Statistical Analysis Plan

Protocol Title:

An Open-Label, Multicenter, Phase 2 Study to Evaluate Growth and Safety of LUM-201 Following 12 Months of Daily rhGH Treatment in Children with Idiopathic Growth Hormone Deficiency who have Previously Completed the LUM-201-01 Trial

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Parent Document	*SOP-STS-001*

STATISTICAL ANALYSIS PLAN

An Open-Label, Multicenter, Phase 2 Study to Evaluate Growth and Safety of LUM-201 Following 12 Months of Daily rhGH Treatment in Children with Idiopathic Growth Hormone Deficiency who have Previously Completed the LUM-201-01 Trial

PROTOCOL NO.: **LUM-201-04**

PRODUCT CODE: LUM-201

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14-Feb-2025	0.1	2024-8800	Initial SAP
01-Apr-2025	0.2	2024-8800	Revise SAP per sponsor comments
10-Apr-2025	1.0	2024-8800	Final SAP before DBL
28-Apr-2025	1.1	2024-8800	Updated the list of tables
01-Jul-2025	1.2	2024-8800	Amendment mock shells, add summary of bone age and change from baseline, corresponding listing
30-Jul-2025	1.3	2024-8800	Amendment SAP per sponsor request to calculate IGF-1 SDS at each timepoint

SAP APPROVAL

By my signature, I confirm that this SAP has been reviewed by Lumos Pharma, Inc. and has been approved for use on the LUM-201-04 study:

Name	Title / Company	Signature	Date
Prepared by: [redacted]	Biostatistician/Novotech	[redacted]	
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Approved by: [redacted]	Chief Medical Officer / Lumos Pharma	[redacted]	

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List of Abbreviations

Abbreviation	Description
AE	Adverse Event
AHV	annualized height velocity
ATC	Anatomical Therapeutic Class
BMI	Body Mass Index
CS	Clinically Significant
CSR	Clinical Study Report
DBP	Diastolic blood pressure
ECG	12-Lead Electrocardiogram
eCRF	Electronic Case Report Form
GCP	Good Clinical Practice
GH	growth hormone
GHD	growth hormone deficiency
HT-SDS	height standard deviation score
HV	Height velocity
IGFBP-3	insulin-like growth factor binding protein 3
IGF-1	insulin-like growth factor 1
MedDRA	Medical Dictionary for Regulatory Activities
MPH	Mid-parental height
N/A	Not Applicable
NCS	Not Clinically Significant
NK	Not Known
PI	Principal Investigator
PD	Pharmacodynamic
PT	Preferred Term
SDS	Standard deviation score
SAE	Serious Adverse Event
SAP	Statistical Analysis Plan
SBP	Systolic blood pressure
SD	Standard Deviation
S.I.	International System of Units
SOC	System Organ Class
SOP	Standard Operating Procedure
SRC	Safety Review Committee
TEAE	Treatment Emergent Adverse Event
rhGH	recombinant human growth hormone
WHO-DD	World Health Organization Drug Dictionary

1. INTRODUCTION

The following Statistical Analysis Plan (SAP) provides the outline for the statistical analysis of the data collected from the LUM-201-04 study (protocol version 2.0 dated 19 January 2022).

The planned analyses identified in this SAP may be included in clinical study reports (CSRs), regulatory submissions, or future manuscripts. Also, post hoc exploratory analyses not necessarily identified in this SAP may be performed to further examine study data. Any post hoc, or unplanned, exploratory analyses performed will be clearly identified as such in the final CSR.

Important features of the analysis including the methods used, adjustments made for demographic or baseline measurements or concomitant therapy, handling of dropouts and missing data, adjustments for multiple comparisons, special analyses of multicenter studies, and adjustments for interim analyses, should be discussed.

2. PROJECT OVERVIEW

2.1 Study Design

This is a multiple center, 12-month, open-label in children with growth hormone deficiency (GHD) who completed 12 months of recombinant human growth hormone (rhGH) treatment in the LUM-201-01 Trial. Up to 20 subjects will be enrolled.

Following successful screening, subjects will be assigned to the 3.2 mg/kg daily oral dose of LUM-201. IGF-1 samples will be collected throughout the study. If an insulin-like growth factor 1 (IGF-1) standard deviation score (SDS) value > +2.0 is confirmed by a follow-up (unscheduled) lab, the LUM-201 dose may be reduced to 1.6 mg/kg/day.

The follow-up safety history of subjects will be collected by telephone visit 30 days after their final visit.

2.2 Study Objectives and Endpoints

Categories	Objectives	Endpoints
Primary objective	The primary objective(s) of this study is assessing the growth response to LUM-201 administration in children with idiopathic growth hormone deficiency (GHD) previously treated with daily rhGH for 12 months in the LUM-201-01 trial.	Growth parameters of efficacy (Month 6 [visit 4] and Month 12 [visit 6]) will be assessed based on: 1. Annualized height velocity. 2. Change in HT-SDS 3. Change in Weight and Weight SDS 4. Change in BMI and BMI SDS

		5. Change in Bone Age (Only Month 6 [visit 4]) PD: Serum LUM-201 PD markers including growth hormone (GH), IGF-1, and IGFBP-3 concentrations. IGF-1 SDS will be calculated from measured IGF-1 concentrations
Secondary objective(s)	The secondary objective(s) of this study is assessing safety in response to LUM-201 treatment.	Safety of LUM-201 will be assessed by AEs and SAEs, routine laboratory tests (hematology, chemistry panel including liver enzymes and urinalysis), HbA1c, blood glucose, free thyroxine (T4), triiodothyronine (T3), thyroid-stimulating hormone (TSH), lipids, physical examinations, ECGs and vital signs

2.3 Sample Size

Up to 20 subjects may enter this study.

2.4 Randomization

Not applicable for this open-label study.

3. STATISTICAL CONSIDERATIONS

Data will be handled and processed per the sponsor's representative (Novotech) Standard Operating Procedures (SOPs), which are written based on the principles of good clinical practice (GCP).

3.1 General Considerations

All data collected on the electronic case report form (eCRF) will be presented in the data listings and will be listed and sorted by treatment group where applicable. All descriptive summaries will be presented by the treatment group and nominal visit/time point (where applicable).

All safety summaries will be presented by the treatment group. All disposition and, concomitant medication descriptive summaries will be presented by treatment sequence and nominal visit/time point (where applicable).

Unless otherwise stated, the following methods will be applied:

- Continuous variables: Descriptive statistics will include the number of non-missing values (n), arithmetic mean, standard deviation (SD), standard error (SE), median, minimum, and maximum values.

  The minimum and maximum values will be displayed to the same decimal precision as the source data, the arithmetic mean, SD, and median values will be displayed to one more decimal than the source data for the specific variable.

  95% Confidence Intervals (CIs), mean differences (among treatments and from baseline) and least-square (LS-Means) values will be displayed to one more decimal than the source data for a specific variable. P-values will be displayed to 3 decimal places.

  The appropriate precision for derived variables will be determined based on the precision of the data on which the derivations are based, and statistics will be presented in accordance with the above mentioned rules.

- Categorical variables: Descriptive statistics will include counts and percentages per category. The denominator in all percentage calculations will be the number of participants in the relevant analysis population with non-missing data, unless specifically stated otherwise. Percentages will be displayed to one decimal place. Proportions will be displayed to 3 decimal places.

  95% Confidence Intervals (CIs), difference in proportions, odds ratio's and other categorical parameters will be displayed to one decimal place for percentages. Proportions will be displayed to 3 decimal places. P-values will be displayed to 3 decimal places.

- Repeat/unscheduled assessments: Only values collected at scheduled study visits/time points will be presented in summary tables. If a repeat assessment was performed, the result from the original assessment will be presented as the result at the specific visit/time point. All collected data will be included in the data listings.
- Result display convention: Results will be center aligned in all summary tables and listings. Participant identifiers visit and parameter labels may be left-aligned if required.
- Date and time display conventions: The following display conventions will be applied in all outputs where dates and/or times are displayed:

  Date only: YYYY-MM-DD

  Date and time: YYYY-MM-DD HH:MM

If only partial information is available, unknown components of the date or time will be presented as 'NK' (not known), i.e., '2016-NK-NK'. Times will be reported in military time.

3.2 Key Definitions

The following definitions will be used:

- Baseline: The baseline value is defined as the last available valid (quantifiable continuous or categorical value), non-missing observation for each participant prior to first study drug administration. Repeat and unscheduled assessments will be included in the derivation of the baseline values.
- Change from Baseline: The change from baseline value is defined as the difference between the result collected/derived at a post-baseline visit/time point and the baseline value.

  The change from baseline value at each post-baseline visit/time point will be calculated for all continuous parameters using the following formula:

  Change from Baseline Value = Result at Visit/Time Point – Baseline Value

  The change from baseline value will only be calculated if the specific post-baseline visit/time point result and the baseline value for the parameter are both available and will be treated as missing otherwise.
- Study day: The study day of an event is defined as the relative day of the event starting with the date of the first study drug administration (reference date) as Day 1 (there will be no Day 0).

  The study day of events occurring before the first study drug administration will be calculated as:

  Study Day = (Date of Event - Date of First Study Drug Administration)

  For events occurring on or after Day 1, study day will be calculated as:

  Study Day = (Date of Event - Date of First Study Drug Administration) + 1

  Study days will only be calculated for events with complete dates and will be undefined for events that are 'Ongoing' at the end of the study.

  Relative days compared to an alternative reference point will be calculated similarly, but the alternative starting reference start date will be used instead of the date of the first study drug dosing.
- Prior Medications: Prior medications are defined as any medication where the use was stopped prior to the first administration of the study medication.
- Concomitant Medications: Concomitant medications are defined as any medication (other than the study drug) that was used at least once after the first administration of the study drug. Medications that were stopped on the same date as the first study drug administration will be defined as concomitant medications. If a clear determination cannot be made (partial medication end dates) the medication will be classified as concomitant
- Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication.
- Standard Deviation Scores (SDS) for height, weight, and BMI will be computed using the WHO 2007 Child Growth Standards available from the World Health Organization (WHO)

website. SDS for IGF-1 will be computed using reference standards for healthy children, based on IGF-1 concentration values provided by the central laboratory.

- Growth response including change in HT-SDS, change in Weight and Weight-SDS, change in BMI and BMI-SDS, and change in Bone Age (baseline data from previously study last visit data, this study only collected Month 6 data).
- Puberty is defined as Tanner Stage 2 breast development in girls and testicular volumes ≥ 4 mL in boys.

3.3 Inferential Analyses

Descriptive statistics will be used to summarize the safety data. No formal hypothesis testing is planned.

3.4 Multiple Comparisons and Multiplicity Adjustments.

Not applicable for this study.

3.5 Handling of Missing Data

For height related endpoints, missing results will be imputed by carrying the last non-missing height value forward and using this value to derive HV and HT-SDS at the analysis time points. This is a conservative estimate of HV and change in HT-SDS, as it assumes no further change in height after the last measurement.

For the classification of Treatment emergent adverse event (TEAE) and Concomitant medication, the following will be applied in the following order:

a. If all dates/times (start and stop) are missing, the event/medication will automatically be classified as a TEAE/Concomitant medication.

b. For AEs with a missing start date/time, if the event end date/time is prior to first study drug administration, the event will not be classified as a TEAE.

c. If only the AE start year/ medication end year is present and is the same or is after the first study drug administration year unit, the event/medication will be classified as a TEAE/Concomitant medication.

d. If the AE start month and year/medication end month and year are present and are the same or after the first study drug administration month and year units, the event/medication will be classified as a TEAE/Concomitant medication.

3.6 Coding of Events and Medications

Medical history and AE verbatim terms will be coded using the Medical Dictionary for Regulatory Activities (MedDRA) using version 23.1 at the time of study commencement. Terms will be coded to the full MedDRA hierarchy, but the system organ class (SOC) and preferred terms (PT) will be of primary interest for the analysis.

Prior and concomitant medications will be coded using the World Health Organization Drug Dictionary using 2020 Q3 at the time of study commencement. Medications will be mapped to the full WHO-DD Anatomical Therapeutic Chemical (ATC) class hierarchy, but PTs will be of primary interest in this analysis.

3.7 Treatment Groups

- LUM-201 3.2 mg/kg/day

4. ANALYSIS POPULATIONS (ANALYSIS SETS)

In this study 3 analysis populations are defined: The Safety, the Intent-to-Treat (ITT), and the Per-Protocol (PP) population.

4.1 Population Descriptions

1. Intent To Treat (ITT) Population (Full Analysis Set)

The Full Analysis Set includes all enrolled subjects who have received at least one dose of study treatment. This set will be used for the safety analysis.

All disposition and demographic data analysis will be based on the ITT population. All listings will be presented by the ITT population.

2. Per Protocol (PP) population (Analysis Set)

The Per Protocol Population includes all enrolled subjects who complete Month 12 (visit 6) and have not missed more than 10% of scheduled doses and who do not have any major protocol deviations.

The Per Protocol Population will be used for the primary evaluation, the growth response to treatment.

Pharmacodynamic (PD) markers including GH, IGF-1, and IGFBP-3 concentrations.

5. SUMMARY OF STUDY DATA

5.1 Participant disposition and analysis populations

Participant disposition and analysis population analysis will be based on the ITT population. Participant disposition and analysis populations will be summarized descriptively as described in section 3.1 (categorical descriptive analysis).

a. Participant Disposition

Participant disposition will include the number of participants who completed the study as planned, participants withdrawn from the study, as well as the primary reason for early termination. Participant disposition will be summarized descriptively.

A listing of patient disposition with study discontinuation and treatment discontinuation will be presented. Whether a patient had any ongoing AEs, the date of last dose, reason for treatment discontinuation (if applicable), date of last contact (or date of death) and date of withdrawal of consent (if applicable) will be included.

Listings will be provided by treatment groups (section 3.7) sorted by participant ID for all enrolled participants along with the date the participant provided informed consent, date and

time of first/last received study treatment, and date of last visit, whether the participant completed the study and reason for early withdrawal.

b. Analysis Populations

The number of participants included in each study populations will be summarized descriptively.

In addition, the inclusion of each participant into/from each of the defined analysis populations will be presented in the by-participant data listings.

Listings will be provided by treatment groups (section 3.7) sorted by participant ID for all enrolled participants, along with the date and time of first study drug administration, date of last visit, and whether the participant completed the study.

5.2 Protocol deviations

All major protocol deviations will be categorized and summarized descriptively, as per data collected based on ITT population. Protocol deviations will be presented for each participant in the by-participant data listings. Listing of all protocol deviations and the details will be provided by treatment group (section 3.7) sorted by participant ID for ITT population.

Prior to database lock, all protocol deviations will be reviewed by medical monitors and assigned a status of major or minor if qualifying as such.

Protocol deviations and major protocol deviations will be categorized and provided by sponsor.

5.3 Demographic and baseline information

Demographic and baseline information analysis will be based on the ITT population. Demographic and baseline information will be summarized descriptively as described in section 3.1.

Demographics and Baseline Characteristics

The following demographic and baseline characteristic parameters will be analyzed:

Continuous descriptive analysis:

- Age (months)
- Height (cm): height is the average of the three height measurements collected.
    - Height-SDS: SDS values is calculated using WHO growth charts
    - Target Height-SDS
- Weight (kg)
    - Birth weight and gestational age
    - Birth weight percentile for gestational age
    - Weight-SDS: SDS values is calculated using WHO growth charts
- BMI (kg/m^2)
    - BMI-SDS: SDS values is calculated using WHO growth charts

- Bone Age (years): measured at Month 6
- Baseline IGF-1 and IFG-1 SDS
- Maternal and paternal heights for calculation of mid-parental height (MPH)

Categorical descriptive analysis:

- Sex
- Race
- Ethnicity
- Tanner Stage

5.4 Medical history

Medical history will be coded using the MedDRA® version 23.1. Participants with any medical history will be summarized by SOC and PT using descriptive statistics in accordance with section 3.1 for the safety population by treatment groups. Medical history will be ordered from the highest frequency in the overall column by SOC and PT.

Medical history listings will be provided by treatment grouping (section 3.7) sorted by participant ID for the ITT population.

5.5 Treatment exposure

Treatment exposure will be summarized using descriptive statistics in accordance with section 3.1 based on the PP Population.

All study drug administration information (date and start and end time of administration, total dose administered, tablet strength, total number of tablets), whether dose details will be presented in the by-participant data listings and sorted by participant ID and visit for the PP population, as well as the dates and times of study drug administration.

5.6 Concomitant medications

Concomitant medications will be summarized by Anatomical Therapeutic Chemical (ATC) class Level 3 and PT as noted in section 3.1 (categorical descriptive analysis) using ITT population. Participant who used the same medication on multiple occasions will only be counted once in the specific category (PT). PTs will be sorted alphabetically. In addition to the summaries by the coded terms, the number of participants who used at least one concomitant medication during the study will be presented.

Listing of full details of concomitant medications will be provided by treatment group (section 3.7) sorted by participant ID and visit for the ITT population, sequence number, concomitant medications/therapy classification.

6. EFFICACY

All efficacy analysis will be based on the Per Protocol population.

Efficacy endpoints will be summarized descriptively as described in section 3.1 (continuous descriptive analysis).

Descriptive statistics will be compiled for 6-month and 12-month AHV, change in HT-SDS, change in weight and weight SDS, change in BMI and BMI SDS and change in bone age. In addition, these measures will be estimated from least square means from a General Linear Model with age, Baseline HT-SDS, and bone age delay as covariates.

Target HT-SDS will be computed from the observed maternal and paternal heights, their height SDS as determined from the WHO Child Growth Standards, and the calculation method of Hermanussen and Cole [4].

To calculate the Target HT-SDS, the formula is defined as follows,

Target Height-SDS = MPH-SDS (derived using WHO growth chart values at age = 228 months).

AHV at Month 12 that is < 70% of the 1st year growth on rhGH will be summarized. The AHV at the time point t2 is defined as follows,

$$\left(\frac{h_2 - h_1}{t_2 - t_1}\right) 365.25,$$

where $h2$ and $h1$ are the heights (cm) measured at $t2$ and $t1$ (in days), respectively. Of note, $h1$ is the height measured at Baseline visit (visit 1).

Derivation of IGF-1 SDS values

Inputs:

1. IGF-1 concentration values from the central lab.
2. CRF collect sex and age in months at the screening visit and date of the screening visit.
3. Date at which the IGF-1 sample was drawn.
4. LMS tables (female and male) were provided from sponsor.

Method:

1. Determine IGF-1 concentration in units of ng/mL from the laboratory results in original results. If the original results are in nmol/L, covert to ng/mL using the conversion calculation below. DO NOT round result value.

   $X\ ng/mL = Y\ nmol/L * 7.63$

2. Derive age at Visit in Half Years using the calculations below.

$$age\ at\ visit = \frac{(age\ at\ screening * 30.44) + (visit\ date - screening\ date + 1)}{365.25}$$

$$age\ at\ visit\ in\ half\ years = \frac{floor(age\ at\ visit * 2)}{2}$$

3. Merge data with LMS tables by sex and age at visit in half years, to obtain values for the following LMS parameter: lambda(λ), mu(μ), and sigma(σ).
4. Calculate SDS values from the LMS parameter values using calculation below.

$$SDS = round\left(\frac{((X\ ng/mL)/\mu)^{\lambda} - 1}{\lambda * \sigma}, 0.001\right)$$

5. Incorporate normal ranges for the SDS values:
   a. X < -2.0 = Low
   b. -2.0 < X < 2.0 = Normal
   c. X > 2.0 = High

6.1 Estimand

No estimand is planned for this study.

6.2 Pharmacodynamic (PD) markers

PD endpoints (Serum IGF-1, IGFBP-3 and GH) will be summarized descriptively as described in continuous descriptive analysis.

7. SAFETY

Safety endpoints will be analyzed using the ITT population. Safety endpoints will be summarized descriptively as described in section 3.1.

7.1 Adverse Events

All AEs and SAEs including will be coded using MedDRA version 23.1. All AE summaries will be restricted to TEAEs only. Summary tables will include the number of participants (%) experiencing an event and the number of events. Participants will be counted only once for each SOC and PT level (categorical descriptive analysis).

The TEAE summaries will include:

- Overall summary of TEAEs.
  - Number of TEAEs
  - Number of Serious TEAEs
  - Number of Study Treatment Related TEAE
  - Number of TEAE Leading to Study Drug Interrupted
  - Number of TEAE Leading to Study Discontinuation
  - Number of TEAE Leading to Death
- Summary of TEAEs by SOC and PT.

- Summary of Serious TEAEs by SOC and PT.
- Summary of TEAEs by SOC, PT and Severity.
- Summary of Serious TEAEs by SOC, PT and Severity.
- Summary of TEAEs by SOC, PT and Relationship to Study Drug.
- Summary of Serious TEAEs by SOC, PT and Relationship to Study Drug.
- Summary of TEAEs Leading to the Study Drug Discontinuation by SOC and PT.
- Summary of TEAEs Leading to Death by SOC and PT

All AEs will be listed and will include the verbatim term, PT, SOC, severity (CTCAE v5.0 grades), SAE/TEAE flag, action taken with regards to the study drug for AE, outcome and other information captured in the CRF. Separate listings will be created for SAEs. These listings will be presented by treatment group (section 3.7) sorted by participant ID and AE start date along with age, sex of the participant for the ITT population.

7.2 Safety Laboratory Assessments

Blood and urine samples will be collected at the time points specified in the Schedule of Events (refer to the Protocol) to conduct hematology, chemistry and urinalysis (including microscopic examinations) analyses.

Laboratory parameters will be summarized by treatment group (section 3.7) using descriptive statistics at baseline and at each on-study evaluation, also including the change from baseline. Shift tables for laboratory parameters will be produced showing the number and percentage of subjects with changes to or from the normal range (e.g., normal to low, normal to high, low to normal, etc.) from baseline to each subsequent visit.

The clinical laboratory tests (Hematology, Serum Chemistry, Urinalysis and other test) will be performed within each of the specified test panels (refer to the Protocol session 19.3 Appendix 3: Clinical Laboratory Analytes).

All laboratory data collected at scheduled and unscheduled visits will be included in the listings, but only results collected as scheduled visits will be included in the summary tables.

Results for individual parameters may be reported in different units depending on the analyzing laboratory. If required, the results (and the corresponding normal range cut-off values) for individual parameters may be converted to International System of Units (S.I.) units to summarize the data.

For all the parameters where a unit value has been reported, the parameter names that will be used in the outputs will comprise of the test name and the unit of measure, for example, ‘Albumin (g/L)’. For the urinalysis parameters, the parameter name will be the reported test name only. Parameters will be sorted alphabetically within tables and listings.

For all parameters where a normal range limit value was reported, the normal range will be derived based on the available lower and upper limit values and any reported mathematical symbols. If both a lower and upper limit value is available, the normal range will be presented as ‘(Lower, Upper)’.

The reported results for each parameter with a defined normal range will be classified ('Low', 'Normal', 'High') in relation to the defined normal range limits. If a result is equal to the normal range cut-off value, the result will be considered 'Normal'. If laboratory assays values below the lower limit of quantification will be treated as 0 for statistical summaries.

The hematology and chemistry results tables will present summary statistics for each laboratory parameter within the specific test panel. For each parameter, summaries will be presented for the baseline and each scheduled post-baseline visit. In addition, summaries will be presented for the change from baseline values at each scheduled post-baseline visit (continuous descriptive analysis).

The decimal precision to which the summaries for each parameter will be based on the maximum number of decimals to which the reported result or the normal range limits are presented to in the raw data. The results and normal ranges will be displayed to the same decimal precision in the listings.

Additionally, counts (%) of number participants with values out of normal range at each scheduled time point will also be presented along with shift tables that will represent the changes in normal range categories across post-baseline time points (categorical descriptive analysis).

The urinalysis table will present counts and percentages of normal, abnormal clinically significant and clinically significant for the reported results at baseline and each post-baseline visit for all parameters (categorical descriptive analysis).

7.3 Vital Signs

The following vital signs measurements will be taken at the time points specified in the Schedule of Events (refer to the Protocol):

- Pulse (beats/min);
- Systolic blood pressure (SBP) (mmHg)
- Diastolic blood pressure (DBP) (mmHg)
- Respiratory rate (breaths/min)
- Temperature (ºC)

All vital signs data collected at scheduled and unscheduled visits will be included in the listings, but only results collected as scheduled visits will be included in the summary tables.

The parameter names that will be used in the outputs will comprise of the test name and the unit of measure, for example, 'Systolic Blood Pressure (mmHg)'. Parameters will be sorted in the order that the measurements were collected in on the Vital Signs eCRF page within the tables and listings.

Vital signs measurements will present summary statistics for the results at the baseline and each scheduled post-baseline visit for each of the parameters. In addition, summaries will be presented for the change from baseline values at each scheduled post-baseline visit (continuous descriptive analysis).

The decimal precision to which the summaries for each parameter will presented will be based on the maximum number of decimals to which the results were reported on the eCRF.

7.4 12-Lead Electrocardiogram (ECG)

The following ECG measurements will be taken at the time points specified in the Schedule of Events (refer to the Protocol):

- Heart Rate (beats/min)
- PR Interval (msec)
- QT Interval (msec)
- QTcF Interval (msec)
- QRS Duration (msec)
- Overall Interpretation

All ECG data collected at scheduled and unscheduled visits will be included in the listings, but only results collected as scheduled visits will be included in the summary tables.

The parameter names that will be used in the outputs will comprise of the test name and the unit of measure, for example, 'Ventricular Rate (beats/min)'. Parameters will be sorted in the order that the measurements were collected in on the ECG eCRF page within the tables and listings.

ECG measurements will present summary statistics for the results at the baseline and each scheduled post-baseline visit for each of the parameters. In addition, summaries will be presented for the change from baseline values at each scheduled post-baseline visit (continuous descriptive analysis).

If multiple tracings are done as part of one assessment, the mean of the three tracings per parameter and the worst clinical interpretation per timepoint will be summarized for each participant. All values will be listed including the mean value per parameter and the worst clinical interpretation. Changes from baseline will be calculated based on the mean values of the triplicates where appropriate.

The decimal precision for each parameter will be based on the maximum number of decimals to which the results were reported on the eCRF.

The summary of overall interpretation findings table will present counts and percentages for the reported results at baseline and each post-baseline visit/time point. Result categories will be ordered as 'Normal ECG', 'Otherwise Normal ECG', and 'Abnormal ECG' (categorical descriptive analysis).

7.5 Physical Examinations

Complete physical examination will be performed at Baseline and Month 12 visits. Tanner staging of pubertal development will be performed at Screening/Baseline (visit 1), Month 3 (visit 3), Month 6 (visit 4), Month 9 (visit 5) and Month 12 (visit 6). Tanner staging to include breast development, pubic and axillary hair and, in males, testicular volumes.
For each body system, an assessment is being made as to whether the body system is normal, abnormal and clinically significant and abnormal but not clinically significant.

Counts and percentages for each the body system classified into: normal, abnormal and clinically significant and abnormal but not clinically significant at each scheduled visit will be summarized for ITT population for treatment groups defined in section 3.7.
The listing of physical examinations will include all the information collected, with baseline value flagged.

7.6 Pregnancy Test Results

All information related to pregnancy testing (urine and serum based) and contraception status will be presented in the by-participant data listings.

8. IMMUNOGENICITY

Not applicable.

9. CHANGES TO THE PLANNED ANALYSIS

Not applicable.

10. FINAL ANALYSIS (END OF STUDY)

The final analysis will be conducted after all participants have completed the study, the clinical database has been locked, the analysis populations have been approved.

The final analysis will be based on the final version of the SAP. Any deviations from the planned analysis will be documented in the CSR.

11. SOFTWARE

- SAS® Version 9.2 or higher (SAS Institute, Cary, North Carolina, USA).

12. TABLES

Table Number	Table Title	Population
14.1	**Demographics and Other Baseline Characteristics**	
14.1.1	*Summary of Participant Enrolment and Disposition*	*All Enrolled Participants*
14.1.2	*Summary of Protocol Deviations*	*ITT*
14.1.3	*Summary of Demographics and Baseline Characteristics*	*ITT*
14.1.4	*Treatment Exposure*	*PP*
14.1.5	*Summary of Medical History*	*ITT*
14.1.6	*Summary of Prior Medications*	*ITT*
14.1.7	*Summary of Concomitant Medications*	*ITT*
14.2	**Efficacy/PK/PD**	
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13. LISTINGS

Listing Number	Listing Title	Population
16.2.1	**Participant Disposition**	
16.2.1.1	*Analysis Populations*	*All Participants*
16.2.1.2	*Participant Disposition*	*All Enrolled Participants*
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16.2.8	**Laboratory Parameters**	
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Listing Number	Listing Title	Population
16.2.8.3	*Urinalysis*	*ITT*
16.2.9	**Other Safety**	
16.2.9.1	*Vital Signs*	*ITT*
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16.2.9.3	*Physical Examination*	*ITT*
16.2.9.4	*Tanner Stage*	*ITT*

14. REFERENCES

1) ICH E3 Guideline
2) ICH E6 Guideline
3) ICH E9 Guideline
4) Hermanussen M, Cole J. The calculation of target height reconsidered. Horm Res 2003;59:180-183.
5) SDTM v1.7
6) SDTMIG v3.3
7) ADaM v2.1
8) ADaMIG v1.1
9) Medical Dictionary for Regulatory Activities (MedDRA) version 23.1
10) World Health Organization Drug Dictionary (WHO-DD) 2020 Q3

Mock Tables

Table of Contents

GENERAL COMMENTS

- Where a count is 0, the percentage will not be shown (e.g. 0(0.0%) will be displayed as 0) and statistical values (mean, median, SD, etc.) are represented as 0.
- Unless otherwise states, parameters will be listed in alphabetical order.
- The minimum and maximum values will be presented to the same number of decimal places as recorded in the electronic Case Report Form (eCRF)
- Mean, SD, and Median will be presented to one more decimal place than the raw data
- Percentages will be rounded to one decimal place, with the denominator being the number of subjects in the relevant population with non-missing data, unless otherwise specified
- Change from Baseline:
  Change from Baseline will be calculated as:
  Change from Baseline = post baseline value – baseline value
- Unscheduled visits will be excluded from summary tables but included in all by-participant listings.
- **Names and order of Treatment Groups**
  - LUM-201 3.2 mg/kg/day
- **Names of visits**
  - Screening/Baseline/Visit 1
  - Month 1/Visit 2
  - Month 3/Visit 3
  - Month 6/Visit 4
  - Month 9/Visit 5
  - Month 12/Visit 6/Early Withdrawal
  - Follow up
  - Unscheduled (Listing only)
- Column widths and text-wrapping may be altered in final output in order to best present the data.
- Footnotes may be added/amended if required.

Document History- <Amendment 1.0> / <Addendum x.x>

Following Tables /Listings are added : **e.g. L16.2.8.4, L16.2.8.5** **Reason for additional / update for existings**

TFL number	**Title**
T14.2.13 Added the new table using Month 6 bone age data from Lumos-201-01 as baseline for Lumos-201-04. These additions were made due to the absence of baseline bone age data in Lumos-201-04.	Summary of Bone Age and Change from Baseline
L16.2.6.5 Added the new listing using Month 6 bone age data from Lumos-201-01 as baseline for Lumos-201-04. These additions were made due to the absence of baseline bone age data in Lumos-201-04.	Bone Age

Table 14.1.1 Summary of Participant Enrolment and Disposition
(All Enrolled Participants)

	LUM-201 3.2 mg/kg/day (N=xx)
Number of Participants Enrolled	xx
Intent-to-treat Population (Intent to Treat) [1]	xx (xx.x%)
Per Protocol Population [2]	xx (xx.x%)
Number of Participants who Completed the Study	xx (xx.x%)
Primary Reason for Non-Completion of Study	
Screen Failure	xx (xx.x%)
Subject Withdrew Study Participation	xx (xx.x%)
Lost to Follow-up	xx (xx.x%)
Termination of Study	xx (xx.x%)
Investigator Decision	xx (xx.x%)
Medical Monitor Decision	xx (xx.x%)
Protocol Violation/Non-compliance	xx (xx.x%)
Adverse Event	xx (xx.x%)
COVID-19 Disruption	xx (xx.x%)
Other	xx (xx.x%)

Source Listing: 16.2.1.1, 16.2.1.2.
Note: Percentages are calculated (the denominator used for the calculation) based on the number of enrolled participants (N).
[1] Intent-to-treat Population (Intent to Treat) consists of all enrolled participants who have received at least one dose of study treatment.
[2] Per Protocol population consists of all enrolled participants who complete month 12 (visit 6) and have not missed more than 10% of scheduled doses and who do not have any significant protocol deviations.

Table 14.1.2 Summary of Protocol Deviations
(Intent to Treat Population)

	LUM-201 3.2 mg/kg/day (N=xx) n (%)
Participants with at Least One PD	xx (xx.x%)
By PD Severity	xx (xx.x%)
Major	xx (xx.x%)
Minor	xx (xx.x%)
By PD Category for Major PD	
xxxxxxx	xx (xx.x%)
xxxxxxx	xx (xx.x%)
xxxxxxx	xx (xx.x%)
xxxxxxx	xx (xx.x%)

Source Listing: 16.2.2
PD: Protocol deviation
If a participant has multiple occurrences of a PD, the participant is presented only once in the Participant count (n) column.
Percentages are calculated (the denominator used for the calculation) based on the number of safety analysis set in each treatment group (N).

Table 14.1.3 Summary of Demographics and Baseline Characteristics
(Intent to Treat Population)

Parameter	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
Age (months) at Screening	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	x.x
	Minimum, Maximum	xx.x, xx.x
Sex, n (%)	Female	xx (xx.x%)
	Male	xx (xx.x%)
Race, n (%)	American Indian or Alaska Native	xx (xx.x%)
	Asian	xx (xx.x%)
	Black or African American	xx (xx.x%)
	Native Hawaiian or other Pacific Islander	xx (xx.x%)
	White	xx (xx.x%)
	Other	xx (xx.x%)
Ethnicity, n (%)	Hispanic or Latino	xx (xx.x%)
	Not Hispanic or Latino	xx (xx.x%)
Bone Age (years) at Month 6	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	x.x
	Minimum, Maximum	xx.x, xx.x

Table 14.1.3 Summary of Demographics and Baseline Characteristics
(Intent to Treat Population)

Parameter	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
Weight (kg) at Screening	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Baseline Weight-SDS [1]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Birth Weight (kg)	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Gestational Age (weeks) at Birth	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Height (cm) at Screening [1]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x

Parameter	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
	Minimum, Maximum	xx.x, xx.x
Baseline Height-SDS [1]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Target Height-SDS [2]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Body Mass Index(kg/m^2) at Screening [3]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Baseline Body Mass Index-SDS [1]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Tanner Stage at Screening, n (%)	I	xx (xx.x%)
	II	xx (xx.x%)
	III	xx (xx.x%)
	IV	xx (xx.x%)
	V	xx (xx.x%)

Parameter	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
IGF-1 (nmol/L) at Screening	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Baseline IGF-1 SDS [4]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Baseline MPH height (cm) [5]	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x

Source Listing: 16.2.4.1
SD: Standard Deviation. SE: Standard Error. MPH: Mean Parental Height
Note: Percentages are calculated (the denominator used for the calculation) based on the number of participants in Intent to Treat population who received at least one dose of study treatment (N).
[1] Height is the average of the three height measurements collected. SDS values for height, weight, and BMI are calculated using WHO 2007 Child Growth Standards charts.
[2] Target Height-SDS will be computed from the observed maternal and paternal height, their height SDS are calculated using WHO Child Growth Standards charts. Target Height-SDS = MPH-SDS, MPH-SDS is derived using WHO growth chart values at age=228 months.
[3] Body Mass Index(kg/m^2) = weight (kg)/height $(m)^2$
[4] IGF-1 SDS values are provided by the laboratory and are derived from the IGF-1 concentration using the formula specified in the SAP.
[5] MPH = average of the parental heights +6.5 cm (for boys) or -6.5 cm (for girls).

Table 14.1.4 Treatment Exposure (Per Protocol Population)

Parameter	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
Treatment Duration (days)	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Number of Tablets Expected	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Number of Tablets Taken	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x
Compliance (%)	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	SE	xx.xxx
	Median	xx.x
	Minimum, Maximum	xx.x, xx.x

Source Listing: 16.2.5.2
SD: Standard Deviation.
Treatment duration (days) = Last dose date - First dose date + 1
Expected tablets = Treatment duration (days) * tablets per day.
Compliance (%) = (Taken tablets / Expected tablets) * 100%.

Table 14.1.5 Summary of Medical History
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Subjects with at least one Medical History	xx (xx.x%) x
SOC 1	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
….	xx (xx.x%) x
SOC 2	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
……	

Source: Listing 16.2.4.2

Note: If a participant has multiple occurrences of a medical history event, the participant is presented only once in the participant count (n) column for a given ATC and PT. Occurrences are counted each time in the mentions/occurrence (m) column.
Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1.

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.1.6 Summary of Prior Medications
(Intent to Treat Population)

Anatomical Therapeutic Class (ATC) [Level 3] Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Subjects with at least one Prior Medication	xx (xx.x%) x
ATC 1	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
….	xx (xx.x%) x
ATC 2	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
……	

Source: Listing 16.2.4.3

Prior medications are defined as any medication where the use was stopped prior to the first administration of the study medication. If a participant has multiple occurrences of a concomitant medication, the participant is presented only once in the participant count (n) column for a given ATC and PT. Occurrences are counted each time in the mentions/occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
World Health Organization-Drug Dictionary WHODrug Global Version Q3, 2020

Programming Note: *Table will be sorted by ATC/PT alphabetically.*

Table 14.1.7 Summary of Concomitant Medications
(Intent to Treat Population)

Anatomical Therapeutic Class (ATC) [Level 3] Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Subjects with at least one Concomitant Medication	xx (xx.x%) x
ATC 1	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
….	xx (xx.x%) x
ATC 2	xx (xx.x%) x
PT 1	xx (xx.x%) x
PT 2	xx (xx.x%) x
……	

Source: Listing 16.2.4.4

Concomitant medications are defined as any medication (other than the study drug) that was used at least once after the first administration of the study drug. If a participant has multiple occurrences of a concomitant medication, the participant is presented only once in the participant count (n) column for a given ATC and PT. Occurrences are counted each time in the mentions/occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
World Health Organization-Drug Dictionary WHODrug Global Version Q3, 2020

Programming Note: *Table will be sorted by ATC/PT alphabetically.*

Table 14.2.1 Summary of Annualized Height Velocity (cm/year) and Change from Baseline
(Per ProtocolPopulation)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

t1 to t2 Visit [1]	AHV Value (cm/year)						Change from Baseline [2]					
	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
Day 1 to Month 6	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
Day 1 to Month 12	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx

….

Source: Listing 16.2.6.1
AHV: Annualized Height Velocity. SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable. LOCF: Last Observation Carried Forward.
Height is the average of the three height measurements collected.
LOCF imputation method was applied to missing height data, up to and including Month 12.
[1] AHV at the time point t2 is defined by ((h2-h1) / (t2-t1)) * 365.25, where h2 and h1 are the heights (cm) measured at t2 and t1 (in days), respectively.
[2] Baseline is defined as Day 1 to Month 6.

Programming Note:

Table 14.2.2 Summary of Annualized Height Velocity (cm/year) Analysis of Covariance
(Per Protocol Population)

Visit	Statistic	LUM-201 3.2 mg/kg/day (N=xx)
Month 6 AHV (cm/year)	n	xx
	Least Squares Mean (LSM)	xx.x
	Standard Error of LSM	xx.x
	Lower 95% CI	xx.x
	Upper 95% CI	xx.x
Month 12 AHV (cm/year)	n	xx
	Least Squares Mean (LSM)	xx.x
	Standard Error of LSM	xx.x
	Lower 95% CI	xx.x
	Upper 95% CI	xx.x

Source: Listing 16.2.6.1
AHV: Annualized Height Velocity. LOCF: Last Observation Carried Forward.
An ANCOVA model was used for analysis of AHV at 6 and 12 months, with model terms for age, baseline HT-SDS, bone age delay.
LOCF imputation method was applied to missing height data, up to and including Month 12.

Programming Note:.

Table 14.2.3 Summary of Height (cm) and Change from Baseline
(Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

	Actual Value						Change from Baseline [1]					
Visit	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx

….

Source: Listing 16.2.6.1
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable. LOCF: Last Observation Carried Forward.
Height is the average of the three height measurements collected.
LOCF imputation method was applied to missing height data, up to and including Month 12.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.

***Programming Note**: Need to present data for all scheduled visits*

Table 14.2.4 Summary of Height-SDS and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.1
SDS: standard deviation score. SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable. LOCF: Last Observation Carried Forward.
Height is the average of the three height measurements collected.
LOCF imputation method was applied to missing height data, up to and including Month 12.
Standard deviation scores (SDS) were computed using percentile, and z-score data files from the World Health Organization (WHO) 2007 Child Growth Standards
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.

***Programming Note**: Need to present data for all scheduled visits.*

Table 14.2.5 Summary of Weight (kg) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.2

Table 14.2.6 Summary of Weight-SDS and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.2
SDS: standard deviation score. SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
Standard deviation scores (SDS) were computed using percentile, and z-score data files from the World Health Organization (WHO) 2007 Child Growth Standards

Table 14.2.7 Summary of BMI (kg/m2) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.3

Table 14.2.8 Summary of BMI-SDS and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.3
SDS: standard deviation score. SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
Standard deviation scores (SDS) were computed using percentile, and z-score data files from the World Health Organization (WHO) 2007 Child Growth Standards

Table 14.2.9 Summary of Growth Hormone (ng/mL) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.4

Table 14.2.10 Summary of IGF-1 (nmol/L) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.4

Table 14.2.11 Summary of IGF-1 SDS and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.4
SDS: standard deviation score. SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
IGF-1 SDS will be computed using reference standards for healthy children, based on IGF-1 concentration values provided by the central laboratory and calculated using the formula specified in the SAP.

Table 14.2.12 Summary of IGFBP-3 (nmol/L) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.4

Table 14.2.13 Summary of Bone Age (year) and Change from Baseline
(Per Protocol Population)

Same format as Table 14.2.3
Source: Listing 16.2.6.5
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
[1] Baseline data from Month 6 of Lum-201-01 study.

Table 14.3.3.1 Overall Summary of Treatment Emergent Adverse Events
(Intent to Treat Population)

	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Number of Participants Reporting at Least:	
One TEAE	xx (xx.x%) xx
One Serious TEAE	xx (xx.x%) xx
One Study Treatment Related TEAE	xx (xx.x%) xx
One TEAE Leading to Study Drug Interrupted	xx (xx.x%) xx
One TEAE Leading to Study Discontinuation	xx (xx.x%) xx
One TEAE Leading to Death	xx (xx.x%) xx

Source: Listing 16.2.7.1

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Table 14.3.3.2 Summary of Treatment Emergent Adverse Events by System Organ Class and Preferred Term
(Intent to Treat Population)

System Organ Class (SOC)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One TEAE	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
PT1	xx (xx.x%) xx
…..	
SOC2	xx (xx.x%) xx
PT1	xx (xx.x%) xx
PT2	xx (xx.x%) xx
PT3	xx (xx.x%) xx
PT4	xx (xx.x%) xx
….	

Source: Listing 16.2.7.1

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication.
If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.
Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.3 Summary of Serious Treatment Emergent Adverse Events by System Organ Class and Preferred Term
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One Serious TEAE	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
PT1	xx (xx.x%) xx
…..	
SOC2	xx (xx.x%) xx
PT1	xx (xx.x%) xx
PT2	xx (xx.x%) xx
PT3	xx (xx.x%) xx
PT4	xx (xx.x%) xx
….	

Source: Listing 16.2.7.2

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication.
If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.
Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.4 Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One TEAE	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
PT1	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
…..	

Source: Listing 16.2.7.1

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.5 Summary of Serious Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One Serious TEAE	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
PT1	xx (xx.x%) xx
Mild (Grade 1)	xx (xx.x%) xx
Moderate (Grade 2)	xx (xx.x%) xx
Severe (Grade 3)	xx (xx.x%) xx
Life-threatening (Grade 4)	xx (xx.x%) xx
Death (Grade 5)	xx (xx.x%) xx
…..	

Source: Listing 16.2.7.2

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.6 Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Relationship to Study Drug
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One TEAE	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
PT1	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
…..	

Source: Listing 16.2.7.1

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.7 Summary of Serious Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Relationship to Study Drug
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One Serious TEAE	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
PT1	xx (xx.x%) xx
Related	xx (xx.x%) xx
Not Related	xx (xx.x%) xx
…..	

Source: Listing 16.2.7.2

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.8 Summary of Treatment Emergent Adverse Events Leading to Study Drug Discontinuation by System Organ Class and Preferred Term
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One TEAE Leading to Study Drug Discontinuation	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
PT1	xx (xx.x%) xx
…..	
SOC2	xx (xx.x%) xx
PT1	xx (xx.x%) xx
PT2	xx (xx.x%) xx
PT3	xx (xx.x%) xx
PT4	xx (xx.x%) xx
….	

Source: Listing 16.2.7.3

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication.
If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.
Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.3.9 Summary of Treatment Emergent Adverse Events Leading to Death by System Organ Class and Preferred Term
(Intent to Treat Population)

System Organ Class (SOC) Preferred Term (PT)	LUM-201 3.2 mg/kg/day (N=xx) n (%) m
Participants with at Least One TEAE Leading to Death	xx (xx.x%) xx
SOC1	xx (xx.x%) xx
PT1	xx (xx.x%) xx
…..	
SOC2	xx (xx.x%) xx
PT1	xx (xx.x%) xx
PT2	xx (xx.x%) xx
PT3	xx (xx.x%) xx
PT4	xx (xx.x%) xx
….	

Source: Listing 16.2.7.1

Treatment Emergent Adverse Events (TEAEs) are defined as adverse events that occurred following the first administration of study medication. If a participant has multiple occurrences of a TEAE, the participant is presented only once in the Participant count (n) column for a given System Organ Class and Preferred Term. Occurrences are counted each time in the mentions/Occurrence (m) column.

Percentages are calculated (the denominator used for the calculation) based on the number of participants in the Intent to Treat population (N).
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Programming Note: *Table will be sorted by SOC/PT alphabetically.*

Table 14.3.4.1.1 Summary of Hematology and Change from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

		Actual Value						Change from Baseline [1]					
Parameter (Unit)	Visit	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
Etc.	….												

Source: Listing 16.2.8.1
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for all Hematology parameters at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.4.1.2 Summary of Hematology and Shift from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

				Post-Baseline		
Parameter (Unit)	Visit	Result Classification	Baseline[1] n(%)	Low n(%)	Normal n(%)	High n(%)
xxxxxxx (unit)	Baseline [1]	n	xx	N/A		
		Low	xx (xx.x%)			
		Normal	xx (xx.x%)			
		High	xx (xx.x%)			
	Month x	n	xx	xx	xx	xx
		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%)
xxxxxxx (unit)	Baseline [1]	n	xx	N/A		
		Low	xx (xx.x%)			
		Normal	xx (xx.x%)			
		High	xx (xx.x%)			
Etc.	*Etc.*	*Etc*				

Source: Listing 16.2.8.1
N/A: Not Applicable.
Number of participants at each visit (n) will be used as the denominator for the calculation of all percentages.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for all Hematology parameters at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.4.2.1 Summary of Serum Chemistry and Change from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

		Actual Value						Change from Baseline [1]					
Parameter (Unit)	Visit	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
Etc.	….												

Source: Listing 16.2.8.2
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for all chemistry parameters visits and sorted by parameters alphabetically.*

Table 14.3.4.2.2 Summary of Serum Chemistry and Shift from Baseline
(Intent to Treat Population)

Same format as Table 14.3.4.1.2
Source: Listing 16.2.8.2

N/A: Not Applicable.
Number of participants at each visit (n) will be used as the denominator for the calculation of all percentages.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for all Chemistry parameters at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.4.2.3 Summary of Thyroid Function and Change from Baseline
(Intent to Treat Population)

Same format as Table 14.3.4.2.1
Source: Listing 16.2.8.1

Table 14.3.4.2.4 Summary of Thyroid Function and Shifts from Baseline
(Intent to Treat Population)

Same format as Table 14.3.4.1.2
Source: Listing 16.2.8.1

Table 14.3.4.3.1 Summary of Urinalysis and Change from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

		Actual Value						Change from Baseline [1]					
Parameter (Unit)	Visit	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
xxxxxxx (unit)	Baseline [1]	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
	Month 1	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
Etc.	….												

Source: Listing 16.2.8.3
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for Urinalysis parameters as appropriate (Specific Gravity, pH) at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.4.3.2 Summary of Urinalysis (categorical) and Shift from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

				Post-Baseline				
Parameter (Unit)	Visit	Result Classification	Baseline[1] n(%)	Large n(%)	Moderate n(%)	Negative n(%)	Small n(%)	Trace n(%)
xxxxxxx (unit)	Baseline [1]	N	xx	N/A				
		Large	xx (xx.x%)					
		Moderate	xx (xx.x%)					
		Negative	xx (xx.x%)					
		Small	xx (xx.x%)					
		Trace	xx (xx.x%)					
	Month x	n	xx	xx	xx	xx	xx	xx
		Large	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)
		Moderate	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)
xxxxxxx (unit)	Baseline [1]	n	xx	N/A				
		Large	xx (xx.x%)					
		Moderate	xx (xx.x%)					
	Month x	n	xx	xx	xx	xx	xx	xx
		Large	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)
		Moderate	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)	xx (xx.x%)
Etc.	*Etc.*	Etc.						

Source: Listing 16.2.8.3
N/A: Not Applicable.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
For baseline, the number of participants (n) will be used as the denominator for the calculation of all percentages.
For post-baseline percentage calculations, use the number of subjects who classified the results at baseline as the denominator.
***Programming Note**: Need to present data for Urinalysis parameters as appropriate (Protein, Glucose, Blood, Ketones, Leukocytes) at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.5.1 Summary of Vital Signs and Change from Baseline
(Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day (N=xx)

			Actual Value						Change from Baseline [1]				
Parameter (Unit)		Timepoint	n	Mean (SD)	SE	Median	Min	Max	n	Mean (SD)	SE	Median	Min	Max
xxxxxxx (unit)	Baseline [1]	Pre-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
		1 H Post-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
		2 H Post-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month 1		xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x		xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
xxxxxxx (unit)	Baseline [1]	Pre-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	N/A					
		1 H Post-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
		2 H Post-Dose	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month 1		xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	Xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
	Month x		xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	Xx	xx	xx	xx.xx (xx.xxxx)	xx.xxxx	xx.xx	xx	xx
Etc.		….												

Source: Listing 16.2.9.1
SD: Standard Deviation. SE: Standard Error. Min: Minimum. Max: Maximum. N/A: Not Applicable. H: hour
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.

Programming Note: *Need to present data for all Vital Signs parameters at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.5.2.1 Summary of 12-Lead Electrocardiograms and Change from Baseline
(Intent to Treat Population)

Same format as Table 14.3.4.1.2
Source: Listing 16.2.9.2

N/A: Not Applicable.
Number of participants at each visit (n) will be used as the denominator for the calculation of all percentages.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for 12-Lead ECG (exclude overall interpretation) parameters at all scheduled visits and sorted by parameters alphabetically.*

Table 14.3.5.2.2 Summary of 12-Lead Electrocardiograms Interpretation
(Intent to Treat Population)

Parameter: Overall Interpretation of ECG

Visit	Timepoint	Result/Clinically Significant	LUM-201 3.2 mg/kg/day (N=xx) n(%)
Baseline [1]	Pre-Dose	n	xx
		Normal	Xx (xx.x%)
		Otherwise Normal	Xx (xx.x%)
		Abnormal	Xx (xx.x%)
	1 H Post-Dose	n	xx
		Normal	Xx (xx.x%)
		Otherwise Normal	Xx (xx.x%)
		Abnormal	Xx (xx.x%)
Month 1		n	xx
		Normal	Xx (xx.x%)
		Otherwise Normal	Xx (xx.x%)
		Abnormal	Xx (xx.x%)
Month x	Etc.	n	xx
		Normal	Xx (xx.x%)
		Otherwise Normal	Xx (xx.x%)
		Abnormal	Xx (xx.x%)
Etc.	Etc.	…	

Source: Listing 16.2.9.2

N/A: Not Applicable.
Number of participants at each visit (n) will be used as the denominator for the calculation of all percentages.
"Otherwise Normal" is noted as the overall interpretation on and ECG when minor findings are noted, but do not warrant an "Abnormal" interpretation.

[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.

Table 14.3.5.3 Summary of Physical Examination
(Intent to Treat Population)

Parameter: Overall Interpretation of ECG

Parameter	Visit	Result/Clinically Significant	LUM-201 3.2 mg/kg/day (N=xx) n (%)
Xxxxxxxx	Baseline [1]	n	xx
		Normal	Xx (xx.x%)
		Abnormal, NCS	Xx (xx.x%)
		Abnormal, CS	Xx (xx.x%)
	Month 12	n	xx
		Normal	Xx (xx.x%)
		Abnormal, NCS	Xx (xx.x%)
		Abnormal, CS	Xx (xx.x%)
Xxxxxxxx	Baseline [1]	n	xx
		Normal	Xx (xx.x%)
		Abnormal, NCS	Xx (xx.x%)
		Abnormal, CS	Xx (xx.x%)
	Month 12	n	xx
		Normal	Xx (xx.x%)
		Abnormal, NCS	Xx (xx.x%)
		Abnormal, CS	Xx (xx.x%)
Etc.	Etc.	…	

Source: Listing 16.2.9.3

NCS: Not Clinically significant. CS: Clinically significant.
Number of participants at each visit (n) will be used as the denominator for the calculation of all percentages.
[1] Baseline is defined as the last available, valid, non-missing observation prior to first study drug administration.
***Programming Note**: Need to present data for all Physical Examination parameters at all scheduled visits and sorted by parameters alphabetically.*

Listing 16.2.1.1 Analysis Populations (All Participants)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Intent to Treat Population/ If no, Reason for Exclusion	Safety Population/ If no, Reason for Exclusion	Per Protocol Population/ If no, Reason for Exclusion
XXXXXXX/Female	Yes	Yes	Yes
XXXXXXX/Male	Yes	Yes	Yes
XXXXXXX/Xxx	Yes	Yes	Yes
XXXXXXX/Xxx	Yes	No/xxxxxx	No/xxxxxx

Listing 16.2.1.2 Participant Disposition (All Enrolled Participants)

Treatment Arm: LUM-201 3.2 mg/kg/day

	Informed Consent			Re-consent						
Participant ID/Sex	Date (YYYY-MM-DD HH:MM)	Protocol Version	Informed Consent Version	Date (YYYY-MM-DD)	Protocol Version	Did the Participant Complete the Study?	Study Completion/ Early Termination Date (YYYY-MM-DD)	Primary Reason for Non-completion	Date of Last Dose (YYYY-MM-DD)	Reason for Withdrawal
XXXXXXX/F	YYYY-MM-DD HH:MM	x.x	xx.x	N/A		Yes	YYYY-MM-DD			
XXXXXXX/M	YYYY-MM-DD HH:MM	x.x	xx.x	N/A		Yes	YYYY-MM-DD			
XXXXXXX/X	YYYY-MM-DD HH:MM	x.x	xx.x	YYYY-MM-DD	x.x	No	YYYY-MM-DD	Withdrawal by Subject	YYYY-MM-DD	xxxxxxxxxxx
XXXXXXX/X	YYYY-MM-DD HH:MM	x.x	xx.x	YYYY-MM-DD	x.x	Yes	YYYY-MM-DD			
….										

F: Female. M: Male. N/A: Not Applicable.

Listing 16.2.1.3 Eligibility Criteria (All Participants)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Participant Eligible	Category Failed (Inclusion/ Exclusion)	Inclusion/ Exclusion Criterion not met
XXXXXXX/M	Screening	Yes		
XXXXXXX/F	Screening	Yes		
XXXXXXX/F	Screening	Yes		
XXXXXXX/M	Screening	No	Inclusion and Exclusion	Inclusion: INCL06, INCL09 Exclusion: EXCL02

F: Female. M: Male.

Listing 16.2.2 Protocol Deviations (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Date of Deviation (YYYY-MM-DD)	Type of Deviation	Deviation Description	Important Deviation
XXXXXXX/M	YYYY-MM-DD	YYYYYYYYYYYYYYY	XXXXXXXXXXXXX	No
XXXXXXX/F	YYYY-MM-DD	YYYYYYYYYYYYYYY	XXXXXXXXXXXXX	No
….				

F: Female. M: Male.

Listing 16.2.4.1 Demographics and Baseline Characteristics (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Date of Informed Consent (YYYY-MM-DD)	Age at Informed Consent (months)	Sex	Race	Ethnicity	Height (cm)	Target HT-SDS	HT-SDS	Weight (kg)	Weight-SDS	BMI (kg/m^2)
XXXXXXX/M	YYYY-MM-DD	xx	Female	Asian	xxxxx	xxx	xxxx	xxxxx	xx.x	xxxx	xx.x
XXXXXXX/F	YYYY-MM-DD	xx	Male	Asian	xxxxx	xxx	xxxx	xxxxx	xx.x	xxxx	xx.x
….											

F: Female. M: Male. N/A: Not Applicable. BMI: Body Mass Index. SDS: standard deviation score. MPH: mid-parental height

Participant ID/Sex	BMI-SDS	Tanner Stage	Date of Last rhGH Dose (YYYY-MM-DD)	Bone Age (years)	IGF-1 (nmol/L)	IGF-1-SDS [1]	Maternal Height (cm)	Paternal Height (cm)	MPH (cm)
XXXXXXX/M	xx.x	xx	YYYY-MM-DD	xx	xx	xx	xxx.x	xxx.x	
XXXXXXX/F	xx.x	xx	YYYY-MM-DD	xx	xx	xx	xxx.x	xxx.x	
….									

HT: Height. SDS: Standard deviation score. BMI: Body Mass Index. MPH: Mid-parental height
[1] IGF-1 SDS values are provided by the laboratory and are derived from the IGF-1 concentration using the formula specified in the SAP.

Listing 16.2.4.2 Medical History (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sequence number	Medical History Term/ System Organ Class /Preferred Term	Start Date (YYYY-MM-DD)	End Date (YYYY-MM-DD)
XXXXXXX/Male	1	XXXXXXXXXX/ YYYYYYYYY/ ZZZZZZZZZ	YYYY-MM-D/	YYYY-MM-DD
XXXXXXX/Female	2	XXXXXXXXXX/ YYYYYYYYY/ ZZZZZZZZZ	YYYY-MM-DD	Ongoing
…..	3			

Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Listing 16.2.4.3 Prior Medications (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sequence Number	Prior Medication Therapy/ Anatomical Therapeutic Class (ATC) [Level 3]/ Preferred Term (PT)	Indication	Start Date (YYYY-MM-DD) End Date (YYYY-MM-DD)	Dose	Unit	Form	Frequency	Route
XXXXXXX/M	xx	XXXXXXXXXXXXXXXXXXXXXX/ ZZZZZZZZZZZZZZZZZZZZZ/ YYYYYYYYYYYYYYYYYYYYY	Adverse Event	YYYY-MM-DD/ YYYY-MM-DD	xx	XX	Tablet	YYYYYY	ZZZZZ
		XXXXXXXXXXXXXXXXXXXXXX/ ZZZZZZZZZZZZZZZZZZZZZ/ YYYYYYYYYYYYYYYYYYYYY	Other:xxx	YYYY-MM-DD/ Ongoing	xx	XX	Capsule	YYYYYY	ZZZZZ
…		…							

F: Female. M: Male.
Prior medications are defined as any medication where the use was stopped prior to the first administration of the study medication.
World Health Organization-Drug Dictionary (WHO-DD) Version Q3, 2020.

Listing 16.2.4.4 Concomitant Medications (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sequence Number	Concomitant Medication Therapy/ Anatomical Therapeutic Class (ATC) [Level 3]/ Preferred Term (PT)	Indication	Start Date (YYYY-MM-DD) End Date (YYYY-MM-DD)	Dose	Unit	Form	Frequency	Route
XXXXXXX/M	xx	XXXXXXXXXXXXXXXXXXXXXX/ ZZZZZZZZZZZZZZZZZZZZZ/ YYYYYYYYYYYYYYYYYYYYY	Adverse Event	YYYY-MM-DD/ YYYY-MM-DD	xx	XX	Tablet	YYYYYY	ZZZZZ
		XXXXXXXXXXXXXXXXXXXXXX/ ZZZZZZZZZZZZZZZZZZZZZ/ YYYYYYYYYYYYYYYYYYYYY	Other:xxx	YYYY-MM-DD/ Ongoing	xx	XX	Capsule	YYYYYY	ZZZZZ
…		…							

F: Female. M: Male.
Concomitant medications are defined as any medication (other than the study drug) that was used at least once after the first administration of the study drug.
World Health Organization-Drug Dictionary (WHO-DD) Version Q3, 2020.

Listing 16.2.5.1 Study Drug Administration (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Was Dose Admin/ If No, Reason	Date of Admin (YYYY-MM-DD)	Time of Admin (HH:MM)	Tablet Strength (mg)	Number of Tablets
XXXXXXX/M	Month x	Yes	YYYY-MM-DD	HH:MM	Xx	Xx
XXXXXXX/F	Month x	Yes	YYYY-MM-DD	HH:MM	Xx	xx
XXXXXXX/F	Month x	No/xxxxxxxx				
...						

F: Female. M: Male. Admin: Administration. N/A: Not Applicable.

Listing 16.2.5.2 Study Drug Accountability (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Drug Dispensed at the Previous Visit	Date of Dispensed (YYYY-MM-DD)	If Yes, Number of Tablets Dispensed	Drug Accountability Performed at This Visit	If No, Reason for Not Done	Miss Any Doses of Study Medication Since Last Visit	If Yes, Meet a Stopping Rule or Treatment of Intercurrent Illness	If Yes, Number of Tablets Missed
XXXXXXX/M	Month x	Yes	YYYY-MM-DD	xx	No	xxxxx	No		
XXXXXXX/F	Month x	No	YYYY-MM-DD		Yes		Yes	No	xxxxxx
XXXXXXX/F	Month x	xxx	YYYY-MM-DD		xxx				
...									

F: Female. M: Male. N/A: Not Applicable.

Participant ID/Sex	Visit	Date of Returned (YYYY-MM-DD)	If Yes, When Were the Tablets not Taken	Total Number of Tablets Taken	Number of Tablets Returned	Any Tablets Lost or Unaccounted	If Yes, Number of Tablets lost or Unaccounted
XXXXXXX/M	Month x	YYYY-MM-DD	xxxxxx	xxxxxx	xxxxxx	No	
XXXXXXX/F	Month x	YYYY-MM-DD	xxxxxx	xxxxxx	xxxxxx	Yes	xxxxxx
XXXXXXX/F	Month x	YYYY-MM-DD	xxxxxx	xxxxxx	xxxxxx		
...							

Listing 16.2.6.1 Height and Annualized Height Velocity (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sex/Age (months)	Visit	Date of Assessment (YYYY-MM-DD)	Height (cm)	AHV Value (cm/year)	Height-SDS
XXXXXXX/Male	Male/xx	Screening	YYYY-MM-DD	xxx.x		
		Month 6	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month 12	YYYY-MM-DD	xxx.x	xx.xx	XX
XXXXXXX/Female	Female/xx	Screening	YYYY-MM-DD	xxx.x		
		Month 6	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month 12	YYYY-MM-DD	xxx.x	xx.xx	XX
...						

AHV: Annualized Height Velocity. SDS: Standard Deviation Scores. LOCF: Last Observation Carried Forward.
LOCF imputation method was applied to missing height data, up to and including Month 12.
AHV value is calculated at the time point t2 is defined by ((h2-h1) / (t2-t1)) * 365.25, where h2 and h1 are the heights (cm) measured at t2 and t1 (in days), respectively.
Height-SDS is calculated using World Health Organization (WHO) 2007 Child Growth Standards charts.

Listing 16.2.6.2 Weight (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sex/Age (months)	Visit	Date of Assessment (YYYY-MM-DD)	Weight (kg)	Change in Weight (kg)	Weight-SDS
XXXXXXX/Male	Male/xx	Screening	YYYY-MM-DD	xxx.x		
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
XXXXXXX/Female	Female/xx	Screening	YYYY-MM-DD	xxx.x		
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
...						

SDS: Standard Deviation Scores.
Weight-SDS is calculated using World Health Organization (WHO) 2007 Child Growth Standards charts.

Listing 16.2.6.3 Body Mass Index (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Sex/Age (months)	Visit	Date of Assessment (YYYY-MM-DD)	BMI (kg/m^2)	Change in BMI (kg/m^2)	BMI-SDS
XXXXXXX/Male	Male/xx	Screening	YYYY-MM-DD	xxx.x		
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
XXXXXXX/Female	Female/xx	Screening	YYYY-MM-DD	xxx.x		
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
		Month x	YYYY-MM-DD	xxx.x	xx.xx	XX
...						

BMI: Body Mass Index. SDS: Standard Deviation Scores.
BMI-SDS is calculated using World Health Organization (WHO) 2007 Child Growth Standards charts.

Listing 16.2.6.4 Serology (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx (unit)

Participant ID/Sex	Visit	Sample Collected/ If No, Reason	Collection Date/ Time (YYYY-MM-DD/ HH:MM)	Actual Value	Reference Range	Change from Baseline
XXXXXXX/Male						
	Screening (Baseline)	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A
	Month 1	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A
	Month 3	No/xxxxx				N/A
	Month 6	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx
	Month 12	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx
….	xxxxx	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx

N/A: Not Applicable.
IGF-1 SDS will be computed using reference standards for healthy children, based on IGF-1 concentration values provided by the central reference laboratory and calculated using the formula specified in the SAP.

Programming note: *Repeat using the same format by replacing the relevant parameters (Growth Hormone, IGF-1, IGF-1 SDS, IGFBP-3 and IGFBP-3 SDS) and parameters sorted by parameters alphabetically.*

Listing 16.2.6.5 Bone Age (Per Protocol Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Assessment Performed/ If No, Reason	Assessment Date (YYYY-MM-DD)	Method Used	Radiograph Date (YYYY-MM-DD)	Result (Year)	Change from Baseline [1]	Radiograph Sent to Central Reader
XXXXXXX/Male	Baseline [1]	Yes			YYYY-MM-DD	xx.x	N/A	
	Month 6	No/xxxxx	YYYY-MM-DD	GREULICH-PYLE			N/A	Yes
XXXXXXX/Male	Baseline [1]	Yes			YYYY-MM-DD	xx.x	N/A	
	Month 6	Yes	YYYY-MM-DD	Other:xxxxx	YYYY-MM-DD	xx.x	xx	
….	xxxxx	Yes	YYYY-MM-DD	xxxxxxx	YYYY-MM-DD	xx.x	xx	

N/A: Not Applicable.
[1] The baseline data are from Month 6 of LUM-201-01 study.

Listing 16.2.7.1 Adverse Events (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Adverse Event Verbatim/ System Organ Class/ Preferred Term	Start Date/ End Date/ (YYYY-MM-DD)	Start Time/ End Time/ (HH:MM)	Relationship to Study Treatment	CTCAE Grade	Action Taken/ Other Action	Outcome	AE Leading to Study Discontinue	SAE/ TEAE
XXXXXXX/M	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ YYYY-MM-DD	HH:MM/ HH:MM	Related	Mild	None/ None	Recovered/ Resolved	No	Yes/ Yes
XXXXXXX/F	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ Ongoing	HH:MM	Not Related	Moderate	Drug Therapy / Medication Given	Recovering / Resolving	No	Yes/ Yes

….

F: Female. M: Male. SAE: Serious Adverse Event. TEAE: A treatment-emergent adverse event.

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Listing 16.2.7.2 Serious Adverse Events (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Adverse Event Verbatim/ System Organ Class/ Preferred Term	Start Date/ End Date/ (YYYY-MM-DD)	Start Time/ End Time/ (HH:MM)	SAE Criteria	TEAE	CTCAE Grade	Relationship to Study Treatment	Action Taken/Other Action	Outcome
XXXXXXX/M	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ YYYY-MM-DD	HH:MM/ HH:MM	Requires or Prolongs Hospitalization	Yes	Mild	Related	None/xxx	Recovered/ Resolved
XXXXXXX/F	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ Ongoing	HH:MM	Life Threatening	Yes	Moderate	Not Related	Drug withdrawn/xxx	Recovering/ Resolving

….

F: Female. M: Male. SAE: Serious Adverse Event. TEAE: A treatment-emergent adverse event.
Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Listing 16.2.7.3 Adverse Events Leading to Study Drug Discontinuation (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Adverse Event Verbatim/ System Organ Class/ Preferred Term	Start Date/ End Date/ (YYYY-MM-DD)	Start Time/ End Time/ (HH:MM)	SAE	CTCAE Grade	Relationship to Study Treatment	Action Taken	Concomitant Medication/ Other Action	Outcome	TEAE
XXXXXXX/F	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ YYYY-MM-DD	HH:MM/ HH:MM	Yes	Mild	Related	N/A	Yes	Recovered/ Resolved	Yes
XXXXXXX/M	XXXXXXXXXXXXXXXXXX ZZZZZZZZZZZZZZZZZZ YYYYYYYYYYYYYYYYYY	YYYY-MM-DD/ Ongoing	HH:MM	Yes	Moderate	Not Related	Dose not changed	No/ xxxxxxxxxxxx	Recovering / Resolving	Yes

….

F: Female. M: Male. SAE: Serious Adverse Event. TEAE: A treatment-emergent adverse event. N/A: Not Applicable.

Medical Dictionary for Regulatory Activities (MedDRA) Version 23.1

Listing 16.2.8.1 Hematology (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx(unit)

Participant ID/Sex	Visit	Sample Collected/ If No, Reason	Collection Date/ Time (YYYY-MM-DD/ HH:MM)	Actual Value	Reference Range	Change from Baseline [1]	Abnormal Flag	Clinically Significant
XXXXXXX/Male	Screening (Baseline) [1]	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A	H	Yes
	Month 1	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A	L	No
	Month 3	No/xxxxx				N/A		
	Month 6	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		No
	Month 12	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		No
….	xxxxx	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		Yes

N/A: Not Applicable. H: High (Above Normal Range). L: Low (Below Normal Range)
[1] Baseline is defined as the last available valid, non-missing observation prior to first study drug administration

***Programming note**: Repeat using the same format by replacing the relevant parameters (including HbA1c, T3, T4, and TSH) and parameters sorted by parameters alphabetically.*

Listing 16.2.8.2 Serum Chemistry (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx (unit)

Participant ID/Sex	Visit	Sample Collected/ If No, Reason	Collection Date/ Time (YYYY-MM-DD/ HH:MM)	Actual Value	Reference Range	Change from Baseline [1]	Abnormal Flag	Clinically Significant
XXXXXXX/Male	Screening (Baseline) [1]	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A	H	Yes
	Month 1	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	N/A	L	No
	Month 3	No/xxxxx				N/A		
	Month 6	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		No
	Month 12	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		No
….	xxxxx	Yes	YYYY-MM-DD/ HH:MM	xx	xx, xx	xx		Yes

N/A: Not Applicable. H: High (Above Normal Range). L: Low (Below Normal Range)
[1] Baseline is defined as the last available valid, non-missing observation prior to first study drug administration

***Programming note**: Repeat using the same format by replacing the relevant parameters and parameters sorted by parameters alphabetically.*

Listing 16.2.8.3 Urinalysis (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx (unit)

Participant ID/Sex	Visit	Sample Collected/ If No, Reason	Collection Date/ Time (YYYY-MM-DD/ HH:MM)	Result	Change from Baseline [1]	Clinically Significant
XXXXXXX/Male	Screening (Baseline) [1]	Yes	YYYY-MM-DD/ HH:MM	xx		
	Month 1	No/xxxxx				
	Month x	Yes	YYYY-MM-DD/ HH:MM	xx	xx	No
	Month x	Yes	YYYY-MM-DD/ HH:MM	xx	xx	No
……	Screening	Yes	YYYY-MM-DD/ HH:MM	xx		Yes

N/A: Not Applicable.
[1] Baseline is defined as the last available valid, non-missing observation prior to first study drug administration

Programming note: *Repeat using the same format by replacing the relevant parameters and parameters sorted by parameters alphabetically.*

Listing 16.2.9.1 Vital Signs (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx (unit)

Participant ID/Sex	Visit	Time Point	Performed/ If No, Reason	Assessment Date/ Time (YYYY-MM-DD/ HH:MM)	Actual Value	Change from Baseline [1]	Clinical Significant
XXXXXXX/Male	Screening		Yes	YYYY-MM-DD/ HH:MM	Xx/xx		Yes
	Month 1 (Baseline) [1]	Pre-Dose	Yes	YYYY-MM-DD/ HH:MM	Xx/xx	xx	No
		1 hour	Yes	YYYY-MM-DD/ HH:MM	Xx/xx	xx	
		2 hours	Yes	YYYY-MM-DD/ HH:MM	Xx/xx		
	Month x		Yes	YYYY-MM-DD/ HH:MM	Xx/xx		
	Month x			YYYY-MM-DD/ HH:MM	Xx/xx		
				YYYY-MM-DD/ HH:MM	Xx/xx		
				YYYY-MM-DD/ HH:MM	Xx/xx		

….

N/A: Not Applicable. CS: Clinically Significant, NCS: Not Clinically Significant.
[1] Baseline is defined as the last available valid, non-missing observation prior to first study drug administration
Programming note: *Repeat using the same format by replacing the relevant parameters and parameters sorted by parameters alphabetically.*

Listing 16.2.9.2 12-Lead Electrocardiograms (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day
Parameter: xxxxxxx (mmHg)

Participant ID/Sex	Visit	Time Point	Performed/ If No, Reason	Assessment Date/ Time (YYYY-MM-DD/ HH:MM)	Actual Value	Change from Baseline [1]
XXXXXXX/Male	Screening (Baseline) [1]	Pre-Dose	Yes	YYYY-MM-DD/ HH:MM	Xx/xx	
		1 hour	Yes	YYYY-MM-DD/ HH:MM	Xx/xx	xx
	Month 1			YYYY-MM-DD/ HH:MM	Xx/xx	xx
	Month x		Yes	YYYY-MM-DD/ HH:MM	Xx/xx	
	Month x		No/xxxx			
….	Screening		Yes	YYYY-MM-DD/ HH:MM	Xx/xx	

N/A: Not Applicable. CS: Clinically Significant, NCS: Not Clinically Significant.
"Otherwise Normal" is noted as the overall interpretation on and ECG when minor findings are noted, but do not warrant an "Abnormal" interpretation.
[1] Baseline is defined as the last available valid, non-missing observation prior to first study drug administration

Programming note: *Repeat using the same format by replacing the relevant parameters and parameters sorted by parameters alphabetically.*

Listing 16.2.9.3 Physical Examination (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Performed/ If No, Reason	Assessment Date (YYYY-MM-DD)	Body System	Result	Description of Abnormality
XXXXXXX/Male	Screening (Baseline)	Yes	YYYY-MM-DD	General Appearance	Normal	
			YYYY-MM-DD	HEENT	Abnormal, CS	YYYYYYYYYY
			YYYY-MM-DD	Mouth	Normal	
			YYYY-MM-DD	Skin	Normal	
			YYYY-MM-DD	…		
	Month x	Yes	YYYY-MM-DD	General Appearance	Normal	
			YYYY-MM-DD	HEENT	Normal	
			YYYY-MM-DD	Mouth	Normal	
			YYYY-MM-DD	Skin	Normal	
			YYYY-MM-DD	…	Normal	
XXXXXXX/Male	Screening (Baseline)	Yes	YYYY-MM-DD	General Appearance	Normal	
		No/xxxx	YYYY-MM-DD	HEENT	Not done	
			YYYY-MM-DD	Mouth	Abnormal, NCS	ZZZZZZZZZZZ
			YYYY-MM-DD	Skin	Normal	
			YYYY-MM-DD	….		
			YYYY-MM-DD	….		

HEENT: Head, Eyes, Ears, Nose, Throat. CS: Clinically Significant, NCS: Not Clinically Significant.

Listing 16.2.9.4 Tanner Stage (Intent to Treat Population)

Treatment Arm: LUM-201 3.2 mg/kg/day

Participant ID/Sex	Visit	Tanner Staging Performed/ If No, Reason	Assessment Date (YYYY-MM-DD)	Breast Development Stage Assessed	Testicular Volume Assessed (ml)	Public Hair Development
XXXXXXX/Male	Screening (Baseline)	Yes	YYYY-MM-DD		x.x	III
	Month 3	No/xxxxx				
	Month x	Yes	YYYY-MM-DD		x.x	III
	Month x	Yes	YYYY-MM-DD		x.x	III
	Month x	Yes	YYYY-MM-DD		x.x	III
XXXXXXX/Female	Screening (Baseline)	Yes	YYYY-MM-DD	II		

…….