Statistical Analysis Plan

Protocol Number:	SB-318-1502
Title:	A Phase 1/2, Multicenter, Open-Label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of SB-318, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis I (MPS I)
Date of Protocol:	12Dec2019
SAP Version/ Date:	V2.0/ 22Jan2022
BB-IND:	16821
EudraCT:	2018-000206-28
NCT:	NCT02702115

Sangamo Therapeutics, Inc

SB-318-1502

A Phase 1/2, Multicenter, Open-Label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of SB-318, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis I (MPS I)

10Jan2022

Statistical Analysis Plan

Final 2.0

Prepared by:

PPD
3575 Quakerbridge Road, Suite 201
Hamilton, NJ 08619

PPD	PPD Biostatistics and Programming
	Statistical Analysis Plan (SAP) Client Approval Form

Client:	**Sangamo Therapeutics, Inc**
Protocol Number:	**SB-318-1502**

Document Description:	Final Statistical Analysis Plan and Shells
SAP Title:	A Phase I, Multicenter, Open-label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of SB-318, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis I (MPS I)
SAP Version Number:	SAP version 2.0, Shells version 4.0
Effective Date:	1/10/2022

Author(s):

For PPD: Lina Al Mufti, Senior Biostatistician

Approved by:

Lina Al Mufti
Senior Biostatistician
I approve this document
14 Jan 2022 15:05:43 +01:00

DocuSign

Lina Al Mufti, Senior Biostatistician **Biostatistics and Programming, PPD**	Date
DocuSigned by: Michael Chen Signer Name: Michael Chen Signing Reason: I approve this document Signing Time: 1/18/2022 | 11:53 PST 132A1FACD973421B934699D5FEC79D67 **Michael Chen, Senior Director** **Biometrics, Sangamo Therapeutics**	1/18/2022 | 11:53 PST Date
DocuSigned by: Maozhen Tian Signer Name: Maozhen Tian Signing Reason: I approve this document Signing Time: 1/18/2022 | 10:16 PST 0A4BCB22BFC0424098D7CE80AC1A7B40 **Maozhen Tian, Senior Manager** **Statistical Programming, Sangamo Therapeutics**	1/18/2022 | 10:16 PST Date

DocuSigned by:
Bettina Cockroft
Signer Name: Bettina Cockroft
Signing Reason: I approve this document
Signing Time: 1/18/2022 | 10:17 PST

Bettina Cockroft, Senior Vice President and Chief Medical Officer Medical Monitor, Sangamo Therapeutics	Date

DocuSign

Certificate Of Completion

Envelope Id: 855137EF1E6C4014B815650752BB8227
Status: Completed
Subject: Please DocuSign: Sangamo SB-318-1502 SAP and Shells Approval Form.pdf
Source Envelope:
Document Pages: 2
Signatures: 3
Envelope Originator:
Certificate Pages: 5
Initials: 0
Maozhen Tian
AutoNav: Enabled
501 Canal Blvd Ste A
EnvelopeId Stamping: Disabled
Richmond, CA 94804
Time Zone: (UTC-08:00) Pacific Time (US & Canada)
MTian@sangamo.com
IP Address: 4.53.139.193

Record Tracking

Status: Original
1/18/2022 10:08:03 AM
Holder: Maozhen Tian
MTian@sangamo.com
Location: DocuSign

Signer Events	Signature	Timestamp
Bettina Cockroft BCockroft@sangamo.com SVP & CMO Bettina Cockroft Security Level: Email, Account Authentication (Required)	Bettina Cockroft Signature Adoption: Pre-selected Style Signature ID: C958E0AE-B676-4A24-8B95-4170DD8203AA Using IP Address: 75.141.215.19 With Signing Authentication via DocuSign password With Signing Reasons (on each tab): I approve this document	Sent: 1/18/2022 10:15:52 AM Viewed: 1/18/2022 10:17:08 AM Signed: 1/18/2022 10:17:40 AM
Electronic Record and Signature Disclosure: Accepted: 11/9/2020 1:55:02 PM ID: 6227dc47-16e0-4f01-b87e-77ca1eb75fa5		
Maozhen Tian mtian@sangamo.com Sangamo Therapeutics, Inc Security Level: Email, Account Authentication (Required)	Maozhen Tian Signature Adoption: Pre-selected Style Signature ID: 0A4BCB22-BFC0-4240-98D7-CE80AC1A7B40 Using IP Address: 4.53.139.193 With Signing Authentication via DocuSign password With Signing Reasons (on each tab): I approve this document	Sent: 1/18/2022 10:15:52 AM Viewed: 1/18/2022 10:16:12 AM Signed: 1/18/2022 10:16:39 AM
Electronic Record and Signature Disclosure: Not Offered via DocuSign		

Signer Events	Signature	Timestamp
Michael Chen MChen@sangamo.com Sangamo Therapeutics, Inc Security Level: Email, Account Authentication (Required)	 Signature Adoption: Pre-selected Style Signature ID: 132A1FAC-D973-421B-9346-99D5FEC79D67 Using IP Address: 4.53.139.193 With Signing Authentication via DocuSign password With Signing Reasons (on each tab): I approve this document	Sent: 1/18/2022 10:15:52 AM Viewed: 1/18/2022 11:52:47 AM Signed: 1/18/2022 11:53:31 AM
Electronic Record and Signature Disclosure: Not Offered via DocuSign		

In Person Signer Events	Signature	Timestamp

Editor Delivery Events	Status	Timestamp

Agent Delivery Events	Status	Timestamp

Intermediary Delivery Events	Status	Timestamp

Certified Delivery Events	Status	Timestamp

Carbon Copy Events	Status	Timestamp

Witness Events	Signature	Timestamp

Notary Events	Signature	Timestamp

Envelope Summary Events	Status	Timestamps
Envelope Sent	Hashed/Encrypted	1/18/2022 10:15:52 AM
Certified Delivered	Security Checked	1/18/2022 11:52:47 AM
Signing Complete	Security Checked	1/18/2022 11:53:31 AM
Completed	Security Checked	1/18/2022 11:53:31 AM

Payment Events	Status	Timestamps

Electronic Record and Signature Disclosure

ELECTRONIC RECORD AND SIGNATURE DISCLOSURE

From time to time, Sangamo Therapeutics (we, us or Company) may be required by law to provide to you certain written notices or disclosures. Described below are the terms and conditions for providing to you such notices and disclosures electronically through the DocuSign system. Please read the information below carefully and thoroughly, and if you can access this information electronically to your satisfaction and agree to this Electronic Record and Signature Disclosure (ERSD), please confirm your agreement by selecting the check-box next to 'I agree to use electronic records and signatures' before clicking 'CONTINUE' within the DocuSign system.

Getting paper copies

At any time, you may request from us a paper copy of any record provided or made available electronically to you by us. You will have the ability to download and print documents we send to you through the DocuSign system during and immediately after the signing session and, if you elect to create a DocuSign account, you may access the documents for a limited period of time (usually 30 days) after such documents are first sent to you. After such time, if you wish for us to send you paper copies of any such documents from our office to you, you will be charged a $0.00 per-page fee. You may request delivery of such paper copies from us by following the procedure described below.

Withdrawing your consent

If you decide to receive notices and disclosures from us electronically, you may at any time change your mind and tell us that thereafter you want to receive required notices and disclosures only in paper format. How you must inform us of your decision to receive future notices and disclosure in paper format and withdraw your consent to receive notices and disclosures electronically is described below.

Consequences of changing your mind

If you elect to receive required notices and disclosures only in paper format, it will slow the speed at which we can complete certain steps in transactions with you and delivering services to you because we will need first to send the required notices or disclosures to you in paper format, and then wait until we receive back from you your acknowledgment of your receipt of such paper notices or disclosures. Further, you will no longer be able to use the DocuSign system to receive required notices and consents electronically from us or to sign electronically documents from us.

All notices and disclosures will be sent to you electronically

Unless you tell us otherwise in accordance with the procedures described herein, we will provide electronically to you through the DocuSign system all required notices, disclosures, authorizations, acknowledgements, and other documents that are required to be provided or made available to you during the course of our relationship with you. To reduce the chance of you inadvertently not receiving any notice or disclosure, we prefer to provide all of the required notices and disclosures to you by the same method and to the same address that you have given us. Thus, you can receive all the disclosures and notices electronically or in paper format through the paper mail delivery system. If you do not agree with this process, please let us know as described below. Please also see the paragraph immediately above that describes the consequences of your electing not to receive delivery of the notices and disclosures electronically from us.

How to contact Sangamo Therapeutics:

You may contact us to let us know of your changes as to how we may contact you electronically, to request paper copies of certain information from us, and to withdraw your prior consent to receive notices and disclosures electronically as follows:
To contact us by email send messages to: tseto@sangamo.com

To advise Sangamo Therapeutics of your new email address

To let us know of a change in your email address where we should send notices and disclosures electronically to you, you must send an email message to us at tseto@sangamo.com and in the body of such request you must state: your previous email address, your new email address. We do not require any other information from you to change your email address.

If you created a DocuSign account, you may update it with your new email address through your account preferences.

To request paper copies from Sangamo Therapeutics

To request delivery from us of paper copies of the notices and disclosures previously provided by us to you electronically, you must send us an email to tseto@sangamo.com and in the body of such request you must state your email address, full name, mailing address, and telephone number. We will bill you for any fees at that time, if any.

To withdraw your consent with Sangamo Therapeutics

To inform us that you no longer wish to receive future notices and disclosures in electronic format you may:

i. decline to sign a document from within your signing session, and on the subsequent page, select the check-box indicating you wish to withdraw your consent, or you may;

ii. send us an email to tseto@sangamo.com and in the body of such request you must state your email, full name, mailing address, and telephone number. We do not need any other information from you to withdraw consent.. The consequences of your withdrawing consent for online documents will be that transactions may take a longer time to process..

Required hardware and software

The minimum system requirements for using the DocuSign system may change over time. The current system requirements are found here: https://support.docusign.com/guides/signer-guide-signing-system-requirements.

Acknowledging your access and consent to receive and sign documents electronically

To confirm to us that you can access this information electronically, which will be similar to other electronic notices and disclosures that we will provide to you, please confirm that you have read this ERSD, and (i) that you are able to print on paper or electronically save this ERSD for your future reference and access; or (ii) that you are able to email this ERSD to an email address where you will be able to print on paper or save it for your future reference and access. Further, if you consent to receiving notices and disclosures exclusively in electronic format as described herein, then select the check-box next to 'I agree to use electronic records and signatures' before clicking 'CONTINUE' within the DocuSign system.

By selecting the check-box next to 'I agree to use electronic records and signatures', you confirm that:

- You can access and read this Electronic Record and Signature Disclosure; and
- You can print on paper this Electronic Record and Signature Disclosure, or save or send this Electronic Record and Disclosure to a location where you can print it, for future reference and access; and
- Until or unless you notify Sangamo Therapeutics as described above, you consent to receive exclusively through electronic means all notices, disclosures, authorizations, acknowledgements, and other documents that are required to be provided or made available to you by Sangamo Therapeutics during the course of your relationship with Sangamo Therapeutics.

TABLE OF CONTENTS

List of Abbreviations

AAV	adeno-associated virus
AAV2/6	adeno-associated virus serotype 2/6
AE	adverse event
AFP	alpha-fetoprotein
ALT	alanine aminotransferase (SGPT)
ALP	alkaline phosphatase
AST	aspartate aminotransferase (SGOT)
ATC	Anatomical Therapeutic Chemical
CBC	complete blood count
CRF	case report form
CSF	cerebrospinal fluid
CTCAE	Common Terminology Criteria for Adverse Events
DS	dermatan sulfate
ECG	electrocardiogram
ECHO	echocardiogram
ERT	enzyme replacement therapy
FVC	forced vital capacity
GAG	glycosaminoglycan
HBV	hepatitis B virus
HCV	hepatitis C virus
HIV	human immunodeficiency virus
hIDUA	human iduronidase
HS	heparin sulfate
HSCT	hematopoietic stem cell transplant
IDUA	iduronidase enzyme
ITT	intent-to-treat
IV	intravenous
JROM	Joint range of motion
LDH	lactate dehydrogenase
MedDRA	Medical Dictionary for Regulatory Activities
MPS I	Mucopolysaccharidosis I
MPS IH	Hurler syndrome
MPS IHS	Hurler-Scheie syndrome
MPS IS	Scheie syndrome
MRI	magnetic resonance imaging
PCR	polymerase chain reaction
PFT	Pulmonary function test
rAAV	recombinant adeno-associated virus
rAAV2/6	recombinant adeno-associated virus serotype 2/6
RBC	red blood cell
SAE	serious adverse event
SAP	statistical analysis plan
sEGFR	Soluble epidermal growth factor receptor
SMC	Safety Monitoring Committee

SOC	system organ class
TEAE	treatment-emergent adverse events
VG	vector genome
WASI-II	Wechsler Abbreviated Scale of Intelligence, Second Edition (Shapiro et al. 2015)
WBC	white blood cell
WHO	World Health Organization
ZFN	zinc finger nuclease
6MWT	6-minute walk test

1. Introduction

Mucopolysaccharidosis type I (MPS I) is a lysosomal storage disease caused by deficiency of α-L-iduronidase (IDUA). IDUA is an enzyme that is required for the degradation of the glycosaminoglycans (GAGs) dermatan sulfate (DS) and heparan sulfate (HS). The enzyme deficiency is the result of mutations in the gene encoding IDUA. The inability to degrade GAGs leads to their accumulation within lysosomes throughout the body, with consequent multiorgan dysfunction and damage. The clinical severity of MPS I depends on the nature of the mutational changes and the degree of residual IDUA enzyme activity.

According to the National Institute of Neurological Disorders and Stroke factsheet for MPS I, the estimated incidence is 1 in about 100,000 newborns for severe MPS I, 1 in about 500,000 newborns for attenuated MPS I, and 1 in about 115,000 newborns for individuals whose disease symptoms fall between severe and attenuated. It has been estimated that 50-80% of all MPS I patients present with the severe form of the disease (Muenzer et al. 2009).

Current therapies for MPS I include hematopoietic stem cell transplantation (HSCT) and enzyme replacement therapy (ERT). HSCT can prevent or reverse most clinical features and is recommended for those with the severe form of the disease (Hurler syndrome [MPS IH]). However, the reported mortality rate after HSCT is 15%, and the survival rate with successful engraftment is 56%, although recent improvements in HSCT procedure have improved overall outcomes (Boelens et al. 2013). Patients with the attenuated forms of the disease (Hurler-Scheie syndrome [MPS IHS], Scheie syndrome [MPS IS]) are treated with ERT using laronidase (recombinant human IDUA; Aldurazyme). The limitations of ERT include the need for life-long treatment; development of neutralizing antibodies; inability to cross the blood brain barrier; continued cardiac, orthopedic, and ocular complications; and the inconvenience of weekly intravenous (IV) infusions.

In this study, the objective and rationale for the proposed SB-318 investigational therapy is to abrogate or decrease the need for ERT using *in vivo* genome editing. The proposed treatment employs recombinant adeno-associated virus (rAAV) comprising engineered zinc finger nucleases (ZFNs) to site-specifically integrate a corrective copy of the human iduronidase enzyme (hIDUA) transgene into the genome of subjects' own hepatocytes *in vivo*. Integration of the hIDUA transgene is targeted to intron 1 of the albumin locus, resulting in stable, high level, liver-specific expression and secretion of hIDUA into the blood. Placement of the hIDUA transgene under the control of the highly expressed endogenous albumin locus is expected to provide permanent, liver-specific expression of hIDUA for the lifetime of an MPS I patient.

The purpose of the Statistical Analysis Plan (SAP) is to describe the analyses and data presentations for Sangamo's protocol SB-318-1502 (Amendment version 7). This SAP outlines the types of analyses that will address the study objectives and explains in detail how the data will be handled and analyzed. It contains the definitions of analysis sets and statistical methods for the analysis of endpoints. Since this is an exploratory Phase I study, all analyses will be descriptive and exploratory in nature. No hypotheses testing will be carried out.

2. Objectives

2.1. Primary Objective

The primary objective of this study is to evaluate the safety and tolerability of SB-318.

2.2. Secondary Objectives

The secondary objectives of this study are to evaluate:

- The change from baseline over time in the following assessments:
  - IDUA activity in blood
  - GAG testing in urine
  - Frequency of ERT administration.
- To evaluate AAV clearance

2.3. Exploratory Objectives

The exploratory objectives of this study are to:

- Evaluate the change from baseline over time in the following assessments:
  - GAG levels in tissues (including blood, liver tissue, and cerebrospinal fluid[CSF])
  - Gene modification at the albumin locus in the liver.
  - Imaging, functional, and neurocognitive testing related to MPS I.
  - Immune response to AAV2/6, ZFNs, and IDUA.

3. Investigational Plan

3.1. Overall Study Design and Plan

This is a Phase 1/2, multicenter, open-label, single-dose, dose-ranging study with sequentially enrolled age cohorts: age ≥18 (adult cohorts 1 through 3), age 12 to 17 (pediatric cohorts 4 and 5), and age 5 to 11 (pediatric cohorts 6 and 7). The duration of study participation will be approximately 39 months for each subject, including approximately 3 months for screening followed by 36 months for treatment and study follow-up.

Up to 27 subjects may be enrolled in this study

Subjects who satisfy all inclusion/exclusion criteria will be enrolled into one of the following treatment cohorts:

Cohort	ZFN 1 (SB-47171) (vg/kg)	ZFN 2 (SB-47898) (vg/kg)	hIDUA Donor (SB-IDUA) (vg/kg)	Total rAAV (vg/kg)
1	1.00E+12	1.00E+12	8.00E+12	1.00E+13
2	5.00E+12	5.00E+12	4.00E+13	5.00E+13
3	1.20E+13	1.20E+13	9.60E+13	1.20E+14
4	TBD	TBD	TBD	TBD
5	TBD	TBD	TBD	TBD
6	TBD	TBD	TBD	TBD
7	TBD	TBD	TBD	TBD

Subjects who received ERT prior to study enrollment will continue to receive ERT during the study and remain on their current schedule per standard of care unless they undergo protocol-specified ERT withdrawal per protocol. However, ERT will be omitted during the week of the SB-318 infusion to facilitate accurate baseline testing (e.g., of GAG levels in urine, blood, and of IDUA activity in blood) and to allow a week free of ERT after the SB-318 infusion.

Cohort #	Age Range (y)	Total Dose (vg/kg)	# Subjects
1	≥18	1.00E+13	1
2	≥18	5.00E+13	2
3	≥18	1.20E+14	2
4	12-17	TBD	2
5	12-17	TBD	2
6	5-11	TBD	2
7	5-11	TBD	2

For each dose cohort after Cohort 1, two subjects will be dosed at least 4 weeks apart. SMC review will occur after all subjects in each cohort have ≥ 4 weeks of safety data.

The pediatric cohorts will be enrolled only after review of cumulative adult safety data by the SMC. The starting dose for pediatric cohorts 4 through 7 will be decided based on SMC review of study data and must meet pre-defined safety criteria.

After being discharged from the hospital or acute care facility, study visits are scheduled on Day 7; Weeks 2, 4, 6, 8, 12, 16, 20, 24, 28, 32, 36, 40, 44, 48, and 52; and Months 15, 18, 21, 24, 27, 30, 33, and 36. Liver function tests (aspartate aminotransferase [AST], alanine aminotransferase [ALT], total and direct bilirubin, alkaline phosphatase (ALP), lactate dehydrogenase (LDH), albumin, and total protein levels) will be conducted for evaluation of AAV mediated immunogenicity twice a week during the first 20 weeks after SB-318 infusion, and then subsequently at all study visits.

3.2. Study Endpoints

3.2.1. Primary Endpoints

The primary endpoint of this study is the incidence of treatment-emergent AE's (including SAEs).

Additional safety evaluation will be included:

- Routine hematology, chemistry and liver function laboratory tests, vital signs, physical exam, ECG, ECHO, and concomitant medications.
- Monitoring of chimerism in post-HSCT subjects.
- Cranial nerve exam and muscle strength testing.
- Serial alpha-fetoprotein (AFP) testing and magnetic resonance imaging (MRI) of liver to evaluate for liver mass.

3.2.2. Secondary Endpoints

The following are secondary endpoints of this study:

1. Change from baseline over time in the following assessments:
    - IDUA activity measured in blood.
    - Total GAG, DS GAG, and HS GAG (expressed as a ratio to creatinine) measured in urine.
    - Monthly and annualized frequency and dose of Aldurazyme (or equivalent ERT).

2. AAV2/6 clearance measured by vector genomes in plasma, saliva, urine, stool, and semen by polymerase chain reaction (PCR).

3.2.3. Exploratory Endpoints

The following are exploratory endpoints for this study:

- Change from baseline over time in the following assessments:
    - Total GAG, DS GAG, and HS GAG measured in tissues (including blood, liver tissue, and CSF).
    - Percentage and durability of gene modification at the albumin locus in liver tissue obtained at biopsy.

- Forced vital capacity (FVC) measured by pulmonary function tests (PFTs).
- Distance walked measured by six-minute walk test (6MWT).
- Joint range motion (JROM).
- MRI of liver to evaluate liver and spleen volume.
- MRI of brain and cervical spine to evaluate clinical soft tissue and/or bone.
- Neurocognitive abilities by WASI-II, WPPSI-IV, or BSID-III, and by VABS-II.
- Eye exam for corneal clouding and vision testing.
- Histopathological exam of liver tissue
- Immune response to AAV2/6, ZFNs, and IDUA measured in serum

Immune response to ZFNs and IDUA have not been performed.

3.3. Treatments

SB-318 is a combination of 3 recombinant adeno-associated virus serotype 2/6 (rAAV2/6) vectors that encode:

- ZFN 1 (SB-47171): Left-side ZFN that targets base pairs 447-461 of the albumin locus relative to the transcription initiation site, labeled as SB-A6P-ZLEFT
- ZFN 2 (SB-47898): Right-side ZFN that targets base pair 468-485 of the albumin locus relative to the transcription initiation site, labeled as SB-A6P-ZRIGHT
- hIDUA Donor (SB-IDUA): DNA repair template that encodes a promotorless hIDUA transgene, labeled as SB-A6P-HRL

The prepared investigational product will be administered via IV infusions while the subject is in hospital or an acute care facility. Subjects will remain in the hospital or acute care facility for at least 24 hours after completion of SB-318 infusion for observation and will be discharged when all AEs and vital signs (temperature, heart rate, respiratory rate, and blood pressure) are stable.

3.4. Dose Modifications

No dose modifications are possible within an individual subject since this is a single infusion study.

4. General Statistical Considerations

Study days will be numbered relative to the date of SB-318 infusion. For assessments on or after the date of SB-318 infusion, the study day of events from SB-318 infusion is calculated as the date of event minus the date of infusion. For assessments prior to the date of SB-318 infusion, the study day of events from SB-318 infusion is calculated as the date of event minus the date of infusion. Thus, the study day of event before the date of the infusion will be negative number, and the study day of the date of infusion will be 0.

Baseline is defined as the last non-missing measurement prior to the SB-318 infusion. Where only date is available, the date of the collection must be prior to the date of SB-318 infusion. Where time is also available, the time of the collection must be prior to the time

of SB-318 infusion. For example, for vital signs parameters, measures that are taken at the latest time prior to the infusion will be defined as baseline values. Measurements that are obtained after infusion will be considered as post-baseline values. If the measurement of a variable is not made on a given subject prior to infusion, then that subject will be considered not to have a baseline value for that variable. Change from baseline is defined as post-baseline assessment minus baseline assessment.

Continuous data will be summarized using the following descriptive statistics: number of subjects (n), mean, standard deviation (SD), median, minimum and maximum, where appropriate. Categorical data will be summarized using the frequency count (n) and percentage (%) of subjects for each category, where appropriate.

Unless specified otherwise, all the summary tables will be presented by treatment cohort and overall, and all the collected data will be presented in the listings, and data displayed in the listings will be sorted by treatment cohort and subject identifier.

No imputation will be applied for missing data unless otherwise specified.

All analyses will be conducted using SAS® Version 9.3 or later. The Medical Dictionary for Regulatory Activities (MedDRA 23.0) will be used for coding adverse events. Concomitant medication data will be coded using the World Health Organization (WHO) Drug Dictionary.

4.1. Sample Size

This study will enroll up to 27 subjects (1 subject in Cohort 2 and 2 subjects in each of the other 6 cohorts with potential enrollment of approximately 2 additional subjects in any cohort).

The sample size for this study was not based on statistical considerations, but is considered sufficient to provide preliminary assessments of the safety and tolerability of SB-318 in subjects with MPS I, as well as biochemical changes related to the pathophysiology of MPS I. Subjects who prematurely discontinue the study prior to the 12 months of study follow-up (i.e., subjects who were enrolled but not dosed, were lost to follow-up, or discontinued prematurely for another reason) may be replaced at the discretion of Sangamo.

4.2. Analysis Set

4.2.1. Enrolled Set

The Enrolled set includes subjects who have signed the informed consent form and have met all inclusion and exclusion criteria.

4.2.2. Safety Set

The Safety set includes all subjects enrolled in the study and receive any portion of the SB-318 infusion.

All collected data used for table summaries will be based on Safety set unless otherwise specified.

5. Subject Disposition

5.1. Disposition

A disposition of subjects includes the number and percentage of subjects for the following categories: subjects who screen failed, subjects who were enrolled, subjects who received any portion of the SB-318 infusion, subjects who completed the study, and subjects who terminated early from the study. The disposition will be summarized by treatment cohort and overall. The percentages will be based on the number of subjects in each treatment cohort and overall. The reasons for early termination of study will also be summarized in the table.

Subject disposition data will be presented in a listing. A separate listing of reasons for screen failure will be provided. The number of screen fails and the screen criteria failed will be summarized in a table.

5.2. Protocol Deviations

Deviations from the protocol including violations of inclusion/exclusion criteria will be assessed as 'significant' in cooperation with the sponsor. Deviations will be defined prior to database lock. Protocol deviations will be presented in a listing and summarized by category. All COVID-19-related protocol deviations will be summarized by category and flagged in the corresponding listing.

6. Demographics and Baseline Characteristics

6.1. Demographics

The demographics will be summarized by treatment cohort and overall. The following variables will be included:

- Age (Years)
- Sex (Male, Female)
- Ethnicity (Hispanic or Latino, Not Hispanic or Latino)
- Race (African American, American Indian or Alaska Native, Asian, Native Hawaiian or Other Pacific Islander, White, Not Provided, or Other)
- Weight and Height at baseline prior to dosing

The demographics data will be presented in a listing as well.

6.2. Baseline Characteristics

6.2.1. Chest X-RAY

Chest X-Ray assessment will be performed at Screening visit only. Corresponding listing include clinically significant abnormal findings will be provided.

6.2.2. MPS I Gene Sequencing

Sample of MPS I gene sequencing will be collected at Screening visit only. The data will be presented in a listing.

6.2.3. Single-Nucleotide Polymorphism (SNP) Assay

Sample of SNP assay will be collected at Screening visit to identify polymorphisms in the ZFN-targeted region of the albumin locus. The data will be presented in a listing.

6.2.4. Viral Load

Testing for human immunodeficiency virus (HIV), hepatitis B virus (HBV), and hepatitis C virus (HCV) will be collected at Screening visit only. The data will be presented in a listing.

6.2.5. Neutralizing Antibodies to AAV2/6

The level of neutralizing antibodies to AAV2/6 will be collected at Screening visit only to assess the subject's pre-existing immune response to AAV2/6. Listing is provided to display the results.

6.3. Medical History

The number and percentage of subjects with a medical history will be summarized by treatment cohort and overall for each body system by system organ class (SOC) and preferred term (PT). Percentages will be calculated based on number of subjects for the Safety set. Corresponding listing will be provided.

MedDRA dictionary Version 23.0 will be used for reporting and will be described in the relevant table and listing footnotes.

7. Study Treatments

The 3 components of SB-318 (ZFN1, ZFN2, and IDUA Donor) will each be added to 200 mL of diluent and adjusted to 0.25% human serum albumin. Total infusion volumes will depend on subject's cohort assignment and body weight (kg). IV infusions will be administered while the subject is in the hospital or acute care facility. The subject will remain in the hospital or acute care facility for at least 24 hours after SB-318 infusion for observation and will be discharged when all AEs and vital signs (temperature, heart rate, respiratory rate, and blood pressure) are stable.

All infusion data including specific details will be presented in a listing.

8. Endpoint Analysis

The primary objective of this study is to evaluate the safety and tolerability of SB-318. All statistical summaries will be descriptive in nature (e.g., means, standard deviations, and percentages). All subjects who receive any portion of the SB-318 infusion will be included

in the analyses, even those who withdraw prematurely from the study. All results will be presented separately for each of the SB-318 dose levels.

8.1. Primary Endpoint Analysis

8.1.1. Adverse Events

A treatment-emergent adverse event (TEAE) is defined as any adverse event with an onset date on or after the date of the SB-318 infusion through the last study visit, whether or not it is considered causally related to the study drug.

Treatment Emergence:

Totally Missing Dates:

- If both the onset date and the end date are, then the adverse event is assumed to be treatment emergent.

Partially Missing Dates:

- If the onset date is missing and the non-missing end date is prior to the infusion date, then the AE is not considered treatment emergent.
- If the partial end date can be assumed to be prior to the infusion date (month and/or year before infusion date), then the AE is not considered treatment emergent.
- If the end date is after the infusion date or the partial end date can be assumed to be on or after the infusion date (based on month and/or year), then the AE is assumed to be treatment emergent.
- If the onset date is partial and can be assumed to be prior to the infusion date (based on the month and/or year) or the non-missing end date is prior to the infusion date then the AE is not considered treatment emergent.
- If the partial onset date can be assumed to be on or after the infusion date, then the AE is assumed to be treatment emergent.

All AEs will be coded according to the most recent version of the MedDRA. Severity will be categorized by toxicity grade according to the Common Terminology Criteria for Adverse Events (CTCAE) Version 4.03.

AEs not listed in the CTCAE version 4.03 will be evaluated by using the following criteria:

- Grade 1, Mild: Symptoms cause no or minimal interference with usual social and functional activities
- Grade 2, Moderate: Symptoms cause greater than minimal interference with usual social and functional activities
- Grade 3, Severe: Symptoms cause inability to perform usual social and functional activities

- Grade 4, Potentially Life-threatening: Symptoms cause inability to perform basic self-care functions OR medical or operative intervention indicated to prevent permanent impairment, persistent disability, or death
- Grade 5: Outcome of AE is death.

Incidence of Adverse Events

The incidence of AEs will be summarized in tables with count and percentage of subjects with AEs by system organ class (SOC) and preferred term (PT). Unless otherwise specified, at each level of SOC or preferred term, a subject with multiple events will only be counted once per SOC or preferred term. Percentages of subjects with AEs will be calculated out of the number of subjects in each summary cohort of the intent-to-treat set. Tables will be sorted by SOC alphabetically in in overall group and PT in decreasing order on total frequency order.

The following categories of AE will be summarized:

- TEAEs
- Severity (CTCAE Grade 1, 2, 3, 4 and 5) of TEAEs
- TEAEs by relationship to study drug
- Grade 3 or greater TEAEs
- Serious TEAEs
- Grade 3 or greater Serious TEAEs
- Grade 3 or greater TEAE related to study treatment
- TEAEs leading to study discontinuation
- TEAEs leading to death

All AEs will be presented in data listings. TEAEs and SAEs will be flagged in the listings.

Severity of Adverse Events

All AEs will be summarized by maximum severity (CTCAE Grade 1, 2, 3, 4 and 5), SOC, and preferred term. At each level of SOC or preferred term, a subject with multiple events will only be counted once by the maximum severity per SOC or preferred term. No imputation will be done for missing severity. Adverse Events with missing severity will be presented in tables and listings as is.

8.1.2. Clinical Laboratory Evaluations

The laboratory assessments include

- Hematology

- Urinalysis with microscopic examination
- Serum chemistry
- Liver panel
- Circulating AFP level.

All summaries will be based on the SI units, and missing values will not be imputed. Summary statistics of the observed values and change from baseline for all laboratory parameters except for Urinalysis, will be provided at the scheduled visits. The number and percentage of subjects with shift on extreme post-baseline values in low/normal/high from baseline will be presented in a shift table for selected laboratory parameters. Extreme laboratory value is the lowest or highest category a patient has post-baseline for the laboratory parameters. If a patient has a value below the normal range and a value above the normal range, the value furthest from the normal range will be chosen.

All laboratory data will also be presented in listings for each summary section with abnormal values flagged.

8.1.3. Vital Sign Measurements

Vital signs include height, weight, temperature, heart rate, respiratory rate, and blood pressure (systolic and diastolic) will be assessed overtime during the study. Descriptive statistics of observed values and change from baseline values will be provided at scheduled visits. The data collected will be summarized by visit. On day 0, where multiple repeat assessments are collected, both the maximum and minimum assessment for each subject will be presented in table summaries. A Corresponding listing will also be provided.

8.1.4. Physical Examination

Physical examination will be assessed overtime during the study. Results of physical examination at screening is categorized into normal, abnormal and not done. Corresponding listing will be provided.

8.1.5. Electrocardiogram

Electrocardiogram assessment will be performed overtime during the study. Descriptive statistics of the observed values for numeric parameters will be summarized by cohort and overall, for example, ventricular rate (beats/min), P-R interval(msec), QRS duration (msec), Q-T interval (msec) and Q-Tc interval (msec). The number and percentage of subjects will be summarized by treatment cohort and overall for categorical parameters, for example, normal sinus rhythm, QRS axis and ECG interpretation. Corresponding listing will be provided.

8.1.6. Echocardiogram

Echocardiogram assessment will be performed at Screening, Week 48, Month 24, and Month 36/end of study visit or early termination visit if applicable. Descriptive statistics of the observed values for left ventricular ejection fraction (%) will be summarized by cohort and overall. The number and percentage of subjects will be summarized by treatment cohort and overall for categorical results (normal, abnormal not clinically significant, abnormal clinically significant). Corresponding listing will be provided.

8.1.7. Concomitant Medications

The Principal Investigator will record all concomitant medications, including over-the-counter medicinal products, dietary supplements, herbal medications, and medications given in treatment of AEs, taken by a subject from Screening throughout the course of the study on the concomitant medications page in the subject's CRF. Subjects who received ERT prior to study enrollment should continue to receive ERT as per standard of care, except during the week of the SB-318 infusion, and such treatment should be recorded as concomitant medication. Treatment with prednisone or equivalent corticosteroid and pre-treatment with acetaminophen and diphenhydramine hydrochloride should also be recorded as concomitant medication. All CRF recorded concomitant medications will be used in summary and listing.

Concomitant medications will be coded using the WHO Drug Dictionary. The dictionary will be updated throughout the life of the project to allow for the most recent version of the dictionary to be used. The medication names will be coded according to the Anatomical Therapeutic Chemical (ATC) class level 4 and preferred terms provided in the dictionary.

Concomitant medications will be summarized by providing the number and percentage of subjects by ATC class level 4 and preferred term for each treatment cohort and overall. ATC classes will be sorted in decreasing order of frequency based on the total number of subjects who take each medication in the total column, while preferred terms within each drug class will be presented in decreasing order of frequency. In addition, the total number of medications and the number and percentage of subjects receiving at least 1 concomitant medication will also be presented. If a subject has multiple medications for a given preferred term the subject will only be counted once.

All concomitant medications will be presented in a listing.

8.1.8. Monitoring of chimerism in post-HSCT subjects

Chimerism assay will be performed at Baseline, Weeks 12 and 48, Month 36/end of study visit or early termination visit if applicable. Descriptive statistics of the observed values for T-cells % recipient, T-cells % donor, B-cells % recipient, B-cells % donor, myeloid cells % recipient and myeloid % donor will be summarized by cohort and overall. Corresponding listing will be provided.

8.1.9. Neurological cranial nerve exam and muscle strength testing

The neurological cranial nerve exam and muscle strength testing will be conducted over time during the study. The neurological cranial nerve exam includes oculomotor nerve, trochlear nerve and abduces nerve assessment, facial nerve assessment, accessory nerve assessment, and hypoglossal assessment. Each assessment has outcome categorized into normal, abnormal non clinical significant and abnormal clinical significant. The muscle strength testing includes scapular assessment, shoulder abduction, elbow flexion, and hand extension. The test result has 6 categories: no contraction, visible/palpable muscle contraction but no movement, movement with gravity eliminated, movement against gravity only, movement against gravity with some resistance, and movement against gravity with full resistance. The number and percentage of subjects in each test result category will be summarized by scheduled visit. Corresponding listing will be displayed.

8.1.10. MRI of Liver

Liver mass will be evaluated through MRI of liver at Screening, Week 48, Month 24 and Month 36/end of study visit or early termination visit if applicable. MRI findings are categorized in to normal and abnormal. The number and percentage of subjects in each category will be summarized by scheduled visit. Corresponding listing will be provided.

8.2. Secondary Endpoint Analysis

The secondary analysis is to evaluate change from baseline over time for the endpoints mentioned in Section 3.2.2.

At each sampling time point, the actual value and the change from baseline for IDUA activity and GAG levels in urine will be summarized using descriptive statistics by treatment cohort.

8.2.1. Plasma and leukocyte IDUA activity

Plasma and leukocyte IDUA activity will be assessed over time through the end of study visit or early termination visit if applicable. Descriptive statistics of the observed values and change from baseline will be provided at the scheduled visits. A corresponding listing and summary table will be provided.

8.2.2. Total GAG, DS GAG, and HS GAG

Total GAG, DS GAG, and HS GAG (expressed as a ratio to creatinine) measured in urine will be assessed over time from Screening till Month 36/end of study visit or early termination visit if applicable. Descriptive statistics of the observed values and change from baseline values will be provided at the scheduled visits. A corresponding listing and summary table will be provided.

8.2.3. ERT Administration and Withdrawal

ERT withdrawal may occur at any time after the Week 12 visit. ERT withdrawal may be repeated if it was found to be unsuccessful previously. Concomitant medications, adverse events, liver panels, GAG, and IDUA testing will be done at each ERT withdrawal monitoring visit. These will be displayed in the relevant listings.

Frequency of ERT administration will be summarized by the following timepoints:

- Prior to first dose of infusion
- Within the first 30 days of infusion
- After the first 30 days of infusion

8.2.4. AAV2/6 clearance in plasma, saliva, urine, stool, and semen

Presence and shedding of AAV2/6 vector DNA, by PCR in plasma, saliva, urine, stool and semen (males only) will be conducted over time from Baseline till Week 48. The level of vector genome from each type of sample will be summarized by visit. A corresponding listing and summary table will be provided.

8.3. Exploratory Endpoint Analysis

The exploratory analysis is to evaluate change from baseline over time for the endpoints mentioned in Section 3.2.3, unless otherwise indicated.

8.3.1. Gene modification at the albumin locus

Percentage and durability of gene modification at the albumin locus in liver tissue obtained at biopsy will be collected at Baseline, Weeks 24 and 48. A corresponding listing will be provided.

8.3.2. Forced vital capacity

Forced vital capacity measured by PFTs will be assessed from Screening till Month 36/end of study visit or early termination visit if applicable. Descriptive statistics of the observed values and change from baseline for FVC % and FVC absolute will be provided at the scheduled visits. Corresponding listing will be provided.

8.3.3. Distance walked measured by Six-Minute Walk Test

Six-Minute walk tests will be assessed over time from Baseline till Month 36/end of study visit or early termination visit if applicable. Descriptive statistics of the observed values for total distance walked (meters) and their corresponding change from baseline values will be provided at schedule visits. Corresponding listing will be provided to include all the parameters, which are total distance walked (meters), heart rate (beats/min) and oxygen

saturation (%) before 6MWT, at the end of 6 MWT and 2 minutes after completion of 6 MWT, overall dyspnea rate and overall fatigue rate before and after 6MWT.

8.3.4. Joint range of motion (ankle, knee, hip, and shoulder)

Joint range of motion for ankle, knee, hip and shoulder will be assessed over time from Baseline till Month 36/end of study visit or early termination visit if applicable. Range of motion is measured in degree for ankle dorsiflexion, knee flexion, knee extension, hip flexion, shoulder forward flexion, and shoulder abduction. Descriptive statistics of the observed values and change from baseline values will be provided at the scheduled visits. Corresponding listing will be provided.

8.3.5. Liver and Spleen Volume

Liver and spleen volume will be measured through MRI of liver at Screening, Week 48, Months 24 and 36/end of study visit or early termination visit if applicable. Exploratory analysis of liver and spleen volume will be performed by Sangamo.

8.3.6. MRI of brain and cervical spine

MRI of brain and cervical spine to evaluate clinical soft tissue and/or bone will be conducted at Baseline, Week 48, Months 24 and 36/end of study visit or early termination visit if applicable. Listing is provided to display the test results.

8.3.7. Neurocognitive abilities by WASI-II Testing

WASI-II testing will be assessed at Baseline, Week 24 and 48, Months 24 and 36/end of study visit, and early termination visit if applicable. Observed values and change of baseline values of T score for block design, vocabulary, matrix reasoning, and similarities, composite IQ score for verbal comprehension index, perceptual reasoning index and full-scale IQ will be summarized by descriptive statistics at scheduled visit. Corresponding listing will be provided.

8.3.8. Eye exam for corneal clouding and vision testing

Eye exam for corneal clouding and vision testing will be assessed at Baseline and Week 24 and 48, Months 24 and 36/end of study visit or early termination visit if applicable. Results for vision testing are interpreted as Normal or Abnormal. Results for corneal clouding exam are interpreted as Present or Absent. The frequency of test/exam results will be summarized by treatment cohort at each scheduled visit. Corresponding listing will be provided.

8.3.9. Histopathological exam of liver tissue

The histopathology result of liver biopsy is categorized to Normal or Abnormal. A corresponding listing and summary table will be provided.

8.3.10. Total GAG, DS GAG, and HS GAG measured in liver tissue and CSF

Lumbar puncture will be conducted to assess total GAG, DS GAG, and HS GAG in liver tissue and CSF at Baseline, Week 24 and 48. Exploratory analysis of GAG levels will be performed by Sangamo. Listing of sample collecting information will be provided.

8.3.11. Immune response to AAV2/6 and ZFNs

ZFN Immunogenicity and antibodies to AAV2/6 will be collected at Baseline, Week 4, 12, 24, 36, and 48, Month 18 and 24. Corresponding exploratory analysis will be performed by Sangamo. Listing with sample collection information will be provided.

8.3.12. AAV-related research biomarkers

Passive transfer AAV2/6, AAV2/8 serum neutralization and other AAV-related biomarker research assays will be assessed at Baseline. Corresponding exploratory analysis will be performed by Sangamo. Listing with sample collection information will be provided.

9. Other Analysis

9.1. Other Lumbar Puncture Results

Results include opening pressure (ml/Hg), glucose, protein, red blood cell (RBC), and white blood cell (WBC). Descriptive statistics of observed values and change from baseline values will be provided at scheduled visits. A corresponding listing will be provided.

9.2. ACTH stimulation (cosyntropin) test

The ACTH stimulation test will be conducted at Baseline and Week 20 only. The data will be presented in the listing.

9.3. Pregnancy Test

Urine pregnancy test will be conducted for females of childbearing potential at Screening and Baseline, at Day 0 if >7 days from Baseline. It will be performed until Week 48 or until 3 consecutive plasma samples are negative for AAV2/6, whichever happens first. If urine pregnancy test result is positive, equivocal or impractical, a serum pregnancy test will be performed to confirm. The data will be presented in a listing.

10. Safety Monitoring Committee

An independent SMC composed of appropriate medical and scientific expertise will provide oversight of the study. For the cohort safety review, the SMC will be convened after the completion of each cohort (at least four weeks after the last subject within that cohort has received the study drug) to determine if it is safe to escalate to the next dose cohort. For the rolling safety review, the SMC will meet to review all available safety data approximately every 4-6 months after the first Cohort Safety Review. These meetings may be combined with subsequent cohort safety review meetings as appropriate.

The SMC may be convened at any time if there are excessive or unexpected toxicities associated with the conduct of the protocol. Specifically, the SMC will be convened earlier if the following occurs:

- Any two Grade 2 AEs in the same SOC that last more than 2 weeks with treatment or any one Grade 3 or greater AE, if these AEs are not related to the primary MPS I disease or treatment of the MPS I disease.
- SAEs not related to the primary MPS I disease.
- Death of a subject.
- Development of a malignancy.

Data analyses for SMC review will be presented in a separate document.

When no further enrolling or dosing decisions are required of the SMC, the SMC will no longer meet. Sangamo will continue to review subject safety data on an ongoing basis.

11. Changes to the Statistical Analysis Section of the Protocol

Secondary endpoint, change of baseline over time of dose of Aldurazyme (or equivalent ERT), will no longer be summarized.

12. References

Shapiro EG, Nestrasil I, Rudser K, Delaney K, Kovac V, Ahmed A, Yund B, Orchard PJ, Eisengart J, Niklason GR, Raiman J, Mamak E, Cowan MJ, Bailey-Olson M, Harmatz P, Shankar SP, Cagle S, Ali N, Steiner RD, Wozniak J, Lim KO, Whitley CB. Neurocognition across the spectrum of mucopolysaccharidosis type I: Age, severity, and treatment. Mol Genet Metab. 2015 Sep-Oct;116(1-2):61-8.

Muenzer J, Wraith JE, Clarke LA; International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines. Pediatrics. 2009 Jan;123(1):19-29.

Boelens JJ, Aldenhoven M, Purtill D, Ruggeri A, Defor T, Wynn R, Wraith E, Cavazzana-Calvo M, Rovelli A, Fischer A, Tolar J, Prasad VK, Escolar M, Gluckman E, O'Meara A, Orchard PJ, Veys P, Eapen M, Kurtzberg J, Rocha V. Outcomes of transplantation using

various hematopoietic cell sources in children with Hurler syndrome after myeloablative conditioning. Blood. 2013 May 9;121(19):3981-7.

Sangamo Therapeutics, Inc

SB-318-1502

A Phase I, Multicenter, Open-label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of SB-318, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis I (MPS I)

10Jan2022

Statistical Analysis Plan Shells

Version 4

Prepared by:

PPD
3575 Quakerbridge Road, Suite 201
Hamilton, NJ 08619

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

Version	Date	Changes
1.0	11Aug2017	Initial draft
2.0	18Jun2018	Address comments and incorporate changes in protocol amendment 2 and 3
2.1	27Jul2018	Address sponsor comments and finalize
3	17Jun2020	Address sponsor comments and finalize
4	10Jan2021	Address sponsor comments and finalize

Table of Contents

Instructions

This document provides specifications for after-text and appendix tables, figures, and data listings.

Header

The following header should appear at the very top of each page of a table, a figure, or a data listing:

```
Sangamo Therapeutics, Inc
Study/Protocol: SB-318-1502
```

Footer

The following footer should appear at the very bottom of each page of a table, figure or listing generated in SAS:

```
Program path: \\area\Sangamo SBSB3181502\Programs\TLF\program name.sas
                                                    Executed: DDMMMYYYY hh:mm
```

Title

At least three (3) lines, in general, should be reserved for the whole title. The first line is for the table/listing/figure number; the second line is for the actual title (title). It is okay to wrap it to the third if needed; and the next line after the actual title is reserved for the analysis population descriptor (set). All titles should be centered, as shown in the following example:

```
Table 14.1.1
Disposition
Enrolled Set
```

Footnotes

- In general, a footnote serves as a brief explanation/clarification/definition/concept of a flag symbol or a character, an abbreviation, a terminology, etc., that appears in or related directly to the displayed content of a table/listing/figure. Detailed/technical elaboration of, for example, a mathematical/statistical formula, a statistical term/test, or an algorithm for deriving a parameter value, should be addressed in the text of the SAP.
- All footnotes should follow immediately after a horizontal solid line. There should be one and only one space between the last footnote and the footer.
- When an abbreviation (e.g. TEAE, SAE, ITT, etc.) appears first time in the whole set of T/L/Fs for a study, a footnote should be provided at least once; and it is up to the study statistician, study TA, and study programmer to decide whether there is a need to repeat the same footnote for the rest of T/L/Fs (if applicable).

- Each line of a complete footnote should end with a period. When a footnote needs more than 1 line, one (1) period is needed.
- If possible the footnotes should not exceed 7 lines.
- Footnotes should be in the format of "Note: followed by 1 space, then the footnote(s)" or "[x] followed by 1 space, then the footnote(s)", as shown in the following two examples:

```
Note: Percentages are calculated based on number of subjects in the
      enrolled set.

[1]Change from baseline: post-baseline value - baseline value. For the
   change from baseline, only subjects with a value at both baseline visit
   and the specific post-baseline visit are included.
```

Page Layout

- All output should be in landscape orientation. A margin of 1, 1, 1, and 1 inch should be on the top, right, left, and bottom, respectively.
- All efforts should be made to present all cohorts in one page.

Page Format

- There should be a solid line at the top of the tables and listings just below the title.
- There should be a solid line just below the column headings that runs completely across the width of the tables and listings.
- There should be a solid line at the bottom of the tables and listings just above the footnote(s) on every page.

Font

- The default font to be used in the actual study tables/listings should be Courier New 10 point.
- The use of Courier New 9 point is optional for some tables/listings and will be determined at the study level by the study statistician, and study programmer. However, it is recommended that this option be used primarily for data listings.

Descriptive Statistics

By default, descriptive statistics in this template covers: **n, Mean, Standard Deviation (SD), Median, Minimum (Min), and Maximum (Max)**. Unless specified in the actual table shells, the mean, median, and the upper and the lower limits of confidence interval (CI) should be displayed to the one more decimal place than the original data (derived analysis data). Standard deviation should be displayed to the two more decimal places than the original data (derived analysis data). The minimum and maximum should be displayed to the same number of decimal places as the original data.

Dates

- The date9. format will be used for all dates.
- Partial dates are presented as: MMMYYYY or YYYY or blank for missing.

Rounding for Percentage

Unless specified in the actual table shells for a study, all percentages will be rounded to 1 decimal place in all T/L/Fs except in the case of the percentage is 100, display (100).

Alignments

- It is recommended that descriptive statistics are aligned to the center in summary tables, as shown in the following example:

	Center Align
n	xxx
Mean (SD)	xx.x (xx.xx)
Median	xx.x
Min, Max	xx.x, xx.x

- Row header should be aligned to the left, with 2 spaces indented for sub-categories or statistics belong to that parameter, and 1 space indented for wrapped line.
- Column header should be bottom aligned.

Use of N versus n

- N = total number of subjects/subjects in the population set.
- n = total number of subjects/subjects in the specific category.
- If N is specified in the column heading then any reference to the number of subjects in the body should be small n, as shown in the following example:

Demographic Parameter	SB-318 1.00E+13 (N=XX)	SB-318 5.00E+13 (N=XX)	Total (N=XX)
Age (years)			
n	xxx	xxx	xxx
Mean (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
Median	xx.x	xx.x	xx.x
Min, Max	xx.x, xx.x	xx.x, xx.x	xx.x, xx.x

- If N is specified in the column heading and percentage calculation is needed, then N is used as the denominator of the calculation, unless otherwise specified, as shown in the following example:

System Organ Class Preferred Term	SB-318 1.00E+13 (N=xxx) n (%)	...
SYSTEM ORGAN CLASS #1	xx (xx.x)	
PREFERRED TERM #1	xx (xx.x)	
PREFERRED TERM #2	xx (xx.x)	

Notes for Tables

Missing category is only presented when it's applicable. For example, for any categorical summaries, if the percentages across all categories do not add up to 100%, add a "Missing" line with the missing counts.

Notes for Listings

1. Observed Dates/AE severity/Relationship to investigational product are used in data listings
2. Sort listings by Cohort group, and Subject ID unless otherwise specified.
3. There is no "/" if two dates are presented in one column.

Display of No Observations

For all outputs, when there are no observations to display, please display "There are no observations to display."

Display of long list categories

Add a group heading with "(cont.)" if it doesn't not fit on a page. For example, for the AE tables it is SOC and PT so if there are many PTs within a SOC that SOC should be repeated on the next page followed by "(cont.)".

Handling Missing AEDECOD and AEBODSYS

If AETERM is not missing or none, and the coded term (AEDECOD and AEBODSYS) are missing, then display as "**UNCODED**" for both PT and SOC. Please display '**UNCODED**' at the top unless otherwise specified.

Table 14.1.1.1
Disposition
Enrolled Set

	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
ALL SCREENED SUBJECTS	xx	xx	xx
SCREEN FAILURE SUBJECTS	xx (xx.x)	xx (xx.x)	xx (xx.x)
ENROLLED SET	xx (xx.x)	xx (xx.x)	xx (xx.x)
SAFETY SET	xx (xx.x)	xx (xx.x)	xx (xx.x)
COMPLETED	xx (xx.x)	xx (xx.x)	xx (xx.x)
EARLY TERMINATED	xx (xx.x)	xx (xx.x)	xx (xx.x)
PRIMARY REASON FOR EARLY TERMINATION [a]			
ADVERSE EVENT	xx (xx.x)	xx (xx.x)	xx (xx.x)
DEATH	xx (xx.x)	xx (xx.x)	xx (xx.x)
LOST TO FOLLOW-UP	xx (xx.x)	xx (xx.x)	xx (xx.x)
STUDY TERMINATED BY SPONSOR	xx (xx.x)	xx (xx.x)	xx (xx.x)
INVESTIGATOR DECISION	xx (xx.x)	xx (xx.x)	xx (xx.x)
SUBJECT WITHDRAWAL OF CONSENT	xx (xx.x)	xx (xx.x)	xx (xx.x)
PROTOCOL DEVIATION	xx (xx.x)	xx (xx.x)	xx (xx.x)
OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Percentages for screen failure subjects and Enrolled Set are calculated based on number of subjects screened. All other percentages are based on the Enrolled Set except indicated in footnote [a].
The Enrolled set includes all subjects who have signed the informed consent form and met all inclusion and exclusion criteria. The Safety set includes all subjects enrolled in the study and receive any portion of the SB-318 infusion.
[a]Percentages are based on early termination subjects.

Source Data: Listing 16.2.1.1

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

Table 14.1.1.2
Reason of not Eligible for Enrollment
Screen Failed Subjects

	n (%)
SUBJECTS WITH SCREEN FAILURE	xx
INCLUSION CRITERIA NOT MET	
Serious intercurrent illness or clinically significant organic disease (unleass secondary to MPS I) such as cardiovascular, hepatic, pulmonary, neurologic, or renal disease.	xx (xx.x)
xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xx (xx.x)
EXCLUSION CRITERIA NOT MET	
xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xx (xx.x)
xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xx (xx.x)
…	

Note: The denominator for exclusion reasons will be the total number of subjects with screen failure.
Subjects could be excluded for more than one reason.
Inclusion/Exclusion criteria are based on the criteria listed in the protocol.
Source Data: Listing 16.2.1.2

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

Table 14.1.2
Demographics and Baseline Characteristics
Safety Set

	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
AGE (Years)			
n	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx
SEX, n (%)			
MALE	xx (xx.x)	xx (xx.x)	xx (xx.x)
FEMALE	xx (xx.x)	xx (xx.x)	xx (xx.x)
RACE, n (%)			
AMERICAN INDIAN OR ALASKA NATIVE	xx (xx.x)	xx (xx.x)	xx (xx.x)
ASIAN	xx (xx.x)	xx (xx.x)	xx (xx.x)
ASIAN, OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)
JAPANESE	xx (xx.x)	xx (xx.x)	xx (xx.x)
BLACK OR AFRICAN AMERICAN	xx (xx.x)	xx (xx.x)	xx (xx.x)
NATIVE HAWAIIAN OR OTHER PACIFIC ISLANDER	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT PROVIDED	xx (xx.x)	xx (xx.x)	xx (xx.x)
OTHER	xx (xx.x)	xx (xx.x)	xx (xx.x)
WHITE	xx (xx.x)	xx (xx.x)	xx (xx.x)

Source Data: Listing 16.2.2.1 and 16.2.2.2

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

Table 14.1.2
Demographics and Baseline Characteristics
Safety Set

	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
ETHNICITY, n (%)			
HISPANIC OR LATINO	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT HISPANIC OR LATINO	xx (xx.x)	xx (xx.x)	xx (xx.x)
NOT PROVIDED	xx (xx.x)	xx (xx.x)	xx (xx.x)
HEIGHT (cm)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
WEIGHT (kg)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
CHEST X-RAY RESULT AT SCREENING, n(%)			
NORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)
ABNORMAL	xx (xx.x)	xx (xx.x)	xx (xx.x)

Source Data: Listing 16.2.2.1 and 16.2.2.2

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

Table 14.1.3
Protocol Deviations
Safety Set

Deviation Type	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
NUMBER OF SUBJECTS WITH AT LEAST ONE PROTOCOL DEVIATION	xx (xx.x)	xx (xx.x)	xx (xx.x)
PROTOCOL DEVIATION			
CATEGORY 1	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 2	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 3	xx (xx.x)	xx (xx.x)	xx (xx.x)
…			
NUMBER OF SUBJECTS WITH AT LEAST ONE COVID-19-RELATED PROTOCOL DEVIATION	xx (xx.x)	xx (xx.x)	xx (xx.x)
COVID-19-RELATED PROTOCOL DEVIATION			
CATEGORY 1	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 2	xx (xx.x)	xx (xx.x)	xx (xx.x)
CATEGORY 3	xx (xx.x)	xx (xx.x)	xx (xx.x)
…			

Note: Subjects could have more than one protocol deviation.
Source Data: Listing 16.2.1.3

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

Table 14.1.4
Medical History
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
SUBJECTS WITH ANY MEDICAL HISTORY	xx (xx.x)	xx (xx.x)	xx (xx.x)
SYSTEM ORGAN CLASS # 1	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM # 1	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM # 2	xx (xx.x)	xx (xx.x)	xx (xx.x)
SYSTEM ORGAN CLASS # 2	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM # 1	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM # 2	xx (xx.x)	xx (xx.x)	xx (xx.x)
…			

Note: At each level of patient summarization, a patient is counted once if the patient reported one or more findings.
Medical history was coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.3.1

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

Prog Note: Medical history will be sorted ***alphabetically by System Organ Class*** *and then by* ***descending frequency of Preferred Term***

Table 14.1.5
Concomitant Medications
Safety Set

Note: Patients may have more than one medication per ATC level 4 category and preferred term. At each level of patient summarization, a patient is counted once if the patient reported one or more medications.
Concomitant medication is defined as any medication, including over-the-counter medicinal products, dietary supplements, herbal medications, and medications given in treatment of AEs, taken by a subject from Screening throughout the course of the study. Subjects who received ERT prior to study enrollment should continue to receive ERT as per standard of care, except during the week of the SB-318 infusion, and such treatment should be recorded as concomitant medication. Treatment with prednisone or equivalent corticosteroid and pre-treatment with acetaminophen and diphenhydramine hydrochloride should also be recorded as concomitant medication.
Concomitant medications were coded with the WHO Drug dictionary dated XXXXXX, 20XX.
ATC classes are sorted in decreasing order of frequency based on the total number subjects who take each medication in the total column. Preferred terms within each drug class are sorted alphabetically
Source Data: Listing 16.2.4.2, Listing 16.2.4.3

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

Table 14.1.5
Concomitant Medications
Safety Set

ATC Level 4 Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
TOTAL NUMBER OF CONCOMITANT MEDICATIONS	xx	xx	xx
NUMBER OF PATIENTS WITH AT LEAST ONE CONCOMITANT MEDICATION	xx (xx.x)	xx (xx.x)	xx (xx.x)
ATC LEVEL 4 #1	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #1	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #2	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #3	xx (xx.x)	xx (xx.x)	xx (xx.x)
PREFERRED TERM #4	xx (xx.x)	xx (xx.x)	xx (xx.x)

Notes are listed on page 1.

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

Prog Note: Medications will be sorted ***in descending order by ATC level 4 category based on the total of all treatment groups.*** *Within each ATC level 4 category, preferred terms will be sorted* ***in decreasing order of frequency based on the total of all treatment groups.***

Table 14.1.6
Summary of Enzyme Replacement Therapy Administration
Safety Set

Cohort	Subject ID	Number of ERT Administrations		
		Prior to First Dose of SB-318 Infusion	0 – 30 Days Post SB-318 Infusion	> 30 Days Post SB-318 Infusion
SB-318 1.00E+13 vg/kg	XXXXXX	XXX	XXX	XXX
SB-318 5.00E+13 vg/kg	XXXXXX	XXX	XXX	XXX

Source Data: Listing 16.2.4.2

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

Table 14.2.1
Change from Baseline in Plasma and Leukocyte IDUA Activity
Safety Set

Parameter: Alpha Iduronidase Plasma (nmol/hr/mL)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.3

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

Prog Notes: Repeat for Alpha Iduronidase *Leukocyte (nmol/hr/mL) paramter. Post baseline visits include Day 7, Weeks 2, 4, 6, 8, 12, 16, 20, 24, 36, 48, Months 15, 18, 21, 24, 27, 30, 33, 36/ EOS, or Early Termination Visit if applicable.*

Table 14.2.2
Change from Baseline in Urine GAG Levels and Urine GAG/Creatinine Ratio
Safety Set

Parameter: Total Glycosaminoglycans (GAGs)(mg/mmol creatinine)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.4

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

Prog Notes: Post-baseline visits to be summarized include Weeks 4, 8, 12, 16, 20, 24, 28, 32, 36, 40, 44, 48, 52, Months 15, 18, 21, 24, 27, 30, 33, 36/EOS, or Early Termination Visit if applicable. Repeat for parameters in the following PARAMCD order: CREAT, MM1DR, MM1DU, MM1HR, and MM1HU.

Table 14.2.3
Change from Baseline in AAV2/6 Clearance
Safety Set

Parameter: AAV2/6-hIDUA (Copies/10 ul Plasma)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.6.1

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

Prog Notes:
1. Please repeat for test AAV2/6-ZFN 47171 and other specimen (Saliva, Urine, Stool and Semen (Males Only)).
2. Post-baseline visit to be summarized include Day 7, Weeks 2, 4, 8, 12, 16, 20, 24, 36, 48.
3. Display visit Day 1 (12hr post-infusion) for Plasma only.

Table 14.2.4
Change from Baseline in Forced Vital Capacity (FVC)
Safety Set

Parameter: FVC (%)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.7

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

Prog Notes: Please repeat for parameter FVC absolute. Post-baseline visit to be summarized include Weeks 24, 48, Months 18, 24, 30, 36/EOS, or Early Termination Visit if applicable.

Table 14.2.5
Change from Baseline in Total Distance (meter) Walked in Six-Minute Walk Test
Safety Set

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.8

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

Prog Notes:
1. Post-baseline visit to be summarized include Weeks 24, 48, Months 18, 24, 30, 36/EOS, or Early Termination Visit if applicable.

Table 14.2.6
Change from Baseline in Joint Range of Motion (JROM)
Safety Set

Parameter: Shoulder forward flexion (degrees)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.9

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

Prog Notes:
1. Please repeat for parameters Shoulder abduction, Hip flexion, Knee flexion, Knee extension, Ankle dorsiflexion. All the parameters should with the same unit (degrees).
2. Post-baseline visit to be summarized include Weeks 24, 48, Months 18, 24, 30, 36/EOS, or Early Termination Visit if applicable.

Table 14.2.7
Change from Baseline in WASI-II Neurocognitive Test
Safety Set

Parameter: T score for block design

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.11

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

Prog Notes:
1. Please repeat for parameters T score for vocabulary, T score for matrix reasoning, T score for similarities, Composite IQ score for verbal comprehension index, Composite IQ score for perceptual reasoning index and Composite IQ score for full scale IQ. Do not repeat for Raw Scores.
2. Post-baseline visits to be summarized include Weeks 24, 48, Months 24, 36/EOS, or Early Termination Visit if applicable.

Table 14.2.8
Summary of Visual Acuity Test and Corneal Clouding Exam
Safety Set

Visit Parameter	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
BASELINE			
Visual Acuity Test n(%)			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Corneal Clouding Exam n(%)			
Left Eye			
Present	xx (xx.x)	xx (xx.x)	xx (xx.x)
Absent	xx (xx.x)	xx (xx.x)	xx (xx.x)
Right Eye			
Present	xx (xx.x)	xx (xx.x)	xx (xx.x)
Absent	xx (xx.x)	xx (xx.x)	xx (xx.x)
VISIT X			
Visual Acuity Test n(%)			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)
…			

Note: Baseline is defined as last non-missing measurement prior to dosing.
Source Data: Listing 16.2.9.12

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

Prog Notes: Visit to be summarized include Baseline, Weeks 24, 48, Months 24, 36/EOS, or Early Termination Visit if applicable.

Table 14.2.9
Summary of Histopathology Result of Liver Biopsy
Safety Set

Visit	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
BASELINE			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)
VISIT X			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)

Note: Baseline is defined as last non-missing measurement prior to dosing.
Source Data: Listing 16.2.9.5.1

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

Prog Notes: Visits to be summarized include Baseline, Weeks 24, 48.

Table 14.2.10
Change from Baseline in Lumbar Puncture
Safety Set

Parameter: Opening Pressure (ml/Hg)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.9.13

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

Prog Notes: Repeat for parameters glucose, protein, RBC and WBC. Post baseline visits include Week 24 and 48.

Table 14.3.1.1
Treatment-Emergent Adverse Events by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)		xx (xx.x)		xx (xx.x)	
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…		xx		xx		xx
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.1.2
Treatment-Emergent Adverse Events by Severity
Safety Set

	SB-318 1.00E+13 vg/kg (N=xxx)											
System Organ Class	Grade 1		Grade 2		Grade 3		Grade 4		Grade 5		Missing	
Preferred Term	n (%)	T	n (%)	T	n (%)	T	n (%)	T	n (%)	T	n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…												

n = number of subjects experiencing the event. T = total number of events.
Note: GRADE 1=MILD, GRADE 2=MODERATE, GRADE 3=SEVERE, GRADE 4=POTENTIALLY LIFE THREATENING, GRADE 5=FATAL
The total number of treatment-emergent AEs counts all treatment-emergent AEs for subjects. At each level of subject summarization, a subject is counted once for the most severe event if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version XX.X.
Source Data: Listing 16.2.7.1

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

Prog Note:
1. Following SB-318 1.00E+13 vg/kg, display SB-318 5.00E+13 vg/kg and Total in the next pages.
*2. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

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

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	 T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	 T	Total (N=xxx) n (%)	 T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
NOT RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
NOT RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
NOT RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
NOT RELATED	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						

n = number of subjects experiencing the event. T = total number of events.

Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs for subjects. At each level of subject summarization, a subject is counted once for the most related event if the subject reported one or more events. Percentages are based on the number of subjects within each cohort. Adverse Events were coded using MedDRA, Version ***XX.X***.
Source Data: Listing 16.2.7.1

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

Prog Note:
1. AEs will be sorted ***alphabetically by SOC****. Within each SOC, AEs will be sorted in* ***decreasing frequency of Preferred Term, based of the total treatment column****.*

Table 14.3.1.4
Treatment-Emergent Adverse Events with Severity Grade 3 or Greater by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT WITH SEVERITY GRADE 3 OR GREATER	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs with severity grade 3 or greater for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

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

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE SERIOUS TREATMENT-EMERGENT ADVERSE EVENT	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of serious treatment-emergent AEs counts all serious treatment-emergent AEs for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.2.2
Serious Treatment-Emergent Adverse Events with Severity Grade 3 or Greater
by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE SERIOUS TREATMENT-EMERGENT ADVERSE EVENT WITH SEVERITY GRADE 3 OR GREATER	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of serious treatment-emergent AEs counts all serious treatment-emergent AEs with severity Grade 3 or greater for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.3.1
Treatment-Emergent Adverse Events Related to Study Treatment with Severity Grade 3 or Greater by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT RELATED TO STUDY TREATMENT WITH SEVERITY GRADE 3 OR GREATER	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs related to study treatment with severity Grade 3 or greater for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.3.2
Treatment-Emergent Adverse Events Leading to Study Discontinuation by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT LEADING TO STUDY DISCONTINUTATION	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	x
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs leading to study discontinuation for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.3.3
Treatment-Emergent Adverse Events Leading to Death by System Organ Class and Preferred Term
Safety Set

System Organ Class Preferred Term	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	T	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	T	Total (N=xxx) n (%)	T
NUMBER OF SUBJECTS WITH AT LEAST ONE TREATMENT-EMERGENT ADVERSE EVENT LEADING TO DEATH	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
SYSTEM ORGAN CLASS #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
SYSTEM ORGAN CLASS #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #1	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
PREFERRED TERM #2	xx (xx.x)	xx	xx (xx.x)	xx	xx (xx.x)	xx
…						
…						

n = number of subjects experiencing the event. T = total number of events.
Note: The total number of treatment-emergent AEs counts all treatment-emergent AEs leading to death for subjects. At each level of subject summarization, a subject is counted once if the subject reported one or more events. Percentages are based on the number of subjects within each cohort.
Adverse Events were coded using MedDRA, Version *XX.X*.
Source Data: Listing 16.2.7.1

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

Prog Note:
*1. AEs will be sorted **alphabetically by SOC**. Within each SOC, AEs will be sorted in **decreasing frequency of Preferred Term, based of the total treatment column**.*

Table 14.3.4.1
Change from Baseline in Hematology
Safety Set

Parameter: LAB TEST #1 (UNIT)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.8.1.1

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

Prog Note: Lab test parameters will be displayed in the same order as the lab test listing. The post-baseline visits include Day 1, Weeks 2, 4, 6, 8, 12, 16, 20, 24, 36, 48, Months 15, 18, 21, 24, 27, 30, 36/EOS, or Early Termination Visit if applicable.

Table 14.3.4.2
Change from Baseline in Serum Chemistry
Safety Set

Parameter: LAB TEST #1 (UNIT)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual **Value**	Change From **Baseline [1]**	Actual **Value**	Change From **Baseline [1]**	Actual **Value**	Change From **Baseline [1]**
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.8.1.3

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

Prog Note: Lab test parameters will be displayed in the same order as the lab test listing. Include all post-baseline visits.

Table 14.3.4.3
Change from Baseline in Circulating Alpha Fetoprotein Level (ng/mL)
Safety Set

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

Note: Baseline is defined as last non-missing measurement prior to dosing.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline visit and the specific post-baseline visit are included.
Source Data: Listing 16.2.8.1.4

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

Prog Note: The post-baseline visits include Weeks 4, 8, 12, 24, 48, Months 18, 24, 30, 36/EOS, or Early Termination Visit if applicable.

Table 14.3.4.4
Shift from Baseline to Worst Post-Baseline for Laboratory Parameters – Serum Chemistry
Safety Set

Parameter: LAB TEST #1 (UNIT)

Cohort Baseline Value	Baseline n (%)	Extreme Value [1] Low n (%)	 Normal n (%)	 High n (%)
SB-318 1.00E+13 vg/kg (N=xxx)				
Low	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
High	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
Total	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
SB-318 5.00E+13 vg/kg (N=xxx)				
Low	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…				

Note: Baseline is defined as last non-missing measurement prior to dosing.
Counts represent patients with a baseline value in addition to having at least one value at post-baseline visits. Baseline percentage is based on N. Percentage for extreme value is based on Baseline n.
'Low' denotes a value below the normal range. 'High' denotes a value above the normal range.
[1] Extreme laboratory value is the lowest or highest category a patient has post-baseline for the laboratory parameter. If a patient has a value below the threshold and above the threshold, the value furthest from the threshold will be chosen.
Source Data: Listing 16.2.8.1.3

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

Table 14.3.4.5
Shift from Baseline to Worst Post-Baseline for Laboratory Parameters - Hematology
Safety Set

Parameter: LAB TEST #1 (UNIT)

Cohort Baseline Value	Baseline n (%)	Extreme Value [1] Low n (%)	 Normal n (%)	 High n (%)
SB-318 1.00E+13 vg/kg (N=xxx)				
Low	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
High	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
Total	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
SB-318 5.00E+13 vg/kg (N=xxx)				
Low	xx (xx.x)	xx (xx.x)	xx (xx.x)	xx (xx.x)
…				

Note: Baseline is defined as last non-missing measurement prior to dosing.
Counts represent patients with a baseline value in addition to having at least one value at post-baseline visits. Baseline percentage is based on N. Percentage for extreme value is based on Baseline n.
'Low' denotes a value below the normal range. 'High' denotes a value above the normal range.
[1] Extreme laboratory value is the lowest or highest category a patient has post-baseline for the laboratory parameter. If a patient has a value below the threshold and above the threshold, the value furthest from the threshold will be chosen.
Source Data: Listing 16.2.8.1.1

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

Table 14.3.5
Change from Baseline in Vital Signs
Safety Set

Parameter: VITAL SIGN PARAMETER #1 (UNIT)

	SB-318 1.00E+13 vg/kg (N=xx)		SB-318 5.00E+13 vg/kg (N=xx)		Total (N=xx)	
Visit Timepoint	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]	Actual Value	Change From Baseline [1]
BASELINE						
n	xx		xx		xx	
MEAN (SD)	xx.x (xx.xx)		xx.x (xx.xx)		xx.x (xx.xx)	
MEDIAN	xx.x		xx.x		xx.x	
MIN, MAX	xx, xx		xx, xx		xx, xx	
DAY 0						
MAXIMUM						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx
MINIMUM						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx
VISIT X						
n	xx	xx	xx	xx	xx	xx
MEAN (SD)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)	xx.x (xx.xx)
MEDIAN	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
MIN, MAX	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx	xx, xx

…

Note: Baseline is defined as last non-missing measurement prior to dosing. The data collected will be summarized by visit. On day 0, where multiple repeat assessments are collected, both the maximum and minimum assessment for each subject are presented.
[1]Change from baseline: post-baseline value - baseline value. For the change from baseline, only subjects with a value at both baseline and the specific post-baseline timepoint are included.
Source Data: Listing 16.2.8.2

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

Prog Note: Vital sign parameters will be displayed in the same order as the vital sign listing.
The post-baseline visits include Day 0, Day 1, Day 7, Weeks 2, 4, 6, 8, 12, 16, 20, 24, 36, 48, Months 15, 18, 21, 24, 27, 30, 33, 36/EOS, or Early Termination Visit if applicable. Minimum and Maximum assessment rows are included for Day 0 only for all parameters except weight. For weight page, summarize Day 0 in one row, as woulud be done for any other Visit.

Table 14.3.6
Summary of Electrocardiogram
Safety Set

Visit Parameter	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
Baseline			
Ventricular Rate (beats/min)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
P-R Interval (msec)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
QRS Duration (msec)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
Q-T Interval (msec)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx

Source Data: Listing 16.2.8.4

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

Table 14.3.6
Summary of Electrocardiogram
Safety Set

Visit Parameter	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
Baseline			
Q-Tc Interval (msec)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
Normal Sinus Rhythm n (%)			
Yes	xx (xx.x)	xx (xx.x)	xx (xx.x)
No	xx (xx.x)	xx (xx.x)	xx (xx.x)
QRS Axis n (%)			
Normal Range	xx (xx.x)	xx (xx.x)	xx (xx.x)
Possible Left Axis Deviation (LAD) Range	xx (xx.x)	xx (xx.x)	xx (xx.x)
Right Axis Deviation (RAD) Range	xx (xx.x)	xx (xx.x)	xx (xx.x)
Extreme Axis Deviation	xx (xx.x)	xx (xx.x)	xx (xx.x)
ECG Interpretation n (%)			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal Not Clinically Significant	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal Clinically Significant	xx (xx.x)	xx (xx.x)	xx (xx.x)
Visit X	...		
...			

Source Data: Listing 16.2.8.4

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

Prog Note: Visit to be included: Baseline, Day 7, Week 24, 48, Months 18, 24, 30, 36/EOS or Early Termination Visit if applicable.

Table 14.3.7
Summary of Echocardiogram
Safety Set

Visit Parameter	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
Baseline			
Left Ventricular Ejection Fraction (%)			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
Results			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal Not Clinically Significant	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal Clinically Significant	xx (xx.x)	xx (xx.x)	xx (xx.x)
Visit X			
...	...		

Source Data: Listing 16.2.8.5

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

Prog Note: Visit to be included: Baseline, Week 48, Months 24, 36/EOS or Early Termination Visit if applicable.

Table 14.3.8
Summary of Chimerism
Safety Set

Visit Parameter	SB-318 1.00E+13 vg/kg (N=xxx)	SB-318 5.00E+13 vg/kg (N=xxx)	Total (N=xxx)
Baseline			
T-Cells % Recipient			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
T-Cells % Donor			
n	xxx	xxx	xxx
MEAN (SD)	xxx.x (xxx.xx)	xxx.x (xxx.xx)	xxx.x (xxx.xx)
MEDIAN	xxx.x	xxx.x	xxx.x
MIN, MAX	xxx, xxx	xxx, xxx	xxx, xxx
...			
Visit X			
...			

Note: Baseline is defined as last non-missing measurement prior to dosing.
Source Data: Listing 16.2.2.6

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

Prog Note: Parameter to be summarized: T-Cells % Recipient, T-Cells % Donor, B-Cells % Recipient, B-Cells % Donor, Myeloid Cells % Recipient, Myeloid Cells % Donor.
Visit to be included: Baseline, Week 12, 48, Month 36/EOS or Early Termination Visit if applicable.

Table 14.3.9
Summary of Neurologic Cranial Nerve Exam and Muscle Strength Testing
Safety Set

Assessment: Neurologic Cranial Nerve Exam

Visit Parameter*	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
Baseline			
Oculomotor nerve, Trochlear Nerve and Abducens Nerve assessment			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal, NCS	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal, CS	xx (xx.x)	xx (xx.x)	xx (xx.x)
Not Done	xx (xx.x)	xx (xx.x)	xx (xx.x)
Facial Nerve assessment			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal, NCS	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal, CS	xx (xx.x)	xx (xx.x)	xx (xx.x)
Not Done	xx (xx.x)	xx (xx.x)	xx (xx.x)
...			
Visit X	...		

* NCS=Not Clinically Significant, CS=Clinically Significant, 0/5=No contraction, 1/5=Visible/palpable muscle contraction but no movement, 2/5=Movement with gravity eliminated, 3/5=Movement against gravity only, 4/5=Movement against gravity with some resistance, 5/5= Movement against gravity with full resistance.
Source Data: Listing 16.2.9.1

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

Table 14.3.9
Summary of Neurologic Cranial Nerve Exam and Muscle Strength Testing
Safety Set

Assessment: Muscle Strength Testing

Visit Parameter*	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
Baseline			
Scapular assessment			
0/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
1/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
2/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
3/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
4/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
5/5	xx (xx.x)	xx (xx.x)	xx (xx.x)
...			
Visit X	...		

* NCS=Not Clinically Significant, CS=Clinically Significant, 0/5=No contraction, 1/5=Visible/palpable muscle contraction but no movement, 2/5=Movement with gravity eliminated, 3/5=Movement against gravity only, 4/5=Movement against gravity with some resistance, 5/5= Movement against gravity with full resistance.
Source Data: Listing 16.2.9.1

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

Prog Notes: Visits include Baseline, Week 24, 48, Months 18, 24, 30, 36/EOS, or Early Termination Visit if applicable.
At each visit, list all available parameters including:
Neurologic Cranial Nerve Exam: (oculomotor nerve, trochlear nerve and abduces nerve assessment), facial nerve, accessory nerve, hypoglossal
Muscle Strength Testing: scapular assessment left side results, scapular assessment right side results, shoulder abduction left side results, shoulder abduction right side results, elbow flexion left side results, elbow flexion right side result, hand extension left side result, hand extension right side results.

Table 14.3.10
Summary of MRI of Liver
Safety Set

Visit	SB-318 1.00E+13 vg/kg (N=xxx) n (%)	SB-318 5.00E+13 vg/kg (N=xxx) n (%)	Total (N=xxx) n (%)
Baseline			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)
...			
VISIT X			
Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
Abnormal	xx (xx.x)	xx (xx.x)	xx (xx.x)
...			

Source Data: Listing 16.2.9.2

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

Prog Note: Visits to be summarized include Baseline, Week 24, Week 48,Month 18, Month 24, Month 30, and 36/EOS, or Early Termination Visit if applicable.

Listing 16.2.1.1
Disposition
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Informed Consent Date	Protocol Version of Enrollment	Eligible to Safety Set	First Dose of Study Treatment Date	Study Completed	Primary Reason of Early Termination	Completion or Termination Date (Day)
XXXXXX	65/M/W	DDMMMYYYY	XXXXXXX	No	DDMMMYYYY	YES		DDMMMYYYY (XX)
XXXXXX	50/M/W	DDMMMYYYY	XXXXXXX	Yes	DDMMMYYYY	No	Lost to Follow-Up	DDMMMYYYY (XX)
XXXXXX	50/M/W	DDMMMYYYY	XXXXXXX	Yes	DDMMMYYYY	No	Other, XXXXXXX	DDMMMYYYY (XX)

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.1.2
Reasons for Screen Failure
Screen Failed Subjects

Subject ID	Age(yrs)/ Sex/ Race	Informed Consent Date	Protocol Version at Screening	Criterion Type	Reason for Screen Failure
XXXXXX	65/M/W	DDMMMYYYY	XXXXXXXX	EXCLUSION	Serious intercurrent illness or clinically significant organic disease (unleass secondary to MPS I) such as cardiovascular, hepatic, pulmonary, neurologic, or renal disease. XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX XXXXX
				INCLUSION	XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX XXXXX
XXXXXX	50/M/W	DDMMMYYYY	XXXXXXXX	INCLUSION	XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX XXXXX

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

Prog Note: Sort by Subject ID.

Listing 16.2.1.3
Protocol Deviations
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Date of Deviation	Category	Description	Significance?	COVID-19 Related?
XXXXXX	50/M/W	DDMMMYYYY	INFORMED CONSENT	XXXXXXXXXXXXXXXXXXXXXXXXXXXX XXXXXXXXXXXXXXXXXXXXXXXXXXXX	YES	YES
XXXXXX	65/F/BL	DDMMMYYYY	STUDY PROCEDURES/ ASSESSMENTS	XXXXXXXXXXXXXXXXXXXXXXXXXXXX XXXXXXXXXXXXXXXXXXXXXXXXXXXX	No	No

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.1.4
Inclusion/Exclusion Criteria
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject	Age(yrs)/ Sex/ Race	Met all Eligibility Criteria?	Inclusion Criteria Not Met	Exclusion Criteria Met
XXXXXX	50/M/W	NO	MRI negative for liver mass as read by radiologist	Known to be unresponsive to ERT. XXXXXXXXXXXXXXXXXXXXXXXXXX
XXXXXX	65/F/BL	No	XXXXXXXXXXXXXXXXXXXXXXXXXX XXXXXXXXXXXXXXXXXXXXXXXXXX XXXXXXXXXXXXXXXXXXXXXXXXXX XXXXXXXXXXXXXXXXXXXXXXXXXX	
XXXXXX	55/F/BL	Yes		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.1
Demographics
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age (Years)	Sex	Ethnicity	Race/Specify if Other	Height (cm)	Weight (kg)
XXXXXX	50	MALE	NOT HISPANIC OR LATINO	WHITE	XXX	XXX
XXXXXX	65	MALE	HISPANIC OR LATINO	XXX	XXX	XXX

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.2
Chest X-Ray
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Date of Chest X-Ray (Day)	Interpretation	Findings if Abnormal Clinically Significant
XXXXXX	50/M/W	DDMMMYYYY (XX)	Normal	
XXXXXX	65/F/BL	DDMMMYYYY (XX)	Abnormal	XXXXXXXXXXXXXXXXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.3
MPS I Gene Sequencing
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/Sex/Race	Collection Date (Day)	Interpretation
XXXXXX	50/M/W	DDMMMYYYY (XX)	IDUA affected
XXXXXX	65/F/BL	DDMMMYYYY (XX)	IDUA heterozygous

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.4
Single Nucleotide Polymorphism Assay
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/Sex/Race	Collection Date (Day)	Result	Reason Test Not Done
XXXXXX	50/M/W	DDMMMYYYY (XX)	A/A	
XXXXXX	65/F/BL	DDMMMYYYY (XX)	A/G	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.5
Viral Load
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Collection Date (day)	HBV Surface Antigen	HBV DNA	HCV RNA	HIV RNA
XXXXXX	50/M/W	DDMMMYYYY (XX)	Negative	Negative	Negative	Negative
XXXXXX	65/F/BL	DDMMMYYYY (XX)				

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.2.6
Chimerism in post-HSCT Subjects
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Date Assay Collected (Day)	Test Name	Result
XXXXXX	50/M/W	Screening	DDMMMYYYY (XX)	T-Cells % Recipient	xxxxx
				T-Cells % Donor	xxxxx
				B-Cells % Recipient	xxxxx
				B-Cells % Donor	xxxxx
				Myeloid Cells % Recipient	xxxxx
				Myeloid Cells % Donor	xxxxx
XXXXXX	40/F/W	Screening	DDMMMYYYY (XX)	T-Cells % Recipient	xxxxx
				T-Cells % Donor	xxxxx
				B-Cells % Recipient	xxxxx
				B-Cells % Donor	xxxxx
				Myeloid Cells % Recipient	xxxxx
				Myeloid Cells % Donor	xxxxx

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Visit, Date of Sample Collection.. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.2.7
Neutralizing Antibodies to AAV2/6
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Date and Time of Sample Collection (Day)	Test Name	Result	Reason Not Done
XXXXXX	50/M/W	Screening	DDMMMYYYY HH:MM (XX)	AAV6 NAb Assay MRD4	xxxxx	
				AAV6 NAb Assay MRD10		
XXXXXX						

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Date of Sample Collection, Time of Sample Collection. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.3.1
Medical History
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Body System Code	System Organ Class/ Preferred Term/ Verbatim Term	Start Date (Day)	Stop Date (Day)	Ongoing
XXXXXX	50/M/W	XXXX	XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXXXXX	DDMMMYYYY (XX)	DDMMMYYYY (XX)	No
XXXXXX	65/M/AA	XXXX	XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXXXXX	DDMMMYYYY (XX)	DDMMMYYYY (XX)	No

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
Medical History was coded using MedDRA, Version ***XX.X***.

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

Prog Note: Sort by Cohort, Subject ID, Start Date, ***and alphabetically by Body System Code, and Verbatim Term.***

Listing 16.2.3.2
Surgery and Procedures
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Did any Surgeries or Procedures Occur?	Date of Surgery or Procedure (Day)	Surgery or Procedure	If HSCT, Clinical Outcome/ Additional Details
XXXXXX	50/M/W	No			
XXXXXX	32/M/W	Yes	DDMMMYYYY (XX)	XXXXXXXXX	
XXXXXX	45/F/AA	Yes	DDMMMYYYY (XX)	XXXXXXXXX	Successful Engraftment/ XXXXXXXXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

<DIRECTORY PATH>PROGRAM NAME Data Extraction Date: DDMMMYYYY Executed: DDMMMYYYY hh:mm

Prog Note: Sort by Cohort, Subject ID, Start Date/Time

Listing 16.2.4.1
Study Treatment Administration
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Infusion Performed?/ If Yes, Date of Infusion	Hospital Admission Date Time (Day)/ Release Date Time (Day)	ZFN1 Lot #/ ZFN2 Lot #/ hIDUA Donor Lot #	Infusion Start/ Stop Time	Dur (Hours)	Total Dose Infused (mL)	Was Pre-med given?	Dose Int/If Yes, Reason (Interruption start time/end time)
XXXXXX	50/M/W	Yes/ DDMMMYYYY	DDMMMYYYY HH:MM (XX)/ DDMMMYYYY HH:MM (XX)	XXXXXXXX/ XXXXXXXX/ XXXXXXXX	HH:MM/ HH:MM	XXX	XX	No	No
XXXXXX	63/M/W	Yes/ DDMMMYYYY	DDMMMYYYY HH:MM (XX)/ DDMMMYYYY HH:MM (XX)	XXXXXXXX/ XXXXXXXX/ XXXXXXXX	HH:MM/ HH:MM	XXX	XX		Yes/ Int 1: XXXXXXXXX XXXXX (Xx:xx/xx:xx) Int2: XXXX (Xx:xx/xx:xx)

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Dur=Duration; Dose Int=Dose Interrupted
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Date of Infusion.

Listing 16.2.4.2
Enzyme Replacement Therapy Administration
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	ATC Level 4/ Preferred Term/ Medication Reported	Start Date Time (Day)/ End Date Time (Day)	Duration (hours)	Dose (Unit)
XXXXXX	50/M/W	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXX/ XXXXXXXXXXX	DDMMMYYY HH:TT (xx)/ DDMMMYYYY HH:TT (xx)	XX	XXX
XXXXXX	63/M/W	XXXXXXXXXXXXXX/ XXXXXXXXXXX/ XXXXXXXXXX	DDMMMYYY HH:TT (xx)/ DDMMMYYYY HH:TT (xx)	XX	XXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Date of ERT Therapy

Listing 16.2.4.3
Prior and Concomitant Medications
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	ATC Level 4/ Preferred Term/ Medication Reported	Start Date (Day)/ End Date (Day)/ Ongoing	Dose	Unit	Route	Indication	Freq
XXXXXX	50/M/W	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX	DDMMMYYYY (XX)/ ONGOING	XXX	XXX	XXXXX	XXXXX	XXXX
XXXXXX	32/M/W	XXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX	DDMMMYYYY (XX)/ DDMMMYYYY (XX)/	XXX	XXX	Other: XXXXX	XXXXX	XXXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Freq=Frequency
Concomitant medications were coded with the WHO Drug dictionary dated *XXXXXX, 20XX*.
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Start Date/Time, ***and alphabetically by Drug Class, Preferred Term, and Medication Reported.***

Listing 16.2.7.1
Adverse Events
Enrolled Set

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
*=Treatment-emergent AE
Day = date of event - date of infusion.
Dur=Duration (Days = Stop date - Start date + 1)
Outcome: 1=Recovered/Resolved, 2=Recovering/Resolving, 3=Recovered/Resolved with sequelae, 4=Not recovered/not resolved, 5=Fatal, 6=Unknown
C/A=Concomitant or Additional Treatment Given?
Sev=Severity: 1=Mild, 2=Moderate, 3=Severe, 4=Potentially Life Threatening, 5=Fatal
Act=Action taken with study treatment: 1=Drug interrupted, 2=Not Applicable
Rel=Relationship to study treatment: 1=Related, 2=Not related
Study Disc=Caused study discontinuation?
Ser=Serious?
Adverse Events were coded using MedDRA, Version 23.0.

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

Listing 16.2.7.1
Adverse Events
Safety Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date (Day)/ Stop Date (Day)	Dur (Days)	Out- come	C/ A	Sev	Act/ Rel	Study Disc/ Ser
XXXXXX	65/F/W	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	126	4	Y	2	1/1	Y/Y
XXXXXX	50/M/W	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXXXX *	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	45	2	Y	1	2/2	Y/Y

Notes are listed on page 1.

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

Prog Note: Sort by Cohort, Subject ID, AE Start Date/Time, ***and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.***

Listing 16.2.7.2
Serious Adverse Event
Enrolled Set

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
*=Treatment-emergent AE
Day = date of event - date of infusion.
** Serious Criteria: If blank, not checked. Hospital=Hospitalization (Init=Initial, Pro=Prolongation, A=Admission, D=Discharge), L=Life threatening, CA=Congenital anomaly or birth defect, OME=Other medically important event, S=Significant disability, Death: (A=autopsy performed, DC=death certificate completed)
Abate=Did the SAE abate after use of study treatment stopped?
Reoccur=Did the SAE reoccur after reintroduction of study treatment?
[1] 1 = Temporal relationship of event to study treatment exposure
    2 = Event is known to be associated with the study treatment or drug class
    3 = Event improved on discontinuation of study treatment
    4 = Event reoccurred on rechallenge with study treatment
    5 = Biological plausibility
    6 = Other
[2] 1 = Event attributed to concomitant medication or disease and specify
    2 = Event not reasonably temporally associated with study treatment administration
    3 = Event is expected in targeted disease and/or population
    4 = Negative dechallenge and/or rechallenge
    5 = Other
Adverse Events were coded using MedDRA, Version ***XX.X***.

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

Listing 16.2.7.2
Serious Adverse Events
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date (Day)/ End Date (Day)	Serious Criteria**					
				Hospital	L	CA	OME	S	Death
XXXXXX	65/M/W	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	PRO A:DATE					DATE A:Y, DC:Y
XXXXXX	35/M/AA	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX *	DDMMMYYYY (XX)/ DDMMMYYYY (XX)		Y				DATE A:N, DC:Y

Notes are listed on page 1.

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

Prog Note: Sort by Cohort, Subject ID, AE Start Date/Time, ***and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.***

Listing 16.2.7.2
Serious Adverse Events
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date (Day)/ End Date (Day)	Abate	Reoccur	Interruption of Study Treatment: Date Stopped/Date Restarted	Interruption of Study Treatment: Type of Sequelae
XXXXXX	65/M/W	XXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXX	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	Yo	Yes	DATE/ DATE	XXXXXXXXXXX
XXXXXX	35/M/W	XXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXX/ XXXXXXXXXXXXX *	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	Unk	Unk		

Notes are listed on page 1.

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

Prog Note: Sort by Cohort, Subject ID, AE Start Date/Time, ***and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.***

Listing 16.2.7.2
Serious Adverse Events
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	System Organ Class/ Preferred Term/ Adverse Event Reported	Start Date (Day)/ End Date (Day)	Related to Study Treatment[1]	Not Related to Study Treatment[2]
XXXXXX	65/M/W	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXX	DDMMMYYYY (XX)/ DDMMMYYYY (XX)	6:XXXXXXXXXXXX	
XXXXXX	35/M/W	XXXXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXXXX/ XXXXXXXXXXXXXXX *	DDMMMYYYY (XX)/ DDMMMYYYY (XX)		1:XXXXXXXXXXXXX

Notes are listed on page 1.

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

Prog Note: Sort by Cohort, Subject ID, AE Start Date/Time, ***and alphabetically by System Organ Class, Preferred Term, and Adverse Event Reported.***

Listing 16.2.8.1.1
Laboratory Results – Hematology
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Laboratory Test	Result	Unit	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX			
			DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.8.1.2
Laboratory Results - Urinalysis with Microscopic Exam
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Laboratory Test	Result	Unit	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX			
			DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.8.1.3
Laboratory Results - Serum Chemistry
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Laboratory Test	Result	Unit	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX			
		XXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.8.1.4
Laboratory Results – Circulating Alpha Fetoprotein Level
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Laboratory Test	Result	Unit	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX			
			DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.8.2
Vital Signs
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit/ Date Time (Day)	Arm Used for Blood Pressure	Blood Pressure		Pulse Rate (bpm)	Resp Rate (bpm)	Temp (C)	Height (cm)/ Weight (kg)
				Systolic (mmHg)	Diastolic (mmHg)				
XXXXXX	50/M/W	XXXXX/ DDMMMYYYY HH:MM (XX)		XXX	XX	XXX	XX	XXX	
				XXX	XX	XXX	XX	XXX	
				XXX	XX	XXX	XX	XXX	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
Pulse Rate (bpm=beats/minute), Resp Rate=Respiratory Rate (bpm=breaths/minute), Temp=Temperature

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

Prog Note: Sort by Cohort, Subject ID, Date. Actual vital signs may be variable depending on the study & visit. All vitals collected in the study should be in the header, while only those collected at each visit will be displayed in the rows (e.g. height may only be displayed at baseline.). Display only visits where vitals signs are collected and tests done.

Listing 16.2.8.3
Physical Examination at Screening
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Exam Date (Day)	Assessment	Result	Specify Abnormality
XXXXXX	50/M/W	DDMMMYYYY (XX)	Skin	Abnormal	XXXXXXXXXXXXXXXXXXXXX
			Head, Eyes, Ears, Nose, Throat	Normal	
			…		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Visit, Exam Date, Assessment. All assessments including Physical Exam, height and weight will be displayed for each subject.

Listing 16.2.8.4
Electrocardiogram
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	ECG Date Time(Day)	Normal Sinus Rhythm	Vent. Rate (bpm)	PR Int (ms)	QRS Dur (ms)	QT Int (ms)	QTc Int (ms)	QRS Axis	Overall Interpretation*
XXXXXX	50/M/W	DDMMMYYYY HH:MM (XX)	YES	XXX	XXX	XXX	XXX	XXX	XXX	AB;NCS
XXXXXX	21/F/W	DDMMMYYYY HH:MM (XX)	NO	XXX	XXX	XXX	XXX	XXX		AB;CS
XXXXXX	65/F/BL	DDMMMYYYY HH:MM (XX)	YES	XXX	XXX	XXX	XXX	XXX	XXX	N

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
Vent. Rate=Ventricular Rate (beats/minute), PR Int=PR Interval(milliseconds), QRS Dur=QRS Duration(milliseconds), QT Int=QT Interval(milliseconds), QTc Int=QT Interval(milliseconds) correction method unspecified
* N=Normal, Ab;NCS=Abnormal;Not Clinically Significant, Ab;CS=Abnormal;Clinically Significant, ND=Not Done

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

Prog Note: Sort by Cohort, Subject ID, ECG Date.

Listing 16.2.8.5
Echocardiogram
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject I	Age(yrs)/Sex/Race	Date of Echocardiogram (Day)	Left Ventricular Ejection Fraction (%)	Results
XXXXXX	50/M/W	DDMMMYYYY (XX)	xx	Normal
XXXXXX	65/F/BL	DDMMMYYYY (XX)	xx	Abnormal not clinically significan

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID.

Listing 16.2.8.6
Pregnancy Test
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date (Day)	Laboratory Test	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Urine	Negative
			DDMMMYYYY (XX)	Urine	Positive
				Serum	Negative/XXX.X

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Laboratory Test Collection Date/Time, Lab test. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.1
Neurologic Cranial Nerve Exam and Muscle Strength Testing
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit/ Assessment Date (Day)	Assessment	Result	Finding of Clinically Significant
X	50/M/W	XXXX/ DDMMMYYYY (XX)	Oculomotor nerve, Trochlear Nerve and Abducens Nerve assessment	Abnormal, significant	XXXXXXXXXXXXXXXXXXXX
			...	Normal	
			…		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date, Assessment. All assessments will be displayed for each subject.

Listing 16.2.9.2
MRI of Liver
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Assessment Date Time(Day)	Results	If Abnormal, Findings
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY HH:MM (XX)	Abnormal	XXXXXXXXXXXXXXX
		XXXXXXXX	DDMMMYYYY HH:MM (XX)		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other,NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date Time.

Listing 16.2.9.3
Plasma and Leukocyte IDUA Activity
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Date of Sample Collected (Day)	Specimen Type	Result	Unit	Normal Range	Interpretation
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Leukocytes	XXX.X	nmol/hr /mg	6 - 71.4	Normal
				Plasma	XXX.X	nmol/hr /mL	3 - 50.2	Normal
			DDMMMYYYY (XX)					

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Date of Sample Collected, Specimen Type. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.4
Urine GAG Levels and Urine GAG/Creatinine Ratio
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Parameter (unit)	Result	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Total GAGs (mg/mmol creatinine)	XXX		0 - 24	
				DS (g/mol creatinine)	XXX		0 - 9.00	
				HS (g/mol creatinine)	XXX	L	0 - 5.71	XXXXXXXXX
				Creatinine (mg/ml)	XXX		0.15 - 1.5	
			DDMMMYYYY (XX)	...				

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, Visit Collection Date/Time, Parameter. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.5.1
Liver Biopsy
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Date of Biopsy (Day)	Findings	Histopathology Result	fRNA Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	Abnormal	
XXXXXX						

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Date of Biopsy.

Listing 16.2.9.5.2
Gene Modification at the Albumin Locus
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date (Day)	Category	Parameter	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x
		XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x
		XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x
XXXXXX	42/F/J	XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x
		XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x
		XXXXXXXX	DDMMMYYYY (xx)	xxxxx	xxxxx	xx.x

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Collection Date, Test.

Listing 16.2.9.6.1
AAV2/6 Clearance
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date (Day)	Test	Specimen Type	Result	Unit	Comments
XXXXXX	50/M/W	XXXXXXXX/ XXXXX	DDMMMYYYY (XX)	AAV2/6-hIDUA	Plasma	XXX.X	Copies/10 ul Plasma	
					Saliva	XXX.X	Copies/100ng Saliva DNA	
					Stool	XXX.X	Copies/100ng Stool DNA	XXXXXXXXX
					Semen	XXX.X	Copies/100ng Semen DNA	
					Urine		Copies/250 ul Urine	
				AAV2/6-ZFN 47171	Plasma	XXX.X	Copies/10 ul Plasma	XXXXXXXXX
					Saliva	XXX.X	Copies/100ng Saliva DNA	
					...			

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Collection Date, Test, Specimen Type. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.6.2
Immunogenicity Antibodies to AAV6 by Visit
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date (Day)	Test	Specimen Type	Result	Interpretation	Units	Analysis Tiers	Comments
XXXXXX	50/M/W	XXXX	DDMMMYYYY (XX)	Anti-AAV6_Ab_Screen	Serum	XXX.X	Potentially Positive	S/N (signal/ noise)	1.35	
				Anti-AAV6_Ab_Confirmatory	Serum	XXX.X	Positive	%inhibition	17.2	
				Anti-AAV6_Ab titer	Serum	XXX.X	Negative	titer	1.35	XXXXXXXXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Collection Date, Test, Specimen Type. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.7
Pulmonary Function Test
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Assessment Date Time (Day)	Quality of Study	Parameter (unit)	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY HH:MM(XX)	Acceptable	Forced Expiratory Volume (FEV1 %)	XXX.X
					Forced Vital Capacity (FVC %)	XXX.X
					Forced Vital Capacity (FVC Absolute)	XXX.X
					Vital Capacity (VC %)	XXX.X
					Vital Capacity (VC Absolute)	XXX.X
					FEV1 (L/sec)	XXX.X
					Adjusted DLCO (ml/mmHg/min)	XXX.X
		...	...	...		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date Time, Parameter. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.8
Six Minute Walk Test (6MWT)
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Assessment Date Time (Day)	Parameter (unit)	Timepoint	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY HH:MM(XX)	Distance Walked (meter)		XXX.X
				Overall Dyspnea Rate	Before Walk Test	XXX
						XXX
				Overall Fatigue Rate	Before Walk Test	XXX
					After Walk Test	XXX
				Heart Rate (beats/min)	Before Walk Test	XXX
					At the End of 6MWT	XXX
					2 Minutes After Completion of 6MWT	XXX
				% Oxygen Saturation	Before Walk Test	XXX
					At the End of 6MWT	XXX
					2 Minutes After Completion of 6MWT	XXX
				Use Supplemental Oxygen?		Yes/xxx
				Stop Time		HH:MM
				Reason for Stopping		XXXXX
		...	...	...		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Visit, Assessment Date Time, Parameter. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.9
Joint Range of Motion
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Assessment Date (Day)	Type	Parameter	Range of Motion (degrees)
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Shoulder	Forward flexion	
					Abduction	
				Hip	Flexion	
				Knee	Flexion	
					Extension	
				Ankle	Dorsiflexion	
		...	...	...		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American,AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date Time. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.10
MRI of Brain and Neck
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/Sex/Race	Visit	Date of Test (Day)	Findings
XXXXXX	50/M/W	Baseline	DDMMMYYYY (XX)	XXXXXXXXXX
XXXXXX				

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Visitnum.

Listing 16.2.9.11
WASI-II Testing
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Administration Date (Day)	Type	Parameter	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Block Design	Raw Score	
					T-score	
				Vocabulary	Raw Score	
					T-score	
				Matrix Reasoning	Raw Score	
					T-score	
				Similarities	Raw Score	
					T-score	
				Composite IQ Score	Verbal Comprehension	
					Perceptual Reasoning	
					Full Scale IQ	
		...	...	...		

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date Time. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.12
Visual Acuity Test and Corneal Clouding Exam
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Assessment Date Time(Day)	Assessment	Result	Corr	If Abnormal/Present, Clinically Significant, Findings
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY	Visual Acuity Test - Left Eye	xx/xx; Normal	Yes	
				Visual Acuity Test - Right Eye	xx/xx; Normal	No	
				Corneal Clouding in left Eye	Absent		
				Corneal Clouding in Right Eye	Absent		
		...	...	...			

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
Corr= Was this test performed with correction?

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

Prog Note: Sort by Cohort, Subject ID, Assessment Date Time. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.13
Lumbar Puncture
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date (Day)	Parameter (Unit)	Result
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	Glucose ()	XXX
				Protein ()	XXX
				RBC ()	XXX
				WBC ()	XXX
			DDMMMYYYY (XX)	Opening Pressure (ml/Hg)	XXX

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.

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

Prog Note: Sort by Cohort, Subject ID, Type, Collection Date, Parameter. For Result, display the number of decimal places where Applicable exactly as they are in the data.

Listing 16.2.9.14
ACTH Stimulation Test
Enrolled Set

Cohort: SB-318 1.00E+13 vg/kg

Subject ID	Age(yrs)/ Sex/ Race	Visit	Collection Date(Day)	Laboratory Test	Result	Unit	Flag	Normal Range	Comments
XXXXXX	50/M/W	XXXXXXXX	DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	L	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX			
			DDMMMYYYY (XX)	XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	XXXXXXXXX
				XXXXXXXXXXXXXXX	XXX.X	XXXXX	H	XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	
				XXXXXXXXXXXXXXX	XXX.X	XXXXX		XXX.X-XXX.X	

Sex: M=Male, F=Female;
Race: W=White, BL=Black or African American, AS=Asian, AI=American Indian or Alaska Native, PI=Native Hawaiian or Other Pacific Islander, O=Other, NS=Not Specified
Day = date of event - date of infusion.
L=Low, H=High

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

Prog Note: Sort by Cohort, Subject ID, and PARAMCD in the following order of PARAMCD: CORBAS_C, COR15_C, COR30_C, AND COR60_C. For Result and Normal Range, display the number of decimal places where Applicable exactly as they are in the data.