Phase III, Single-arm, Open-label, International, Multi-centre Study to Evaluate the Efficacy and Safety of Lomitapide in Paediatric Patients with Homozygous Familial Hypercholesterolaemia (HoFH) on Stable Lipid-lowering Therapy

Amryt Pharma Study No: APH-19
SQN Clinical Study No: OCH19003

Table, Figure and Listing Shells

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TABLE/FIGURE/LISTING CONTENTS

1 PROGRAMMING NOTES

''Summary statistics'' for quantitative data will be recorded as n, mean, standard deviation, minimum, median and maximum, 25th & 75th percentile (as specified in shell). For categorical data, a frequency table (showing n and %) will replace this summary.

Data manipulations will be detailed as appropriate in the SAS code. Derived database (DDB) specifications will be created and maintained by Veristat.

All tables, listings and figures will use recommended ICH numbering.

Complex manipulations should be detailed in the body of the SAP text or as ''programming notes'' on the table shells.

Decimal places will be as follows (where applicable), where x is the precision of the raw data:

- x +2 (standard errors, standard deviations, CV%).
- x +1 (means, medians, confidence intervals).
- x (min and max).
- 1 decimal place (percentages). Percentages will not be displayed where n=0.

Percentages will be based on the number of subjects in the analysis set/population, for the relevant treatment group (regardless of time point), unless otherwise stated.

The 'missing' category, where used, will not include subjects who have withdrawn at earlier visits. Subjects with missing results or assessment not done at visit x will be included in the missing category only if they have a visit x or a visit greater than x in the visit date listing.

Page set-up specifications for the SAS output TFLs:

- Word document landscape
- Word document margins set to:
    - top = 2.54 cm
    - bottom = 1.5 cm
    - left = 2 cm
    - right = 2 cm
    - gutter = 0 cm
    - header = 1.25 cm
    - footer = 1.25 cm
- Word document font: Courier New, 8pt
- SAS output options ls=141 ps=52

All output will be provided as individual and collated .rtf files.

The sponsor study number and sponsor name will be displayed in the top left hand side of the TFLs and page numbers will be displayed in the top right hand side of the TFLs.

For each listing, only the visits/time points where the data was collected will be listed, including unscheduled assessments where applicable. Tables will show results for all visits and time points where data was collected, unless specified otherwise within the shell (see programming notes).

Other standards and conventions:

- General:
  - UK spelling used in all tables, listings and figures.
  - Output spans entire width of page (where possible).
  - Top line spanning output but no line at bottom.
  - Numeric results aligned by decimal place.
- Listings only:
  - Ordered by age group (5-10,11-15 and 16-<=17), visit and subject number (as applicable).
  - All dates will be displayed as DDMONYYYY. Missing days will be displayed in the listings as ‘UK’, missing months as ‘UNK’ and missing years as ‘UKUK’. Add footnote ‘UK/UNK = Unknown.’ where required.
  - Data will be presented in date order.
  - Leave all footnote decodes such as L = …, H = …. even if not actually within data listed.
  - Where adding a footnote for decodes such as Route/Frequency, add all possible values from CRF rather than just those in the data.
  - Where one row per subject – no break lines. Where more than one row per subject – break after subject/visit/time point as applicable.
- Tables only:
  - Remove percentages when count is zero.
  - All results should be aligned. For example, numbers from summary statistics should align with results from frequency counts.

Table 14.1.1.1 Subject Disposition
(Enrolled Set)

	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Screen Failures, n	XX	XX	XX
Enrolled Set [1], n (%)*	XX (XX.X)	XX (XX.X)	XX (XX.X)
Run-in Failures, n (%)*	XX (XX.X)	XX (XX.X)	XX (XX.X)
Completed Run-In Period, n (%)*	XX (XX.X)	XX (XX.X)	XX (XX.X)
Safety Set (SAF) [2], n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Full Analysis Set (FAS) [3], n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Completers Analysis Set (CAS) [4], n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Per Protocol Set (PPS) [5], n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects who completed the Safety Phase (week 104) of the study, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects who prematurely discontinued study, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Reason for study withdrawal n (%)			
Adverse Event	XX (XX.X)	XX (XX.X)	XX (XX.X)
Discontinuation of the study by sponsor	XX (XX.X)	XX (XX.X)	XX (XX.X)
Lost to follow-up	XX (XX.X)	XX (XX.X)	XX (XX.X)
Non-compliance	XX (XX.X)	XX (XX.X)	XX (XX.X)
Pregnancy	XX (XX.X)	XX (XX.X)	XX (XX.X)
Protocol Deviation	XX (XX.X)	XX (XX.X)	XX (XX.X)
Voluntary Withdrawal	XX (XX.X)	XX (XX.X)	XX (XX.X)
COVID-19	XX (XX.X)	XX (XX.X)	XX (XX.X)
Death	XX (XX.X)	XX (XX.X)	XX (XX.X)
Other	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects enrolled. n = the number of subjects meeting the criterion.
[*] Percentages based off the Enrolled Set. All other percentages based off the Safety Analysis Set.
[**] The calculation of study duration excludes screen failure patients.
[1] The Enrolled Set includes all subjects who pass screening and enter into the run-in period, irrespective of whether they receive the study treatment.
[2] The Safety Analysis Set includes all subjects who are treated with at least one dose of the IMP.
[3] The Full Analysis Set includes all subjects who receive at least one dose of the IMP and who have a baseline and at least one post-baseline measurement of LDL-C.
[4] The Completers Analysis Set includes all subjects from the FAS who have not discontinued the Efficacy Phase (up to Week 24) of the study early irrespective of the reason for discontinuation.
[5] The Per Protocol Set includes all patients from the FAS who reasonably adhere to all protocol conditions (i.e. who have met the eligibility criteria and receive planned study medication without important protocol deviations considered to have a serious impact on the efficacy results within the Efficacy Phase of the trial).
Source: Listing 16.2.1.1 and Listing 16.2.3.1

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Table 14.1.1.1 Subject Disposition
(Enrolled Set)

	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Study duration (weeks)**			
n	XX	XX	XX
Mean	XX.X	XX.X	XX.X
SD	XX.XX	XX.XX	XX.XX
Minimum	XX.X	XX.X	XX.X
Median	XX.X	XX.X	XX.X
Maximum	XX.X	XX.X	XX.X
Treatment duration (weeks)			
n	XX	XX	XX
Mean	XX.X	XX.X	XX.X
SD	XX.XX	XX.XX	XX.XX
Minimum	XX.X	XX.X	XX.X
Median	XX.X	XX.X	XX.X
Maximum	XX.X	XX.X	XX.X

N = the number of subjects enrolled. n = the number of subjects meeting the criterion.
[*] Percentages based off the Enrolled Set. All other percentages based off the Safety Analysis Set.
[**] The calculation of study duration excludes screen failure patients.
[1] The Enrolled Set includes all subjects who pass screening and enter into the run-in period, irrespective of whether they receive the study treatment.
[2] The Safety Analysis Set includes all subjects who are treated with at least one dose of the IMP.
[3] The Full Analysis Set includes all subjects who receive at least one dose of the IMP and who have a baseline and at least one post-baseline measurement of LDL-C.
[4] The Completers Analysis Set includes all subjects from the FAS who have not discontinued the Efficacy Phase (up to Week 24) of the study early irrespective of the reason for discontinuation.
[5] The Per Protocol Set includes all patients from the FAS who reasonably adhere to all protocol conditions (i.e. who have met the eligibility criteria and receive planned study medication without important protocol deviations considered to have a serious impact on the efficacy results within the Efficacy Phase of the trial).
Source: Listing 16.2.1.1 and Listing 16.2.3.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.1.2 Subject Study Visits
(Enrolled Set)

	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Run-in period:			
Visit 2 (Enrolment), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 3 (Compliance), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Efficacy Phase:			
Visit 4 (Week 0 / Day 0), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 5 (Week 4 / Day 28), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 6 (Week 8 / Day 56), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 7 (Week 12 / Day 84), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 8 (Week 16 / Day 112), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 9 (Week 20 / Day 140), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 10 (Week 24 / Day 168), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Safety Phase:			
Visit 11 (Week 28 / Day 196), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 12 (Week 32 / Day 224), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 13 (Week 36 / Day 252), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 14 (Week 40 / Day 280), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 15 (Week 44 / Day 308), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 16 (Week 48 / Day 336), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 17 (Week 52 / Day 364), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 18 (Week 56 / Day 392), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 19 (Week 68 / Day 479), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 20 (Week 80 / Day 560), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Visit 21 (Week 92 / Day 644), n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
End Of Treatment:			
Visit 22 (EOT / Week 104)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Follow-up:			
Visit 23 (Week 108)	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects enrolled. n = the number of subjects meeting the criterion.
Percentages based off the Enrolled Set.
Source: Listing 16.2.16.1

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Table 14.1.2.1 Demography
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Sex, n (%)	Male	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Female	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Unknown	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
Age (years)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
Race, n (%)	Black or African American	XX (XX.X)	XX (XX.X)	XX (XX.X)
	American Indian or Alaska Native	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Asian	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Native Hawaiian or other Pacific Islander	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Arabic	XX (XX.X)	XX (XX.X)	XX (XX.X)
	White	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
Ethnicity, n (%)	Hispanic or Latino	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Not Hispanic or Latino	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Not Reported	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Unknown	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] BMI = {weight(kg) / height(m)^2}
[2] BSA (m^2) = √[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Age is calculated in years = [full age in month (months + years) / 12]

Table 14.1.2.1 Demography
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Country, n (%)	Germany	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Spain	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Italy	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Saudi Arabia	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Tunisia	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Israel	XX (XX.X)	XX (XX.X)	XX (XX.X)
Height (cm) at Baseline	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Weight (kg) at Baseline	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] BMI = {weight(kg) / height(m)^2}
[2] BSA (m^2) = √[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Age is calculated in years = [full age in month (months + years) / 12]

Table 14.1.2.1 Demography
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Body mass index (kg/m^2) at Baseline [1]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Body surface area (BSA) at Baseline [2]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Height percentile [3]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] BMI = {weight(kg) / height(m)^2}
[2] BSA (m^2) = $\sqrt{}$[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Age is calculated in years = [full age in month (months + years) / 12]

Table 14.1.2.1 Demography
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Height Z Score [3]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
BMI percentile [3]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
BMI Z Score [3]	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] BMI = {weight(kg) / height(m)^2}
[2] BSA (m^2) = √[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Age is calculated in years = [full age in month (months + years) / 12]

Table 14.1.2.2 Baseline Lipids
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
LDL-C (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Non-HDL-C (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Total Cholesterol (TC) (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
LDL-C, Non-HDL-C, Cholesterol, VLDL-C, Apo B, Triglycerides, HDL-C, Apo A1 summarised using Central Lab values.
Lp(a) summarised using Local Lab values.
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.2.2 Baseline Lipids
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
VLDL-C (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Apo B (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Triglycerides (TG) (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
LDL-C, Non-HDL-C, Cholesterol, VLDL-C, Apo B, Triglycerides, HDL-C, Apo A1 summarised using Central Lab values.
Lp(a) summarised using Local Lab values.
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.2.2 Baseline Lipids
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Apo A1 (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
HDL-C (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Lp(a) (mg/dL)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
LDL-C, Non-HDL-C, Cholesterol, VLDL-C, Apo B, Triglycerides, HDL-C, Apo A1 summarised using Central Lab values.
Lp(a) summarised using Local Lab values.
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.2.2 Baseline Lipids
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Lp(a) (nmol/L)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
TC/HDL-C	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX.X	XX.X	XX.X
	Median	XX.X	XX.X	XX.X
	Maximum	XX.X	XX.X	XX.X
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
LDL-C, Non-HDL-C, Cholesterol, VLDL-C, Apo B, Triglycerides, HDL-C, Apo A1 summarised using Central Lab values.
Lp(a) summarised using Local Lab values.
Source: Listing 16.2.4.1, Listing 16.2.4.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.2.3 Diagnosis of HoFH
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Duration of HoFH diagnosis (weeks) [1]				
	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Genetic confirmation of 2 mutant alleles, n (%)	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Type of mutant allele, n (%)	LDLR gene locus	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Apo B gene locus	XX (XX.X)	XX (XX.X)	XX (XX.X)
	PCSK9 gene locus	XX (XX.X)	XX (XX.X)	XX (XX.X)
	LDLRAP1 gene locus	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Unknown	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
LDL receptor function mutation	LDLR-deficient mutation	XX (XX.X)	XX (XX.X)	XX (XX.X)
	LDLR-defective mutation	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Duration of diagnosis is defined as the time in weeks from diagnosis to baseline.
Source: Listing 16.2.4.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.2.3 Diagnosis of HoFH
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
An untreated LDL-C >500mg/dL (13mmol/L) or treated LDLC ≥300mg/dL (8mmol/L) AND cutaneous or tendon xanthoma before age of 10 years? n (%)	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
An untreated LDL-C >500mg/dL (13mmol/L) or treated LDLC ≥300mg/dL (8mmol/L) AND untreated LDL-C levels consistent with HoFH in both parents? n (%)	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Duration of diagnosis is defined as the time in weeks from diagnosis to baseline.
Source: Listing 16.2.4.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.3.1 Medical History
(Safety Analysis Set)

Ongoing conditions

System organ class Preferred term	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any medical history	XX (XX.X)	XX (XX.X)	XX (XX.X)
System organ class 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
System organ class 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Medical history events were coded using the MedDRA Dictionary, version xx.x.
System organ classes (SOC) are ordered in decreasing frequency of the total number of subjects with medical histories reported in each SOC and preferred terms are ordered within a SOC in decreasing frequency of the total number of subjects with each medical history.
N = the number of subjects in the analysis set. n = the number of subjects with medical history. (%) = n/N*100.
Source: Listing 16.2.4.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for 'Previous conditions'.

Table 14.1.3.2 Cardiovascular History
(Safety Analysis Set)

Ongoing conditions

System organ class Preferred term	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any cardiovascular history	XX (XX.X)	XX (XX.X)	XX (XX.X)
System organ class 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
System organ class 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Preferred term 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Cardiovascular history events were coded using the MedDRA Dictionary, version xx.x.
System organ classes (SOC) are ordered in decreasing frequency of the total number of subjects with cardiovascular histories reported in each SOC and preferred terms are ordered within a SOC in decreasing frequency of the total number of subjects with each cardiovascular history.
N = the number of subjects in the analysis set. n = the number of subjects with medical history. (%) = n/N*100.
Cardiovascular History will be identified using pre-specified SOC and PT terms.
Source: Listing 16.2.4.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for 'Previous conditions'.

Table 14.1.3.3 Cardiovascular Disease History at Screening
(Safety Analysis Set)

Type of cardiovascular disease	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with cardiovascular history at screening, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Valvular disease, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Coronary artery disease, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Other, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Cardiovascular disease ongoing, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Duration of cardiovascular disease (months)*			
n	XX	XX	XX
Mean	XX.X	XX.X	XX.X
SD	XX.XX	XX.XX	XX.XX
Minimum	XX	XX	XX
Median	XX.X	XX.X	XX.X
Maximum	XX	XX	XX
25th percentile	XX.X	XX.X	XX.X
75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = m/n*100.
* Duration of cardiovascular disease (months). For those with ongoing CVD, duration is calculated up to date of screening visit.
Source: Listing 16.2.4.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.4.1 Prior and 'Run-in Medications' Medications
(Safety Analysis Set)

Medication class Standardised medication name	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any prior and/or 'Run-in' medication	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Medications were coded using WHO Drug Dictionary, version xxx.
Medication classes and standardised medication names are ordered by descending frequency.
Prior medications / procedures are defined as those that started and ended prior to the enrolment date visit (visit 2) (i.e. start of the 'run-in period').
Run-in period' medications / procedures are defined as those that are ongoing at the time of starting the 'run-in' period (i.e. Visit 2) or started after the start of the 'run-in' period, but before the time of first administration of study treatment.
N = the number of subjects in the analysis set receiving the treatment. n = the number of subjects taking a prior/'run-in' medication. (%) = n/N*100.
[*] Indicates prior medication only.
Source: Listing 16.2.4.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Please flag (*) medications that are prior only medications.

Table 14.1.4.2 Concomitant Medications
(Safety Analysis Set)

Medication class Standardised medication name	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any concomitant medication	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Medications were coded using WHO Drug Dictionary, version xxx.
Medication classes and standardised medication names are ordered by descending frequency.
Concomitant medications / procedures are defined as those that are ongoing at the time of first administration of study treatment or started on or after the time of first administration of study treatment or started prior to the end / last dose of study treatment. If medication / procedure dates are incomplete and it is not clear whether the medication / procedure was concomitant, it will be assumed to be concomitant.
N = the number of subjects in the analysis set receiving the treatment. n = the number of subjects taking a concomitant medication. (%) = n/N*100.
Source: Listing 16.2.4.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.4.3 Lipid-Lowering-Therapy Medications
(Safety Analysis Set)

Prior and 'Run-in' LLT medications

Medication class Standardised medication name	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any prior and/or 'run-in' LLT medication	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects with >= 2 'run-in' LLT medications	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects with >= 3 'run-in' LLT medications	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Medication class 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Standardised medication name 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Medications were coded using WHO Drug Dictionary, version xxx.
Medication classes and standardised medication names are ordered by descending frequency.
Prior Lipid-lowering therapy medications and procedures are defined as those that started and ended prior to the enrolment visit (visit 2) date (i.e. start of the 'run-in period').
Run-in' periord Lipid-lowering therapy medications and procedures are defined as any lipid-lowering therapy or procedure that are ongoing at the enrolment visit (visit 2) date (i.e. start of the 'run-in period') and / or started after the start of the 'run-in period', but before the time of first administration of study treatment.
N = the number of subjects in the analysis set receiving the treatment. n = the number of subjects with a prior and/or 'run-in' medication.
(%) = n/N*100.
[*] Indicates prior medication only.
Source: Listing 16.2.4.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for Concomitant LLT medications (efficacy phase); Concomitant LLT medications (safety phase).
Update first row table text and footnotes accordingly.
Please flag (*) medications that are prior only medications.

Table 14.1.4.5 Procedures
(Safety Analysis Set)

Procedure category Indication	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects with any procedure	XX (XX.X)	XX (XX.X)	XX (XX.X)
Procedure category 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Procedure category 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
Indication 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
Etc...			

Procedures were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set receiving the treatment. n = the number of subjects with procedure. (%) = n/N*100.
Source: Listing 16.2.4.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.4.6 Days from most recent apheresis to the corresponding LDL-C primary efficacy assessment
(Safety Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Established regimen during run-in	Twice a week	XX (XX.X)	XX (XX.X)
	Weekly	XX (XX.X)	XX (XX.X)
	Every two weeks	XX (XX.X)	XX (XX.X)
	Other	XX (XX.X)	XX (XX.X)
Weighted average number of days from previous LA during run-in (days) [1]	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X
Days from most recent apheresis to LDL-C assessment on [2]:			
Baseline	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X
Week 24/LOCF	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X

Only subjects on apheresis at baseline are presented.
[1] Established apheresis regimen during run-in in days. The weighted average number of days is summarised.
[2] Days from most recent apheresis to baseline LDL-C assessment and to Week 24 are summarised. For subjects discontinuing before Week 24, the duration between the most recent apheresis and the LDL-C assessment that was carried forward is presented.
N = the number of subjects in the analysis set receiving the treatment. n = the number of subjects with procedure. (%) = n/N*100.

Source: Listing 16.2.6.2, Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: For discontinued subjects, note the Week number used for the LOCF in the footnotes.
Add categories for established regimen as applicable.

Table 14.1.5.1 Study Drug Exposure and Dosing
(Safety Analysis Set)

Study Phase

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Exposure (weeks)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Exposure (mg / day)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Maximum Tolerated Dose (mg), n (%)	5mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	10mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	20mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	30mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	40mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	60mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
Exposure to maintenance dose (weeks)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.

[1] Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Additional doses may be added to Maximum Tolerated Dose as appropriate.***
Subject percentage compliance for dosing as per protocol (Efficacy Phase) - Use post-baseline study visits Week 4 to Week 24 inclusive
Subject percentage compliance for dosing as per protocol (Safety Phase) - Use post-baseline study visits Week 28 to Week 104 inclusive
Subject percentage compliance for dosing as per protocol (Overall) - Use post-baseline study visits Week 4 to Week 104 inclusive
If a subject discontinues early during the efficacy phase, Use post-baseline study visits Week 4 to Week 24 and EOT/Week 104, as appropriate.
For the compliant with IMP dosing present only the corresponding to the study phase.

Table 14.1.5.1 Study Drug Exposure and Dosing
(Safety Analysis Set)

Study Phase

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of Doses Received	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Dose Increases	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Dose Reductions	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Additional doses may be added to Maximum Tolerated Dose as appropriate.
Subject percentage compliance for dosing as per protocol (Efficacy Phase) - Use post-baseline study visits Week 4 to Week 24 inclusive

Subject percentage compliance for dosing as per protocol (Safety Phase) - Use post-baseline study visits Week 28 to Week 104 inclusive
Subject percentage compliance for dosing as per protocol (Overall) - Use post-baseline study visits Week 4 to Week 104 inclusive
If a subject discontinues early during the efficacy phase, Use post-baseline study visits Week 4 to Week 24 and EOT/Week 104, as appropriate.
For the compliant with IMP dosing present only the corresponding to the study phase.

Table 14.1.5.1 Study Drug Exposure and Dosing
(Safety Analysis Set)

Study Phase

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of Dose Interruptions	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Dose Reductions due to concomitant medication	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Dose Reductions due to adverse events	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Additional doses may be added to Maximum Tolerated Dose as appropriate.

Subject percentage compliance for dosing as per protocol (Efficacy Phase) - Use post-baseline study visits Week 4 to Week 24 inclusive
Subject percentage compliance for dosing as per protocol (Safety Phase) - Use post-baseline study visits Week 28 to Week 104 inclusive
Subject percentage compliance for dosing as per protocol (Overall) - Use post-baseline study visits Week 4 to Week 104 inclusive
If a subject discontinues early during the efficacy phase, use post-baseline study visits Week 4 to Week 24 and EOT/Week 104, as appropriate.
For the compliant with IMP dosing present only the corresponding to the study phase.

Table 14.1.5.1 Study Drug Exposure and Dosing
(Safety Analysis Set)

Study Phase

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of Dose Interruptions due to concomitant medication	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Dose Interruptions due to adverse events	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Number of Drug Discontinuations due to adverse events	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Additional doses may be added to Maximum Tolerated Dose as appropriate.

Subject percentage compliance for dosing as per protocol (Efficacy Phase) - Use post-baseline study visits Week 4 to Week 24 inclusive
Subject percentage compliance for dosing as per protocol (Safety Phase) - Use post-baseline study visits Week 28 to Week 104 inclusive
Subject percentage compliance for dosing as per protocol (Overall) - Use post-baseline study visits Week 4 to Week 104 inclusive
If a subject discontinues early during the efficacy phase, use post-baseline study visits Week 4 to Week 24 and EOT/Week 104, as appropriate.
For the compliant with IMP dosing present only the corresponding to the study phase.

Table 14.1.5.1 Study Drug Exposure and Dosing
(Safety Analysis Set)

Study Phase

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Dose at Drug Discontinuation due to adverse events (mg), n (%)	2mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	5mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	10mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	20mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	30mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	40mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
	60mg	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subject compliant with IMP dosing, n (%) [1] (Efficacy Phase)	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subject compliant with IMP dosing, n (%) [1] (Safety Phase)	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Additional doses may be added to Maximum Tolerated Dose as appropriate.***
Subject percentage compliance for dosing as per protocol (Efficacy Phase) - Use post-baseline study visits Week 4 to Week 24 inclusive
Subject percentage compliance for dosing as per protocol (Safety Phase) - Use post-baseline study visits Week 28 to Week 104 inclusive
Subject percentage compliance for dosing as per protocol (Overall) - Use post-baseline study visits Week 4 to Week 104 inclusive
If a subject discontinues early during the efficacy phase, use post-baseline study visits Week 4 to Week 24 and EOT/Week 104, as appropriate.
For the compliant with IMP dosing present only the corresponding to the study phase.

Table 14.1.5.2 Summary of Compliance to IMP
(Safety Analysis Set)

Visit	m1	5 to 10 years (N=XX)	m2	11 to 17 years (N=XX)	m	Overall (N=XX)
Week 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Week 8	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Week 12	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Week 16	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
...						
Week 104	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. mx = number of subjects attending the visit. (%) = n/mx*100.
Subject compliant for IMP dosing has been medically reviewed on a patient level basis. Subjects will be considered compliant if 80% to 100% of IMP doses have been taken.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.1.5.3 Time to Dose Reduction and Interruption
(Safety Analysis Set)

Overall / Efficacy Phase / Safety Phase

Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Number of subjects with dose reduction, n (%)	XX (XX.X)	XX (XX.X)
Number of subjects censored, n (%)	XX (XX.X)	XX (XX.X)
Kaplan-Meier estimates of time to dose reduction (weeks)		
25th percentile	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Median	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)
75th percentile	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Number of subjects with dose interruption, n (%)	XX (XX.X)	XX (XX.X)
Number of subjects censored, n (%)	XX (XX.X)	XX (XX.X)
Kaplan-Meier estimates of time to dose interruption (weeks)		
25th percentile	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Median	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)
75th percentile	XX.X	XX.X
95% CI [1]	(XX.XX, XX.XX)	(XX.XX, XX.XX)

Time to dose reduction is defined as the time (weeks) from the date of the start of the respective phase to the time of first dose reduction, if any. For the safety phase, subjects who do not enter the safety phase will be censored at time 0. If a patient discontinues study drug they will be counted as an event at the time of study discontinuation. If a patient has not had a dose reduction and is still on study drug, they will be censored at their last visit assessment date.
Time to dose interruption is defined as the time (weeks) from the date of the start of the respective phase to the time of first dose interruption, if any. For the safety phase, subjects who do not enter the safety phase will be censored at time 0. If a patient discontinues study drug they will be counted as an event at the time of study discontinuation. If a patient has not had a dose interruption and is still on study drug, they will be censored at their last visit assessment date.
[1] 95% Confidence Interval for Kaplan-Meier estimate.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
(%) = n/N*100.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for Safety Phase and Overall.

Table 14.1.5.4 Distribution of Achieved Doses
(Safety Analysis Set)

Visit	Dose	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Week 24	5 mg	XX (XX.X)	XX (XX.X)
	10 mg	XX (XX.X)	XX (XX.X)
	20 mg	XX (XX.X)	XX (XX.X)
	30 mg	XX (XX.X)	XX (XX.X)
	40 mg	XX (XX.X)	XX (XX.X)
	60 mg	XX (XX.X)	XX (XX.X)
Week 56	5 mg	XX (XX.X)	XX (XX.X)
	10 mg	XX (XX.X)	XX (XX.X)
	20 mg	XX (XX.X)	XX (XX.X)
	30 mg	XX (XX.X)	XX (XX.X)
	40 mg	XX (XX.X)	XX (XX.X)
	60 mg	XX (XX.X)	XX (XX.X)
Week 104	5 mg	XX (XX.X)	XX (XX.X)
	10 mg	XX (XX.X)	XX (XX.X)
	20 mg	XX (XX.X)	XX (XX.X)
	30 mg	XX (XX.X)	XX (XX.X)
	40 mg	XX (XX.X)	XX (XX.X)
	60 mg	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
Source: Listing 16.2.5.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Add or remove dose categories as applicable.

Table 14.2.1.1 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks
(Full Analysis Set)

		Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX		
	Mean	XX.X		
	SD	XX.XX		
	Minimum	XX		
	Median	XX.X		
	Maximum	XX		
	25th percentile	XX.X		
	75th percentile	XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
[1] 95% confidence interval for mean percentage change.
[*] Null hypothesis of 0% change. One-sample t-test or Wilcoxon Signed-Rank test.

[**] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[***] Missing data has been imputed using Baseline Observation Carried Forward (BOCF).
[****] Sensitivity analysis: Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect. An unstructured covariance will be used initially. The Akaike Information Criterion (AIC) used to assess model fit for covariance structures.
Percentage change from baseline is interpreted clinically as the greater the negative percentage change from baseline the greater the absolute clinical percentage change (i.e. percentage change of -48.5% is interpreted clinically as a 48.5% decrease from baseline).
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.1 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks
(Full Analysis Set)

		Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
[1] 95% confidence interval for mean percentage change.
[*] Null hypothesis of 0% change. One-sample t-test or Wilcoxon Signed-Rank test.
[**] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[***] Missing data has been imputed using Baseline Observation Carried Forward (BOCF).
[****] Sensitivity analysis: Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect. An unstructured covariance will be used initially. The Akaike Information Criterion (AIC) used to assess model fit for covariance structures.
Percentage change from baseline is interpreted clinically as the greater the negative percentage change from baseline the greater the absolute clinical percentage change (i.e. percentage change of -48.5% is interpreted clinically as a 48.5% decrease from baseline).
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.1 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks
(Full Analysis Set)

		Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (BOCF)*** (Analysis Study Week 24)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X
Efficacy Week 24 (BOCF)*** (Nominal Study Visit 10)	n	XX	XX	XX
	Mean	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX
	Median	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
[1] 95% confidence interval for mean percentage change.
[*] Null hypothesis of 0% change. One-sample t-test or Wilcoxon Signed-Rank test.
[**] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[***] Missing data has been imputed using Baseline Observation Carried Forward (BOCF).
[****] Sensitivity analysis: Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect. An unstructured covariance will be used initially. The Akaike Information Criterion (AIC) used to assess model fit for covariance structures.
Percentage change from baseline is interpreted clinically as the greater the negative percentage change from baseline the greater the absolute clinical percentage change (i.e. percentage change of -48.5% is interpreted clinically as a 48.5% decrease from baseline).
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.1 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks
(Full Analysis Set)

		Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline
Primary Analysis (Analysis Study Week 24)	Mean percentage change			XX.X
	95% Confidence Interval[1]			(X.XXXX, X.XXXX)
	P-Value*			X.XXXX
Sensitivity Analysis (Wilcoxon signed rank)(LOCF)	P-Value*			X.XXXX
Sensitivity Analysis (Nominal Study Visit 10)	Mean percentage change			XX.X
	95% Confidence Interval[1]			(X.XXXX, X.XXXX)
	P-Value*			X.XXXX
Sensitivity Analysis (MMRM)****	Mean percentage change			XX.X
	95% Confidence Interval[1]			(X.XXXX, X.XXXX)
	P-Value*			X.XXXX
Sensitivity Analysis (BOCF)***	Mean percentage change			XX.X
	95% Confidence Interval[1]			(X.XXXX, X.XXXX)
	P-Value*			X.XXXX

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
[1] 95% confidence interval for mean percentage change.
[*] Null hypothesis of 0% change. One-sample t-test or Wilcoxon Signed-Rank test.
[**] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[***] Missing data has been imputed using Baseline Observation Carried Forward (BOCF).
[****] Sensitivity analysis: Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect. An unstructured covariance will be used initially. The Akaike Information Criterion (AIC) used to assess model fit for covariance structures.
Percentage change from baseline is interpreted clinically as the greater the negative percentage change from baseline the greater the absolute clinical percentage change (i.e. percentage change of -48.5% is interpreted clinically as a 48.5% decrease from baseline).
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.2 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)

Table 14.2.1.3 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)

Table 14.2.1.4 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

		5 to 10 years (N=XX)			11 to 17 years (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with age group as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).

[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.4 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

		5 to 10 years (N=XX)			11 to 17 years (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with age group as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.4 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

		5 to 10 years (N=XX)			11 to 17 years (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with age group as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.5 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

		Documented cardiovascular disease history at screening (N=XX)			No documented cardiovascular disease history at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.

Analysis of Covariance (ANCOVA) model with classification of documented cardiovascular disease history as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.5 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)
(Full Analysis Set)

		Documented cardiovascular disease history at screening (N=XX)			No documented cardiovascular disease history at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of documented cardiovascular disease history as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.5 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)
(Full Analysis Set)

		Documented cardiovascular disease history at screening (N=XX)			No documented cardiovascular disease history at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of documented cardiovascular disease history as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.6 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

		Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)			No established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.6 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))
(Full Analysis Set)

		Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)			No established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.6 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))
(Full Analysis Set)

		Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)			No established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) at screening (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis) as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.7 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

		Had concomitant LLT medications/procedures excluding LA (N=XX)			Had concomitant LLT medications/procedures including LA (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.

Analysis of Covariance (ANCOVA) model with classification of concomitant LLT medications/procedures as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.7 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)
(Full Analysis Set)

		Had concomitant LLT medications/procedures excluding LA (N=XX)			Had concomitant LLT medications/procedures including LA (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of concomitant LLT medications/procedures as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.7 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)
(Full Analysis Set)

		Had concomitant LLT medications/procedures excluding LA (N=XX)			Had concomitant LLT medications/procedures including LA (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of concomitant LLT medications/procedures as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.8 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)

(Full Analysis Set)

		No dose reductions (N=XX)			At least one dose reduction (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.

[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.8 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)
(Full Analysis Set)

		No dose reductions (N=XX)			At least one dose reduction (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.8 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)
(Full Analysis Set)

		No dose reductions (N=XX)			At least one dose reduction (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.9 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

		Reached MTD for age group (N=XX)			Did not reach MTD for age group (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.

[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.9 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)
(Full Analysis Set)

		Reached MTD for age group (N=XX)			Did not reach MTD for age group (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.9 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)
(Full Analysis Set)

		Reached MTD for age group (N=XX)			Did not reach MTD for age group (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with classification of dose reduction as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.10 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

		Male (N=XX)			Female (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX			XX		
	Mean	XX.X			XX.X		
	SD	XX.XX			XX.XX		
	Minimum	XX			XX		
	Median	XX.X			XX.X		
	Maximum	XX			XX		
	25th percentile	XX.X			XX.X		
	75th percentile	XX.X			XX.X		
Efficacy Week 24 (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with sex as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).

[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.10 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

		Male (N=XX)			Female (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Efficacy Week 24 (LOCF)** (Analysis Study Week 24)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Efficacy Week 24 (LOCF)** (Nominal Study Visit 10)	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with sex as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.10 Lipid parameter: LDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

		Male (N=XX)			Female (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Analysis Study Week 24	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)
Nominal Study Visit 10	Mean percentage change [1]			XX.X			XX.X
	95% Confidence Interval[2]			(X.XXXX, X.XXXX)			(X.XXXX, X.XXXX)

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set.
Analysis of Covariance (ANCOVA) model with sex as a categorical fixed effect.
[*] Missing data has been imputed using Last Observation Carried Forward (LOCF).
[1] Mean percentage change at week 24 from baseline
[2] 95% confidence interval for mean percentage change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.1.11 Lipid parameter: LDL-C (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of subjects achieving a reduction in LDL-C at Week 24 of:				
>15%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
>25%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
>50%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Number of subjects achieving a reduction in LDL-C at any time from Week 8 of:				
>15%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
>25%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
>50%	n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)

Subjects that discontinued study medication early will be considered a non-responder.

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.2.1 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)
Footnotes as per Table 14.2.1.1 (as applicable)

Table 14.2.2.2 Lipid parameter: Non-HDL-C (mg/dL)and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.2.3 Lipid parameter: Non-HDL-C value (mg/dL) change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.2.4 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.2.5 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.2.6 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.2.7 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.2.8 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)

(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.2.9 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.2.10 Lipid parameter: Non-HDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.2.11 Lipid parameter: Non-HDL-C (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.3.1 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)
Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)

Table 14.2.3.2 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.3.3 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.3.4 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.3.5 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.3.6 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.3.7 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.3.8 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)
(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.3.9 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.3.10 Lipid parameter: TC (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.3.11 Lipid parameter: TC (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.4.1 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)
Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)

Table 14.2.4.2 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.4.3 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.4.4 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.4.5 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.4.6 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.4.7 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.4.8 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)

(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.4.9 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.4.10 Lipid parameter: VLDL-C (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.4.11 Lipid parameter: VLDL-C (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.5.1 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)
Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)

Table 14.2.5.2 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.5.3 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.5.4 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.5.5 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.5.6 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.5.7 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.5.8 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)

(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.5.9 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.5.10 Lipid parameter: Apo B (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.5.11 Lipid parameter: Apo B (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.6.1 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)
Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)

Table 14.2.6.2 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.6.3 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.6.4 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.6.5 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.6.6 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.6.7 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.6.8 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)
(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.6.9 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.6.10 Lipid parameter: TG (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.6.11 Lipid parameter: TG (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.7.1 Lipid parameter: Lp(a) and change from baseline at 24 weeks (Secondary analysis)
(Full Analysis Set)

<Unit>

Repeat of Table 14.2.1.1 separately by unit

Results are based on local labs rather than central labs.

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.1 (as applicable)
Replace Primary Analysis with Secondary Analysis and exclude Sensitivity Analysis (MMRM)
Add a combined p-value using Fisher's method.

Table 14.2.7.2 Lipid parameter: LP(a) and change from baseline at 24 weeks (Supplementary analysis - per protocol set)
(Per Protocol Set)

Repeat of Table 14.2.1.2

Results are based on local labs rather than central labs.

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.2 (as applicable)

Table 14.2.7.3 Lipid parameter: LP(a) and change from baseline at 24 weeks (Supplementary analysis - completer analysis set)
(Completers Analysis Set)

Repeat of Table 14.2.1.3

Results are based on local labs rather than central labs.

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.3 (as applicable)

Table 14.2.7.4 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 1 - Age groups)
(Full Analysis Set)

Repeat of Table 14.2.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.4 (as applicable)

Table 14.2.7.5 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 2 - Documented cardiovascular disease history)

(Full Analysis Set)

Repeat of Table 14.2.1.5

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.5 (as applicable)

Table 14.2.7.6 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 3 - Established cardiovascular disease history (defined as aortic valve disease and/or coronary atherosclerosis))

(Full Analysis Set)

Repeat of Table 14.2.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.6 (as applicable)

Table 14.2.7.7 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 4 - Concomitant LLT medications/procedures)

(Full Analysis Set)

Repeat of Table 14.2.1.7

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.7 (as applicable)

Table 14.2.7.8 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 5 - Dose reductions / dose interruptions)

(Full Analysis Set)

Repeat of Table 14.2.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.8 (as applicable)

Table 14.2.7.9 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 6 - Reached MTD for age group/ did not reach MTD for age group)

(Full Analysis Set)

Repeat of Table 14.2.1.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.9 (as applicable)

Table 14.2.7.10 Lipid parameter: LP(a) (mg/dL) and change from baseline at 24 weeks (Subgroup analysis 7 - Sex)
(Full Analysis Set)

Repeat of Table 14.2.1.10

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.10 (as applicable)

Table 14.2.7.11 Lipid parameter: LP(a) (mg/dL) Responder Analysis (Supplementary analysis)
(Full Analysis Set)

Repeat of Table 14.2.1.11

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.2.1.11 (as applicable)

Table 14.2.8.1 Lipid parameter: LDL-C (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

		5 to 10 years (N=XX)				11 to 17 years (N=XX)				Overall (N=XX)			
Visit	Statistics	Dose	Observed value	Change from baseline	% Change from baseline	Dose	Observed value	Change from baseline	% Change from baseline	Dose	Observed value	Change from baseline	% Change from baseline
Baseline	n	XX	XX			XX	XX			XX	XX		
	Mean	XX.X	XX.X			XX.X	XX.X			XX.X	XX.X		
	SD	XX.X	XX.X			XX.X	XX.X			XX.X	XX.X		
	Minimum	XX	XX			XX	XX			XX	XX		
	Median	XX.X	XX.X			XX.X	XX.X			XX.X	XX.X		
	Maximum	XX	XX			XX	XX			XX	XX		
	25th percentile	XX.X	XX.X			XX.X	XX.X			XX.X	XX.X		
	75th percentile	XX.X	XX.X			XX.X	XX.X			XX.X	XX.X		
XXX	n	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.1 Lipid parameter: LDL-C (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

		5 to 10 years (N=XX)				11 to 17 years (N=XX)				Overall (N=XX)			
Visit	Statistics	Dose	Observed value	Change from baseline	% Change from baseline	Dose	Observed value	Change from baseline	% Change from baseline	Dose	Observed value	Change from baseline	% Change from baseline
Etc.....													
EOT / Week 104	n	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.2 Lipid parameter: Non-HDL-C (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.3 Lipid parameter: TC (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.4 Lipid parameter: VLDL-C (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.5 Lipid parameter: Apo B (g/L) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.6 Lipid parameter: TG (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.7 Lipid parameter: Lp(a) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.8.8 Lipid parameter: TC/HDL-C ratio and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.1 Lipid parameter: LDL-C (mg/dL) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

	Overall (N=XX)			
Visit	Least Square Means Estimate[1]	Standard Error	95% Confidence Interval	P-Value*
Week 4	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 8	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 12	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 16	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 20	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 24	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 28	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 32	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 36	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 40	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 44	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 48	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 52	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 56	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 68	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 80	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
Week 92	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX
EOT / Week 104	XX.X	XX.XX	(X.XXXX, X.XXXX)	X.XXXX

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.2 Lipid parameter: Non-HDL-C (mg/dL) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.3 Lipid parameter: TC (mmol/L) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.4 Lipid parameter: VLDL-C (mg/dL) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.5 Lipid parameter: Apo B (g/L) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.6 Lipid parameter: TG (mg/dL) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.7 Lipid parameter: LP(a) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Results are based on local labs rather than central labs.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.9.8 Lipid parameter: TC/HDL-C ratio and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Results are based on local labs rather than central labs.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.10.1 Treated with standard Lipid Lowering Therapy (LLT) (including Lipoprotein Apheresis (LA)
(Full Analysis Set)

Visit	Number attending visit (m)	LLT 5 to 10 years (N=XX)	LLT 11 to 17 years (N=XX)	LLT Overall (N=XX)	LA 5 to 10 years (N=XX)	LA 11 to 17 years (N=XX)	LA Overall (N=XX)
Baseline	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 4	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 8	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 12	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 16	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 20	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 24	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 28	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 32	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 36	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 40	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 44	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 48	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 52	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 56	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 68	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 80	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 92	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
EOT / Week 104	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. m = number of subjects attending visit. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Lising 16.2.4.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.10.2 Reduction in dose of standard Lipid Lowering Therapy (LLT) / Reduced need for Lipoprotein Apheresis (LA) compared to Week 24 (end of Efficacy Phase)

(Full Analysis Set)

	LLT			LA		
Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of patients treated with LLT / LA at Week 24 (m)	XX	XX	XX	XX	XX	XX
Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
...						

N = the number of subjects in the analysis set. m = number of patients treated with LLT (including LA) at Week 24. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Lising 16.2.4.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.10.3 Discontinue standard Lipid Lowering Therapy (LLT) / Discontinue need for Lipoprotein Apheresis (LA) compared to Week 24 (end of Efficacy Phase)

(Full Analysis Set)

	LLT			LA		
Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of patients treated with LLT / LA at Week 24 (m)	XX	XX	XX	XX	XX	XX
Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
...						

N = the number of subjects in the analysis set. m = number of patients treated with LLT (including LA) at Week 24. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Lising 16.2.4.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.10.4 Increase dose of standard Lipid Lowering Therapy (LLT) / Increased need for Lipoprotein Apheresis (LA) compared to Week 24 (end of Efficacy Phase)

(Full Analysis Set)

	LLT			LA		
Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of patients treated with LLT / LA at Week 24 (m)	XX	XX	XX	XX	XX	XX
Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
High LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Low LDL-C	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						
...						

N = the number of subjects in the analysis set. m = number of patients treated with LLT (including LA) at Week 24. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Lising 16.2.4.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.11.1 Recommended target of LDL-C of <135 mg/dL (i.e. 3.5 mmol/L) - EAS recommendation
(Full Analysis Set)

Visit	Number attending visit (m)	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Baseline	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 4	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 8	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 12	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 16	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 20	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 24	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 28	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 32	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 36	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 40	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 44	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 48	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 52	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 56	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 68	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 80	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 92	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
EOT / Week 104	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Anytime up to Week 24	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)
Anytime up to Week 104	XX	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. m = number of patients attending the study visit. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.11.2 Recommended target of LDL-C of <135 mg/dL (i.e. 3.5 mmol/L) by age group and Lomitapide dose - EAS recommendation
(Full Analysis Set)

Age Group = 5 to 10 years

Visit	Number attending visit (m)	Lomitapide dose at time when LDL-C < 135 mg/dL						
		2mg	5mg	10mg	20mg	30mg	40mg	60mg
Baseline	XX	XX	XX	XX	XX	XX	XX	XX
Week 4	XX	XX	XX	XX	XX	XX	XX	XX
Week 8	XX	XX	XX	XX	XX	XX	XX	XX
Week 12	XX	XX	XX	XX	XX	XX	XX	XX
Week 16	XX	XX	XX	XX	XX	XX	XX	XX
Week 20	XX	XX	XX	XX	XX	XX	XX	XX
Week 24	XX	XX	XX	XX	XX	XX	XX	XX
Week 28	XX	XX	XX	XX	XX	XX	XX	XX
Week 32	XX	XX	XX	XX	XX	XX	XX	XX
Week 36	XX	XX	XX	XX	XX	XX	XX	XX
Week 40	XX	XX	XX	XX	XX	XX	XX	XX
Week 44	XX	XX	XX	XX	XX	XX	XX	XX
Week 48	XX	XX	XX	XX	XX	XX	XX	XX
Week 52	XX	XX	XX	XX	XX	XX	XX	XX
Week 56	XX	XX	XX	XX	XX	XX	XX	XX
Week 68	XX	XX	XX	XX	XX	XX	XX	XX
Week 80	XX	XX	XX	XX	XX	XX	XX	XX
Week 92	XX	XX	XX	XX	XX	XX	XX	XX
EOT / Week 104	XX	XX	XX	XX	XX	XX	XX	XX

m = number of patients attending the study visit. n = the number of subjects meeting the criterion.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for Age Group = 11 to 17 years.
Only include applicable Lomitapide doses applicable for age group
Additional doses may be added in to Lomitapide dose at time when LDL-C < 135 mg/dL as appropriate

Table 14.2.11.3 Recommended targets of LDL-C - Exploratory endpoints
(Full Analysis Set)

Visit	Number attending visit (m)	Recommended target	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Baseline	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 4	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 8	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
...					
EOT / Week 104	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
Anytime up to Week 24	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
Anytime up to Week 104	XX	< 115 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)
		< 110 mg/dL	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. m = number of patients attending the study visit. n = the number of subjects meeting the criterion. (%) = n/m*100.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each visit

Table 14.2.11.4 Recommended targets of LDL-C by age group and Lomitapide dose - Exploratory endpoints
(Full Analysis Set)

Age Group = 5 to 10 years

Visit	Number attending visit (m)	Recommended target	Lomitapide dose at time when LDL-C recommended target was reached						
			2mg	5mg	10mg	20mg	30mg	40mg	60mg
Baseline	XX	< 115 mg/dL	XX	XX	XX	XX	XX	XX	XX
		< 110 mg/dL	XX	XX	XX	XX	XX	XX	XX
Week 4	XX	< 115 mg/dL	XX	XX	XX	XX	XX	XX	XX
		< 110 mg/dL	XX	XX	XX	XX	XX	XX	XX
Week 8	XX	< 115 mg/dL	XX	XX	XX	XX	XX	XX	XX
		< 110 mg/dL	XX	XX	XX	XX	XX	XX	XX
...									
EOT / Week 104	XX	< 115 mg/dL	XX	XX	XX	XX	XX	XX	XX
		< 110 mg/dL	XX	XX	XX	XX	XX	XX	XX

m = number of patients attending the study visit. n = the number of subjects meeting the criterion.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Age Group = 11 to 17 years.***
Only include applicable Lomitapide doses applicable for age group
Additional doses may be added in to Lomitapide dose at time as appropriate

Table 14.2.12.1 Lipid parameter: HDL-C (mg/dL) and change from Baseline to EOT / Week 104 by age group (Summary Measures)
(Full Analysis Set)

Repeat of Table 14.2.8.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.2.12.2 Lipid parameter: HDL-C (mg/dL) and change from Baseline to EOT / Week 104 (Mixed Model Repeated Measures analysis)
(Full Analysis Set)

Repeat of Table 14.2.9.1

N = the number of subjects in the analysis set.
Mixed Model Repeated Measures model with missing at random assumption. Model includes visit as a categorical fixed effect, baseline as a covariate and subject as a random effect. An unstructured covariance will be used initially. The Alkaike Information Criterion (AIC) used to assess model fit for covariance structures.
[1] Mean percentage change from baseline
[*] Null hypothesis of 0% change.
Source: Listing 16.2.8.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.1 Summary of Treatment-Emergent Adverse Events
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Total number of Non-TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Serious TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Serious Related TEAEs [3]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
TEAEs leading to study discontinuation	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Serious TEAEs leading to study discontinuation	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Related TEAEs leading to study discontinuation [3]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Serious Related TEAEs leading to study discontinuation [3]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
TEAEs leading to death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Adverse Events of Special Interest (AESI)	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
AESI leading to study discontinuation	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Study treatment-related AESI	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
NTEAEs leading to death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
[1] If a subject experienced more than one TEAE, the subject is counted once at the most severe or most related event.
[2] Unrelated adverse events are those classified as having a non-reasonable causal relationship to the study treatment.
[3] Related adverse events are those classified as having a reasonable causal relationship to the study treatment.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
*Major Adverse Cardiac Events are defined by the clinical team
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.1 Summary of Treatment-Emergent Adverse Events
(Safety Analysis Set)

Overall / Efficacy Phase / Safety Phase / Follow-up

	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Major Adverse Cardiac Events (MACE)*	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Severity [1]						
Mild	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Moderate	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Severe	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Life Threatening	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Relationship to study treatment [1]						
Related [3]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Unrelated [2]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Action taken with study treatment						
Dose Decreased	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Dose not Changed	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Drug Withdrawn	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Not Applicable	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Unknown						
Other						

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
[1] If a subject experienced more than one TEAE, the subject is counted once at the most severe or most related event.
[2] Unrelated adverse events are those classified as having a non-reasonable causal relationship to the study treatment.
[3] Related adverse events are those classified as having a reasonable causal relationship to the study treatment.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
*Major Adverse Cardiac Events are defined by the clinical team
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.2 Summary of Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Etc...						

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.3 Summary of Serious Adverse Events (SAEs) by MedDRA System Organ Class and Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.1.2 (as applicable)

Table 14.3.1.4 Summary of Treatment-Emergent Adverse Events by MedDRA Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
Preferred term	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)

Etc...

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each preferred term (PT).
PTs are ordered in decreasing frequency of subject incidence.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.5 Summary of Serious Adverse Events (SAEs) by MedDRA Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.1.4 (as applicable)

Table 14.3.1.6 Summary of Treatment-Emergent Adverse Events by MedDRA System Organ Class, Preferred Term and Severity
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	Severity	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
		Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Mild	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Moderate	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Severe	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Life-threatening	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Mild	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Moderate	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Severe	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Life-threatening	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Mild	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Moderate	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Severe	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Life-threatening	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Death	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Etc...							

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT) at the most severe.
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.7 Summary of Serious Adverse Events (SAEs) by MedDRA System Organ Class, Preferred Term and Severity
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.6

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.1.6 (as applicable)

Table 14.3.1.8 Summary of Treatment-Emergent Adverse Events by MedDRA System Organ Class, Preferred Term and Relationship to Study Treatment
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	Relationship to study treatment	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
		Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Unrelated [1]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Related [2]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Unrelated [1]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Related [2]	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Etc...						
Etc...							

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT) using the most related event.
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
[1] Unrelated adverse events are those classified as having no reasonable causal relationship to the study treatment.
[2] Related adverse events are those classified as having a reasonable causal relationship to the study treatment.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.9 Summary of Serious Adverse Events (SAEs) by MedDRA System Organ Class, Preferred Term and Relationship to Study Treatment
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.8

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.1.8 (as applicable)

Table 14.3.1.10 Summary of Adverse Events of Special Interest (AESI) by AESI Category, MedDRA System Organ Class and Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of AESI	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
AESI category 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Etc...						

Adverse events of special interest (AESI) are defined as hepatic, small bowel/intestinal, pancreatic and colorectal tumours, hepatic abnormalities, gastrointestinal effects and major congenital abnormality AEs.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.11 Summary of Gastrointestinal (GI) Adverse Events by MedDRA System Organ Class and Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of GI Adverse Events	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Etc...						

If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.12 Summary of Most Frequent Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
	Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
System organ class 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 1	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 2	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 3	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Preferred term 4	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
Etc...						

Only TEAEs that occurred in at least 2 subjects are presented.
Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Present only TEAEs that occured in at least 2 subjects.

Table 14.3.1.13 Time to First Gastrointestinal Treatment-Emergent Adverse Events
(Safety Analysis Set)

Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of subjects with a gastrointestinal TEAE, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Number of subjects censored, n (%)	XX (XX.X)	XX (XX.X)	XX (XX.X)
Kaplan-Meier estimates of time to first gastrointestinal TEAE (days) [1]			
25th percentile	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Median	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
75th percentile	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Kaplan-Meier estimates of duration of gastrointestinal TEAE (days) [2]			
25th percentile	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
Median	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
75th percentile	XX.X	XX.X	XX.X
95% CI [3]	(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)

[1] Time to first gastrointestinal TEAE is defined as the time (days) from the first dose to the time of first gastrointestinal TEAE, if any.
[2] Duration is derived as: Date of resolution of the TEAE - Date of start of TEAE. Subjects with an ongoing TEAE will be censored at their last assessment date.
[3] 95% Confidence Interval for Kaplan-Meier estimate.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
(%) = n/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.1.14 Time to First Hepatic Treatment-Emergent Adverse Events
(Safety Analysis Set)

Repeat of Table 14.3.1.13

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.1.13 (as applicable)

Table 14.3.1.15 Summary of Treatment-Emergent Adverse Events, by Sex
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.1 by sex

Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
[1] If a subject experienced more than one TEAE, the subject is counted once at the most severe or most related event.
[2] Unrelated adverse events are those classified as having a non-reasonable causal relationship to the study treatment.
[3] Related adverse events are those classified as having a reasonable causal relationship to the study treatment.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
*Major Adverse Cardiac Events are defined by the clinical team
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat of Table 14.3.1.1 by sex (Male, Female) instead of age group

Table 14.3.1.16 Summary of Adverse Events of Special Interest (AESI) by AESI Category, MedDRA System Organ Class and Preferred Term, by Sex
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat of Table 14.3.1.10 by sex

Adverse events of special interest (AESI) are defined as hepatic, small bowel/intestinal, pancreatic and colorectal tumours, hepatic abnormalities, gastrointestinal effects and major congenital abnormality AEs.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat of Table 14.3.1.10 by sex (Male, Female) instead of age group

Table 14.3.1.17 Summary of Elevation in Serum Transaminases Related Treatment-Emergent Adverse Events by MedDRA System Organ Class and Preferred Term, by Country

(Safety Analysis Set)

Overall / Efficacy Phase / Safety Phase / Follow-up

System organ class Preferred term	Country	5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
		Events	Subjects (%)	Events	Subjects (%)	Events	Subjects (%)
Total number of TEAEs	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Germany	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Spain	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	...						
System organ class 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Germany	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Spain	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	...						
Preferred term 1	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Germany	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Spain	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	...						
Preferred term 2	Overall	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Germany	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
	Spain	XX	XX (XX.X)	XX	XX (XX.X)	XX	XX (XX.X)
...	...						

Only elevated serum transaminases related TEAEs are presented.
Treatment emergent adverse events (TEAEs) are defined as an adverse event that started after administration of study drug.
If a subject experienced more than one TEAE, the subject is counted once for each system organ class (SOC) and once for each preferred term (PT).
SOCs are ordered in decreasing frequency of the total number of subjects with TEAEs reported in each SOC and PTs are ordered within a SOC in decreasing frequency of the total number of subjects with each TEAE.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
N = the number of subjects in the analysis set. (%) = Subjects/N*100.
Source: Listing 16.2.7.1 and Listing 16.2.7.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for overall and all study phases.
Include all countries as applicable
Include related events only
Elevated serum transaminases defined as: aedecod in ("Alanine aminotransferase increased", "Aspartate aminotransferase increased", "Hypertransaminasaemia", "Transaminases increased")

Table 14.3.2.1 Haematology Values and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
Visit	Statistics	Observed value	Change from baseline	Observed value	Change from baseline	Observed value	Change from baseline
XXX	n	XX		XX		XX	
	Mean	XX.X		XX.X		XX.X	
	SD	XX.XX		XX.XX		XX.XX	
	Minimum	XX		XX		XX	
	Median	XX.X		XX.X		XX.X	
	Maximum	XX		XX		XX	
	25th percentile	XX.X		XX.X		XX.X	
	75th percentile	XX.X		XX.X		XX.X	
XXX	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.

Table 14.3.2.2 Clinical Significance of Haematology Values over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
...				

CS = clinically significant, NCS = not clinically significant.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number with a value at that visit)*100].
Source: Listing 16.2.8.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.

Table 14.3.2.3 Shift in Haematology Values over Time
(Safety Analysis Set)

Age Group = XXX
Parameter: <Parameter (Unit)>

		Baseline					
Visit	Result	Normal n (%)	CS & Low n (%)	CS & High n (%)	NCS & Low n (%)	NCS & High n (%)	Total n (%)
XXX	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS & Low	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS & High	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS & Low	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS & High	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS & Low	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	CS & High	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS & Low	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	NCS & High	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...							

CS = clinically significant, NCS = not clinically significant.
Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number with a value at that visit)*100].
Source: Listing 16.2.8.9

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all treatment groups, post-baseline visits and parameters.***
Age group = 5 to 10 years; 11 to 17 years; Overall

Table 14.3.3.1 Serum Clinical Chemistry Values and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)			11 to 17 years (N=XX)			Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
XXX	n	XX			XX			XX		
	Mean	XX.X			XX.X			XX.X		
	SD	XX.XX			XX.XX			XX.XX		
	Minimum	XX			XX			XX		
	Median	XX.X			XX.X			XX.X		
	Maximum	XX			XX			XX		
	25th percentile	XX.X			XX.X			XX.X		
	75th percentile	XX.X			XX.X			XX.X		
XXX	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Source: Listings 16.2.8.1, 16.2.8.2, 16.2.8.3, 16.2.8.4, 16.2.8.5, 16.2.8.6, 16.2.8.7, 16.2.8.8
Repeat for all visits and parameters (metabolic panel, LFTS, Fasting Lipid panel (local and central), EFAs, Fat-soluble vitamin levels, hormones, sex hormones, serum lipase).
Footnotes as per Table 14.3.2.1 (as applicable)

Table 14.3.3.2 Clinical Significance of Serum Clinical Chemistry Values over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Table 14.3.2.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Source: Listings 16.2.8.1, 16.2.8.2, 16.2.8.3, 16.2.8.4, 16.2.8.5, 16.2.8.6, 16.2.8.7, 16.2.8.8***
Repeat for all visits and parameters (metabolic panel, LFTS, Fasting Lipid panel (local and central), EFAs, Fat-soluble vitamin levels, hormones, sex hormones, serum lipase).
Footnotes as per Table 14.3.2.1 (as applicable)

Table 14.3.3.3 Shift in Clinical Chemistry Values over Time
(Safety Analysis Set)

Age Group = XXX
Parameter: <Parameter (Unit)>

Repeat of Table 14.3.2.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Source: Listings 16.2.8.1, 16.2.8.2, 16.2.8.3, 16.2.8.4, 16.2.8.5, 16.2.8.6, 16.2.8.7, 16.2.8.8***
Repeat for all visits and parameters (metabolic panel, LFTS, Fasting Lipid panel (local and central), EFAs, Fat-soluble vitamin levels, hormones, sex hormones, serum lipase).
Footnotes as per Table 14.3.2.1 (as applicable)
Age group = 5 to 10 years; 11 to 17 years; Overall

Table 14.3.3.4 Summary of Maximum Clinical Chemistry Values
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Parameter	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Alanine Transaminase	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X
Aspartate Aminotransferase	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Maximum post-baseline values for ALT, AST, ALP and TBL

Table 14.3.3.4 Summary of Maximum Clinical Chemistry Values
(Safety Analysis Set)
Overall / Efficacy Phase / Safety Phase / Follow-up

Parameter	Statistics	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Alkaline Phosphatase	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X
Total Bilirubin	n	XX	XX
	Mean	XX.X	XX.X
	SD	XX.XX	XX.XX
	Minimum	XX	XX
	Median	XX.X	XX.X
	Maximum	XX	XX
	25th percentile	XX.X	XX.X
	75th percentile	XX.X	XX.X

N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Maximum post-baseline values for ALT, AST, ALP and TBL

Table 14.3.3.5 Summary of Subjects Exceeding Hepatotoxicity Thresholds over Time
(Safety Analysis Set)

Visit	Parameter	Statistics	5 to 10 years	11 to 17 years	Overall
XXX	AST	> 3 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 5 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 10 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 20 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
	ALT	> 3 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 5 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 10 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 20 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
	ALP	> 1.5 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
	TBL	> 1.5 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)
		> 2 x ULN	XX (XX.X)	XX (XX.X)	XX (XX.X)

...

ALP = Alkaline Phosphatase. ALT = Alanine Transaminase. AST = Aspartate Aminotransferase. TBL = Total Bilirrubin.
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.8.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each post-baseline visit.

Table 14.3.3.6 Time to Onset of Liver Function Test Abnormality
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

	5 to 10 years (N=XX)	11 to 17 years (N=XX)
Number of subjects with LFT > x 3 ULN, n (%)	XX (XX.X)	XX (XX.X)
Time to onset (days) [1]		
n	XX	XX
Mean	XX.X	XX.X
SD	XX.XX	XX.XX
Minimum	XX	XX
Median	XX.X	XX.X
Maximum	XX	XX
25th percentile	XX.X	XX.X
75th percentile	XX.X	XX.X
Number of subjects with ongoing elevation, n (%)	XX (XX.X)	XX (XX.X)
Duration of abnormality (days) [2]		
n	XX	XX
Mean	XX.X	XX.X
SD	XX.XX	XX.XX
Minimum	XX	XX
Median	XX.X	XX.X
Maximum	XX	XX
25th percentile	XX.X	XX.X
75th percentile	XX.X	XX.X

CI = Confidence Interval. LFT = Liver Function Test. ULN = Upper Limit of Normal.
[1] Time to first LFT abnormality is derived as: Date of the first LFT assessment that is >= 3 x ULN or greater - Date of first dose + 1.
[2] Duration is derived as: Date of resolution of the first LFT abnormality (resolution defined as <3 x ULN) - Date of the first LFT assessment that is ≥3 x ULN. Subjects with an ongoing elevation will be censored at their last laboratory assessment date containing both AST and ALT results.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion.
(%) = n/N*100.
Source: Listing 16.2.8.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Present for ALT and AST abnormalities.

Table 14.3.4.1 Urinalysis Values and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Table 14.3.2.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.2.1 (as applicable)
Repeat for all visits and parameters.
Source: Listing 16.2.8.10

Table 14.3.4.2 Clinical Significance of Urinalysis Values over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Table 14.3.2.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Table 14.3.2.2 (as applicable)
Repeat for all visits and parameters.
Source: Listing 16.2.8.10

Table 14.3.4.3 Shift in Urinalysis Values over Time
(Safety Analysis Set)

Age Group = XXX
Parameter: <Parameter (Unit)>

Repeat of Table 14.3.2.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Footnotes as per Table 14.3.2.3 (as applicable)***
Repeat for all visits and parameters.
Source: Listing 16.2.8.10
Age group = 5 to 10 years; 11 to 17 years; Overall

Table 14.3.5.1 Vital Signs and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)			11 to 17 years (N=XX)			Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
XXX	n	XX			XX			XX		
	Mean	XX.X			XX.X			XX.X		
	SD	XX.XX			XX.XX			XX.XX		
	Minimum	XX			XX			XX		
	Median	XX.X			XX.X			XX.X		
	Maximum	XX			XX			XX		
	25th percentile	XX.X			XX.X			XX.X		
	75th percentile	XX.X			XX.X			XX.X		
XXX	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

...

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.9.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.
**** Present change from baseline for Maximum, Minimum and Last post baseline value for height/BMI percentiles and z-scores only.***

Table 14.3.5.1 Vital Signs and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)			11 to 17 years (N=XX)			Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
Maximum Post-baseline Value *	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Minimum Post-baseline Value *	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
Last Post-baseline Value *	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.

Source: Listing 16.2.9.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.
**** Present change from baseline for Maximum, Minimum and Last post baseline value for height/BMI percentiles and z-scores only.***

Table 14.3.6.1 Summary of Overall ECG Interpretation over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, CS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, CS	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
...				

NCS = not clinically significant, CS = clinically significant.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.10.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.

Table 14.3.6.2 Shift in Overall ECG Interpretation over Time
(Safety Analysis Set)

Age Group = XXX

		Baseline			
Visit	Result	Normal n (%)	Abnormal, NCS n (%)	Abnormal, CS n (%)	Total n (%)
XXX	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, CS	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Normal	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, NCS	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Abnormal, CS	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...					

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4)..
NCS = not clinically significant, CS = clinically significant.
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.10.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all post-baseline visits.
Age group = 5 to 10 years; 11 to 17 years; Overall.

Table 14.3.7.1 Summary of Echocardiography (Standard of Care) over Time
(Safety Analysis Set)

Visit		Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Echocardiography performed?	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Atherosclerosis and/or aortic valve disease?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Thickening of aortic valve leaflets	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (Baseline, week 24, week 56, week 104).

Table 14.3.7.1 Summary of Echocardiography (Standard of Care) over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Fusion of the aortic valve leaflets	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Thickening of the aortic root	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (Baseline, week 24, week 56, week 104).

Table 14.3.7.1 Summary of Echocardiography (Standard of Care) over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Flow acceleration or turbulence over the aortic valve	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Regions present?	Supravalvular region	XX (XX.X)	XX (XX.X)	XX (XX.X)
	In the ascending aorta	XX (XX.X)	XX (XX.X)	XX (XX.X)
Aortic valve regurgitation?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (Baseline, week 24, week 56, week 104).

Table 14.3.7.1 Summary of Echocardiography (Standard of Care) over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Coronary ostial narrowing or flow disturbances?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Ventricular findings?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Left ventricular hypertrophy?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (Baseline, week 24, week 56, week 104).

Table 14.3.7.1 Summary of Echocardiography (Standard of Care) over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Impairment of left ventricular function?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	N/A	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this new?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)
Is this progression of the condition	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	No	XX (XX.X)	XX (XX.X)	XX (XX.X)

...

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (Baseline, week 24, week 56, week 104).

Table 14.3.7.2 Summary of Subjects with Worsening in Echocardiography over Time
(Safety Analysis Set)

Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Number of subjects with a worsening from baseline by:			
Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/N)*100].
Source: Listing 16.2.11.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.8.1.1 Summary of Lipid Accumulation in the Liver over Time
(Safety Analysis Set)

		NMR Scan (N=XX)		Ultrasound Scan (N=XX)		
Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	≤ 10% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 10% and ≤ 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	≤ 10% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 10% and ≤ 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...						

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.

Table 14.3.8.1.2 Summary of Lipid Accumulation in the Liver over Time (Sensitivity Analysis - Excluding Site 12)
(Safety Analysis Set)

Excluding Site 12

Repeat of Table 14.3.8.1.1

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all visits.***
Exclude Site 12 data
Repeat for Site 12 only

Table 14.3.8.2.1 Shift in Lipid Accumulation in the Liver over Time
(Safety Analysis Set)

Age Group = XXX

		Baseline			
Visit	Result	≤ 10% liver fat n (%)	>10% and ≤ 20% liver fat n (%)	>20% liver fat n (%)	Total n (%)
XXX	≤ 10% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 10% and ≤ 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	≤ 10% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 10% and ≤ 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	> 20% liver fat	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
...					

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all post-baseline visits.
Age group = 5 to 10 years; 11 to 17 years; Overall.

Table 14.3.8.2.2 Shift in Lipid Accumulation in the Liver over Time (Sensitivity Analysis - Excluding Site 12)
(Safety Analysis Set)

Excluding Site 12
Age Group = XXX

Repeat of Table 14.3.8.2.1

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all post-baseline visits.***
Age group = 5 to 10 years; 11 to 17 years; Overall.
Exclude Site 12 data
Repeat for Site 12 only

Table 14.3.8.3.1 Summary of Lipid Accumulation in the Liver over Time
(Safety Analysis Set)

		5 to 10 years (N=XX)			11 to 17 years (N=XX)			Overall (N=XX)		
Visit	Statistics	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline	Observed value	Change from baseline	% Change from baseline
XXX	n	XX			XX			XX		
	Mean	XX.X			XX.X			XX.X		
	SD	XX.XX			XX.XX			XX.XX		
	Minimum	XX			XX			XX		
	Median	XX.X			XX.X			XX.X		
	Maximum	XX			XX			XX		
	25th percentile	XX.X			XX.X			XX.X		
	75th percentile	XX.X			XX.X			XX.X		
XXX	n	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

...

Only NMR scan assessments are included.
Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.

Table 14.3.8.3.2 Summary of Lipid Accumulation in the Liver over Time
(Safety Analysis Set)

Excluding Site 12

Repeat of Table 14.3.8.3.1

Only NMR scan assessments are included.
Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all visits and parameters.***
Exclude Site 12 data
Repeat for Site 12 only

Table 14.3.8.4.1 Summary of Lipid Accumulation Increase over Time
(Safety Analysis Set)

Visit		5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	>5%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>10%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>15%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>20%	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	>5%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>10%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>15%	XX (XX.X)	XX (XX.X)	XX (XX.X)
	>20%	XX (XX.X)	XX (XX.X)	XX (XX.X)
...				

Only NMR scan assessments are included.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each visit
The increases will be presented cumulatively.

Table 14.3.8.4.2 Summary of Lipid Accumulation Increase over Time
(Safety Analysis Set)

Excluding Site 12

Repeat of Table 14.3.8.4.1

Only NMR scan assessments are included.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***FOOTNOTE***
Repeat for each visit
The increases will be presented cumulatively.
Present the maximum post-baseline change at the end.
Exclude Site 12 data
Repeat for Site 12 only

Table 14.3.9.1 Summary of Tanner Staging over Time
(Safety Analysis Set)

Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Stage 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 5	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	Stage 1	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 2	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 3	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Stage 5	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
...				

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.12.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.

Table 14.3.9.2 Summary of Sex Hormones in patients with Tanner Stage ≥ 2 over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
Visit	Statistics	Observed value	Change from baseline	Observed value	Change from baseline	Observed value	Change from baseline
XXX	n	XX		XX		XX	
	Mean	XX.X		XX.X		XX.X	
	SD	XX.XX		XX.XX		XX.XX	
	Minimum	XX		XX		XX	
	Median	XX.X		XX.X		XX.X	
	Maximum	XX		XX		XX	
	25th percentile	XX.X		XX.X		XX.X	
	75th percentile	XX.X		XX.X		XX.X	
XXX	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X

…

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set. n = the number of subjects with a measurement.
Source: Listing 16.2.13.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters (serum testosterone, serum oestradiol).

Table 14.3.9.3 Shift in Tanner Staging Over Time
(Safety Analysis Set)

Age Group = XXX

		Baseline Tanner Staging					
Visit	Tanner Staging	1	2	3	3	5	Total
XXX	1	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	2	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	3	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	4	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	5	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	1	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	2	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	3	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	4	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	5	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)	XX (XX.X)
…. Etc							

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set. n = the number of subjects with a measurement. (%) = [(n/number who have a value at that visit)*100].
[-] denotes a zero count in the table.
Source: Listing 16.2.13.2

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all visits***
Age group = 5 to 10 years; 11 to 17 years; Overall.
Please display '-' instead of '0' for zero counts and foornote to say that '-' represents a zero count in the table?

Table 14.3.10.1 Summary of Pulmonary Function Test (PFT) Parameters and Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
Visit	Statistics	Observed value	Change from baseline	Observed value	Change from baseline	Observed value	Change from baseline
XXX	n	XX		XX		XX	
	Mean	XX.X		XX.X		XX.X	
	SD	XX.XX		XX.XX		XX.XX	
	Minimum	XX		XX		XX	
	Median	XX.X		XX.X		XX.X	
	Maximum	XX		XX		XX	
	25th percentile	XX.X		XX.X		XX.X	
	75th percentile	XX.X		XX.X		XX.X	
XXX	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
...							

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each visit time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.13.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters.

Table 14.3.11.1 Summary of Xanthoma Assessment over Time
(Safety Analysis Set)

Visit		Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Pre-existing xanthomas present	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Pre-existing tendon xanthomas present	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Anatomical locations (tendon)	Achilles tendon	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Dorsum of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Pre-existing cutaneous xanthomas present	Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Anatomical locations (cutaneous)	Tuberous, elbow	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Tuberous, knee	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, palm of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, soles of the feet	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Intertriginous, fingers	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Xanthelasmas, eyelids	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	etc...				

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = [(n/number who have a value at that visit)*100].
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits (baseline, week 56, week 104).

Table 14.3.11.2 Changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Pre-existing xanthomas present		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
Pre-existing xanthomas changed since previous visit	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.2 Changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Xanthomas reduced in size	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.2 Changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Xanthomas resolved	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.2 Changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Xanthomas increased in size	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.2 Changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
New xanthomas present*	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.3 Summary of new xanthomas over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Pre-existing xanthomas present		Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
New tendon xanthomas present*	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.3 Summary of new xanthomas over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Anatomical locations (tendon)*	Week 4	Achilles tendon	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Dorsum of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Achilles tendon	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Dorsum of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Achilles tendon	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Dorsum of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Achilles tendon	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Dorsum of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Etc.....				

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.3 Summary of new xanthomas over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
New cutaneous xanthomas present*	Week 4	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.3 Summary of new xanthomas over Time
(Safety Analysis Set)

	Visit	Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Anatomical locations (cutaneous)*	Week 4	Tuberous, elbow	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Tuberous, knee	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, palm of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, soles of the feet	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Intertriginous, fingers	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Xanthelasmas, eyelids	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	Tuberous, elbow	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Tuberous, knee	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, palm of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, soles of the feet	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Intertriginous, fingers	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Xanthelasmas, eyelids	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	Tuberous, elbow	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Tuberous, knee	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, palm of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, soles of the feet	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Intertriginous, fingers	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Xanthelasmas, eyelids	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	Tuberous, elbow	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Tuberous, knee	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, palm of the hands	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Planar, soles of the feet	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Intertriginous, fingers	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Xanthelasmas, eyelids	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Other	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Etc.....				

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. (%) = [(n/number who have pre-existing xanthoma)*100], unless otherwise stated.
[*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.4 Cumulative changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Pre-existing xanthomas present		XX (XX.X)	XX (XX.X)	XX (XX.X)
Xanthomas reduced in size by:	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. [*]
(%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.4 Cumulative changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Xanthomas resolved by:	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. [*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.4 Cumulative changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Xanthomas increased in size by:	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. [*] (%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.11.4 Cumulative changes to pre-existing Xanthoma over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
New xanthomas present by:	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)

At each time point, only subjects with a value at both baseline and that time point are included in the column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement. [*]
(%) = [n / N]
Source: Listing 16.2.17.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.12.1 Carotid Intima-Media Thickness and Flow-mediated Dilation Summary and Percentage Change from Baseline over Time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

		5 to 10 years (N=XX)		11 to 17 years (N=XX)		Overall (N=XX)	
Visit	Statistics	Observed value	% Change from baseline	Observed value	% Change from baseline	Observed value	% Change from baseline
XXX	n	XX		XX		XX	
	Mean	XX.X		XX.X		XX.X	
	SD	XX.XX		XX.XX		XX.XX	
	Minimum	XX		XX		XX	
	Median	XX.X		XX.X		XX.X	
	Maximum	XX		XX		XX	
	25th percentile	XX.X		XX.X		XX.X	
	75th percentile	XX.X		XX.X		XX.X	
XXX	n	XX	XX	XX	XX	XX	XX
	Mean	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	SD	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
	Minimum	XX	XX	XX	XX	XX	XX
	Median	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	Maximum	XX	XX	XX	XX	XX	XX
	25th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
	75th percentile	XX.X	XX.X	XX.X	XX.X	XX.X	XX.X
...							

Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
At each time point, only subjects with a value at both baseline and that time point are included in the change from baseline column.
N = the number of subjects in the analysis set who receive the respective treatment/dose. n = the number of subjects with a measurement.
Source: Listing 16.2.17.2 and 16.2.17.3

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits and parameters (right CIMT, left CIMT, FMD baseline diameter, FMD maximum diameter, %FMD).

Table 14.3.13.1 Summary of Palatability over Time
(Safety Analysis Set)

Visit		Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
XXX	Able to swallow capsule?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Total	XX (XX.X)	XX (XX.X)	XX (XX.X)
	If no, food media used?	Mashed banana	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Apple Sauce	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
	First occasion subject used this food media?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
	If yes, palatability rate?	Super bad	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Bad	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Maybe Good or Maybe Bad	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Good	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Super Good	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
Source: Listing 16.2.17.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.

Table 14.3.13.1 Summary of Palatability over Time
(Safety Analysis Set)

Visit		Result	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
	Parent/Guardian interpretation of child's reaction / facial expression	Pleasant	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Unpleasant	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Not sure	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Parent/Guardian Question: Do you sometimes have problems giving the medication to your child because he/she refuses to take it or throws up immediately after taking it?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Parent/Guardian Question: Do you sometimes have problems in giving the dietary supplement to your child because he/she refuses to take it or throws up immediately after taking it?	Yes	XX (XX.X)	XX (XX.X)	XX (XX.X)
		No	XX (XX.X)	XX (XX.X)	XX (XX.X)
		Missing	XX (XX.X)	XX (XX.X)	XX (XX.X)
XXX	etc...				

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
Source: Listing 16.2.17.4

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.

Table 14.3.14.1 Summary of Diet Compliance over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects compliant with low-fat diet, n(%)	Baseline	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects compliant with their low-fat diet [1]	Efficacy phase	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Safety phase (up to Week 56):	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Safety phase (up to Week 104):	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Compliance is defined as at least 80% of compliance with the low-fat diet during the respective study phase.
Source: Listing 16.2.19.1

PROGRAM NAME:T_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Table 14.3.14.1 Summary of Diet Compliance over Time
(Safety Analysis Set)

	Visit	5 to 10 years (N=XX)	11 to 17 years (N=XX)	Overall (N=XX)
Subjects compliant with dietary supplement regimen, n(%)	Baseline	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 4	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 8	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 12	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 16	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 20	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 24	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 28	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 32	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 36	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 40	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 44	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 48	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 52	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 56	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 68	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 80	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Week 92	XX (XX.X)	XX (XX.X)	XX (XX.X)
	EOT / Week 104	XX (XX.X)	XX (XX.X)	XX (XX.X)
Subjects compliant with their dietary supplement regimen [1]	Efficacy phase	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Safety phase (up to Week 56):	XX (XX.X)	XX (XX.X)	XX (XX.X)
	Safety phase (up to Week 104):	XX (XX.X)	XX (XX.X)	XX (XX.X)

N = the number of subjects in the analysis set. n = the number of subjects meeting the criterion. (%) = n/N*100.
[1] Compliance is defined as at least 80% of compliance with the low-fat diet during the respective study phase.
Source: Listing 16.2.19.1

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Listing 16.2.1.1 Subject Completions and Withdrawals
(Enrolled Set)

Site/Subject number	Age Group	Did the subject complete the study?	Completion/ Withdrawal date (Study day [1])	Primary reason for withdrawal	Relevant Details	Date of last IMP Dose (Study day [1])	Study duration (days) [2]	Treatment duration (days) [3]	Date of Death (Study day [1])
XXX/XXX	5 to 10 years	Yes/No	DDMONYYYY (XX)	XXXXXXXXXX	XXXXXXXXXX	DDMONYYYY (XX)	XX	XX	DDMONYYYY (XX)
XXX/XXX	11 to 17 years	Yes/No	DDMONYYYY (XX)	Other: XXXXX	XXXXXXXXXX	DDMONYYYY (XX)	XX	XX	DDMONYYYY (XX)
XXX/XXX	5 to 10 years	Yes/No	DDMONYYYY (XX)	Adverse event: <AE number>	XXXXXXXXXX	DDMONYYYY (XX)	XX	XX	DDMONYYYY (XX)

[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] Study duration is the number of days between first visit and the date of completion/withdrawal, derived as: (date of completion/withdrawal - date of first visit + 1).
[3] Treatment duration is the number of days between first administration of study treatment in period 1 and the date of completion/withdrawal, derived as: (date of completion/withdrawal - date of first administration of study treatment in period 1 + 1).

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Listing 16.2.2.1 Eligibility
(Enrolled Set)

Site/Subject number	Age group	Were all inclusion/exclusion criteria met?	Inclusion/Exclusion criteria not met	Informed consent given?	Assent given?	Protocol Version
XXX/XXX	5 to 10 years	Yes		Yes/No	Yes/No	XXX
XXX/XXX	11 to 17 years	No	IXXX, EXXX	Yes/No	Yes/No	XXX
XXX/XXX	5 to 10 years	Yes		Yes/No	Yes/No	XXX

Criterion numbers prefixed by I are inclusion criteria, those prefixed by E are exclusion criteria.

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Listing 16.2.2.2 Protocol Deviations
(Safety Analysis Set)

Site/Subject number	Age Group	Major/Minor [2]	Start date (Study day [1])	End date (Study day [1])	Deviation category (I/II/III)	Deviation details	Action Taken
XXX/XXX	5 to 10 years	Major / Minor	DDMONYYYY (XX)	DDMONYYYY (XX)	XXXXXXXX XXXXXXXX XXXXXXXX	XXXXXXXXXX	XXXXXXXXXX
XXX/XXX	11 to 17 years	Major / Minor	DDMONYYYY (XX)	DDMONYYYY (XX)	XXXXXXXX XXXXXXXX XXXXXXXX	XXXXXXXXXX	XXXXXXXXXX
XXX/XXX	11 to 17 years	Major / Minor	DDMONYYYY (XX)	DDMONYYYY (XX)	XXXXXXXX XXXXXXXX XXXXXXXX	XXXXXXXXXX	XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] Major protocol deviations are a subset of protocol deviations that may significantly impact the completeness, accuracy and/or reliability of the study data or that may significantly affect a subject's rights, safety or well-being.

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NOTES: Repeat protocol deviation listing for all protocol deviations regarding visits and assessments due to the COVID-19 pandemic. Add subheader accordingly to identify the PDs regarding the COVID-19 pandemic.

Listing 16.2.2.3 COVID-19 Protocol Deviations
(Safety Analysis Set)

Repeat of Listing 16.2.2.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.2.2

Listing 16.2.3.1 Analysis Sets
(Enrolled Set)

Site/Subject number	Age group	Age/Sex/Race	Eligible for analysis set?				Reason for exclusion
			Safety [1]	FAS [2]	CAS [3]	PPS [4]	
XXX/XXX	5 to 10 years	XX/Female/White	Yes/No	Yes/No	Yes/No	Yes/No	XXXXXXXXXX
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	Yes/No	Yes/No	Yes/No	XXXXXXXXXX
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	Yes/No	Yes/No	Yes/No	XXXXXXXXXX

[1] The Safety Analysis Set includes all subjects who are treated with at least one dose of the IMP.
[2] The Full Analysis Set includes all subjects who receive at least one dose of the IMP and who have a baseline and at least one post-baseline measurement of LDL-C.
[3] The Completers Analysis Set includes all subjects from the FAS who have not discontinued the Efficacy Phase (up to Week 24) of the study early irrespective of the reason for discontinuation.
[4] The Per Protocol Set includes all patients from the FAS who reasonably adhere to all protocol conditions (i.e. who have met the eligibility criteria and receive planned study medication without important protocol deviations considered to have a serious impact on the efficacy results within the Efficacy Phase of the trial).

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Listing 16.2.4.1 Demography
(Safety Analysis Set)

Site/Subject number	Age group	Age (years)	Age (months)	Sex	Race	Ethnicity	Height (cm)	Weight (kg)	Body mass index (kg/m^2) [1]
XXX/XXX	5 to 10 years	XXX	XXX	Male	XXXXXXXXXX	XXXXXXXXXX	XXX	XX.X	XX.X
XXX/XXX	11 to 17 years	XXX	XXX	Female	XXXXXXXXXX	XXXXXXXXXX	XXX	XX.X	XX.X

Height and weight recorded at Screening.
[1] Body mass index (BMI) is calculated as (weight (kg)/height (m)^2).
[2] BSA (m^2) = √[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years.

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Listing 16.2.4.1 Demography
(Safety Analysis Set)

Site/Subject number	Body surface area [2]	Height z score [3]	Height percentile [3]	BMI z score [3]	BMI percentile [3]
XXX/XXX	XX.X	XX.X	XX.X	XX.X	XX.X
XXX/XXX	XX.X	XX.X	XX.X	XX.X	XX.X

Height and weight recorded at Screening.
[1] Body mass index (BMI) is calculated as (weight (kg)/height (m)^2).
[2] BSA (m^2) = √[{height(cm) x weight(kg)} / 3600] [Mosteller formula]
[3] z scores and percentiles calculated using the WHO growth reference indicators for children aged 5 to 19 years.

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Listing 16.2.4.2 Diagnosis of HoFH
(Safety Analysis Set)

Site/Subject number	Age/Sex/Race	Age group	Duration of diagnosis (months)	Genetic confirmation of 2 mutant alleles?	Type of mutant allele	LDL-C receptor function	Untreated LDL-C>500mg/dL or treated LDL-C≥300mg/dL?	Cutaneous or tendon xanthoma before age 10 years?	Untreated LDL-C level consistent with HoFH on both parents?
XXX/XXX	XX/Female/White	5 to 10 years	XX.X	Yes/No	XXXXXXXXXX	XXXXX	Yes/No	Yes/No	Yes/No
XXX/XXX	XX/Female/White	11 to 17 years	XX.X	Yes/No	XXXXXXXXXX	XXXXX	Yes/No	Yes/No	Yes/No

Duration of diagnosis (months) = 12*[(date of baseline - date of HoFH diagnosis)+1]/365.25

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Listing 16.2.4.3 Medical History
(Safety Analysis Set)

Site/Subject number	Age/Sex/Race	Age Group	Reported term System organ class Preferred term	Start date	Stop date
XXX/XXX	XX/Female/White	5 to 10 years	XXXXXXXX XXXXXXXX XXXXXXXX	DDMONYYYY	DDMONYYYY
XXX/XXX	XX/Female/White	11 to 17 years	XXXXXXXX XXXXXXXX XXXXXXXX	DDMONYYYY	Ongoing

Only subjects with medical history are listed.
Medical history events were coded using the MedDRA Dictionary, version xx.x.

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Listing 16.2.4.4 Cardiovascular Medical History
(Safety Analysis Set)

Repeat of Listing 16.2.4.3

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.4.3 (as applicable)

Listing 16.2.4.5 Prior/Run-in/Concomitant Medications
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Timing	Reported name Medication class Standardised medication name	Dose	Dose unit	Frequency	Route
XXX/XXX	11 to 17 years	XX/Female/White	P / R / C	XXXXXXXX XXXXXXXX XXXXXXXX	XXX	XXX	XXX	XXX
XXX/XXX	5 to 10 years	XX/Female/White		XXXXXXXX XXXXXXXX XXXXXXXX	XXX	XXX	XXX	XXX

Only subjects who have recorded any medications during the study are listed.
Medications were coded using WHO Drug Dictionary, version xxx.
[1] Timing of Medication: P = Prior; R = 'Run-in'; C = Concomitant
[2] Study day is relative to the date of first administration of study drug (Day 0).
Prior medications / procedures are defined as those that started and ended prior to the enrolment date visit (visit 2) (i.e. start of the 'run-in period').
Run-in period' medications / procedures are defined as those that are ongoing at the time of starting the 'run-in' period (i.e. Visit 2) or started after the start of the 'run-in' period, but before the time of first administration of study treatment.
Concomitant medications / procedures are defined as those that are ongoing at the time of first administration of study treatment or started on or after the time of first administration of study treatment or started prior to the end / last dose of study treatment. If medication / procedure dates are incomplete and it is not clear whether the medication / procedure was concomitant, it will be assumed to be concomitant.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: If a medication is defined as prior medication only then timing = P.
If a medication is defined as run-in medication then timing = R.
If a medication is defined as concomitant medication then timing = C.
If a medication is taken during run-in and concomitantly then timing = C.

Listing 16.2.4.5 Prior/Run-in/Concomitant Medications
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Timing	Reported name Medication class Standardised medication name	Date (Study day [2]) Start of medication	 End of medication	 Indication
XXX/XXX	11 to 17 years	XX/Female/White	P / R / C	XXXXXXXX XXXXXXXX XXXXXXXX	DDMONYYYY (XX)	DDMONYYYY (XX)	XXXXXXXXXX
XXX/XXX	5 to 10 years	XX/Female/White		XXXXXXXX XXXXXXXX XXXXXXXX	DDMONYYYY (XX)	Ongoing	XXXXXXXXXX

Only subjects who have recorded any medications during the study are listed.
Medications were coded using WHO Drug Dictionary, version xxx.
[1] Timing of Medication: P = Prior; R = 'Run-in'; C = Concomitant
[2] Study day is relative to the date of first administration of study drug (Day 0).
Prior medications / procedures are defined as those that started and ended prior to the enrolment date visit (visit 2) (i.e. start of the 'run-in period').
Run-in period' medications / procedures are defined as those that are ongoing at the time of starting the 'run-in' period (i.e. Visit 2) or started after the start of the 'run-in' period, but before the time of first administration of study treatment.
Concomitant medications / procedures are defined as those that are ongoing at the time of first administration of study treatment or started on or after the time of first administration of study treatment or started prior to the end / last dose of study treatment. If medication / procedure dates are incomplete and it is not clear whether the medication / procedure was concomitant, it will be assumed to be concomitant.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: If a medication is defined as prior medication only then timing = P.
If a medication is defined as run-in medication then timing = R.
If a medication is defined as concomitant medication then timing = C.
If a medication is taken during run-in and concomitantly then timing = C.

Listing 16.2.4.6 Lipid-lowering-therapy (LLT) Medications
(Safety Analysis Set)

Repeat of Listing 16.2.4.5

Only subjects who have recorded any medications during the study are listed.
Medications were coded using WHO Drug Dictionary, version xxx.
Procedures were coded using the MedDRA Dictionary, version xx.x.
[1] <LLTMDEF>
[2] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.4.5 (as applicable)

Listing 16.2.4.7 Prior/Run-in/Concomitant Procedures
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Study Phase	Timing	Reported name Procedure Category Indication	Frequency	Date (Study day [2]) Start of procedure	 End of procedure
XXX/XXX	5 to 10 years	XX/Female/White	Efficacy / Safety	P / R / C	XXXXXXXX XXXXXXXX XXXXXXXX	XXX	DDMONYYYY (XX)	DDMONYYYY (XX)
XXX/XXX	11 to 17 years	XX/Female/White			XXXXXXXX XXXXXXXX XXXXXXXX	XXX	DDMONYYYY (XX)	Ongoing

Only subjects who have recorded any procedures during the study are listed.
Procedures were coded using the MedDRA Dictionary, version xx.x.
[1] Timing of Procedure: P = Prior; R = 'Run-in'; C = Concomitant
[2] Study day is relative to the date of first administration of study drug (Day 0).
Prior medications / procedures are defined as those that started and ended prior to the enrolment date visit (visit 2) (i.e. start of the 'run-in period').
Run-in period' medications / procedures are defined as those that are ongoing at the time of starting the 'run-in' period (i.e. Visit 2) or started after the start of the 'run-in' period, but before the time of first administration of study treatment.
Concomitant medications / procedures are defined as those that are ongoing at the time of first administration of study treatment or started on or after the time of first administration of study treatment or started prior to the end / last dose of study treatment. If medication / procedure dates are incomplete and it is not clear whether the medication / procedure was concomitant, it will be assumed to be concomitant.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.5.1 IMP Administration
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Study Drug Administration*	Date of Dispensing (Study day [1])	Strength and Number of Capsules Dispensed [2]	Daily Dose (Increase (I), Decrease (D), Unchanged (U)) (mg)**	Reason Dose Increased/Decreased/Unchanged
XXX/XXX	11 to 17 years	XXX	Yes (F)	DDMONYYYY (XX)	XXXXXXXXXX n=xx	XXX	XXXXXXXXXX
XXX/XXX	5 to 10 years	XXX	Yes	DDMONYYYY (XX)	XXXXXXXXXX n=xx	XXX (I)(MTD) (Maint)	XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] Capsules are dispensed as 2mg, 5mg, 10mg and 20mg
[3] Derived total number of weeks exposure to study medication = [(date of last dose - date of first dose)+1]/7
[4] Derived total exposure (mg/day) to study medication = ([number of doses received*treatment dose(mg)] / number of days exposed
[5] Derived total number of weeks of exposure to maintenance dose = [(date of last dose - date when maintenance dose is reached)+1]/7.
*F denotes date of first dose (i.e. date of first study drug administration)
** MTD denotes maximum tolerated dose. Maint denotes maintenance dose.
PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.5.1 IMP Administration
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Were Capsules Returned?	Strength and number of capsules returned [2]	Has subject complied with IMP dosing as per protocol?	Reason missed doses	Number missed	Consecutively or intermittently
XXX/XXX	11 to 17 years	XXX	No		Yes/No			
XXX/XXX	5 to 10 years	XXX	Yes	XXXXXXXXXX n=xx	Yes/No	XXXXXXXXXX	XX	XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] Capsules are dispensed as 2mg, 5mg, 10mg and 20mg
[3] Derived total number of weeks exposure to study medication = [(date of last dose - date of first dose)+1]/7
[4] Derived total exposure (mg/day) to study medication = ([number of doses received*treatment dose(mg)] / number of days exposed
[5] Derived total number of weeks of exposure to maintenance dose = [(date of last dose - date when maintenance dose is reached)+1]/7.
*F denotes date of first dose (i.e. date of first study drug administration)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.5.1 IMP Administration
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Was the treatment interrupted?	Reason for interruption	Date (Study Day [1]) Start of interruption	Date (Study Day [1]) End of interruption	Derived total number of weeks exposure to study medication [3]	Derived total exposure (mg/day) to study medication [4]	Derived total number of weeks exposure to maintenance dose [5]	Time (weeks) to dose reduction	Time (weeks) to dose interruption
XXX/XXX	11 to 17 years	XXX					XXX	XXX	XXX	XXX	XXX
XXX/XXX	5 to 10 years	XXX	Yes/No	XXXXXXXXXX	DDMONYYYY (XX)	DDMONYYYY (XX)	XXX	XXX	XXX	XXX	XXX

[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] Capsules are dispensed as 2mg, 5mg, 10mg and 20mg
[3] Derived total number of weeks exposure to study medication = [(date of last dose - date of first dose)+1]/7
[4] Derived total exposure (mg/day) to study medication = ([number of doses received*treatment dose(mg)] / number of days exposed
[5] Derived total number of weeks of exposure to maintenance dose = [(date of last dose - date when maintenance dose is reached)+1]/7.
*F denotes date of first dose (i.e. date of first study drug administration)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.6.1 Lipoprotein Apheresis (LA) History
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Receiving / Received (i.e.previous 60 days) LA treatment?	Date LA started [1]	Receive LA during run-in period and through Week 24?	Planned frequency of LA treatments
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	DDMONYYYY (XX)	Yes/No	XXXXXXXXXX
XXX/XXX	5 to 10 years	XX/Female/White	Yes/No	DDMONYYYY (XX) ...	Yes/No	Other: XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.6.2 Lipoprotein Apheresis (LA) Treatment
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Study Phase	Visit	Received LA treatment since last visit?	Date of LA [1]	Scheduled / Unscheduled	Days since previous LA	Change to ConMeds?	Fasting lipid panel local lab sample taken before scheduled LA?	Days to next LDL-C assessment
XXX/XXX	11 to 17 years	XX/Female/White	Efficacy / Safety	XXX	Yes/No	DDMONYYYY (XX)	Scheduled / Unscheduled	XX	Yes/No	Yes/No	XX
				Run-in weighted average*				XX			
						...					

[1] Study day is relative to the date of first administration of study drug (Day 0).
* Weighted averaged regimen during run-in.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits.
Study Phase: Efficacy - up to (and including) Week 24; Safety - up to (and including) Week 104.
After run-in, add Run-in weighted average.

Listing 16.2.7.1 Adverse Events
(Enrolled Set)

Site/Subject number	Age Group	Age/Sex/Race	TE? [1]	Reported term System organ class Preferred term	Date (Study day [2]) Time [3] Start	 End	Time to onset [4] [5] (days)	Duration [5] (d:hh:mm)
XXX/XXX	5 to 10 years	XX/Female/White	Yes/No	XXXXXXXX XXXXXXXX XXXXXXXX G	DDMONYYYY (XX) HH:MM	DDMONYYYY (XX) HH:MM	XXX	d:hh:mm
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	XXXXXXXX XXXXXXXX XXXXXXXX	DDMONYYYY (XX) HH:MM	Ongoing	XXX	UK

G = Gastrointestinal event, H = Hepatic event.
Only subjects with any adverse event are listed.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
[1] TE = Treatment-emergent adverse event defined as an adverse event that started after administration of study drug.
[2] Study day is relative to the date of first administration of study drug (Day 0).
[3] 24-hour clock (HH:MM).
[4] Time from date of first administration of study drug to onset of adverse event.
[5] For missing or partial dates and times, not calculated and is reported as unknown (UK).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Listing can go over multiple pages. Data for a given subject will appear on the consecutive page

Listing 16.2.7.1 Adverse Events
(Enrolled Set)

Site/Subject number	Age Group	Age/Sex/Race	TE? [1]	Reported term System organ class Preferred term	Was adverse event serious?	Serious Criteria	Adverse Event of Special Interest? If yes, reason	Severity
XXX/XXX	5 to 10 years	XX/Female/White	Yes/No	XXXXXXXX XXXXXXXX XXXXXXXX	Yes	XXXXXXXXXX	Yes, XXXXXXXXXX	XXX
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	XXXXXXXX XXXXXXXX XXXXXXXX	No	XXXXXXXXXX	No	XXX

Only subjects with any adverse event are listed.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
[1] TE = Treatment-emergent adverse event defined as an adverse event that started after administration of study drug.
[2] Study day is relative to the date of first administration of study drug (Day 0).
[3] 24-hour clock (HH:MM).
[4] Time from date of first administration of study drug to onset of adverse event.
[5] For missing or partial dates and times, not calculated and is reported as unknown (UK).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Listing can go over multiple pages. Data for a given subject will appear on the consecutive page

Listing 16.2.7.1 Adverse Events
(Enrolled Set)

Site/Subject number	Age Group	Age/Sex/Race	TE? [1]	Reported term Preferred term	System organ class	Relationship to study treatment	Action taken with study treatment	Other action taken	Was a concomitant treatment given for this adverse event?	Outcome
XXX/XXX	5 to 10 years	XX/Female/White	Yes/No	XXXXXXXX	XXXXXXXX	XXX	XXX	XXX	Yes	XXX
XXX/XXX	11 to 17 years	XX/Female/White	Yes/No	XXXXXXXX	XXXXXXXX	XXX	XXX	XXX	No	XXX

Only subjects with any adverse event are listed.
Adverse events were coded using the MedDRA Dictionary, version xx.x.
[1] TE = Treatment-emergent adverse event defined as an adverse event that started after administration of study drug.
[2] Study day is relative to the date of first administration of study drug (Day 0).
[3] 24-hour clock (HH:MM).
[4] Time from date of first administration of study drug to onset of adverse event.
[5] For missing or partial dates and times, not calculated and is reported as unknown (UK).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Listing can go over multiple pages. Data for a given subject will appear on the consecutive page

Listing 16.2.7.2 Serious Adverse Events
(Enrolled Set)

Repeat of Listing 16.2.7.1 but just for Serious AEs

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as Listing 16.2.7.1 (as appropriate)

Listing 16.2.8.1 Serum Clinical Chemistry (Fasting Lipid Panel) Values
(Safety Analysis Set)

	Local lab					Central lab			
Parameter	Unit	Site	Age range	Sex	Normal range	Unit	Age range	Sex	Normal range

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.8.1 Serum Clinical Chemistry (Fasting Lipid Panel) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Lomitapide Dose (mg)	Date of collection (Study day [1])	Time [2]	Local Lab	
							Observed	% Change from baseline
XXX/XXX	5 to 10 years	XX/Female/White	XXX	2 / 5 / 10 / 20 / 30 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X B NCS H	
			XXX	2 / 5 / 10 / 20 / 30 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X	XX.X

CS = clinically significant, NCS = not clinically significant.
H = High, L = Low. * = Maximum post-baseline value.
B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] 24-hour clock (HH:MM).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits and all parameters (i.e. LDL-C, Non-HDL-C, TC, VLDL-C, apo B, TG, Lp(a) in this order).

Listing 16.2.8.1 Serum Clinical Chemistry (Fasting Lipid Panel) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Lomitapide Dose (mg)	Date of collection (Study day [1])	Time [2]	Central lab	Central Lab	
							Observed	Change from baseline	% Change from baseline
XXX/XXX	5 to 10 years	XX/Female/White	XXX	2 / 5 / 10 / 20 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X B NCS		
			XXX	2 / 5 / 10 / 20 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X	XX.X	XX.X

CS = clinically significant, NCS = not clinically significant.
B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] 24-hour clock (HH:MM).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits and all parameters (i.e. LDL-C, Non-HDL-C, TC, VLDL-C, apo B, TG, Lp(a) in this order).

Listing 16.2.8.2 Serum Clinical Chemistry (Metabolic Panel) Values
(Safety Analysis Set)

		Local lab			
Parameter	Unit	Site	Age range	Sex	Normal range

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.8.2 Serum Clinical Chemistry (Metabolic Panel) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Lomitapide Dose (mg)	Date of collection (Study day [1])	Time [2]	Local Lab		
							Observed	Change from baseline	% Change from baseline
XXX/XXX	11 to 17 years	XX/Female/White	XXX	2 / 5 / 10 / 20 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X B NCS H		
						HH:MM	XX.X	XX.X	XX.X

CS = clinically significant, NCS = not clinically significant.
H = High, L = Low.
B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] 24-hour clock (HH:MM).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits and all parameters.

Listing 16.2.8.3 Serum Clinical Chemistry (Liver Function Tests) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Footnotes as per Listing 16.2.8.2 (as applicable)***
Add *, ^, +, ! flags with footnote:
[*] = > 3 x ULN (ALP and AST) or > 1.5 x ULN (ALP and TBL). [^] = > 5 x ULN (ALT and AST) or > 2 x ULN (TBL).
[+] = > 10 x ULN (ALT and AST). [!] = > 20 x ULN (ALT and AST).

Listing 16.2.8.4 Serum Clinical Chemistry (Essential Fatty Acids) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

	Central lab			
Parameter	Unit	Age range	Sex	Normal range

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Columns for site, age range and sex will be included in the final listing output if required.

Listing 16.2.8.4 Serum Clinical Chemistry (Essential Fatty Acids) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Lomitapide Dose (mg)	Date of collection (Study day [1])	Time [2]	Central Lab		
							Observed	Change from baseline	% Change from baseline
XXX/XXX	11 to 17 years	XX/Female/White	XXX	2 / 5 / 10 / 20 / 40 / 60	DDMONYYYY (XX)	HH:MM	XX.X B NCS H		
						HH:MM	XX.X	XX.X	XX.X

CS = clinically significant, NCS = not clinically significant.
H = High, L = Low.
B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0).
[2] 24-hour clock (HH:MM).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits and all parameters.

Listing 16.2.8.5 Serum Clinical Chemistry (Fat Soluble Vitamin Levels) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.4

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.4 (as applicable)

Listing 16.2.8.6 Serum Clinical Chemistry (Hormones) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.2 (as applicable)

Listing 16.2.8.7 Serum Clinical Chemistry (Sex Hormones) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.2 (as applicable)

Listing 16.2.8.8 Serum Clinical Chemistry (Serum Lipase) Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.2 (as applicable)

Listing 16.2.8.9 Serum Haematology Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.2 (as applicable)

Listing 16.2.8.10 Urinalysis Values
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Repeat of Listing 16.2.8.2

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Footnotes as per Listing 16.2.8.2 (as applicable)

Listing 16.2.9.1 Vital Signs
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Date of collection (Study day [1])	Observed	Change from baseline	% Change from baseline
XXX/XXX*	11 to 17 years	XX/Female/White	XXX	DDMONYYYY (XX)	XX.X B		
			XXX	DDMONYYYY (XX)	XX.X Min	XX.X	XX.X

* = Subject just met inclusion criterion #3 of having a body weight ≥15 kg.
B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
Min = Minimum Post Baseline Value, Max=Maximum Post Baseline Value, Last=Last Post Baseline Value (for height and weight parameters only).
[1] Study day is relative to the date of first administration of study drug (Day 0)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments should be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits. Parameters (i.e. weight, height, BMI, BSA, heart rate, respiration rate, SBP, DBP, body temp, BMI, height & weight percentile, BMI, height & weight Z score).

Listing 16.2.10.1 ECG Results
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Date of assessment (Study day [1])	Overall interpretation
XXX/XXX	11 to 17 years	XXX	DDMONYYYY (XX)	Normal
XXX/XXX	5 to 10 years	XXX	DDMONYYYY (XX)	Abnormal, NCS
XXX/XXX	11 to 17 years	XXX	DDMONYYYY (XX)	Abnormal, CS

CS = clinically significant, NCS = not clinically significant.
[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.

Listing 16.2.11.1 Echocardiography Results
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Scan performed?	Date of assessment (Study day [1])	Atherosclerosis and/or aortic valve disease?	Thickening aortic valve leaflets New (N): Progression (P):	Fusion of aortic valve leaflets New (N): Progression (P):	Thickening of aortic root New (N): Progression (P):
XXX/XXX	5 to 10 years	XXX	Yes/No	DDMONYYYY (XX)	Yes / No / N/A	Yes / No / N/A N: Yes/No P: Yes/No	Yes / No / N/A N: Yes/No P: Yes/No	Yes / No / N/A N: Yes/No P: Yes/No

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.

Listing 16.2.11.1 Echocardiography Results
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Scan performed?	Flow acceleration / turbulence over aortic valve New (N): Progression (P): Region (Re):	Aortic valve regurgitation New (N): Progression (P):	Coronary ostial narrowing / flow disturbances New (N): Progression (P):
XXX/XXX	5 to 10 years	XXX	Yes/No	Yes / No / N/A N: Yes/No P: Yes/No Re: Supravalvular / ascending aorta	Yes / No / N/A N: Yes/No P: Yes/No	Yes / No / N/A N: Yes/No P: Yes/No

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.

Listing 16.2.11.1 Echocardiography Results
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Scan performed?	Ventricular findings	Left ventricular hypertrophy New (N): Progression (P):	Impairment of left ventricular function New (N): Progression (P):	Other relevant findings
XXX/XXX	5 to 10 years	XXX	Yes/No	Yes / No / N/A	Yes / No / N/A N: Yes/No P: Yes/No	Yes / No / N/A N: Yes/No P: Yes/No	Yes/No Yes: XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.

Listing 16.2.12.1 Lipid Accumulation in the Liver Results
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	NMR Scan performed?	Reason not performed?	Date of scan (Study day [1])	Ultrasound Scan performed?	Reason not performed?	Date of scan (Study day [1])	Result	Change from baseline	% Change from baseline
XXX/XXX	11 to 17 years	XXX	Yes/No		DDMONYYYY (XX)	Yes/No		DDMONYYYY (XX)	XXX	XXX	XXX
XXX/XXX	5 to 10 years	XXX	Yes/No	XXXXXXXXXX	DDMONYYYY (XX)	Yes/No	XXXXXXXXXX				

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments will be included in the listing and flagged as such in the visit column.

Listing 16.2.13.1 Physical Examination
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Assessment performed?	Reason not performed?	Date of examination (Study day [1])	Body System	Physical Examination Findings	Comments
XXX/XXX	5 to 10 years	XXX	Yes/No		DDMONYYYY (XX)	XXXXXXXXXX	Normal / Abnormal / Not Done	XXXXXXXXXX
XXX/XXX	11 to 17 years	XXX	Yes/No	XXXXXXXXXX	DDMONYYYY (XX)	XXXXXXXXXX	Normal / Abnormal / Not Done	XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits recorded

Listing 16.2.13.2 Tanner Staging
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Visit	Date (Study Day [1])	Was assessment performed?	Reason not performed?	Tanner Stage
XXX/XXX	11 to 17 years	XX/Female/White	XXX	DDMMMYYYY (XX)	Y		XXX
XXX/XXX	5 to 10 years	XX/Female/White	XXX	DDMMMYYYY (XX)	N	XXXXXXXXXX	

B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments should be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.

Listing 16.2.14.1 Pulmonary Function Test
(Safety Analysis Set)

Parameter: <Parameter (Unit)>

Site/Subject number	Age Group	Age/Sex/Race	Visit	Was assessment performed?	Reason not performed?	Date of assessment (Study day [1])	Observed	Change from baseline
XXX/XXX	5 to 10 years	XX/Female/White	XXX	Yes		DDMONYYYY (XX)	XX.X B	
			XXX	Yes		DDMONYYYY (XX)	XX.X	XX.X
			XXX	No	XXXXXXXXXX			

B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments should be included in the listing and flagged as such in the visit column.

Listing 16.2.15.1 Pregnancy Test
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Was a pregnancy test performed?	If No, reason pregnancy test not performed	If Yes, date of pregnancy test (Study day [1])	Result
XXX/XXX	5 to 10 years	XXX	Yes/No		DDMONYYYY (XX)	Negative/Positive
XXX/XXX	11 to 17 years	XXX	Yes/No	Subject is premenarchal		
XXX/XXX	11 to 17 years	XXX	Yes/No	Subject is male		

Pregnancy test data was collected at Screening.
[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.16.1 Visit Dates
(Enrolled Set)

Site/Subject number	Age Group	Screening	Baseline (Study Day 1)	Week 4 (Study Day 28)	Week 8 (Study Day 56)	Week 12 (Study Day 84)	Week 16 (Study Day 112)	Week 20 (Stusy Day 140)	Week 24 (Study Day 168)
XXX/XXX	5 to 10 years	DDMONYYYY	DDMONYYYY (1)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)

Study Day in relation to Baseline visit date (Study Day 1) presented in brackets.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.16.1 Visit Dates
(Enrolled Set)

Site/Subject number	Age Group	Week 28 (Study Day 196)	Week 32 (Study Day 224)	Week 36 (Study Day 252)	Week 40 (Study Day 280)	Week 44 (Study Day 308)	Week 48 (Study Day 336)	Week 52 (Study Day 364)	Week 56 (Study Day 392)
XXX/XXX	5 to 10 years	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)

Study Day in relation to Baseline visit date (Study Day 1) presented in brackets.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.16.1 Visit Dates
(Enrolled Set)

Site/Subject number	Age Group	Week 68 (Study Day 476)	Week 80 (Study Day 560)	Week 92 (Study Day 644)	Week 104 (Study Day 728)	Week 108 (Study Day 756)
XXX/XXX	5 to 10 years	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)	DDMONYYYY (XX)

Study Day in relation to Baseline visit date (Study Day 1) presented in brackets.

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Listing 16.2.17.1 Xanthoma Examination
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Was assessment performed?	Reason not performed?	Date of assessment (Study day [1])	Were there pre-existing xanthomas present?	Were there pre-existing tendon xanthomas present?	Anatomical location	Pre-existing cutaneous xanthomas present	Anatomical location
XXX/XXX	5 to 10 years	XXX	Y		DDMONYYYY (XX)	Yes/No	Yes/No	XXXXXXXXXX	Yes/No	XXXXXXXXXX
XXX/XXX	11 to 17 years	XXX	N	XXXXXXXXXX	DDMONYYYY (XX)	Yes/No	Yes/No	Other: XXXXXXXXXX	Yes/No	Other: XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits recorded

Listing 16.2.17.1 Xanthoma Examination
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Pre-existing xanthomas changed since last visit?	If yes, have they reduced in size?	Have they resolved? (Date of resolution)	Have they increased in size?	Were new xanthomas present?	Were new tendon xanthomas present?	Anatomical location	Were new cutaneous xanthomas present?	Anatomical location
XXX/XXX	5 to 10 years	XXX	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No	XXXXXXXXXX	Yes/No	XXXXXXXXXX
XXX/XXX	11 to 17 years	XXX	Yes/No	Yes/No	Yes/No (DDMONYYYY)	Yes/No	Yes/No	Yes/No	Other: XXXXXXXXXX	Yes/No	Other: XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits recorded

Listing 16.2.17.2 Carotid Intima-Media Thickness (CIMT)
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Visit	Was CIMT assessment performed?	Reason not performed?	Date of collection (Study day [1])	Parameter	Unit	Observed	Change from baseline	% Change from baseline
XXX/XXX	11 to 17 years	XX/Female/White	XXX	Y		DDMONYYYY (XX)	XXX	XXX	XX.X B		
			XXX	N	XXXXXXXXXX	DDMONYYYY (XX)	XXX	XXX	XX.X	XX.X	XX.X

B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments should be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits.

Listing 16.2.17.3 Flow-mediated Dilation (FMD)
(Safety Analysis Set)

Site/Subject number	Age Group	Age/Sex/Race	Visit	Was FMD assessment performed?	Reason not performed?	Date of collection (Study day [1])	Parameter	Unit	Observed	Change from baseline	% Change from baseline
XXX/XXX	5 to 10 years	XX/Female/White	XXX	Y		DDMONYYYY (XX)	XXX	XXX	XX.X B		
			XXX	N	XXXXXXXXXX	DDMONYYYY (XX)	XXX	XXX	XX.X	XX.X	XX.X

B = baseline value. Baseline is defined as the last non-missing value (including scheduled and unscheduled assessments) prior to the subject receiving study treatment in the Efficacy Phase (Visit 4).
[1] Study day is relative to the date of first administration of study drug (Day 0)

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Unscheduled assessments should be included in the listing and flagged as such in the visit column.
The listing may go over multiple pages to capture all data.
Repeat for all visits.

Listing 16.2.17.4 Palatability
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Was assessment performed?	Reason not performed?	Date of assessment (Study day [1])	Able to swallow capsule?	If no, food media used?	First occasion used this food media	If yes, rate palatability
XXX/XXX	11 to 17 years	XXX	Y		DDMONYYYY (XX)	Yes/No			
XXX/XXX	5 to 10 years	XXX	N	XXXXXXXXXX	DDMONYYYY (XX)	Yes/No	XXXXXXXXXX	Yes/No	XXXXXXXXXX

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits recorded

Listing 16.2.17.4 Palatability
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Was assessment performed?	Parent / guardian interpretation of child's reaction / facial expression	Problems giving medication to child as refuses to take or throws up immediately after?	Problems giving dietary supplements to child as refuses to take or throws up immediately after?
XXX/XXX	11 to 17 years	XXX	Y	XXXXXXXXXX	Yes/No	Yes/No
XXX/XXX	5 to 10 years	XXX	N	XXXXXXXXXX	Yes/No	Yes/No

[1] Study day is relative to the date of first administration of study drug (Day 0).

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all visits recorded

Listing 16.2.19.1 Diet and Dietary Supplement Compliance
(Safety Analysis Set)

Site/Subject number	Age Group	Visit	Were 2-day diet records completed?	Were 2-day diet records checked?	Dietary discussion took place?	Subject compliant with low fat diet?	Dietary supplements dispensed?	Subject compliant with dietary supplement regimen?
XXX/XXX	11 to 17 years	XXX	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No
XXX/XXX	5 to 10 years	XXX	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No	Yes/No

PROGRAM NAME:L_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for all study visits

Figure 14.1.5.1 Kaplan-Meier Plot of Time to Dose Reduction
(Safety Analysis Set)

Kaplan-Meier plot of time to dose reduction

Time to dose reduction is defined as the time (weeks) from the date of the start of the safety phase to the time of first dose reduction, if any. Subjects who do not enter the safety phase will be censored at time 0. If a patient discontinues study drug they will be counted as an event at the time of study discontinuation. If a patient has not had a dose reduction and is still on study drug, they will be censored at their last visit assessment date.
N = the number of subjects in the analysis set.
Source: Listing 16.2.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Treatment group N counts should be presented in the legend.

Figure 14.1.5.2 Kaplan-Meier Plot of Time to Dose Interruption
(Safety Analysis Set)

Kaplan-Meier plot of time to dose interruption

Time to dose interruption is defined as the time (weeks) from the date of the start of the safety phase to the time of first dose interruption, if any. Subjects who do not enter the safety phase will be censored at time 0. If a patient discontinues study drug they will be counted as an event at the time of study discontinuation. If a patient has not had a dose interruption and is still on study drug, they will be censored at their last visit assessment date.
N = the number of subjects in the analysis set.
Source: Listing 16.2.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Treatment group N counts should be presented in the legend.

Figure 14.2.1.1 Waterfall Plot of Percentage Change (%) from Baseline in Fasting Lipid Panel at Week 24 by age group
(Full Analysis Set)

Parameter: <Parameter (Unit)>

y-axis: percentage change from baseline at Week 24.
x-axis: subject.
Use 3 colours for the blocks representing the respective age group for the subject and overall.

Source: Listing 16.2.8.1, Table 14.2.1.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all parameters.***
Include reference line at 0.
Please present LDL-C first and then same order as specified in SAP rather than alphabetically.
Update table source according to parameter Table 14.2.1.1 (LDL-C), Table 14.2.2.1 (Non-HDL-C), Table 14.2.3.1 (TC), Table 14.2.4.1 (VLDL-C), Table 14.2.5.1 (Apo B), Table 14.2.6.1 (TG), Table 14.2.7.1 (Lp(a)
Use analysis study visit data.

Figure 14.2.1.2 Forest Plot of Sub-group analysis for primary end point (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

x-axis: Mean difference in percentage change at week 24.
y-axis: Overall estimate and Sub-group analysis references (i.e. SG1 to SG7).
Point estimate and 95% confidence interval. Point estimate will be estimated as the mean percentage change and corresponding 95% CI calculated from the ANCOVA model.

Source: Listing 16.2.8.1, Table 14.2.1.4 (SG1), Table 14.2.1.5 (SG2), Table 14.2.1.6 (SG3), Table 14.2.1.7 (SG4), Table 14.2.1.8 (SG5), Table 14.2.1.9 (SG6), Table 14.2.1.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group.
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.3 Profile Plot of LSMeans percentage change from baseline over time in Fasting Lipid Panel
(Full Analysis Set)

Parameter: <Parameter (Unit)>

x-axis: time (weeks) relative to baseline.
y-axis: dynamic based on the parameter <Parameter (Unit)>.
Point estimate and corresponding error bar. LS mean percentage change at time point with corresponding standard error. The mean and median dose at each timepoint will be added.

Source: Listing 16.2.8.1, Table 14.2.9.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all parameters.***
Include reference line at 0.
Please present Week 104 as EOT / Week 104 (as we do in the tables)
Update table source according to parameter Table 14.2.9.1 (LDL-C), Table 14.2.9.2 (Non-HDL-C), Table 14.2.9.3 (TC), Table 14.2.9.4 (VLDL-C), Table 14.2.9.5 (Apo B), Table 14.2.9.6 (TG), Table 14.2.9.7 (Lp(a)), Table 14.2.9.8 (TC/HDL-C) and Table 14.2.12.2 (HDL-C)
Use analysis study visit data.

Figure 14.2.1.4 Forest Plot of Sub-group analysis for key secondary endpoint: Non-HDL-C (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

Repeat of Figure 14.2.1.2 for NON-HDL-C

Source: Listing 16.2.8.1, Table 14.2.2.4 (SG1), Table 14.2.2.5 (SG2), Table 14.2.2.6 (SG3), Table 14.2.2.7 (SG4), Table 14.2.2.8 (SG5), Table 14.2.2.9 (SG6), Table 14.2.2.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.5 Forest Plot of Sub-group analysis for key secondary endpoint: TC (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

Repeat of Figure 14.2.1.2 for TC

Source: Listing 16.2.8.1, Table 14.2.3.4 (SG1), Table 14.2.3.5 (SG2), Table 14.2.3.6 (SG3), Table 14.2.3.7 (SG4), Table 14.2.3.8 (SG5), Table 14.2.3.9 (SG6), Table 14.2.3.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.6 Forest Plot of Sub-group analysis for key secondary endpoint: VLDL-C (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

Repeat of Figure 14.2.1.2 for VLDL-C

Source: Listing 16.2.8.1, Table 14.2.4.4 (SG1), Table 14.2.4.5 (SG2), Table 14.2.4.6 (SG3), Table 14.2.4.7 (SG4), Table 14.2.4.8 (SG5), Table 14.2.4.9 (SG6), Table 14.2.4.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.7 Forest Plot of Sub-group analysis for key secondary endpoint: Apo B (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

Repeat of Figure 14.2.1.2 for Apo B

Source: Listing 16.2.8.1, Table 14.2.5.4 (SG1), Table 14.2.5.5 (SG2), Table 14.2.5.6 (SG3), Table 14.2.5.7 (SG4), Table 14.2.5.8 (SG5), Table 14.2.5.9 (SG6), Table 14.2.5.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.8 Forest Plot of Sub-group analysis for key secondary endpoint: TG (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit

Repeat of Figure 14.2.1.2 for TG

Source: Listing 16.2.8.1, Table 14.2.6.4 (SG1), Table 14.2.6.5 (SG2), Table 14.2.6.6 (SG3), Table 14.2.6.7 (SG4), Table 14.2.6.8 (SG5), Table 14.2.6.9 (SG6), Table 14.2.6.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for Nominal Study Visit.***
Include reference line at 0.

Figure 14.2.1.9 Forest Plot of Sub-group analysis for key secondary endpoint: Lp(a) (percentage change at week 24)
(Full Analysis Set)

Analysis Study Visit
<Unit>

Repeat of Figure 14.2.1.2 for Lp(a)

Source: Listing 16.2.8.1, Table 14.2.7.4 (SG1), Table 14.2.7.5 (SG2), Table 14.2.7.6 (SG3), Table 14.2.7.7 (SG4), Table 14.2.7.8 (SG5), Table 14.2.7.9 (SG6), Table 14.2.7.10 (SG7)
SG1: Age cohorts i.e. 2 age groups).
SG2: Documented CVD vs no documented CVD history at screening (pooled across age groups).
SG3: Established CVD history (defined as aortic valve disease and/or coronary atherosclerosis) vs no established CVD history (as defined previously) at screening (pooled across age groups).
SG4: Taken concomitant LLT medications vs not taken concomitant LLT medications (pooled across age groups).
SG5: No dose reductions vs at least one dose reduction (pooled across age groups).
SG6: Reached MTD for age group vs did not reach MTD for age group
SG7: Male vs Female (pooled across age groups).

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for Nominal Study Visit.
Include reference line at 0.

Figure 14.2.1.10 Profile Plot of percentage change from baseline over time in Fasting Lipid Panel
(Full Analysis Set)

Parameter: <Parameter (Unit)>

x-axis: time (weeks) relative to baseline.
y-axis: dynamic based on the parameter <Parameter (Unit)>.
Percentage change at time point. The mean and median dose at each timepoint will be added.

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for all parameters.***
Include reference line at 0.
Please present Week 104 as EOT / Week 104 (as we do in the tables)
Update table source according to parameter Table 14.2.8.1 (LDL-C), Table 14.2.8.2 (Non-HDL-C), Table 14.2.8.3 (TC), Table 14.2.8.4 (VLDL-C), Table 14.2.8.5 (Apo B), Table 14.2.8.6 (TG), Table 14.2.8.7 (Lp(a)), Table 14.2.8.8 (TC/HDL-C) and Table 14.2.12.1 (HDL-C)
Use analysis study visit data.
Include reference line at 0.

Figure 14.3.2.1 Cumulative distribution function (CDF) plot of LDL-C at Week 24
(Full Analysis Set)

x-axis: LDL-C (mg/dL) at Week 24.
y-axis: cumulative percentage.
The cumulative distribution function for LDL-C at week 24 will be plotted with a reference line at 135 mg/dL.
Use 3 different coloured lines to represent each of the 2 age groups and overall.

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Include reference line at 135.***
Use analysis study visit Week 24.

Figure 14.3.2.2 Cumulative distribution function (CDF) plot of LDL-C over time
(Full Analysis Set)

x-axis: LDL-C (mg/dL) at Week X.
Y-axis: cumulative percentage.
The cumulative distribution function for LDL-C at Week X will be plotted with a reference line at 135 mg/dL.
Use 3 different coloured lines to represent each of the 2 age groups and overall.

Source: Listing 16.2.8.1, Table 14.2.11.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each analysis study visit within the efficacy and safety phase (except week 24). Replace Week X with corresponding analysis study visit for each run,

Figure 14.3.2.3 Cumulative distribution function (CDF) plot of percentage change in LDL-C at Week 24
(Full Analysis Set)

x-axis: percentage change in LDL-C (%) at Week 24.
y-axis: cumulative percentage.
The cumulative distribution function for percentage change in LDL-C at week 24 will be plotted with a reference line at -15, -25 and -50%.
Use 3 different coloured lines to represent each of the 2 age groups and overall.

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Include reference line at -15, -25 and -50.***
Use analysis study visit Week 24.

Figure 14.3.2.4 Cumulative distribution function (CDF) plot of lowest percentage change in LDL-C from Week 8
(Full Analysis Set)

x-axis: lowest percentage change in LDL-C (%) from Week 8.
y-axis: cumulative percentage.
The cumulative distribution function for percentage change in LDL-C from Week 8 will be plotted with a reference line at -15, -25 and -50%.
Use 3 different coloured lines to represent each of the 2 age groups and overall.

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Include reference line at -15, -25 and -50.

Figure 14.3.1.1 Subject profile for distribution of Treatment-Emergent adverse events and dose at time of event
(Safety Analysis Set)

All subjects

Each subject would have a horizontal bar. The length of the bar would represent the time on the study.
For each subject bar, the colour of the bar would change over time, with each colour representing the dose of the drug the subject was on at that time.
Any periods the subject was unable to take any dose should be clearly marked in a grey or white colour (for example).
Overlayed on each subject bar will be a symbol to represent the TEAE PT (e.g. vomiting, abdominal pain, diarrhoea, etc).
The symbol will be placed at the relevant timepoint the TEAE began and an arrow added to represent the duration of the TEAE.
Each TEAE experienced by the subject will be represented in a similar manner.
The colour of the arrow for each TEAE will correspond to the TEAE being related / unrelated to lomitapide.
The line style of the arrow for each TEAE will correspond to the TEAE severity.
Y-axis = subject number.
X-axis = Time (weeks).
Note: Time (weeks) should correspond with visits and days used for TEAE onset and recovery.

Source: Listing 16.2.7.1, Table 14.3.1.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Repeat for: Compliant with diet - All subjects; Compliant with diet - All subjects aged 5 to 10 years; Compliant with diet - All subjects aged 11 to 17 years.
Repeat for: Non-compliant with diet - All subjects; Non- compliant with diet - All subjects aged 5 to 10 years; Non-compliant with diet - All subjects aged 11 to 17 years.

Figure 14.3.1.2 Age profile for distribution of Treatment-Emergent adverse events and MTD
(Safety Analysis Set)

Overall - All subjects

Each subject would have a vertical bar.
The length of the bar would represent the time on study.
Under the x-axis, rather than the subject number being displayed the subject's age will be displayed on the graphic.
The patients will be ordered by age starting with the youngest age closest to the y-axis and moving in an ascending order of age.
For each patient bar, the colour of bar would represent the MTD the subject achieved, with each colour representing the MTD the subject achieved.
Y-axis = Time (weeks).
X-axis = Subject.
Note: Time (weeks) should correspond with visits.
Overlayed on each patient bar will be a symbol to represent the TEAE PT (e.g. vomiting, abdominal pain, diarrhoea, etc). The symbol will be placed at the relevant timepoint the TEAE began. Each TEAE experienced by the subject will be represented in a similar manner.

Source: Listing 16.2.7.1, Table 14.3.1.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Repeat for: Related TEAEs - All subjects; Related TEAEs - All subjects aged 5 to 10 years; Related TEAEs - All subjects aged 11 to 17 years.

Figure 14.3.1.3 Histogram of Time to First Gastrointestinal Treatment-Emergent Adverse Event
(Safety Analysis Set)

Overall

Histogram showing the time (days) of onset from the first dose to the first gastrointestinal TEAE

Source: Listing 16.2.7.1, Table 14.3.1.13

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Subjects aged 5 to 10 years; Subjects aged 11 to 17 years.

Figure 14.3.1.4 Histogram of Time to First Hepatic Treatment-Emergent Adverse Event
(Safety Analysis Set)

Overall

Histogram showing the time of onset from the first dose to the first hepatic TEAE

Source: Listing 16.2.7.1, Table 14.3.1.14

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Subjects aged 5 to 10 years; Subjects aged 11 to 17 years.

Figure 14.3.3.1 Individual subject profiles of liver function data
(Safety Analysis Set)

Subject XXXX

For each patient, at each time point the corresponding liver function tests value will be plotted.
Heptic fat will also be added with a second y-axis with the hepatic fat scale.
A line joining all plotted values will be added to show the patient profile of the liver function test over time.
A separate line will be generated for each liver function test profile over time.
Each line will be colour coordinated by the liver function test (e.g. AST, ALT, GGT, Alk Phos, Total bilirubin, serum albumin).
Each patient would have their own plot to show all liver function tests on one page.
Patient's dose of drug included under the x-axis at each of the analysis study weeks.
Y-axis = liver function scale.
Y-axis2 = hepatic fat scale.
X-axis = Analysis Study Week.

Source: Listing 16.2.8.3, Table 14.3.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Screening will be defined at week -12.***
Time (week) on x-axis will use the analysis study week window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.2 AST profiles over time, by subject age
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding AST liver function tests value will be plotted.
A line joining all plotted values will be added to show the patient profile of the liver function test over time.
Each line will be colour coordinated by patient's age.
Y-axis = AST liver function scale.
X-axis = time (weeks).

Source: Listing 16.2.8.3, Table 14.3.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.3 ALT profiles over time, by subject age
(Safety Analysis Set)

Subject XXXX

For each patient, at each time point the corresponding ALT liver function tests value will be plotted.
A line joining all plotted values will be added to show the patient profile of the liver function test over time.
Each line will be colour coordinated by patient's age.
Y-axis = ALT liver function scale.
X-axis = time (weeks).

Source: Listing 16.2.8.3, Table 14.3.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.4 AST profiles over time, by subject MTD
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding AST liver function tests value will be plotted.
A line joining all plotted values will be added to show the patient profile of the liver function test over time.
Each line will be colour coordinated by patient's MTD.
Y-axis = AST liver function scale.
X-axis = time (weeks).

Source: Listing 16.2.8.3, Table 14.3.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.5 ALT profiles over time, by subject MTD
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding ALT liver function tests value will be plotted.
A line joining all plotted values will be added to show the patient profile of the liver function test over time.
Each line will be colour coordinated by patient's MTD.
Y-axis = ALT liver function scale.
X-axis = time (weeks).

Source: Listing 16.2.8.3, Table 14.3.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.6 Individual subject profiles of LDL-C data
(Safety Analysis Set)

Subject XXXX

For each patient, at each time point the corresponding LDL-C value will be plotted.
A line joining all plotted values will be added to show the patient profile of the LDL-C over time.
Markers will be added to each LDL-C profile line to denote the treatment dose the patient was on at that time point.
Any periods the subject was unable to take any dose should also be clearly marked using a different marker.
Dose adjustments will be identified with a different marker and lipoprotein apheresis with a vertical line.
Each patient would have their own plot to show their LDL-C profile.
A horizontal line denoting an LDL-C of 110 mg/dL will be added.
Y-axis = LDL-C scale.
X-axis = time (weeks).

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Add a reference line at y=110 mg/dL.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.7 LDL-C profiles over time, by subject age
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding LDL-C value will be plotted.
A line joining all plotted values will be added to show the patient profile of the LDL-C over time.
Each line will be colour coordinated by patient's age.
Y-axis = LDL-C scale.
X-axis = time (weeks).

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the analysis study week window for statistical analyses as defined in the SAP to determine study week for the time axis.
Patients who discontinue early and use EOT/Week 104, please use the calculated study day the EOT was completed to assign the subject to the relevant analysis study week window (e.g. patients 01-02 and 01-03).
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.8 LDL-C profiles over time, by subject MTD
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding LDL-C value will be plotted.
A line joining all plotted values will be added to show the patient profile of the LDL-C over time.
Each line will be colour coordinated by patient's MTD.
Y-axis = LDL-C scale.
X-axis = time (weeks).

Source: Listing 16.2.8.1, Table 14.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Screening will be defined at week -12.
Time (week) on x-axis will use the study say window for statistical analyses as defined in the SAP to determine study week for the time axis.
Include unscheduled visits in figure (as approporiate).

Figure 14.3.3.9 Spaghetti plot of lipid profiles over time
(Safety Analysis Set)

Parameter: <Parameter (Unit)>
Overall - All subjects

For each patient, at each time point the corresponding lipid value will be plotted.
A separate line will be generated for each patient to show their individual parameter value over time.
Y-axis = Lipid value.
X-axis = time (weeks).

Source: Listing 16.2.8.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years. Inlcude assessments from Run-in up to Week 104/EoT.

Figure 14.3.3.10 eDISH plot of Maximum Post-Baseline ALT by Maximum Post-Baseline Total Bilirubin
(Safety Analysis Set)

Overall / Efficacy Phase / Safety Phase / Follow-up

x-axis: Maximum post-baseline ALT (x ULN)
y-axis: Maximum post-baseline TBL (x ULN)
Plot maximum post baseline ALT by maximum post baseline TBL for each patient. Use log scale for both sets of axes.

Include the following reference lines: x-axis (vertical line) = 3, y-axis (horizontal line) = 2.
Include reference lines also where x = y = 1 (will create a 'box' in the lower left of the plot).
Label the 4 quadrants created by the reference lines: Top left = Hyperbilirubinemia; Top right = Hy's Law range; Bottom right = Temple's Corollary range; Bottom left: Normal Range (inside 'box' created by x = y = 1 reference lines).

ALT = Alanine Aminotransaminase, TBL = Total Bilirubin, ULN = Upper Limit of Normal.
Source: Listing 16.2.8.3

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Different coulour/symbol for each age group***
Include any abnormal observation (scheduled or unscheduled)
Repeat for each study phase

Figure 14.3.3.11 eDISH plot of Maximum Post-Baseline AST by Maximum Post-Baseline Total Bilirubin
(Safety Analysis Set)

Overall / Efficacy Phase / Safety Phase / Follow-up

Repeat Figure 14.3.3.9 for AST

AST = Aspartate Aminotransferase, TBL = Total Bilirubin, ULN = Upper Limit of Normal.
Source: Listing 16.2.8.3

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Different coulour/symbol for each age group***
Include any abnormal observation (scheduled or unscheduled)
Repeat for each study phase

Figure 14.3.3.12 Subject profile for distribution of Liver Function Test Abnormalities and dose at time of event
(Safety Analysis Set)

All subjects

Each subject would have a horizontal bar.
Each subject experiencing an LFT abnormality will be identified with a bar starting at LFT elevation start and ending at the date when the LFT abnormality ends.
Dose taken will be marked in the plot. Any periods the subject was unable to take any dose should be clearly marked.
Overlayed on each subject bar will be a symbol to represent the Hepatic TEAE PT (e.g. vomiting, abdominal pain, diarrhoea, etc).
The symbol will be placed at the relevant timepoint the TEAE began and an arrow added to represent the duration of the TEAE.
Each Hepatic TEAE experienced by the subject will be represented in a similar manner.

Y-axis = subject number.
X-axis = Time (weeks).

Source: Listing 16.2.5.1, Listing 16.2.7.1, Listing 16.2.8.3

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.5.1 Boxplot of Observed Vital Signs over Time
(Safety Analysis Set)

|L|Parameter: <Parameter (Unit)>

x-axis: Time (weeks)
y-axis: Observed value

Boxplot of the observed value for the respective parameter over time. A boxplot will be displayed for each age group at each side of the tick mark, Use a different colour for each age group.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Different coulour/symbol for each age group

Figure 14.3.5.2 Boxplot of Change from Baseline Vital Signs over Time
(Safety Analysis Set)

|L|Parameter: <Parameter (Unit)>

Repeat Figure 14.3.5.1 for change from baseline

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Different coulour/symbol for each age group

Figure 14.3.5.3 Waterfall plot of change in BMI at Week 24, 56 and 104, by patient sex
(Safety Analysis Set)

Week 24
Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change in BMI (baseline to the respective week, as applicable).
Order the vertical bars in decreasing order of size of change in BMI.
For each patient bar, the colour of the bar will represent the sex of the patient.
Y-axis = change in BMI.
X-axis = subject.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.8.1 Scatter plot of change in hepatic fat up with change in BMI Z score up to week 56 and 104, by subject age
(Safety Analysis Set)

Week 56
Overall - All subjects

Each patient will be represented with a mark on the graphic displaying their change in BMI Z score and corresponding change in hepatic fat.
Each symbol will be colour coded to represent the patient's age in years.
Y-axis = change in hepatic fat.
X-axis = change in BMI Z score.

Only NMR scan assessments are included.
Source: Listing 16.2.12.1, Table 14.3.8.1.1, Table 14.1.2.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.
Repeat for Week 104

Figure 14.3.8.2 Scatter plot of change in hepatic fat with change in BMI Z score up to week 56 and 104, by subject MTD
(Safety Analysis Set)

Week 56
Overall – All subjects

Each patient will be represented with a mark on the graphic displaying their change in BMI Z score and corresponding change in hepatic fat.
Each symbol will be colour coded to represent the patient's MTD they achieved.
Y-axis = change in hepatic fat.
X-axis = change in BMI Z score.

Only NMR scan assessments are included.
Source: Listing 16.2.12.1, Table 14.3.8.1.1, Table 14.1.2.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall – All subjects aged 5 to 10 years; Overall – All subjects aged 11 to 17 years.***
Repeat for week 104

Figure 14.3.8.3 Scatter plot of change in hepatic fat up to week 56 and 104 and time fom Lomitapide administration, by subject age
(Safety Analysis Set)

Week 56
Overall - All subjects

Each patient will be represented with a mark on the graphic displaying their time from lomitapide administration and corresponding change in hepatic fat up to week 56.
Each symbol will be colour coded to represent the patient's age in years.
Y-axis = change in hepatic fat.
X-axis = time from lomitapide administration.

Only NMR scan assessments are included.
Source: Listing 16.2.12.1, Table 14.3.8.1.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.
Repeat for week 104

Figure 14.3.8.4 Scatter plot of change in hepatic fat up to week 56 and 104 and time from Lomitapide administration, by subject MTD
(Safety Analysis Set)

Week 56
Overall - All subjects

Each patient will be represented with a mark on the graphic displaying their time from lomitapide administration and corresponding change in hepatic fat up to week 56.
Each symbol will be colour coded to represent the patient's MTD they achieved.
Y-axis = change in hepatic fat.
X-axis = time from lomitapide administration.

Only NMR scan assessments are included.
Source: Listing 16.2.12.1, Table 14.3.8.1.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.
Repeat for Week 104

Figure 14.3.8.5 Cumulative distribution function (CDF) plot of percentage change in hepatic fat over time
(Safety Analysis Set)

Analysis Study Week XX

x-axis: Percentage change from baseline in hepatic fat.
y-axis: cumulative percentage.
The cumulative distribution function for percentage change in hepatic fat will be plotted with a reference line at 5, 10, 15 and 20%.
Use 3 different coloured lines to represent each of the 2 age groups and overall.

Only NMR scan assessments are included.
Source: Listing 16.2.12.1, Table 14.3.8.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Include reference line at 5, 10, 15 and 20%.***
Repeat for each analysis study week.

Figure 14.3.8.6 Scatter plot of ALT vs hepatic fat over time
(Safety Analysis Set)

Imaging technology: NMR
All subjects

x-axis: Hepatic fat
y-axis: Observed ALT (in x ULN)
Scatter plot of ALT vs hepatic fat will be plotted at baseline, Week 24, Week 56 and Week 104/EOT.
Use different colour to represent each age group. Use different symbols for each time point.
Add Pearson's correlation coefficient (NMR) or Spearman's Rank correlation coefficient (Ultrasound)

Source: Listing 16.2.8.3, Listing 16.2.12.1, Table 14.3.8.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each age group and for ultrasound scan
Present ALT as x times ULN.

Figure 14.3.8.7 Scatter plot of AST vs hepatic fat over time
(Safety Analysis Set)

Imaging technology: NMR
All subjects

Repeat of Figure 14.3.8.6

Source: Listing 16.2.8.3, Listing 16.2.12.1, Table 14.3.8.3.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for each age group and for ultrasound scan
Present AST as x times ULN.

Figure 14.3.9.1 Waterfall plot of change in BMI Z score at Week 24, 56 and 104, by patient age
(Safety Analysis Set)

Week XX
Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change in BMI Z score (baseline to week 24, 56 or 104).
Order the vertical bars in decreasing order of size of change in BMI Z score.
For each patient bar, the colour of the bar will represent the age (years) of the patient.
Y-axis = change in BMI Z score.
X-axis = subject.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.
Present for Weeks 24, 56 and 104.

Figure 14.3.9.2 Waterfall plot of change in BMI Z score at Week 24, 56 and 104, by patient sex
(Safety Analysis Set)

Week XX
Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change in BMI Z score (baseline to week 24, 56 or 104).
Order the vertical bars in decreasing order of size of change in BMI Z score.
For each patient bar, the colour of the bar will represent the age (years) of the patient.
Y-axis = change in BMI Z score.
X-axis = subject.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.
Present for Week 24, 56 and 104.

Figure 14.3.9.3 Spaghetti plot of BMI Z score over time, by patient age and sex
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding BMI Z score will be plotted.
A separate line will be generated for each patient to show their individual BMI Z score profile over time.
Each line will be colour co-ordinated by the patient's age.
The symbol used for the patient to generate their profile spaghetti line will represent the patient's sex.
Y-axis = BMI Z score value.
X-axis = time (weeks).

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.9.4 Spaghetti plot of BMI absolute value over time, by patient age and sex
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding BMI absolute value will be plotted.
A separate line will be generated for each patient to show their individual BMI absolute value profile over time.
Each line will be colour co-ordinated by the patient's age.
The symbol used for the patient to generate their profile spaghetti line will represent the patient's sex.
Y-axis = BMI absolute value.
X-axis = time (weeks).

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.9.5 Spaghetti plot of weight absolute value over time, by patient age and sex
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding weight absolute value will be plotted.
A separate line will be generated for each patient to show their individual weight absolute value profile over time.
Each line will be colour co-ordinated by the patient's age.
The symbol used for the patient to generate their profile spaghetti line will represent the patient's sex.
Y-axis = weight absolute value.
X-axis = time (weeks).

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.9.6 Spaghetti plot of sexual maturation development over time
(Full Analysis Set)

Age Group = 5 to 10 years; Sex = Male/Female

x-axis: time (weeks) relative to baseline.
y-axis: tanner staging category (1 to 5).
Separate line for each subject plotting tanner stage category over time.
Generate 4 different plots for each age group and sex.

Source: Listing 16.2.13.2, Table 14.3.9.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Figure 14.3.9.7 Spaghetti plot of growth development over time
(Full Analysis Set)

Age Group = 5 to 10 years; Sex = Male/Female

x-axis: time (weeks) relative to baseline.
y-axis: BMI percentile (scale 1 to 100).
Separate line for each subject plotting BMI percentile over time.
Generate 4 different plots for each age group and sex.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Figure 14.3.9.8 Waterfall plot of change in BMI Z-score at Week 24, 56 and 104, by baseline BMI for Age Z-score
(Safety Analysis Set)

Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change in BMI Z score (baseline to week 24, 56 or 104).
Order the vertical bars in decreasing order of size of change in BMI Z-score.
For each patient bar, the colour of the bar will represent the baseline BMI for Age Z-score of the patient.
Y-axis = change in BMI Z-score.
X-axis = subject.

BMI for Age Z-score cut-points are lower than -2.0 (wasted), between -2.0 and 1.0 (normal), between 1.0 and 2.0 (risk of overweight), between 2.0 and 3.0 (overweight) and more than 3.0 (obese).
Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Group baseline BMI for Age Z score in the following categories: <-2.0 (wasted); >= -2.0 and <= 1.0 (normal); > 1.0 and <= 2.0 (risk of overweight); > 2.0 and <= 3.0 (overweight); > 3.0 (obese).
Present for weeks 24, 56 and 104.

Figure 14.3.9.9 Spaghetti plot of BMI Z-score over time, by baseline BMI for Age Z-score
(Safety Analysis Set)

Overall - All subjects

For each patient, at each time point the corresponding BMI Z-score will be plotted.
A separate line will be generated for each patient to show their individual BMI Z-score profile over time.
Each line will be colour co-ordinated by the patient's baseline BMI for Age Z-score (<-2, -2 <= x <= 1, 1 < x <= 2, 2 < x <= 3 and > 3).
The symbol used for the patient to generate their profile spaghetti line will represent the patient's sex.
Y-axis = BMI Z-score value.
X-axis = time (weeks).

BMI for Age Z-score cut-points are lower than -2.0 (wasted), between -2.0 and 1.0 (normal), between 1.0 and 2.0 (risk of overweight), between 2.0 and 3.0 (overweight) and more than 3.0 (obese).
Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: ***Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.***
Group baseline BMI for Age Z-score in the following categories: <-2.0 (wasted); >= -2.0 and <= 1.0 (normal); > 1.0 and <= 2.0 (risk of overweight); > 2.0 and <= 3.0 (overweight); > 3.0 (obese).

Figure 14.3.9.10 Individual spaghetti plot of weight and Z scores over time
(Safety Analysis Set)

Subject XXX

For each patient, at each time point the corresponding Weight and Z scores will be plotted.
There will be one Y axis for Z-scores and a second Y-axis for absolute weight.
A separate line will be generated for each parameter over time.

Y-axis = Height, Weight and BMI Z scores values.
Y2-axis = absolute weight.
X-axis = time (weeks).

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

Figure 14.3.9.11 Waterfall plot of change from baseline to maximum weight loss
(Safety Analysis Set)

Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change form basleline to the minimum post-baseline weight (baseline to the respective week, as applicable).
Order the vertical bars in decreasing order of size of change in weight.
For each patient bar, the colour of the bar will represent the sex of the patient.
Y-axis = change in BMI.
X-axis = subject.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.

Figure 14.3.9.12 Waterfall plot of change from baseline to minimum BMI z-score change
(Safety Analysis Set)

Overall - All subjects

Each patient will be represented by a vertical bar, where the length of the bar represents the change form baseline to the minimum BMI z-score change (baseline to the respective week, as applicable).
Order the vertical bars in decreasing order of size of change in z-score.
For each patient bar, the colour of the bar will represent the sex of the patient.
Y-axis = Change from baseline to minimum BMI Z-score.
X-axis = subject.

Source: Listing 16.2.9.1, Table 14.3.5.1

PROGRAM NAME:F_XXX_01.SAS DATE OF RUN:XXXXXXXXX TIME OF RUN: XX:XX:XX DATE OF EXTRACTION:XXXXXXXXX

NOTES: Repeat for: Overall - All subjects aged 5 to 10 years; Overall - All subjects aged 11 to 17 years.