ClinicalTrials.gov Protocol and Results Registration System (PRS) Receipt
Release Date: 10/27/2014

ClinicalTrials.gov ID: NCT00607373

Study Identification

Unique Protocol ID: 301012CS5

Brief Title: Study to Assess the Safety and Efficacy of ISIS 301012 (Mipomersen) in Homozygous Familial Hypercholesterolemia (RADICHOL 1)

Official Title: A Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of Mipomersen as Add-on Therapy in Homozygous Familial Hypercholesterolemia Subjects

Secondary IDs: 2005-003449-15 [EudraCT Number]

Study Status

Record Verification: October 2014

Overall Status: Completed

Study Start: July 2007

Primary Completion: March 2009 [Actual]

Study Completion: March 2009 [Actual]

Sponsor/Collaborators

Sponsor: Genzyme, a Sanofi Company

Responsible Party: Sponsor

Collaborators: Isis Pharmaceuticals

Oversight

FDA Regulated?: Yes

IND/IDE Protocol?:	Yes
IND/IDE Information:	Grantor: CDER IND/IDE Number: 70,969 Serial Number: SN0026 Has Expanded Access? No
Review Board:	Approval Status: Approved Approval Number: 07/06/2007 Board Name: Schulman Associates IRB Board Affiliation: Schulman Associates Phone: 513-761-4100 Email: coordinatorsTeam@sairb.com
Data Monitoring?:	Yes
Oversight Authorities:	United States: Food and Drug Administration Canada: Health Canada United Kingdom: Medicines and Healthcare Products Regulatory Agency South Africa: Medicines Control Council Singapore: Health Sciences Authority Taiwan: Department of Health Brazil: National Health Surveillance Agency

Study Description

Brief Summary: The purpose of this study is to evaluate the safety and efficacy of mipomersen (ISIS 301012) in subjects with homozygous familial hypercholesterolemia on lipid-lowering therapy. This study consisted of a 26-week treatment period and a 24-week post-treatment follow-up period. Following treatment and Week 28 evaluations, participants could elect to enroll in an open-label extension study (301012-CS6; NCT00694109). Participants who were not eligible or elected not to enroll in the open-label extension study or who discontinued during the 28-week treatment period were followed in this study for 24 weeks from administration of the last dose of study drug.

Detailed Description: Familial hypercholesterolemia (FH) is an autosomal dominant genetic disorder of lipoprotein metabolism characterized by markedly elevated low density lipoprotein (LDL), premature onset of atherosclerosis and development of xanthomata. Patients with homozygous familial hypercholesterolemia (HoFH) have a severe disease that presents in childhood with total cholesterol typically in the 650 to 1000 mg/dL range.

This was a randomized, double-blind, placebo-controlled study, which consisted of a 4-week screening period, 26 weeks of treatment, and a 24-week post- treatment follow-up period (with the exception of patients who enrolled in the open-label extension study, Study 301012-CS6; NCT00694109). Eligible patients were randomized in a 2:1 ratio to receive 200 mg mipomersen or matching volume placebo subcutaneous (SC) injections weekly. Patients who weighed <50 kg received a lower dose of 160 mg mipomersen or matching volume of placebo SC injections weekly. Patients were to have been on a stable (>=12 weeks) regimen of allowed lipid-lowering therapies at screening, and were required to remain on the same dose and regimen throughout the study.

Patients returned to the study center for clinical evaluation every other week during the first 4 weeks of treatment, once every 4 to 5 weeks for the remainder of the treatment period, and monthly during the post-treatment evaluation (follow-up) period. The primary

endpoint assessment was at Week 28. Following treatment and Week 28 evaluations, eligible patients who tolerated the study drug could elect to enroll in the open-label extension study (Study 301012-CS6; NCT00694109). Patients who did not participate in the open-label extension study were required to return to the study center for clinical evaluation at least twice during the post-treatment follow-up period, including an end-of-study termination visit at the end of this 24-week period.

Conditions

Conditions:	Lipid Metabolism, Inborn Errors Hypercholesterolemia, Autosomal Dominant Hyperlipidemias Metabolic Diseases Hyperlipoproteinemia Type II Metabolism, Inborn Errors Genetic Diseases, Inborn Infant, Newborn, Diseases Metabolic Disorder Congenital Abnormalities Hypercholesterolemia Hyperlipoproteinemias Dyslipidemias Lipid Metabolism Disorders
Keywords:	Apolipoprotein B Homozygous Familial Hypercholesterolemia LDL-receptor gene

Study Design

Study Type:	Interventional
Primary Purpose:	Treatment
Study Phase:	Phase 3
Intervention Model:	Parallel Assignment
Number of Arms:	2
Masking:	Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor)
Allocation:	Randomized
Endpoint Classification:	Safety/Efficacy Study
Enrollment:	51 [Actual]

Arms and Interventions

Arms	Assigned Interventions
Experimental: Mipomersen Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.	Drug: mipomersen 200 mg mipomersen administered once a week for 26 weeks as a 1 mL subcutaneous injection. Subjects weighing less than 50 kg received a lower dose of 160 mg (0.8mL) mipomersen. Other Names: • ISIS 301012 • mipomersen sodium • Kynamro™
Placebo Comparator: Placebo Participants received placebo as a subcutaneous injection once a week for 26 weeks.	Drug: Placebo 1 mL subcutaneous injection once a week for 26 weeks. Subjects weighing less than 50 kg received 0.8 mL subcutaneous injection. Other Names: • placebo

Outcome Measures

[See Results Section.]

Eligibility

Minimum Age: 12 Years

Maximum Age:

Gender: Both

Accepts Healthy Volunteers?: No

Criteria: Inclusion Criteria:

- Diagnosis of Homozygous Familial Hypercholesterolemia (HoFH)
- Stable lipid-lowering therapy for 12 weeks
- Stable weight for 6 weeks
- Stable low fat diet for 8 weeks

Exclusion Criteria:

- Significant health problems in the recent past including heart attack, stroke, blood disorders, cancer, or digestive problems

Contacts/Locations

Study Officials: Medical Monitor
Genzyme Corporation

Locations: Canada, Quebec
Ste Foy, Quebec, Canada, G1V 4G2

Chicoutimi, Quebec, Canada, G7H 5H6

United States, Ohio
Cincinnati, Ohio, United States, 45212

United States, North Carolina
Charlotte, North Carolina, United States, 28204

South Africa
Observatory, South Africa, 7925

United Kingdom
London, United Kingdom, WC1N 3BG

Taiwan
Taipei, Taiwan, 11217

Singapore
Mistri Wing, Singapore, 168752

South Africa
Parktown, South Africa, 2193

Brazil
Sao Paulo, SP, Brazil, 05403-000

References

Citations:

Links:

Participant Flow

Pre-Assignment Details	Sixty-one patients were screened and fifty-one randomized. Eligible patients were randomized in a 2:1 ratio to receive 200 mg mipomersen or matching volume placebo subcutaneous (SC) injections weekly.

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Treatment Period

	Placebo	Mipomersen
Started	17	34
Completed	17 [1]	28 [2]
Not Completed	0	6
Adverse Event	0	4
Physician Decision	0	1
Withdrawal by Subject	0	1

[1] 16 enrolled in open-label extension study NCT00694109
[2] 23 enrolled in open-label extension study NCT00694109

Follow-up Period

	Placebo	Mipomersen
Started	1 [1]	11 [1]
Completed	0	6
Not Completed	1	5
Not specified	0	1
Protocol Violation	0	1
Withdrawal by Subject	1	3

[1] Participants enrolled in the open-label extension (NCT00694109) or continued in the follow-up period

Baseline Characteristics

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Baseline Measures

	Placebo	Mipomersen	Total
Number of Participants	17	34	51
Age, Continuous [units: years] Mean (Standard Deviation)	33.0 (14.1)	30.4 (11.5)	31.3 (12.4)
Gender, Male/Female [units: participants]			
Female	10	19	29
Male	7	15	22
Race/Ethnicity, Customized [units: participants]			
Hispanic or Latino	1	5	6
Not Hispanic or Latino	16	29	45
Race/Ethnicity, Customized [units: participants]			
White	13	25	38
Asian	3	8	11
Black	1	1	2
Body Mass Index [units: kg/m^2] Mean (Standard Deviation)	26.32 (4.41)	25.97 (5.81)	26.08 (5.34)
Waist/hip ratio [units: ratio] Mean (Standard Deviation)	0.83 (0.07)	0.85 (0.06)	0.84 (0.07)

	Placebo	Mipomersen	Total
Metabolic syndrome [1] [units: participants]			
No	16	27	43
Yes	1	7	8
Tobacco Use [units: participants]			
Current	3	7	10
Non-current	3	4	7
Never	11	23	34
Alcohol Use [units: participants]			
Current	6	14	20
Non-current	3	3	6
Never	8	17	25
Fasting serum insulin [units: microIU/mL] Mean (Standard Deviation)	9.72 (5.98)	11.54 (14.78)	10.93 (12.51)
Fasting hemoglobin A1c [units: percentage of total hemoglobin] Mean (Standard Deviation)	5.47 (0.22)	5.34 (0.37)	5.38 (0.33)
Weight [2] [units: participants]			
<50 kg	2	4	6
>=50 kg	15	30	45

[1] Yes if 3 or more risk factors are present:

1) Abdominal obesity 2) Triglycerides >=150 mg/dl * 3) High density lipoprotein cholesterol (men <40 mg/dl) (women <50 mg/dl) * 4) Systolic blood pressure >=130 or diastolic >=85 mmHg * 5) Fasting glucose >=100 mg/dl *

* = or on medication for condition

[2] Participants who weighed <50 kg received the lower dose of 160 mg mipomersen or matching placebo. All other patients received a dose of 200 mg or matching placebo.

Outcome Measures

1. Primary Outcome Measure:

Measure Title	Percent Change From Baseline in Low-density Lipoprotein Cholesterol (LDL-C) at Primary Efficacy Time Point
Measure Description	LDL-C was measured in mg/dL. Samples were taken following an overnight fast. For patients with triglycerides <400 mg/dL, LDL-C was obtained using Friedewald's calculation; and for patients with triglycerides >=400 mg/dL, LDL-C was directly measured by the central laboratory using ultracentrifugation. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. If the Study Day 1 and screening LDL-C values were >12% different (relative to the maximum value), then the screening value was not used, because the Study Day 1 value represents the best estimate of the patient's condition at the beginning of study drug administration. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set (FAS). The FAS, which represents the practically-feasible intent-to-treat (ITT) population as delineated in ICH Guideline E9, consists of treated participants with a valid baseline and at least one post-baseline LDL-C measure.

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percent Change From Baseline in Low-density Lipoprotein Cholesterol (LDL-C) at Primary Efficacy Time Point [units: percentage of baseline] Mean (Standard Deviation)	-3.31 (17.06)	-24.66 (19.85)

Statistical Analysis 1 for Percent Change From Baseline in Low-density Lipoprotein Cholesterol (LDL-C) at Primary Efficacy Time Point

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	Based upon prior clinical study experience with mipomersen, it was estimated that the standard deviation of the percent change in LDL-C is approximately 22%. With 15 patients in the control group and 30 patients in the mipomersen-treated group, this study would have at least 80% power to detect a 20 percentage point difference between the 2 treatment groups. Fifty-one patients were enrolled to allow for patient withdrawals and potential exclusions from analysis sets.
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.001
	Comments	Statistical significance was concluded if p≤0.05
	Method	t-test, 2 sided
	Comments	[Not specified]

2. Primary Outcome Measure:

Measure Title	LDL-C at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
LDL-C at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	400.2 (141.5)	438.9 (138.6)
PET	388.2 (150.5)	326.2 (121.3)

3. Secondary Outcome Measure:

Measure Title	Percent Change From Baseline in Apolipoprotein B (Apo-B) at Primary Efficacy Time Point
Measure Description	Apo-B was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percent Change From Baseline in Apolipoprotein B (Apo-B) at Primary Efficacy Time Point [units: percentage of baseline]	-2.54 (12.56)	-26.77 (17.04)

	Placebo	Mipomersen
Mean (Standard Deviation)		

Statistical Analysis 1 for Percent Change From Baseline in Apolipoprotein B (Apo-B) at Primary Efficacy Time Point

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.001
	Comments	Inflation of type 1 error was controlled by specifying a small number of secondary parameters and a sequential inferential approach in which inferential conclusions about each successive parameter required statistical significance of the prior one.
	Method	t-test, 2 sided
	Comments	[Not specified]

4. Secondary Outcome Measure:

Measure Title	Apo-B at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28)
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Apo-B at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	259.2 (84.4)	283.1 (78.4)
PET	252.6 (85.0)	205.4 (70.0)

5. Secondary Outcome Measure:

Measure Title	Percentage Change From Baseline in Total Cholesterol at Primary Efficacy Time Point (PET)
Measure Description	Total cholesterol was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percentage Change From Baseline in Total Cholesterol at Primary Efficacy Time Point (PET) [units: percentage of baseline]	-1.98 (14.82)	-21.20 (17.69)

	Placebo	Mipomersen
Mean (Standard Deviation)		

Statistical Analysis 1 for Percentage Change From Baseline in Total Cholesterol at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.001
	Comments	Inferential conclusions about this parameter require statistical significance of the previous secondary outcome measure (i.e., percent change from baseline in Apo B at PET).
	Method	t-test, 2 sided
	Comments	[Not specified]

6. Secondary Outcome Measure:

Measure Title	Total Cholesterol at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Total Cholesterol at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	460.5 (132.0)	502.4 (144.5)
PET	452.1 (144.6)	389.7 (125.3)

7. Secondary Outcome Measure:

Measure Title	Percentage Change From Baseline in Non-High-Density Lipoprotein Cholesterol (Non-HDL-C) at Primary Efficacy Time Point (PET)
Measure Description	Non-HDL-C was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34

	Placebo	Mipomersen
Percentage Change From Baseline in Non-High-Density Lipoprotein Cholesterol (Non-HDL-C) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Mean (Standard Deviation)	-2.90 (16.32)	-24.50 (19.17)

Statistical Analysis 1 for Percentage Change From Baseline in Non-High-Density Lipoprotein Cholesterol (Non-HDL-C) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.001
	Comments	Inferential conclusions about this parameter require statistical significance of the previous secondary outcome measure (i.e., percent change from baseline in total cholesterol at PET).
	Method	t-test, 2 sided
	Comments	[Not specified]

8. Secondary Outcome Measure:

Measure Title	Non-HDL-C at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Non-HDL-C at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	418.9 (144.5)	464.3 (145.4)
PET	409.1 (156.6)	345.8 (126.6)

9. Other Pre-specified Outcome Measure:

Measure Title	Percentage Change From Baseline in Triglycerides at Primary Efficacy Time Point (PET)
Measure Description	Triglycerides were measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percentage Change From Baseline in Triglycerides at Primary Efficacy Time Point (PET) [units: percentage of baseline] Median (Inter-Quartile Range)	0.9 (-25.0 to 29.5)	-17.5 (-36.0 to -4.8)

Statistical Analysis 1 for Percentage Change From Baseline in Triglycerides at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	0.013
	Comments	Statistical significance was concluded if p ≤0.05
	Method	Other [Wilcoxon rank sum test]
	Comments	[Not specified]

10. Other Pre-specified Outcome Measure:

Measure Title	Triglycerides at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Triglycerides at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Median (Inter-Quartile Range)		
Baseline	92 (80 to 105)	91 (73 to 141)
PET	85 (65 to 117)	76 (52 to 116)

11. Other Pre-specified Outcome Measure:

Measure Title	Percentage Change From Baseline in Lipoprotein(a) at Primary Efficacy Time Point (PET)
Measure Description	Lipoprotein(a) was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percentage Change From Baseline in Lipoprotein(a) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Mean (Standard Deviation)	-7.87 (21.87)	-31.10 (23.02)

Statistical Analysis 1 for Percentage Change From Baseline in Lipoprotein(a) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	0.001
	Comments	Statistical significance was concluded if p ≤0.05
	Method	t-test, 2 sided
	Comments	[Not specified]

12. Other Pre-specified Outcome Measure:

Measure Title	Lipoprotein(a) at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Lipoprotein(a) at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	66.3 (53.1)	64.3 (41.0)
PET	61.6 (52.6)	43.8 (32.1)

13. Other Pre-specified Outcome Measure:

Measure Title	Percentage Change From Baseline in Very-Low-Density Lipoprotein Cholesterol (VLDL-C) at Primary Efficacy Time Point (PET)
Measure Description	VLDL-C was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percentage Change From Baseline in Very-Low-Density Lipoprotein Cholesterol (VLDL-C) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Median (Inter-Quartile Range)	2.3 (-25.0 to 28.6)	-17.3 (-37.1 to -3.0)

Statistical Analysis 1 for Percentage Change From Baseline in Very-Low-Density Lipoprotein Cholesterol (VLDL-C) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	0.009
	Comments	Statistical significance was concluded if p ≤0.05
	Method	Other [Wilcoxon rank sum test]
	Comments	[Not specified]

14. Other Pre-specified Outcome Measure:

Measure Title	VLDL-C at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
VLDL-C at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Median (Inter-Quartile Range)		
Baseline	18 (16 to 21)	18 (15 to 28)
PET	17 (13 to 23)	15 (10 to 23)

15. Other Pre-specified Outcome Measure:

Measure Title	Change From Baseline in Ratio of Low-density Lipoprotein Cholesterol (LDL-C) to High-density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET)
Measure Description	LDL-C and HDL-C were measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Change From Baseline in Ratio of Low-density Lipoprotein Cholesterol (LDL-C) to High-density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Mean (Standard Deviation)	-6.22 (18.81)	-34.32 (21.00)

Statistical Analysis 1 for Change From Baseline in Ratio of Low-density Lipoprotein Cholesterol (LDL-C) to High-density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.001
	Comments	Statistical significance was concluded if p ≤0.05
	Method	t-test, 2 sided
	Comments	[Not specified]

16. Other Pre-specified Outcome Measure:

Measure Title	Ratio of LDL-C to HDL-C at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Ratio of LDL-C to HDL-C at Baseline and the Primary Efficacy Time Point (PET) [units: ratio] Mean (Standard Deviation)		
Baseline	12.14 (7.675)	13.02 (6.115)
PET	11.37 (7.095)	8.13 (3.921)

17. Other Pre-specified Outcome Measure:

Measure Title	Percent Change From Baseline in Apolipoprotein A1 (Apo-A1) at Primary Efficacy Time Point (PET)
Measure Description	Apo-A1 was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percent Change From Baseline in Apolipoprotein A1 (Apo-A1) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Mean (Standard Deviation)	5.35 (10.63)	9.27 (17.59)

Statistical Analysis 1 for Percent Change From Baseline in Apolipoprotein A1 (Apo-A1) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	0.328
	Comments	Statistical significance was concluded if p ≤0.05
	Method	t-test, 2 sided
	Comments	[Not specified]

18. Other Pre-specified Outcome Measure:

Measure Title	Apo-A1 at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Apo-A1 at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Mean (Standard Deviation)		
Baseline	118.6 (33.0)	111.5 (27.9)
PET	124.5 (34.9)	118.8 (20.5)

19. Other Pre-specified Outcome Measure:

Measure Title	Percentage Change From Baseline in High-Density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET)
Measure Description	HDL-C was measured in mg/dL. Samples were taken following an overnight fast. Baseline was defined as the average of the screening and Study Day 1 (pre-treatment) assessments. An assessment was not included in this calculation if it was associated with a non-fasting blood draw or was drawn more than 4 weeks prior to Study Day 1. The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
Percentage Change From Baseline in High-Density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET) [units: percentage of baseline] Median (Inter-Quartile Range)	4.1 (-2.0 to 13.2)	14.8 (3.3 to 27.0)

Statistical Analysis 1 for Percentage Change From Baseline in High-Density Lipoprotein Cholesterol (HDL-C) at Primary Efficacy Time Point (PET)

Statistical Analysis Overview	Comparison Groups	Placebo, Mipomersen
	Comments	[Not specified]
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	0.035
	Comments	Statistical significance was concluded if p ≤0.05
	Method	Other [Wilcoxon rank sum test]
	Comments	[Not specified]

20. Other Pre-specified Outcome Measure:

Measure Title	HDL-C at Baseline and the Primary Efficacy Time Point (PET)
Measure Description	The PET was the post-baseline visit, for which LDL-C was assessed, closest to 14 days after the last dose of study drug.
Time Frame	Baseline (the average of the screening and Day 1 pre-treatment assessments) and the Primary Efficacy Time point (PET) up to week 28
Safety Issue?	No

Analysis Population Description

Full analysis set

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Measured Values

	Placebo	Mipomersen
Number of Participants Analyzed	17	34
HDL-C at Baseline and the Primary Efficacy Time Point (PET) [units: mg/dL] Median (Inter-Quartile Range)		
Baseline	38 (27 to 49)	35 (32 to 44)
PET	43 (28 to 53)	43 (37 to 48)

Reported Adverse Events

Time Frame	Day 1 to week 28. On-treatment AEs started on/after the first study drug dose and on/before the end of the treatment period. The treatment period was the time study drug was administered until the later of the PET or 14 days after last study drug dose.
Additional Description	The Safety Set includes all randomized patients who receive at least 1 injection of the study treatment. In the event a single participant has experienced both a serious and a non-serious form of the same adverse event term, the individual has been included in the numerator ("number of affected participants") of both adverse event tables.

Reporting Groups

	Description
Placebo	Participants received placebo as a subcutaneous injection once a week for 26 weeks
Mipomersen	Participants received mipomersen 200 mg as a subcutaneous injection once a week for 26 weeks.

Serious Adverse Events

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Total	1/17 (5.88%)	2/34 (5.88%)
Cardiac disorders		
Acute coronary syndrome [A] †	0/17 (0%)	1/34 (2.94%)
Injury, poisoning and procedural complications		
Ankle fracture [A] †	0/17 (0%)	1/34 (2.94%)
Renal and urinary disorders		
Nephrolithiasis [A] †	1/17 (5.88%)	0/34 (0%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA 11.1

Other Adverse Events

Frequency Threshold Above Which Other Adverse Events are Reported: 0%

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Total	13/17 (76.47%)	30/34 (88.24%)
Blood and lymphatic system disorders		
Anaemia [A] †	1/17 (5.88%)	2/34 (5.88%)
Cardiac disorders		
Angina pectoris [A] †	0/17 (0%)	1/34 (2.94%)
Aortic valve disease [A] †	0/17 (0%)	1/34 (2.94%)
Cardiac discomfort [A] †	0/17 (0%)	1/34 (2.94%)
Palpitations [A] †	0/17 (0%)	1/34 (2.94%)
Ear and labyrinth disorders		
Ear pain [A] †	0/17 (0%)	1/34 (2.94%)
Endocrine disorders		

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Hypothyroidism [A] †	1/17 (5.88%)	1/34 (2.94%)
Gastrointestinal disorders		
Abdominal pain [A] †	0/17 (0%)	2/34 (5.88%)
Abdominal pain upper [A] †	0/17 (0%)	1/34 (2.94%)
Constipation [A] †	1/17 (5.88%)	2/34 (5.88%)
Diarrhoea [A] †	0/17 (0%)	1/34 (2.94%)
Dry mouth [A] †	1/17 (5.88%)	0/34 (0%)
Dyspepsia [A] †	0/17 (0%)	1/34 (2.94%)
Gastrooesophageal reflux disease [A] †	0/17 (0%)	1/34 (2.94%)
Nausea [A] †	1/17 (5.88%)	6/34 (17.65%)
Toothache [A] †	0/17 (0%)	1/34 (2.94%)
Vomiting [A] †	1/17 (5.88%)	0/34 (0%)
General disorders		
Asthenia [A] †	0/17 (0%)	1/34 (2.94%)
Chest pain [A] †	0/17 (0%)	4/34 (11.76%)
Chills [A] †	0/17 (0%)	1/34 (2.94%)
Fatigue [A] †	0/17 (0%)	1/34 (2.94%)
Influenza like illness [A] †	0/17 (0%)	3/34 (8.82%)
Injection site anaesthesia [A] †	0/17 (0%)	1/34 (2.94%)
Injection site discolouration [A] †	0/17 (0%)	10/34 (29.41%)
Injection site discomfort [A] †	0/17 (0%)	3/34 (8.82%)
Injection site erythema [A] †	1/17 (5.88%)	19/34 (55.88%)

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Injection site haematoma [A] †	2/17 (11.76%)	12/34 (35.29%)
Injection site haemorrhage [A] †	0/17 (0%)	1/34 (2.94%)
Injection site induration [A] †	0/17 (0%)	2/34 (5.88%)
Injection site inflammation [A] †	0/17 (0%)	1/34 (2.94%)
Injection site irritation [A] †	1/17 (5.88%)	1/34 (2.94%)
Injection site macule [A] †	0/17 (0%)	5/34 (14.71%)
Injection site oedema [A] †	0/17 (0%)	2/34 (5.88%)
Injection site pain [A] †	1/17 (5.88%)	12/34 (35.29%)
Injection site pallor [A] †	0/17 (0%)	2/34 (5.88%)
Injection site papule [A] †	0/17 (0%)	4/34 (11.76%)
Injection site paraesthesia [A] †	0/17 (0%)	1/34 (2.94%)
Injection site pruritus [A] †	1/17 (5.88%)	10/34 (29.41%)
Injection site rash [A] †	0/17 (0%)	2/34 (5.88%)
Injection site recall reaction [A] †	0/17 (0%)	1/34 (2.94%)
Injection site swelling [A] †	0/17 (0%)	4/34 (11.76%)
Injection site warmth [A] †	0/17 (0%)	1/34 (2.94%)
Oedema peripheral [A] †	0/17 (0%)	1/34 (2.94%)
Pyrexia [A] †	1/17 (5.88%)	3/34 (8.82%)
Hepatobiliary disorders		
Hyperbilirubinaemia [A] †	0/17 (0%)	1/34 (2.94%)
Infections and infestations		
Cystitis [A] †	1/17 (5.88%)	0/34 (0%)

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Ear infection [A] †	0/17 (0%)	1/34 (2.94%)
Fungal infection [A] †	1/17 (5.88%)	0/34 (0%)
Gastroenteritis [A] †	0/17 (0%)	1/34 (2.94%)
Influenza [A] †	2/17 (11.76%)	2/34 (5.88%)
Nasopharyngitis [A] †	1/17 (5.88%)	0/34 (0%)
Rhinitis [A] †	0/17 (0%)	1/34 (2.94%)
Tooth abscess [A] †	1/17 (5.88%)	0/34 (0%)
Upper respiratory tract infection [A] †	4/17 (23.53%)	1/34 (2.94%)
Urinary tract infection [A] †	2/17 (11.76%)	0/34 (0%)
Injury, poisoning and procedural complications		
Head injury [A] †	1/17 (5.88%)	0/34 (0%)
Procedural pain [A] †	0/17 (0%)	1/34 (2.94%)
Investigations		
Alanine aminotransferase increased [A] †	0/17 (0%)	5/34 (14.71%)
Aspartate aminotransferase increased [A] †	1/17 (5.88%)	4/34 (11.76%)
Blood creatine phosphokinase increased [A] †	0/17 (0%)	1/34 (2.94%)
Hepatic enzyme increased [A] †	1/17 (5.88%)	0/34 (0%)
Protein urine present [A] †	1/17 (5.88%)	0/34 (0%)
Red blood cell macrocytes present [A] †	1/17 (5.88%)	0/34 (0%)
Metabolism and nutrition disorders		
Anorexia [A] †	2/17 (11.76%)	0/34 (0%)
Hyperglycaemia [A] †	0/17 (0%)	1/34 (2.94%)
Musculoskeletal and connective tissue disorders		

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Arthralgia [A] †	1/17 (5.88%)	0/34 (0%)
Back pain [A] †	0/17 (0%)	1/34 (2.94%)
Intervertebral disc protrusion [A] †	1/17 (5.88%)	0/34 (0%)
Musculoskeletal pain [A] †	0/17 (0%)	1/34 (2.94%)
Pain in extremity [A] †	0/17 (0%)	2/34 (5.88%)
Nervous system disorders		
Dizziness [A] †	0/17 (0%)	2/34 (5.88%)
Facial palsy [A] †	0/17 (0%)	1/34 (2.94%)
Headache [A] †	2/17 (11.76%)	5/34 (14.71%)
Neuralgia [A] †	0/17 (0%)	1/34 (2.94%)
Somnolence [A] †	0/17 (0%)	1/34 (2.94%)
Psychiatric disorders		
Anxiety [A] †	0/17 (0%)	1/34 (2.94%)
Stress [A] †	0/17 (0%)	1/34 (2.94%)
Renal and urinary disorders		
Proteinuria [A] †	0/17 (0%)	1/34 (2.94%)
Reproductive system and breast disorders		
Amenorrhoea [A] †	0/17 (0%)	1/34 (2.94%)
Galactorrhoea [A] †	0/17 (0%)	1/34 (2.94%)
Menorrhagia [A] †	1/17 (5.88%)	1/34 (2.94%)
Respiratory, thoracic and mediastinal disorders		
Asthma [A] †	1/17 (5.88%)	0/34 (0%)
Cough [A] †	1/17 (5.88%)	0/34 (0%)

	Placebo	Mipomersen
	Affected/At Risk (%)	Affected/At Risk (%)
Epistaxis [A] †	1/17 (5.88%)	0/34 (0%)
Oropharyngeal pain [A] †	1/17 (5.88%)	0/34 (0%)
Painful respiration [A] †	0/17 (0%)	1/34 (2.94%)
Productive cough [A] †	0/17 (0%)	1/34 (2.94%)
Upper respiratory tract congestion [A] †	0/17 (0%)	1/34 (2.94%)
Skin and subcutaneous tissue disorders		
Dermatitis allergic [A] †	0/17 (0%)	1/34 (2.94%)
Dry skin [A] †	1/17 (5.88%)	0/34 (0%)
Eczema [A] †	1/17 (5.88%)	0/34 (0%)
Pruritus generalised [A] †	1/17 (5.88%)	0/34 (0%)
Rash [A] †	0/17 (0%)	1/34 (2.94%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA 11.1

Limitations and Caveats

[Not specified]

More Information

Certain Agreements:

Principal Investigators are NOT employed by the organization sponsoring the study.

There IS an agreement between the Principal Investigator and the Sponsor (or its agents) that restricts the PI's rights to discuss or publish trial results after the trial is completed.

The first publication for a multi-centre trial must address all centers. Institution will submit for review a proposed publication or presentation at least 60 days prior to submission date. Sponsor has the right to delay publication or presentation for not more than 6 months (contracts have variable timeframes) to address patent applications. Sponsor also has the right to demand in writing the deletion of confidential information, as long as such removal will not preclude publication.

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