HIV/HCV/HBV NAT Test: Infusions may begin before the results are back if the Investigator is not aware of, and the patient isn't exhibiting clinical signs of, any viral infection.
8) If the Day 3 preinfusion platelet count >50x10^9/L, the Day 3 infusion will not be administered. If the Baseline platelet count is still ≤50x10^9/L, the Day 3 infusion will be administered.
9) Vital Signs: at the start of the infusion and every 60 minutes (±15 minutes) thereafter until the end of the infusion. In addition, vital sign measurements will be recorded just prior to every infusion rate change (-10 minutes), 15 minutes (+15 minutes) after each infusion rate change, and approximately 30 minutes (+15 minutes) after the end of the infusion.
10) The start date and start time will be collected for any adverse events starting at Day 1 through Day 32/EOS visit.
11) Prior medications will be collected for the 3 months. The start date and start time will be collected for any concomitant medications taken on Day 1 through Day 32/EOS visit.
12) Days 5, 8, 15, and 22 visits may be performed locally by patient's primary care physician or a local laboratory service upon agreement with the site Principal Investigator, the IRB, and the Sponsor.
13) Day 32 visit will correspond to the Early Termination visit for patients who are withdrawn early from the study.

5.2. Inclusion – Exclusion Criteria and Study Population

At least 20 female or male pediatric patients ≥1 year to <18 years old with chronic ITP patients who meet inclusion and exclusion criteria were originally planned to be enrolled. Note: Patients who turn 18 years old during study participation must be re-consented at their next study visit with an adult informed consent form. The inclusion and exclusion criteria defined in the protocol apply to all patients and are not repeated herein the SAP.

5.3. Randomization and Blinding

This is an open-label, non-randomized study; therefore, no randomization code list or procedure for code release are required. Patients will not be stratified by age group.

5.4. Analysis Variables

Variables to be analyzed include demographics, baseline characteristics, efficacy and safety variables as described throughout this SAP.

6. SAMPLE SIZE

The initial plan was to enroll at least 20 patients who meet all eligibility criteria for the study. However, when the study was terminated early in Q4 2023, a total of only 6 patients had been enrolled.

The purpose of this study is to evaluate the efficacy and safety of PANZYGA in pediatric patients with chronic ITP; the initially chosen number of 20 patients to be enrolled was not derived from statistical considerations of power but driven by feasibility constraints with respect to finding pediatric ITP patients eligible and willing to participate in this study.

The true proportion of responders is expected to be comparable to results from similar studies in adult patients as there is no published data or expert statement that would indicate otherwise.

As discussed in the protocol, even though the initially planned number of 20 pediatric patients was not sufficient 'to power' the study, it would still have allowed the Sponsor to gather enough clinical evidence to obtain a sound and meaningful medical assessment of the efficacy and safety of PANZYGA in pediatric patients with chronic ITP. This goal is of course further compromised by the achieved sample size of only 6 patients; the planned analyses are thus reduced to data listings only and a small number of summary tables.

7. GENERAL CONSIDERATIONS

7.1. Analysis Populations

Due to the low number of enrolled patients, only a single analysis population that comprises all enrolled patients will be used for reporting. This set of patients coincides with the initially planned Safety Analysis Set, and for reasons of simplicity we will continue to use this term.

7.1.1. Safety Analysis Set

Safety Analysis Set (SAF): All patients who received at least part of one infusion of PANZYGA.

7.2. Covariates and Subgroups

None planned.

7.3. Management of Analysis Data

7.3.1. Data Handling

Unscheduled laboratory tests will be included with the time of the nearest scheduled test. If there is a scheduled test and one or more unscheduled tests assigned to the same time point, the most conservative test (i.e., most extreme test result from a safety standpoint) will be used. All laboratory values, for all visits, will be provided in by-patient listings.

7.3.2. Missing Data

No replacement of missing data values is planned, but only observed results including platelet count) will be included in the analyses.

Every effort will be made to collect all data. However, despite best efforts, missing or incomplete data may be reported. All missing or partial data will be presented in the patient data listing, as they are recorded on the eCRF.

Patients lost to follow-up or withdrawn will be included in all data presentations up to the point of their last evaluation. Unless otherwise specified, as noted below, in general, no imputation of values for missing data will be performed.

7.3.2.1. Handling of Missing Date Values

Partial or missing dates and times will not be replaced.

If the start date and time of an AE are partially or completely missing, the AE will be assumed to be treatment-emergent if it cannot be definitely shown that the AE did not occur or worsen during the treatment emergent period (worst case approach).

A medication will be assumed to be concomitant if it cannot be definitely shown that the medication was not administered during the study participation of a patient.

7.3.2.2. Imputation Methods

No data will be imputed for this study. All data will be observed cases, without imputation.

7.3.3. Handling of Early Termination Visit Information

In the event that a patient is terminated early from this study, the early termination visit data will be analyzed at the closest scheduled visit. If the closest visit has valid data, the early termination data will be assigned to the next available visit.

7.3.4. Pooling of Investigational Sites

In general, the data from all study centers will be pooled together for analyses.

7.3.5. Coding Conventions for Events and Medications

Event/Medication	Coding/Mapping Convention
Adverse Events Coding	MedDRA version 26.1
Medical History	MedDRA version 26.1
Prior and Concomitant Medications	WHODDE version 2023 Q3

7.3.6. Baseline

Baseline is defined as the last measurement taken prior to first exposure to study drug, including Day 1 measurements taken pre-dosing. Note that Baseline platelet evaluations must be completed and evaluated by the Investigator no more than 2 days prior to the Day 1 visit.

7.3.7. Analysis Software

Data manipulation, tabulation of descriptive statistics, calculation of inferential statistics, and graphical representations will be performed primarily using SAS (release 9.4 or higher) for Windows. If the use of other software is warranted, the final clinical study report will detail what software was used and for what purposes.

7.3.8. Study Data

Study data identified in Flow Chart of Assessments (Table 1) are collected and source verified within the electronic data capture tool: DFDiscover Version 5.4.0. Laboratory results and units are collected in the EDC tool; site local laboratory ranges will be collected from the CRAs and entered by the Data Management CRO into a database separate from EDC. Local laboratory range data will be provided along with the EDC CRF data to the Statistical CRO.

7.4. Planned Study Analyses

7.4.1. Statistical Summaries: Descriptive and Inferential

As detailed in section 6, this study is not powered for inferential statistics.

All collected data will be presented in listings. In addition, a few summary tables will be prepared as appropriate; if appropriate, these tables will rather list the individual values of the 6 patients and not summary statistics.

7.4.2. Independent Data Monitoring Committee

The IDMC composed of hematology and/or critical care experts will review relevant data periodically during the study and will give advice on the continuation, modification, or termination of the study. A written study-specific procedure will define in detail the composition, responsibilities, and procedures of the IDMC.

7.4.3. Final Analysis

The treatment period with PANZYGA will be up to 3 days. The final study analysis will be completed after all patients have completed their final visit assessments at 32 days (±5) days and the database has been locked.

7.5. Multiple Testing Procedures

There will be no adjustments for multiplicity and no formal multiple testing procedures are to be implemented with this analysis plan.

8. SUMMARY OF STUDY DATA

8.1. Patient Disposition

Due to the low enrolment of patients (N=6), only one analysis population will be considered. In this plan, this population will be referred to as the safety analysis set (SAF).

A by-patient data listing of study completion information including the reason for premature study withdrawal will be presented.

8.2. Protocol Deviations

Protocol deviations will be listed for the SAF.

8.3. Demographics and Baseline Characteristics

All demographic and baseline information will be listed by patient.

8.4. Medical History

Patient medical and surgical history will be collected during the screening period. The dates and descriptions of past events will be documented in source documents and captured in the relevant eCRF. Medical history will be coded using the MedDRA (version 26.1). Patient medical history data will be presented in a listing.

8.5. Prior and Concomitant Medications

All prior and concomitant medications will be presented in listings that will also include the preferred term and the ATC-codes, level 2 and 4, coded with WHODrug DDE, version Q3/2023

A concomitant medication is defined as any medication taken after the start date and time of the Day 1 infusion. Concomitant medication will be collected up to the Day 32/EOS visit. A prior medication is defined as any medication taken and stopped prior to the date and time of the Day 1 infusion. Prior medications taken during the 3 months prior to first dose date will be reported.

8.6. Treatment Compliance

Study drug PANZYGA exposure will be listed in full detail for each of two possible treatment days for PANZYGA

9. EFFICACY ANALYSES

Variables for the evaluation of the primary and secondary objectives will be derived as initially planned, but not subjected to descriptive statistics in general. All derived variables will be included in data listings.

9.1. Platelet Count and Response Rate as Primary Endpoint

Platelet count data and change from baseline will be listed individually, including whether the patient is a responder, as defined below.

Individual profiles of platelet count over time will be presented as Trellis plots.

The primary endpoint parameter 'response' is defined as an increase in platelet count at least once to $\geq 50x10^9$/L within 7 days after the first infusion, i.e. by Day 8 (increase must occur at least once on any day up to and including Day 8). If a patient receives emergent ITP treatment other than PANZYGA prior to achieving a platelet count ≥50x109/L during this assessment period, the patient will be considered a non-responder for the primary endpoint analysis.

9.2. Time to Platelet Response (Platelet count ≥ 50 x 10^9/L)

Time to platelet response is defined as the time from first infusion to the first time reaching a platelet count of $\geq$ 50 x 10^9/L. Patients receiving emergent ITP treatment other than PANZYGA prior to achieving a platelet count ≥50x109/L will be considered as not achieving responder status.

Time to platelet response will be listed individually.

9.3. Duration of Platelet Response (Platelet Count Maintained ≥ 50 x 10^9/L)

Duration of platelet response is defined as the number of days the platelet count remains above $\geq$ 50 x 10^9/L. The duration of platelet response will be included in the listing of derived efficacy parameters.

9.4. Maximum Platelet Count

The maximum platelet will also be included in the listing of derived efficacy parameters.

10. SAFETY ANALYSES

Safety analyses will be performed on the SAF and will address the secondary objective. All safety data will be presented in listings. Unless otherwise noted in Section 7.3.1, unscheduled or repeated visit results will not be summarized but will be included in patient data listings.

10.1. Adverse Events

AEs will be collected from the start of the first infusion on Day 1. Any illnesses captured between signing the ICF and the first infusion will be captured under medical history.

An adverse event is defined as treatment-emergent if first onset or worsening occurs any time after start of the first infusion of PANZYGA through the end of the safety follow-up period. Only treatment-emergent adverse events (TEAEs) are accounted for in the analysis. All TEAEs collected during scheduled, unscheduled, and repeat visits will be included in the analysis.

An infusional AE is defined as any AE that occurs from the time of infusion and within the 72-hour period after end of infusion. An infusional AE flag will be derived rather than captured on the CRF.

An adverse drug reaction (ADR) is defined as any noxious and unintended response to an investigational medicinal product (IMP) causally related (i.e. at least possibly related) to any dose. The phrase 'response to an IMP' means that a causal relationship between the IMP and an AE carries at least a reasonable possibility (i.e. the relationship cannot be ruled out). The causality assessment will be included in the TEAE listing.

All adverse events will be coded using the Medical Dictionary for Regulatory Activities (MedDRA), version 26.1.

AE Severity grade will be assessed as mild, moderate, or severe.

All AEs reported will be included in patient level listings, also featuring the derived flags for treatment-emergency, and infusional AE. Because of the limited number of AEs reported no summary tables will be prepared.

10.2. Vital Signs, ECG, Laboratory Tests and Physical Examinations

Vital signs, laboratory results and PE will be listed only.

10.2.1. Vital Signs

All observed vital sign data will be listed by visit and time point, including systolic blood pressure, diastolic blood pressure, temperature, heart rate, and respiration rate. Note that vitals

can be collected more than once during a visit. Specifically, vital signs will be recorded at the start of the infusion and every 60 minutes (±15 minutes) thereafter until the end of the infusion. In addition, vital sign measurements will be recorded just prior to every infusion rate change (-10 minutes), 15 minutes (+15 minutes) after each infusion rate change, and approximately 30 minutes (+15 minutes) after the end of the infusion on Day 1 and Day 3.

10.2.2. Clinical Laboratory Evaluations

Listings of individual laboratory parameters for hematology, serum chemistry, hemolysis and viral markers will be presented by patient. These listings will include date collected, study day, the observed laboratory value, and the reference range if applicable.

10.2.3. Physical Examination

Physical examination data will be presented in listings.

10.3. Other Safety Measures

Safety data including urine and blood pregnancy data will be presented in listings.

If after study results are reviewed, or the IDMC or Sponsor recommend additional safety parameters or analyses be completed, they will be fully described and documented in the final CSR. The SAP will not be amended to complete any other safety measures identified post-hoc.

11. REPORTING CONVENTIONS

The following reporting conventions will be adopted for the presentation of study data. These conventions will enhance the review process and help to standardize presentation with common notations.

11.1 General Reporting Conventions

- All tables and data listings will be developed in Landscape Orientation, unless presented as part of the text in a CSR, where they may be in Landscape or Portrait Orientation.
- Figures will be presented in Landscape Orientation, unless presented as part of the text in a CSR, where they may be in Landscape or Portrait Orientation.
- Legends will be used for all figures with more than one variable or item displayed.
- Specialized text styles, such as bolding, italics, borders, shading, superscripted and subscripted text will not be used in tables, figures, and data listings unless they add significant value to the table, figure, or data listing.
- Only standard keyboard characters should be used in tables and data listings.

Special characters, such as non-printable control characters, printer-specific, or font-specific characters, will not be used on a table, figure, or data listing. Hexadecimal character representations are allowed (e.g., μ, α, β).

- The ICH numbering convention is to be used for all tables, figures, and data listings.
- All footnotes will be left justified and at the bottom of a page. Footnotes must be present on the page where they are first referenced. Footnotes should be used sparingly and must add value to the table, figure, or data listing. If more than four footnote lines are planned, then a cover page may be used to display footnotes.
- Missing values for both numeric and character variables will be presented as blanks in a table or data listing. A zero (0) may be used if appropriate to identify when the frequency of a variable is not observed.
- All date values will be presented as DD/MMM/YYYY format.
- All observed time values will be presented using a 24-hour clock HH:MM format.
- Time durations will be reported in mixed HH MM notation (e.g., 5h 32m, or 27h 52m). The use of decimal notation to present (display) time durations should be avoided (e.g. 0.083h = 5m) unless it is necessary to show the computation of time differences in a table, figure, or data listing, in which case both notations may be used to display the time duration.
- All analysis programs developed for a table, figure, or data listing display will be self-contained to facilitate transfer of programs to multiple computing environments and transfer to a regulatory agency (if requested).

11.2 Population Summary Conventions

- Since only one population will be analyzed (referring to as SAF) in this study, there will be no patient disposition. Because all tables, listings or figures refer to the SAF, this will not be specified on each page.

12 REFERENCES

[4] ICH E9 Expert Working Group. Statistical Principles for Clinical Trials: ICH Harmonized Tripartite Guideline., September 1998

13 APPENDICES

13.1 List of Planned Tables and Figures

Note: additional tables may be prepared if requested during the reporting phase.

Table 14.1 Primary Endpoint: Number of responders
Table 14.2 Listing of Derived Efficacy Parameters
Figure 14.3 Observed Platelet Count Through End of Treatment

13.2 List of Planned Listings

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