



Clinical trial results:

A Single Arm, Prospective, Open-label, Multi-center Study to Evaluate Efficacy and Safety in Chinese Patients with Late Onset Pompe Disease with Alglucosidase Alfa Treatment

Summary

| | |
|--------------------------|----------------|
| EudraCT number | 2024-000461-24 |
| Trial protocol | Outside EU/EEA |
| Global end of trial date | 25 July 2024 |

Results information

| | |
|--------------------------------|------------------|
| Result version number | v1 (current) |
| This version publication date | 01 February 2025 |
| First version publication date | 01 February 2025 |

Trial information

Trial identification

| | |
|-----------------------|------------------------|
| Sponsor protocol code | ALGMYL09010 / LPS15677 |
|-----------------------|------------------------|

Additional study identifiers

| | |
|------------------------------------|-----------------|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | NCT04676373 |
| WHO universal trial number (UTN) | U1111-1238-1267 |

Notes:

Sponsors

| | |
|------------------------------|--|
| Sponsor organisation name | Sanofi China Investment Co.,Ltd |
| Sponsor organisation address | Floor 7, No. 112, Jianguo Road, Chaoyang District, Beijing, China, |
| Public contact | Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.com |
| Scientific contact | Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.com |

Notes:

Paediatric regulatory details

| | |
|--|-----|
| Is trial part of an agreed paediatric investigation plan (PIP) | No |
| Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial? | No |
| Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial? | Yes |

Notes:

Results analysis stage

| | |
|--|------------------|
| Analysis stage | Final |
| Date of interim/final analysis | 11 November 2024 |
| Is this the analysis of the primary completion data? | No |
| Global end of trial reached? | Yes |
| Global end of trial date | 25 July 2024 |
| Was the trial ended prematurely? | No |

Notes:

General information about the trial

Main objective of the trial:

- To evaluate the effect of alglucosidase alfa (Myozyme) treatment on motor function (Six-minute walk test [6MWT] and lung function (predicted Forced vital capacity [FVC]) among Chinese late-onset pompe disease (LOPD) participants above 5 years old.
- To evaluate the safety of alglucosidase alfa (Myozyme) 20 milligram per kilogram (mg/kg), intravenous (IV) biweekly in Chinese LOPD participants above 3 years old.

Protection of trial subjects:

The study was conducted by investigators experienced in the treatment of pediatric participants. The parent(s) or guardian(s) as well as the children were fully informed of all pertinent aspects of the clinical trial as well as the possibility to discontinue at any time. In addition to the consent form for the parent(s)/guardian(s), as assent form in child-appropriate language was provided and explained to the child. Repeated invasive procedures were minimized. The number of blood samples as well as the amount of blood drawn were adjusted according to age and weight. A topical anesthesia may have been used to minimize distress and discomfort.

Background therapy: -

Evidence for comparator: -

| | |
|---|---------------|
| Actual start date of recruitment | 10 March 2021 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | No |

Notes:

Population of trial subjects

Subjects enrolled per country

| | |
|--------------------------------------|-----------|
| Country: Number of subjects enrolled | China: 41 |
| Worldwide total number of subjects | 41 |
| EEA total number of subjects | 0 |

Notes:

Subjects enrolled per age group

| | |
|---|----|
| In utero | 0 |
| Preterm newborn - gestational age < 37 wk | 0 |
| Newborns (0-27 days) | 0 |
| Infants and toddlers (28 days-23 months) | 0 |
| Children (2-11 years) | 2 |
| Adolescents (12-17 years) | 14 |

| | |
|----------------------|----|
| Adults (18-64 years) | 25 |
| From 65 to 84 years | 0 |
| 85 years and over | 0 |

Subject disposition

Recruitment

Recruitment details:

The study was conducted at 10 centers in China. A total of 43 participants were screened from 10 March 2021 to 01 June 2023, of which 2 were screen failures. Screen failures were mainly due to not meeting the eligibility criteria.

Pre-assignment

Screening details:

A total of 41 participants with LOPD received alglucosidase alfa in the study.

Period 1

| | |
|------------------------------|--------------------------------|
| Period 1 title | Overall study (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Not applicable |
| Blinding used | Not blinded |

Arms

| | |
|------------------|--------------------|
| Arm title | Alglucosidase alfa |
|------------------|--------------------|

Arm description:

Participants received alglucosidase alfa at a dose of 20 mg/kg body weight every 2 weeks as an IV infusion for up to 52 weeks.

| | |
|--|----------------------|
| Arm type | Experimental |
| Investigational medicinal product name | Alglucosidase alfa |
| Investigational medicinal product code | |
| Other name | MYOZYME® |
| Pharmaceutical forms | Powder for injection |
| Routes of administration | Intravenous use |

Dosage and administration details:

Participants received alglucosidase alfa at a dose of 20 mg/kg body weight every 2 weeks as an IV infusion using an infusion pump, over approximately 4 hours.

| Number of subjects in period 1 | Alglucosidase alfa |
|-------------------------------------|--------------------|
| Started | 41 |
| Completed | 36 |
| Not completed | 5 |
| Consent withdrawn by subject | 3 |
| Adverse event, non-fatal | 1 |
| Coronavirus Disease-2019 (COVID-19) | 1 |

Baseline characteristics

Reporting groups

| | |
|-----------------------|--------------------|
| Reporting group title | Alglucosidase alfa |
|-----------------------|--------------------|

Reporting group description:

Participants received alglucosidase alfa at a dose of 20 mg/kg body weight every 2 weeks as an IV infusion for up to 52 weeks.

| Reporting group values | Alglucosidase alfa | Total | |
|------------------------|--------------------|-------|--|
| Number of subjects | 41 | 41 | |
| Age Categorical | | | |
| Units: Subjects | | | |

| | | | |
|---|-----------|----|--|
| Age Continuous | | | |
| Units: years | | | |
| arithmetic mean | 24.2 | | |
| standard deviation | ± 11.17 | - | |
| Gender Categorical | | | |
| Units: Participants | | | |
| Female | 21 | 21 | |
| Male | 20 | 20 | |
| Percentage of Predicted Forced Vital Capacity (FVC) | | | |
| FVC is a measurement of pulmonary function which is defined as the volume of air that can forcibly be blown out after full inspiration. It was assessed using the spirometry system with the participant in upright seated position. Percent of predicted FVC = (actual FVC measurement)/(predicted value of FVC) x 100. | | | |
| Units: Percentage of Predicted FVC | | | |
| arithmetic mean | 49.777 | | |
| standard deviation | ± 14.992 | - | |
| Six-Minute Walk Test (6MWT) | | | |
| The 6MWT is a practical simple test that measures the distance that a participant can quickly walk on a flat, hard surface in a period of 6 minutes. It evaluates the global and integrated responses of all the systems involved during exercise, including the pulmonary and cardiovascular systems, systemic circulation, peripheral circulation, blood, neuromuscular units, and muscle metabolism. | | | |
| Units: Meters | | | |
| arithmetic mean | 390.282 | | |
| standard deviation | ± 101.928 | - | |

End points

End points reporting groups

| | |
|--|--------------------|
| Reporting group title | Alglucosidase alfa |
| Reporting group description: Participants received alglucosidase alfa at a dose of 20 mg/kg body weight every 2 weeks as an IV infusion for up to 52 weeks. | |

Primary: Change From Baseline in six-Minute Walk Test (6MWT) for Participants Greater Than or Equal to (\geq) 5-Year old at Month 12

| | |
|-----------------|--|
| End point title | Change From Baseline in six-Minute Walk Test (6MWT) for Participants Greater Than or Equal to (\geq) 5-Year old at Month 12 ^[1] |
|-----------------|--|

End point description:

The 6MWT is a practical simple test that requires a 100-ft hallway but no exercise equipment or advanced training for technicians. This test measures the distance that a participant can quickly walk on a flat, hard surface in a period of 6 minutes. It evaluates the global and integrated responses of all the systems involved during exercise, including the pulmonary and cardiovascular systems, systemic circulation, peripheral circulation, blood, neuromuscular units, and muscle metabolism. Baseline was defined as the last available value before the treatment. The primary endpoint is estimated based on last observation carried forward (LOCF) method. Intent-to-treat (ITT) population included all enrolled participants treated with alglucosidase alfa.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

Baseline (Day 1) and Month 12

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: As the endpoint was descriptive in nature, no statistical analysis was provided.

| | | | | |
|---|---------------------------|--|--|--|
| End point values | Alglucosidase alfa | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 41 | | | |
| Units: Meters | | | | |
| arithmetic mean (confidence interval 95%) | 43.637 (17.461 to 69.813) | | | |

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Treatment-Emergent Adverse Events (TEAEs) and Treatment-Emergent Serious Adverse Events (TESAEs) for all Participants

| | |
|-----------------|--|
| End point title | Number of Participants With Treatment-Emergent Adverse Events (TEAEs) and Treatment-Emergent Serious Adverse Events (TESAEs) for all Participants ^[2] |
|-----------------|--|

End point description:

An AE was defined as any untoward medical occurrence in a participant or clinical investigation participant administered a pharmaceutical product and which does not necessarily have to have a causal

relationship with this treatment. TEAEs were defined as the AEs that developed, worsened or became serious during the treatment-emergent period (defined as the time from first dose of study treatment [Day 1] up to 30 days after the last dose of study treatment). A serious adverse event (SAE) was defined as any untoward medical occurrence that at any dose: resulted in death, was life-threatening, required inpatient hospitalization or prolongation of existing hospitalization, resulted in persistent or significant disability/incapacity, was a congenital anomaly/birth defect or was a medically important event. Safety population included all the participants who actually received at least 1 dose or part of a dose of study treatment.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

From first dose of study drug (Day 1) up to 30 days after last dose, approximately 14.4 months

Notes:

[2] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: As the endpoint was descriptive in nature, no statistical analysis was provided.

| | | | | |
|-----------------------------|--------------------|--|--|--|
| End point values | Alglucosidase alfa | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 41 | | | |
| Units: Participants | | | | |
| Any TEAE | 32 | | | |
| Any TESAE | 7 | | | |

Statistical analyses

No statistical analyses for this end point

Primary: Change From Baseline in Percent Predicted Forced Vital Capacity (%FVC) in Upright Position for Participants \geq 5-Year old at Month 12

| | |
|-----------------|--|
| End point title | Change From Baseline in Percent Predicted Forced Vital Capacity (%FVC) in Upright Position for Participants \geq 5-Year old at Month 12 ^[3] |
|-----------------|--|

End point description:

FVC is a measurement of pulmonary function which is defined as the volume of air that can forcibly be blown out after full inspiration. It was assessed using the spirometry system with the participant in upright seated position. Percent of predicted FVC = (actual FVC measurement)/(predicted value of FVC) x 100. Baseline was defined as the last available value before the treatment. The endpoint is estimated based on LOCF method. ITT population included all enrolled participants treated with alglucosidase alfa.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

Baseline (Day 1) and Month 12

Notes:

[3] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: As the endpoint was descriptive in nature, no statistical analysis was provided.

| | | | | |
|---|-------------------------|--|--|--|
| End point values | Alglucosidase alfa | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 41 | | | |
| Units: Percentage of Predicted FVC | | | | |
| arithmetic mean (confidence interval 95%) | 2.430 (-0.852 to 5.713) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Maximal Inspiratory Pressure (MIP) in Upright Position for Participants ≥ 5 -Year old at Week 52

| | |
|-----------------|---|
| End point title | Change From Baseline in Maximal Inspiratory Pressure (MIP) in Upright Position for Participants ≥ 5 -Year old at Week 52 |
|-----------------|---|

End point description:

MIP is a measurement of inspiratory muscle strength which is defined as how much air pressure force a participant creates by inhaling through the mouth as hard as possible. It was assessed using the pneumography with the participant in upright seated position. Baseline was defined as the last available value before the treatment. ITT population included all enrolled participants treated with alglucosidase alfa. Only those participants with data collected at Baseline and at Week 52 are reported.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Day 1) and Week 52

| | | | | |
|--|-------------------------|--|--|--|
| End point values | Alglucosidase alfa | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 31 | | | |
| Units: centimeter of water column (cmH ₂ O) | | | | |
| least squares mean (confidence interval 95%) | 2.059 (-0.582 to 4.699) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Maximal Expiratory Pressure (MEP) in Upright Position for Participants ≥ 5 -Year old at Week 52

| | |
|-----------------|--|
| End point title | Change From Baseline in Maximal Expiratory Pressure (MEP) in Upright Position for Participants ≥ 5 -Year old at Week 52 |
|-----------------|--|

End point description:

MEP is a measurement of expiratory muscle strength which is defined as the greater pressure generated during maximal expiration. It was assessed using the pneumography with the participant in upright seated position. Baseline was defined as the last available value before the treatment. ITT population included all enrolled participants treated with alglucosidase alfa. Only those participants with data collected at Baseline and at Week 52 are reported.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Day 1) and Week 52

| End point values | Alglucosidase alfa | | | |
|--|-------------------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 31 | | | |
| Units: cmH2O | | | | |
| least squares mean (confidence interval 95%) | 0.073 (-2.921 to 3.066) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Manual Muscle Test (MMT) for Participants ≥ 5 -Year old at Week 52

| | |
|-----------------|---|
| End point title | Change From Baseline in Manual Muscle Test (MMT) for Participants ≥ 5 -Year old at Week 52 |
|-----------------|---|

End point description:

MMT was assessed according to expanded Medical Research Council (MRC) scale. MMT is used to measure body strength for deltoid muscle, quadriceps femoris, iliopsoas, and neck stretch flexor. Each individual item score range: 0 to 5 points with subdivisions in + or -, where a plus sign corresponds to an increase of one-third of score point and minus sign corresponds to a decrease of one-third of score point. Total scores are a sum of each individual item score, ranging from 0 (no muscle strength) to 40 (high muscle strength) with higher scores indicating better muscle strength. Baseline was defined as the last available value before the treatment. ITT population included all enrolled participants treated with alglucosidase alfa. Only those participants with data collected at Baseline and at Week 52 are reported.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Day 1) and Week 52

| End point values | Alglucosidase alfa | | | |
|--|-----------------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 36 | | | |
| Units: Score on a scale | | | | |
| least squares mean (confidence interval 95%) | 2.53 (1.559 to 3.493) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Quick Motor Function Test (QMFT) Scores for Participants ≥ 5 -Year old at Week 52

| | |
|-----------------|--|
| End point title | Change From Baseline in Quick Motor Function Test (QMFT) |
|-----------------|--|

End point description:

The QMFT is a reliable and valid test for assessing motor function in participants with Pompe's disease. QMFT comprised of 16 items specifically difficult for participants with Pompe's disease. Each item was scored separately on a 5-point ordinal scale which ranged from 0 to 4; higher scores indicated better outcomes. Total QMFT score was obtained by adding the scores of all items and ranged from 0 (unable to perform motor function tests) to 64 (normal muscle function); higher scores represented better outcomes. Baseline was defined as the last available value before the treatment. ITT population included all enrolled participants treated with alglucosidase alfa. Only those participants with data collected at Baseline and at Week 52 are reported.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Day 1) and Week 52

| End point values | Alglucosidase alfa | | | |
|--|--------------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 37 | | | |
| Units: Score on a scale | | | | |
| least squares mean (confidence interval 95%) | 5.6 (4.41 to 6.81) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in 12-Item Short-Form Health Survey Scores for Participants ≥ 5 -Year old at Week 52

| | |
|-----------------|--|
| End point title | Change From Baseline in 12-Item Short-Form Health Survey Scores for Participants ≥ 5 -Year old at Week 52 |
|-----------------|--|

End point description:

The 12-Item Short Form Health Survey (SF-12) which was developed for the medical outcomes evaluation of participants with chronic conditions. The SF-12 has 12 questions covering 8 health domains commonly represented in health surveys: physical functioning, role functioning physical, bodily pain, general health, vitality, social functioning, role functioning emotional, and mental health. Results are expressed in terms of 2 meta-scores: the Physical Component Summary (PCS) and the Mental Component Summary (MCS). The score ranges from 0 to 100; higher scores indicated better outcomes. Baseline was defined as the last available value before the treatment. ITT population included all enrolled participants treated with alglucosidase alfa. Only those participants with data collected at Baseline and at Week 52 are reported.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Day 1) and Week 52

| | | | | |
|---|----------------------------|--|--|--|
| End point values | Alglucosidase alfa | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 37 | | | |
| Units: Score on a scale | | | | |
| least squares mean (confidence interval 95%) | | | | |
| PCS-12 | 3.762 (1.252 to 6.271) | | | |
| MCS-12 | 0.608 (-1.986 to 3.203) | | | |

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Adverse events: From first dose of study treatment (Day 1) up to 30 days after last dose, approximately 14.4 months. All-cause mortality (death): From first dose of study treatment (Day 1) up to end of study, approximately 40.56 months.

Adverse event reporting additional description:

Analysis was performed on Safety population.

| | |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

Dictionary used

| | |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

| | |
|--------------------|------|
| Dictionary version | 27.0 |
|--------------------|------|

Reporting groups

| | |
|-----------------------|--------------------|
| Reporting group title | Alglucosidase alfa |
|-----------------------|--------------------|

Reporting group description:

Participants received alglucosidase alfa at a dose of 20 mg/kg body weight every 2 weeks as an IV infusion for up to 52 weeks.

| Serious adverse events | Alglucosidase alfa | | |
|---|--------------------|--|--|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 7 / 41 (17.07%) | | |
| number of deaths (all causes) | 0 | | |
| number of deaths resulting from adverse events | 0 | | |
| Injury, poisoning and procedural complications | | | |
| Fracture | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Lumbar Vertebral Fracture | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Nervous system disorders | | | |
| Cerebral Infarction | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Gastrointestinal disorders | | | |

| | | | |
|---|----------------|--|--|
| Intestinal Obstruction | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Respiratory, thoracic and mediastinal disorders | | | |
| Respiratory Failure | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Infections and infestations | | | |
| Pneumonia | | | |
| subjects affected / exposed | 2 / 41 (4.88%) | | |
| occurrences causally related to treatment / all | 0 / 2 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Pneumonia Bacterial | | | |
| subjects affected / exposed | 1 / 41 (2.44%) | | |
| occurrences causally related to treatment / all | 0 / 2 | | |
| deaths causally related to treatment / all | 0 / 0 | | |

Frequency threshold for reporting non-serious adverse events: 5 %

| | | | |
|---|--------------------|--|--|
| Non-serious adverse events | Alglucosidase alfa | | |
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 24 / 41 (58.54%) | | |
| Investigations | | | |
| Blood Creatine Phosphokinase Increased | | | |
| subjects affected / exposed | 9 / 41 (21.95%) | | |
| occurrences (all) | 10 | | |
| Blood Lactate Dehydrogenase Increased | | | |
| subjects affected / exposed | 7 / 41 (17.07%) | | |
| occurrences (all) | 7 | | |
| Carbon Dioxide Combining Power Increased | | | |
| subjects affected / exposed | 3 / 41 (7.32%) | | |
| occurrences (all) | 5 | | |

| | | | |
|--|---------------------|--|--|
| Creatinine Urine Decreased subjects affected / exposed occurrences (all) | 3 / 41 (7.32%) 4 | | |
| Electrocardiogram T Wave Peaked subjects affected / exposed occurrences (all) | 2 / 41 (4.88%) 2 | | |
| Gastrointestinal disorders Diarrhoea subjects affected / exposed occurrences (all) | 3 / 41 (7.32%) 3 | | |
| Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all) | 3 / 41 (7.32%) 4 | | |
| Nasal Turbinate Hypertrophy subjects affected / exposed occurrences (all) | 1 / 41 (2.44%) 1 | | |
| Oropharyngeal Pain subjects affected / exposed occurrences (all) | 3 / 41 (7.32%) 3 | | |
| Pulmonary Hypertension subjects affected / exposed occurrences (all) | 4 / 41 (9.76%) 4 | | |
| Respiratory Failure subjects affected / exposed occurrences (all) | 1 / 41 (2.44%) 1 | | |
| Skin and subcutaneous tissue disorders Dermatitis subjects affected / exposed occurrences (all) | 1 / 41 (2.44%) 2 | | |
| Infections and infestations Covid-19 subjects affected / exposed occurrences (all) | 4 / 41 (9.76%) 4 | | |
| Nasopharyngitis | | | |

| | | | |
|-----------------------------------|------------------|--|--|
| subjects affected / exposed | 2 / 41 (4.88%) | | |
| occurrences (all) | 2 | | |
| Upper Respiratory Tract Infection | | | |
| subjects affected / exposed | 10 / 41 (24.39%) | | |
| occurrences (all) | 13 | | |

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

| Date | Amendment |
|-------------|---|
| 29 May 2020 | Statistical considerations were modified. Updates were made in study flow chart, determination of sample size, compliance, and graphical study design. Analysis of primary and secondary efficacy endpoints were modified. Multiplicity considerations and other analyses were updated. Clarifications were provided in duration of study participation for each participant, selection of participants and visit schedule. Safety and other safety endpoints were updated. |

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported