Clinical trial results:
A Phase 2 Double-Blind, Randomized, Placebo-Controlled Study to Evaluate the Efficacy, Safety, and Tolerability of PF-06823859 in Adult Subjects With Dermatomyositis

Summary

EudraCT number	2020-004228-41
Trial protocol	DE PL HU IT ES
Global end of trial date	28 November 2022

Results information

Result version number	v1 (current)
This version publication date	12 July 2023
First version publication date	12 July 2023

Trial information

Trial identification

Sponsor protocol code	C0251002

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03181893
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Pfizer Inc.
Sponsor organisation address	235E 42nd Street, New York, United States, 10017
Public contact	Pfizer ClinicalTrials.gov Call Center, Pfizer Inc., +1 8007181021, ClinicalTrials.gov_Inquiries@pfizer.com
Scientific contact	Pfizer ClinicalTrials.gov Call Center, Pfizer Inc., +1 8007181021, ClinicalTrials.gov_Inquiries@pfizer.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?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	28 November 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	28 November 2022
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the efficacy, safety, and tolerability of PF 06823859 in participants with moderate to severe DM.

Protection of trial subjects:

This study was conducted in compliance with the ethical principles originating in or derived from the Declaration of Helsinki and in compliance with all International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) Good Clinical Practice (GCP) Guidelines. In addition, all local regulatory requirements were followed, in particular, those affording greater protection to the safety of trial subjects.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	23 January 2018
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Germany: 1
Country: Number of subjects enrolled	Hungary: 2
Country: Number of subjects enrolled	Poland: 4
Country: Number of subjects enrolled	Spain: 2
Country: Number of subjects enrolled	United States: 66
Worldwide total number of subjects	75
EEA total number of subjects	9

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)	0

Adolescents (12-17 years)	0
Adults (18-64 years)	62
From 65 to 84 years	13
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

32, 9, 16 and 18 subjects were treated in Stage 1, Stage 2, Amended Stage 2 and Stage 3, respectively. A fixed sequence design with crossover at Week 12 was employed in Amended Stage 2 and Stage 3 to provide all subjects with the opportunity to receive active drug during the treatment period.

Pre-assignment

Screening details:

A total of 75 subjects were randomized at 19 centers in 5 countries

Period 1

Period 1 title	Baseline to Week 12 (All Stages)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo (Stage 1)

Arm description:

Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 1.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo administration took place on Day 1, Week 4 and Week 8 in Stage 1 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg intravenous (IV) (Stage 1)

Arm description:

Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 1.

Arm type	Experimental
Investigational medicinal product name	PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

PF 06823859 100 mg/mL solution for Injection administered on Day 1, Week4, and Week 8 in Stage 1 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo (Stage 2)

Arm description:

Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 2.

Arm type	Placebo

Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo administration took place on Day 1, Week 4 and Week 8 in Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 150 mg IV (Stage 2)

Arm description:

Subjects in this group were randomized to receive PF-06823859 150 mg on Day 1, Week 4, and Week 8 in Stage 2.

Arm type	Experimental
Investigational medicinal product name	PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

PF 06823859 100 mg/mL solution for Injection administered on Day 1, Week4, and Week 8 in Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg IV (Stage 2)

Arm description:

Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 2.

Arm type	Experimental
Investigational medicinal product name	PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

PF 06823859 100 mg/mL solution for Injection administered on Day 1, Week4, and Week 8 in Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo then PF-06823859 150 mg IV (Amended Stage 2)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 150 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo then PF-06823859 600 mg IV (Amended Stage 2)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day

1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 150 mg IV then Placebo (Amended Stage 2)

Arm description:

Subjects in this group were randomized to PF-06823859 150 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg IV then Placebo (Amended Stage 2)

Arm description:

Participants in this group were randomized to PF-06823859 600 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20 . After the treatment period ended at Week 24, participants then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo then PF-06823859 600 mg IV (Stage 3)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental

Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Stage 3 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg IV then placebo (Stage 3)

Arm description:

Subjects in this group were randomized to 600 mg PF-06823859 then placebo with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Stage 3 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Number of subjects in period 1	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)
Started	10	22	1
Completed	9	20	1
Not completed	1	2	0
Consent withdrawn by subject	-	1	-
Adverse event, non-fatal	1	1	-

Number of subjects in period 1	PF-06823859 150 mg IV (Stage 2)	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)
Started	5	3	2
Completed	5	3	2
Not completed	0	0	0
Consent withdrawn by subject	-	-	-
Adverse event, non-fatal	-	-	-

Number of subjects in period 1	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Started	1	10	3

Completed	1	10	3
Not completed	0	0	0
Consent withdrawn by subject	-	-	-
Adverse event, non-fatal	-	-	-

Number of subjects in period 1	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)
Started	9	9
Completed	9	9
Not completed	0	0
Consent withdrawn by subject	-	-
Adverse event, non-fatal	-	-

Period 2

Period 2 title	Weeks 12-24 (Amended Stage 2, Stage 3)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo then PF-06823859 150 mg IV (Amended Stage 2)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 150 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo then PF-06823859 600 mg IV (Amended Stage 2)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and seek 20. After the treatment period ended at Week 24, Subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental

Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 150 mg IV then Placebo (Amended Stage 2)

Arm description:

Subjects in this group were randomized to PF-06823859 150 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg IV then Placebo (Amended Stage 2)

Arm description:

Subjects in this group were randomized to PF-06823859 600 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20 . After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Amended Stage 2 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	Placebo then PF-06823859 600 mg IV (Stage 3)

Arm description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Stage 3 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Arm title	PF-06823859 600 mg IV then placebo (Stage 3)

Arm description:

Subjects in this group were randomized to 600 mg PF-06823859 then placebo with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Arm type	Experimental
Investigational medicinal product name	Placebo and PF-06823859
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Placebo or PF-06823859 administration (as dedicated by the treatment sequence) took place on Day 1, Weeks 4, 8, 12, 16 and 20 in Stage 3 over the course of approximately 60 minutes as an IV infusion, using a calibrated infusion pump.

Number of subjects in period 2[1]	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Started	2	1	10
Completed	2	1	9
Not completed	0	0	1
Consent withdrawn by subject	-	-	-
Adverse event, non-fatal	-	-	-
Unspecified	-	-	1

Number of subjects in period 2[1]	PF-06823859 600 mg IV then Placebo (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)
Started	3	9	9
Completed	2	8	8
Not completed	1	1	1
Consent withdrawn by subject	-	1	-
Adverse event, non-fatal	-	-	1
Unspecified	1	-	-

Notes:

[1] - The number of subjects starting the period is not consistent with the number completing the preceding period. It is expected the number of subjects starting the subsequent period will be the same as the number completing the preceding period.
Justification: The number of subjects (2 subjects) started from Week 12 is same as the number of subjects completing the preceding period (2 subjects).

Baseline characteristics

Reporting groups

Reporting group title	Placebo (Stage 1)
Reporting group description:	
Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 1.	
Reporting group title	PF-06823859 600 mg intravenous (IV) (Stage 1)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 1.	
Reporting group title	Placebo (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	PF-06823859 150 mg IV (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 150 mg on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	PF-06823859 600 mg IV (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	Placebo then PF-06823859 150 mg IV (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 150 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	Placebo then PF-06823859 600 mg IV (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 600 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to PF-06823859 150 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Reporting group description:	
Participants in this group were randomized to PF-06823859 600 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20 . After the treatment period ended at Week 24, participants then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	Placebo then PF-06823859 600 mg IV (Stage 3)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 600 mg with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 600 mg IV then placebo (Stage 3)
Reporting group description:	
Subjects in this group were randomized to 600 mg PF-06823859 then placebo with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12,	

subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)
Number of subjects	10	22	1
Age Categorical Units: Participants			
18-64 Years	8	18	1
65-84 Years	2	4	0
>=85 Years	0	0	0
Age Continuous Units: years			
arithmetic mean	50.20	54.41	42.00
standard deviation	± 14.054	± 13.154	± 99999
Sex: Female, Male Units: Participants			
Female	9	20	1
Male	1	2	0
Race/Ethnicity, Customized Units: Subjects			
White	9	20	1
Black or African American	0	0	0
Asian	0	0	0
American Indian or Alaska Native	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Other	0	0	0
Multiracial	1	0	0
Not reported	0	2	0
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	2	2	1
Not Hispanic or Latino	8	20	0
Unknown	0	0	0
Not Reported	0	0	0

Reporting group values	PF-06823859 150 mg IV (Stage 2)	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)
Number of subjects	5	3	2
Age Categorical Units: Participants			
18-64 Years	4	2	1
65-84 Years	1	1	1
>=85 Years	0	0	0
Age Continuous Units: years			
arithmetic mean	51.60	45.67	64.00
standard deviation	± 15.726	± 23.714	± 1.414

Sex: Female, Male Units: Participants			
Female	5	3	2
Male	0	0	0
Race/Ethnicity, Customized Units: Subjects			
White	5	3	2
Black or African American	0	0	0
Asian	0	0	0
American Indian or Alaska Native	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Other	0	0	0
Multiracial	0	0	0
Not reported	0	0	0
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	0	1	0
Not Hispanic or Latino	5	2	2
Unknown	0	0	0
Not Reported	0	0	0

Reporting group values	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Number of subjects	1	10	3
Age Categorical Units: Participants			
18-64 Years	1	8	3
65-84 Years	0	2	0
>=85 Years	0	0	0
Age Continuous Units: years			
arithmetic mean	44	53.90	47.00
standard deviation	± 99999	± 10.999	± 13.115
Sex: Female, Male Units: Participants			
Female	1	10	2
Male	0	0	1
Race/Ethnicity, Customized Units: Subjects			
White	1	9	3
Black or African American	0	0	0
Asian	0	1	0
American Indian or Alaska Native	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Other	0	0	0
Multiracial	0	0	0
Not reported	0	0	0
Ethnicity (NIH/OMB) Units: Subjects			

Hispanic or Latino	0	1	1
Not Hispanic or Latino	1	9	2
Unknown	0	0	0
Not Reported	0	0	0

Reporting group values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)	Total
Number of subjects	9	9	75
Age Categorical Units: Participants			
18-64 Years	8	8	62
65-84 Years	1	1	13
>=85 Years	0	0	0
Age Continuous Units: years			
arithmetic mean	47.44	42.44	
standard deviation	± 12.126	± 16.697	-
Sex: Female, Male Units: Participants			
Female	6	7	66
Male	3	2	9
Race/Ethnicity, Customized Units: Subjects			
White	8	8	69
Black or African American	0	0	0
Asian	1	0	2
American Indian or Alaska Native	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Other	0	0	0
Multiracial	0	0	1
Not reported	0	1	3
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	3	2	13
Not Hispanic or Latino	6	7	62
Unknown	0	0	0
Not Reported	0	0	0

End points

End points reporting groups

Reporting group title	Placebo (Stage 1)
Reporting group description:	
Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 1.	
Reporting group title	PF-06823859 600 mg intravenous (IV) (Stage 1)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 1.	
Reporting group title	Placebo (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive placebo on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	PF-06823859 150 mg IV (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 150 mg on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	PF-06823859 600 mg IV (Stage 2)
Reporting group description:	
Subjects in this group were randomized to receive PF-06823859 600 mg on Day 1, Week 4, and Week 8 in Stage 2.	
Reporting group title	Placebo then PF-06823859 150 mg IV (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 150 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	Placebo then PF-06823859 600 mg IV (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 600 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Reporting group description:	
Subjects in this group were randomized to PF-06823859 150 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Reporting group description:	
Participants in this group were randomized to PF-06823859 600 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20 . After the treatment period ended at Week 24, participants then entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	Placebo then PF-06823859 600 mg IV (Stage 3)
Reporting group description:	
Subjects in this group were randomized to placebo then PF-06823859 600 mg with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.	
Reporting group title	PF-06823859 600 mg IV then placebo (Stage 3)
Reporting group description:	
Subjects in this group were randomized to 600 mg PF-06823859 then placebo with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12,	

subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	Placebo then PF-06823859 150 mg IV (Amended Stage 2)

Reporting group description:

Subjects in this group were randomized to placebo then PF-06823859 150 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	Placebo then PF-06823859 600 mg IV (Amended Stage 2)

Reporting group description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and seek 20. After the treatment period ended at Week 24, Subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	PF-06823859 150 mg IV then Placebo (Amended Stage 2)

Reporting group description:

Subjects in this group were randomized to PF-06823859 150 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	PF-06823859 600 mg IV then Placebo (Amended Stage 2)

Reporting group description:

Subjects in this group were randomized to PF-06823859 600 mg then placebo in Amended Stage 2. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20 . After the treatment period ended at Week 24, subjects then entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	Placebo then PF-06823859 600 mg IV (Stage 3)

Reporting group description:

Subjects in this group were randomized to placebo then PF-06823859 600 mg with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Reporting group title	PF-06823859 600 mg IV then placebo (Stage 3)

Reporting group description:

Subjects in this group were randomized to 600 mg PF-06823859 then placebo with a treatment switch at Week 12 in Stage 3. Study intervention or placebo administration (as dictated by the treatment sequence) occurred on Day 1, Week 4, Week 8, Week 12, Week 16, and Week 20. After Week 12, subjects were switched to the other treatment in the sequence. After the treatment period ended at Week 24, subjects entered a 4-month follow-up period or rolled over to the long-term extension study.

Primary: Change From Baseline in Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI) Activity Score at Week 12 (Stage 1, Stage 2 and Amended Stage 2)

End point title	Change From Baseline in Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI) Activity Score at Week 12 (Stage 1, Stage 2 and Amended Stage 2)[1]

End point description:

The treatment effect was defined as the difference (mean chg from baseline at Week12 in the active treatment group minus that in the placebo group) in the mean change of CDASI activity score from baseline at Week 12. The score (range: 0-100) consists of the extent score (ES), Gottorn hands score (GHS), peringual score (PS) and allopecia score (AS). ES (range: 0-90) was obtained by summing up scores for the total erythema (ER [0-45], redness of the skin or mucous membranes), scaling (SC [0-30], peeling of the skin) and erosion/ulceration (EU [0-15], presence of the deeper wound). Total ER, SC and EU scores were calculated as a sum of the contributions from 15 individual areas of the body. GHS characterizes papules (swellings) on hand and is a sum of the papule’s characterization score (0-6) and ulceration score (0-1). PS (0-2) characterizes abnormalities around nails. The AS (0-1) characterizes hair loss. Higher scores indicate greater disease severity.

End point type	Primary

End point timeframe:

Baseline and Week 12

Notes:

[1] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is primary endpoint for arms in Stage 1, Stage 2, and amended Stage 2. The statistics for arms in Stage 3 are reported in the secondary endpoint.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	9	21	1	5
Units: Units on a scale				
arithmetic mean (standard deviation)	-3.44 (± 5.270)	-19.62 (± 9.140)	5.00 (± 99999)	-17.40 (± 9.290)

End point values	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	2	1	10
Units: Units on a scale				
arithmetic mean (standard deviation)	-26.00 (± 7.937)	-3.00 (± 8.485)	3.00 (± 99999)	-16.40 (± 5.835)

End point values	PF-06823859 600 mg IV then Placebo (Amended Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: Units on a scale				
arithmetic mean (standard deviation)	-15.33 (± 6.028)			

Statistical analyses

Statistical analysis title	PF-06823859 600 mg vs Placebo (Stage 1)
Comparison groups	Placebo (Stage 1) v PF-06823859 600 mg intravenous (IV) (Stage 1)

Number of subjects included in analysis	30
Analysis specification	Pre-specified
Analysis type	other
P-value	< 0.0001
Method	LANCOVA-P model
Parameter estimate	Mean difference (final values)
Point estimate	-14.82
Confidence interval	
level	90 %
sides	2-sided
lower limit	-20.26
upper limit	-9.37
Variability estimate	Standard error of the mean
Dispersion value	3.183

Primary: Number of Subjects With Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAE) (Stage 3)

End point title	Number of Subjects With Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAE) (Stage 3)[2][3]

End point description:

Adverse event (AE) was any untoward medical occurrence in a participant temporally associated with the use of study intervention, whether or not considered related to the study intervention. SAE was any untoward medical occurrence that at any dose resulted in any of following outcomes/deemed significant for any other reason: death; initial /prolonged inpatient hospitalization; life-threatening; persistent or significant disability/incapacity; congenital anomaly/birth defect and suspected transmission via a Pfizer product of an infectious agent, pathogenic or non-pathogenic. AEs included both serious (if occurred) and all non-serious adverse events. TEAEs are events between first dose of study drug and up to Week 40 that were absent before treatment or that worsened relative to pretreatment state.

End point type	Primary

End point timeframe:

Up to Week 40

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: No statistical analyses for the safety endpoint.

[3] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Subjects				
TEAEs	7	8		
SAEs	0	2		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Clinically Significant Laboratory Abnormalities (Stage 3)

End point title	Number of Subjects With Clinically Significant Laboratory Abnormalities (Stage 3)[4][5]

End point description:

Hemoglobin(HGB),hematocrit,erythrocytes(ery.),HDL cholesterol(chl.)<0.8*lower limit of normal(LLN);reticulocytes (ret.), ret./ery.(%)<0.5*LLN,>1.5*upper limit of normal (ULN);ery. mean corpuscular(EMC) volume,EMC HGB concentration,potassium,chloride,calcium,bicarbonate<0.9*LLN,>1.1*ULN;platelets<0.5*LLN,>1.75*ULN; leukocytes(leu.),glucose<0.6*LLN,>1.5*ULN;lymphocytes(lym.), lym./leu.(%),neutrophils (neu.), neu./leu.(%), protein,albumin<0.8*LLN,>1.2*ULN;basophils(bas.), bas./leu.(%), eosinophils(eos.), eos./leu., monocytes(mon.), mon./leu.(%), urate>1.2*ULN;bilirubin (total, direct,indirect)>1.5*ULN;aspartate/alanine aminotransferase,gamma glutamyl transferase,lactate dehydrogenase,alkaline phosphatase>3.0*ULN;urea nitrogen,creatinine,triglycerides, chl.>1.3*ULN; sodium <0.95*LLN,>1.05*ULN; creatine kinase >2.0*ULN;Urine: pH<4.5,>8;glucose, ketones,protein,

End point type	Primary

End point timeframe:

Up to Week 40

Notes:

[4] - 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: The endpoints were reported by stages. All the arms in the baseline period reported statistics.
[5] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analyses for the safety endpoint.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Subjects	0	1		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Vital Sign Abnormalities (Stage 3)

End point title	Number of Subjects With Vital Sign Abnormalities (Stage 3)[6][7]

End point description:

Abnormality in vital signs: Sitting pulse rate <40 beats per minute (bpm) to >120 bpm, sitting diastolic blood pressure (DBP) < 50 millimeter of mercury (mmHg), sitting systolic blood pressure (SBP) <90 mmHg.

End point type	Primary

End point timeframe:

Baseline up to Week 40

Notes:

[6] - 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: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

[7] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analyses for the safety endpoint.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Subjects				
Sitting SBP Value <90 mmHg	0	0		
Sitting DBP Value <50 mmHg	0	0		
Sitting Pulse Rate Value <40 bpm	0	0		
Sitting Pulse Rate Value >120 bpm	0	0		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Electrocardiogram (ECG) Abnormalities (Stage 3)

End point title	Number of Subjects With Electrocardiogram (ECG) Abnormalities (Stage 3)[8][9]

End point description:

ECG abnormalities criteria included: 1) QTc interval adjusted according to Fridericia formula (QTcF) (msec): >450, >480, >500, increase from baseline >=30, increase from baseline >=60; 2) Pulse rate (PR) (msec): >=300, change from baseline (Chg) >=25% or 50%; 3) QT (msec): >=500; 4) QRS (msec): >=200, Chg >=25% or 50%. Categories, with at least 1 participant having ECG abnormality in any of the reporting arms, were reported in this outcome measure.

End point type	Primary

End point timeframe:

Baseline up to Week 40

Notes:

[8] - 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: No statistical analyses for the safety endpoint.

[9] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Subjects				
PR Interval Aggregate Value >=300 msec	0	0		
QRS Duration Aggregate Value >=200 msec	0	0		
QT Interval Aggregate Value >=500 msec	0	0		

QTcF Interval Aggregate 450<=Value<480 msec	0	1		
QTcF Interval Aggregate 480<=Value<500 msec	1	0		
QTcF Interval Aggregate Value >=500 msec	0	0		
PR %Chg >=25% or >=50%	0	0		
QRS Duration %Chg >=25% or >=50%	0	0		
30<=QTcF Chg (msec)<60	0	0		
QTcF Chg (msec) >=60	0	1		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With TEAEs and SAEs (Stage 1 and Stage 2)

End point title	Number of Subjects With TEAEs and SAEs (Stage 1 and Stage 2)[10]

End point description:

AE was any untoward medical occurrence in a participant temporally associated with the use of study intervention, whether or not considered related to the study intervention. SAE was any untoward medical occurrence that at any dose resulted in any of following outcomes/deemed significant for any other reason: death; initial /prolonged inpatient hospitalization; life-threatening; persistent or significant disability/incapacity; congenital anomaly/birth defect and suspected transmission via a Pfizer product of an infectious agent, pathogenic or non-pathogenic. AEs included both serious (if occurred) and all non-serious adverse events. TEAEs are events between first dose of study drug and up to Week 28 that were absent before treatment or that worsened relative to pretreatment state.

End point type	Secondary

End point timeframe:

Up to Week 28

Notes:

[10] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Subjects				
TEAEs	8	20	1	5
SAEs	1	2	0	0

End point values	PF-06823859 600 mg IV (Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			

Units: Subjects				
TEAEs	3			
SAEs	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Clinically Significant Laboratory Abnormalities (Stage 1 and Stage 2)

End point title	Number of Subjects With Clinically Significant Laboratory Abnormalities (Stage 1 and Stage 2)[11]

End point description:

HGB,hematocrit,ery.,HDL chl.<0.8*LLN;ret., ret./ery. (%)<0.5*LLN,>1.5*ULN;EMC volume,EMC HGB,EMC HGB concentration,potassium,chloride,calcium,bicarbonate<0.9*LLN,>1.1*ULN;platelets<0.5*LLN,>1.75*ULN;leu.,glucose<0.6*LLN,>1.5*ULN;lym., lym./leu.(%), neu., neu./leu. (%), protein,albumin <0.8*LLN,>1.2*ULN;bas., bas./leu.(%), eos., eos./leu., mon., mon./leu.(%), urate >1.2*ULN;bilirubin (total, direct, indirect)>1.5*ULN;aspartate/alanine aminotransferase, gamma glutamyl transferase, lactate dehydrogenase, alkaline phosphatase>3.0*ULN;urea nitrogen, creatinine, triglycerides, chl.>1.3*ULN; sodium <0.95*LLN,>1.05*ULN; creatine kinase >2.0*ULN;Urine: pH<4.5,>8;glucose, ketones, protein, HGB, urobilinogen,bilirubin,nitrite,leukocyte esterase>=1;ery., leu.>= 20;hyaline casts>1;bacteria>20. Clinical significance of laboratory parameters was determined at the investigator's

End point type	Secondary

End point timeframe:

Up to Week 28

Notes:

[11] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Subjects	0	0	0	0

End point values	PF-06823859 600 mg IV (Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: Subjects	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With TEAEs and SAEs (Amended Stage 2)

End point title	Number of Subjects With TEAEs and SAEs (Amended Stage

End point description:

AE was any untoward medical occurrence in a participant temporally associated with the use of study intervention, whether or not considered related to the study intervention. SAE was any untoward medical occurrence that at any dose resulted in any of following outcomes/deemed significant for any other reason: death; initial /prolonged inpatient hospitalization; life-threatening; persistent or significant disability/incapacity; congenital anomaly/birth defect and suspected transmission via a Pfizer product of an infectious agent, pathogenic or non-pathogenic. AEs included both serious (if occurred) and all non-serious adverse events. TEAEs are events between first dose of study drug and up to Week 40 that were absent before treatment or that worsened relative to pretreatment state.

End point type	Secondary

End point timeframe:

Up to Week 40

Notes:

[12] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	2	1	10	3
Units: Subjects				
TEAEs	1	1	8	3
SAEs	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Vital Sign Abnormalities (Stage 1 and Stage 2)

End point title	Number of Subjects With Vital Sign Abnormalities (Stage 1 and Stage 2)[13]

End point description:

Abnormality in vital signs: Sitting pulse rate <40 bpm to >120 bpm, sitting DBP < 50 mmHg, sitting SBP <90 mmHg.

End point type	Secondary

End point timeframe:

Up to Week 28

Notes:

[13] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Subjects				
Sitting SBP Value <90 mmHg	0	1	0	0
Sitting DBP Value <50 mmHg	0	0	0	0
Sitting Pulse Rate Value <40 bpm	0	0	0	0
Sitting Pulse Rate Value >120 bpm	0	0	0	0

End point values	PF-06823859 600 mg IV (Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: Subjects				
Sitting SBP Value <90 mmHg	0			
Sitting DBP Value <50 mmHg	0			
Sitting Pulse Rate Value <40 bpm	0			
Sitting Pulse Rate Value >120 bpm	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Clinically Significant Laboratory Abnormalities (Amended Stage 2)

End point title	Number of Subjects With Clinically Significant Laboratory Abnormalities (Amended Stage 2)[14]

End point description:

HGB,hematocrit,ery.,HDL chl.<0.8*LLN;ret., ret./ery. (%)<0.5*LLN,>1.5*ULN;EMC volume,EMC HGB,EMC HGB concentration,potassium,chloride,calcium,bicarbonate<0.9*LLN,>1.1*ULN;platelets<0.5*LLN,>1.75*ULN;leu.,glucose<0.6*LLN,>1.5*ULN;lym., lym./leu.(%), neu., neu./leu. (%), protein,albumin <0.8*LLN,>1.2*ULN;bas., bas./leu.(%), eos., eos./leu., mon., mon./leu.(%), urate >1.2*ULN;bilirubin (total, direct, indirect)>1.5*ULN;aspartate/alanine aminotransferase, gamma glutamyl transferase, lactate dehydrogenase, alkaline phosphatase>3.0*ULN;urea nitrogen, creatinine, triglycerides, chl.>1.3*ULN; sodium <0.95*LLN,>1.05*ULN; creatine kinase >2.0*ULN;Urine: pH<4.5,>8;glucose, ketones, protein, HGB, urobilinogen,bilirubin,nitrite,leukocyte esterase>=1;ery., leu.>= 20;hyaline casts>1;bacteria>20. Clinical significance of laboratory parameters was determined at the investigator's

End point type	Secondary

End point timeframe:

Up to Week 40

Notes:

[14] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	2	1	10	3
Units: Subjects	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Vital Sign Abnormalities (Amended Stage 2)

End point title	Number of Subjects With Vital Sign Abnormalities (Amended Stage 2)[15]

End point description:

Abnormality in vital signs: Sitting pulse rate <40 bpm to >120 bpm, sitting DBP < 50 mmHg, sitting SBP <90 mmHg.

End point type	Secondary

End point timeframe:

Up to Week 40

Notes:

[15] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	2	1	10	3
Units: Subjects				
Sitting SBP Value <90 mmHg	0	0	0	0
Sitting DBP Value <50 mmHg	0	0	0	0
Sitting Pulse Rate Value <40 bpm	0	0	0	0
Sitting Pulse Rate Value >120 bpm	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With ECG Abnormalities (Stage 1 and Stage 2)

End point title	Number of Subjects With ECG Abnormalities (Stage 1 and Stage 2)[16]

End point description:

ECG abnormalities criteria included: 1) QTc interval adjusted according to Fridericia formula (QTcF)

(msec): >450, >480, >500, increase from baseline >=30, increase from baseline >=60; 2) Pulse rate (PR) (msec): >=300, change from baseline (Chg) >=25% or 50%; 3) QT (msec): >=500; 4) QRS (msec): >=200, Chg >=25% or 50%. Categories, with at least 1 participant having ECG abnormality in any of the reporting arms, were reported in this outcome measure.

End point type	Secondary

End point timeframe:

Up to Week 28

Notes:

[16] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Subjects				
PR Interval Aggregate Value >=300 msec	0	0	0	0
QRS Duration Aggregate Value >=200 msec	0	0	0	0
QT Interval Aggregate Value >=500 msec	0	0	0	0
QTcF Interval Aggregate 450<=Value<480 msec	3	1	0	1
QTcF Interval Aggregate 480<=Value<500 msec	0	0	0	0
QTcF Interval Aggregate Value >=500 msec	0	1	0	0
PR %Chg >=25% or >=50%	0	0	0	0
QRS Duration %Chg >=25% or >=50%	0	0	0	0
30<=QTcF Chg (msec)<60	0	0	0	0
QTcF Chg (msec) >=60	0	1	0	0

End point values	PF-06823859 600 mg IV (Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: Subjects				
PR Interval Aggregate Value >=300 msec	0			
QRS Duration Aggregate Value >=200 msec	0			
QT Interval Aggregate Value >=500 msec	0			
QTcF Interval Aggregate 450<=Value<480 msec	0			
QTcF Interval Aggregate 480<=Value<500 msec	0			
QTcF Interval Aggregate Value >=500 msec	0			

PR %Chg >=25% or >=50%	0			
QRS Duration %Chg >=25% or >=50%	0			
30<=QTcF Chg (msec)<60	0			
QTcF Chg (msec) >=60	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With ECG Abnormalities (Amended Stage 2)

End point title	Number of Subjects With ECG Abnormalities (Amended Stage 2)[17]

End point description:

ECG abnormalities criteria included: 1) QTc interval adjusted according to Fridericia formula (QTcF) (msec): >450, >480, >500, increase from baseline >=30, increase from baseline >=60; 2) Pulse rate (PR) (msec): >=300, change from baseline (Chg) >=25% or 50%; 3) QT (msec): >=500; 4) QRS (msec): >=200, Chg >=25% or 50%. Categories, with at least 1 participant having ECG abnormality in any of the reporting arms, were reported in this outcome measure.

End point type	Secondary

End point timeframe:

Up to Week 40

Notes:

[17] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoints were reported by stages. All the arms in the baseline period reported statistics.

End point values	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)	PF-06823859 600 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	2	1	10	3
Units: Subjects				
PR Interval Aggregate Value >=300 msec	0	0	0	0
QRS Duration Aggregate Value >=200 msec	0	0	0	0
QT Interval Aggregate Value >=500 msec	0	0	0	0
QTcF Interval Aggregate 450<=Value<480 msec	1	0	3	0
QTcF Interval Aggregate 480<=Value<500 msec	0	0	0	0
QTcF Interval Aggregate Value >=500 msec	0	0	0	0
PR %Chg >=25% or >=50%	0	0	0	0
QRS Duration %Chg >=25% or >=50%	0	0	0	0
30<=QTcF Chg (msec)<60	1	0	1	0
QTcF Chg (msec) >=60	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in CDASI Activity Score at at All Scheduled Timepoints Through Week 12 (Stage 1, Stage 2 and Amended Stage 2)

End point title	Change From Baseline in CDASI Activity Score at at All Scheduled Timepoints Through Week 12 (Stage 1, Stage 2 and Amended Stage 2)[18]

End point description:

The treatment effect was defined as the difference (mean change from baseline at Weeks 1, 4, 8 in the active treatment group minus the mean change from baseline at Weeks 1, 4, 8 in the placebo group) in the mean change of CDASI activity score from baseline at scheduled timepoints. The score (range: 0-100) consists of the ES, GHS, PS and AS. ES (range: 0-90) was obtained by summing up scores for the total erythema (ER [0-45], redness of the skin or mucous membranes), scaling (SC [0-30], peeling of the skin) and erosion/ulceration (EU [0-15], presence of the deeper wound). Total ER, SC and EU scores were calculated as a sum of the contributions from 15 individual areas of the body. GHS characterizes papules (swellings) on hand and is a sum of the papule's characterization score (0-6) and ulceration score (0-1). PS (0-2) characterizes abnormalities around nails. The AS (0-1) characterizes hair loss. Higher scores indicate greater disease severity.

End point type	Secondary

End point timeframe:

Baseline, Week 1, Week 4, and Week 8 (except for Week 12 which is a primary outcome measure)

Notes:

[18] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: Change from baseline in CDASI activity score for arms in Stage 3 was reported in the next end point.

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	21	1	5
Units: Units on a scale				
arithmetic mean (standard deviation)				
Week 1	-2.60 (± 4.326)	-19.62 (± 9.140)	1.00 (± 99999)	-9.80 (± 12.174)
Week 4	-2.44 (± 8.126)	-12.00 (± 10.277)	3.00 (± 99999)	-11.20 (± 10.986)
Week 8	-4.22 (± 6.942)	-17.19 (± 9.595)	4.00 (± 99999)	-14.20 (± 5.020)

End point values	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)

Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	2	1	10
Units: Units on a scale				
arithmetic mean (standard deviation)				
Week 1	-2.33 (± 1.528)	-6.50 (± 6.364)	0.00 (± 99999)	-8.70 (± 5.638)
Week 4	-15.00 (± 7.550)	-3.00 (± 7.071)	-1.00 (± 99999)	-14.40 (± 7.648)
Week 8	-18.67 (± 10.066)	-2.50 (± 7.778)	-2.00 (± 99999)	-17.80 (± 7.540)

End point values	PF-06823859 600 mg IV then Placebo (Amended Stage 2)			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: Units on a scale				
arithmetic mean (standard deviation)				
Week 1	-6.67 (± 2.517)			
Week 4	-14.00 (± 2.000)			
Week 8	-14.00 (± 4.000)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in CDASI Activity Score at All Scheduled Timepoints Through Week 12 (Stage 3)

End point title	Change From Baseline in CDASI Activity Score at All Scheduled Timepoints Through Week 12 (Stage 3)[19]

End point description:

The treatment effect was defined as the difference (mean change from baseline at Weeks 1, 4, 8,12 in the active treatment group minus the mean change from baseline at Weeks 1, 4, 8, 12 in the placebo group) in the mean change of CDASI activity score from baseline at scheduled timepoints. The score (range: 0-100) consists of the ES, GHS, PS and AS. ES (range: 0-90) was obtained by summing up scores for the total erythema (ER [0-45], redness of the skin or mucous membranes), scaling (SC [0-30], peeling of the skin) and erosion/ulceration (EU [0-15], presence of the deeper wound). Total ER, SC and EU scores were calculated as a sum of the contributions from 15 individual areas of the body. GHS characterizes papules (swellings) on hand and is a sum of the papule's characterization score (0-6) and ulceration score (0-1). PS (0-2) characterizes abnormalities around nails. The AS (0-1) characterizes hair loss. Higher scores indicate greater disease severity.

End point type	Secondary

End point timeframe:

Baseline, Week 1, Week 4, Week 8 and Week 12

Notes:

[19] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistics for other arms were reported in the previous end point.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Units on a scale				
arithmetic mean (standard deviation)				
Week 1	-2.00 (± 3.808)	-2.11 (± 2.522)		
Week 4	-3.78 (± 5.333)	-5.11 (± 5.555)		
Week 8	-5.00 (± 7.382)	-7.78 (± 6.667)		
Week 12	-5.89 (± 8.177)	-8.56 (± 7.923)		

Statistical analyses

No statistical analyses for this end point

Secondary: Absolute Values of CDASI Activity Score at All Scheduled Timepoints Through Week 12 (All Stages)

End point title	Absolute Values of CDASI Activity Score at All Scheduled Timepoints Through Week 12 (All Stages)

End point description:

The CDASI activity score (range: 0-100) consists of the ES, GHS, PS and AS. ES (range: 0-90) was obtained by summing up scores for the total erythema (ER [0-45], redness of the skin or mucous membranes), scaling (SC [0-30], peeling of the skin) and erosion/ulceration (EU [0-15], presence of the deeper wound). Total ER, SC and EU scores were calculated as a sum of the contributions from 15 individual areas of the body. GHS characterizes papules (swellings) on hand and is a sum of the papule's characterization score (0-6) and ulceration score (0-1). PS (0-2) characterizes abnormalities around nails. The AS (0-1) characterizes hair loss. Higher scores indicate greater disease severity.

End point type	Secondary

End point timeframe:

Baseline, Week 1, Week 4, Week 8 and Week 12

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	31.50 (± 11.750)	33.23 (± 10.323)	23.00 (± 99999)	28.60 (± 9.017)

Week 1	28.90 (± 12.982)	27.68 (± 8.962)	24.00 (± 99999)	18.80 (± 6.535)
Week 4	28.11 (± 16.136)	21.23 (± 11.182)	26.00 (± 99999)	17.40 (± 2.408)
Week 8	26.33 (± 13.892)	15.29 (± 6.157)	27.00 (± 99999)	14.40 (± 5.857)
Week 12	27.11 (± 14.903)	12.86 (± 5.876)	28.00 (± 99999)	11.20 (± 4.817)

End point values	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	2	1	10
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	37.00 (± 9.644)	26.00 (± 5.657)	37.00 (± 99999)	35.40 (± 13.226)
Week 1	34.67 (± 9.713)	19.50 (± 0.707)	37.00 (± 99999)	26.70 (± 12.667)
Week 4	22.00 (± 12.490)	23.00 (± 1.414)	36.00 (± 99999)	21.00 (± 10.677)
Week 8	18.33 (± 5.508)	23.50 (± 2.121)	35.00 (± 99999)	17.60 (± 10.895)
Week 12	11.00 (± 3.464)	23.00 (± 2.828)	40.00 (± 99999)	19.00 (± 14.087)

End point values	PF-06823859 600 mg IV then Placebo (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	3	9	9	
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	30.00 (± 7.937)	17.22 (± 11.595)	12.56 (± 8.095)	
Week 1	23.33 (± 9.018)	15.22 (± 9.846)	10.44 (± 6.894)	
Week 4	16.00 (± 9.539)	13.44 (± 8.819)	7.44 (± 5.503)	
Week 8	16.00 (± 11.269)	12.22 (± 10.883)	4.78 (± 4.116)	
Week 12	14.67 (± 9.504)	11.33 (± 9.206)	4.00 (± 3.464)	

Statistical analyses

No statistical analyses for this end point

Secondary: Absolute Values of CDASI Damage Score at All Scheduled Timepoints Through Week 12 (All Stages)

End point title	Absolute Values of CDASI Damage Score at All Scheduled Timepoints Through Week 12 (All Stages)

End point description:

The Damage Score (DS) was calculated as a sum of the total poilkiloderma score (POLS), total calcinosis score (CALS) and Gotorn's hands damage score (GHDS). The POLS characterizes specific dispigmentation in the particulal area and calcinosis score characterizes calcification of the skin in the particular area. The POLS and the CALS are summed up over 15 individual areas in the body and each of them has range 0-15. The GHDS has the range 0-2 so that the DS has the range 0-32.

End point type	Secondary

End point timeframe:

Baseline, Week 1, Week 4, Week 8 and Week 12

End point values	Placebo (Stage 1)	PF-06823859 600 mg intravenous (IV) (Stage 1)	Placebo (Stage 2)	PF-06823859 150 mg IV (Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	22	1	5
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	4.30 (± 5.397)	5.50 (± 3.901)	3.00 (± 99999)	4.20 (± 3.701)
Week 1	4.90 (± 5.021)	5.41 (± 4.159)	4.00 (± 99999)	3.80 (± 3.347)
Week 4	5.11 (± 5.278)	5.23 (± 3.337)	4.00 (± 99999)	3.00 (± 2.449)
Week 8	5.89 (± 5.231)	5.00 (± 4.427)	5.00 (± 99999)	4.40 (± 2.881)
Week 12	6.33 (± 5.679)	5.05 (± 3.905)	6.00 (± 99999)	3.60 (± 3.286)

End point values	PF-06823859 600 mg IV (Stage 2)	Placebo then PF-06823859 150 mg IV (Amended Stage 2)	Placebo then PF-06823859 600 mg IV (Amended Stage 2)	PF-06823859 150 mg IV then Placebo (Amended Stage 2)
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	2	1	10
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	7.00 (± 1.732)	7.50 (± 4.950)	7.00 (± 99999)	4.70 (± 4.373)
Week 1	6.33 (± 3.215)	6.00 (± 7.071)	7.00 (± 99999)	3.90 (± 3.542)
Week 4	9.00 (± 1.732)	7.00 (± 5.657)	7.00 (± 99999)	4.40 (± 4.248)
Week 8	8.33 (± 0.577)	5.50 (± 3.536)	7.00 (± 99999)	3.70 (± 3.945)
Week 12	6.33 (± 1.528)	4.50 (± 4.950)	7.00 (± 99999)	3.10 (± 4.358)

End point values	PF-06823859 600 mg IV	Placebo then PF-06823859	PF-06823859 600 mg IV	

	then Placebo (Amended Stage 2)	600 mg IV (Stage 3)	then placebo (Stage 3)	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	3	9	9	
Units: Units on a scale				
arithmetic mean (standard deviation)				
Baseline	7.00 (± 3.000)	5.56 (± 6.044)	2.00 (± 1.871)	
Week 1	4.67 (± 2.887)	6.00 (± 5.809)	1.89 (± 1.764)	
Week 4	6.33 (± 1.528)	5.22 (± 4.631)	1.56 (± 2.297)	
Week 8	4.67 (± 3.215)	5.11 (± 4.859)	1.00 (± 1.118)	
Week 12	5.00 (± 1.000)	5.22 (± 5.019)	1.33 (± 1.581)	

Statistical analyses

No statistical analyses for this end point

Secondary: Absolute Values for Total Improvement Score (TIS) at Week 12 and Intermediate Scheduled Time Points (Stage 3)

End point title	Absolute Values for Total Improvement Score (TIS) at Week 12 and Intermediate Scheduled Time Points (Stage 3)[20]

End point description:

The TIS was the sum of all 6 improvement scores (PhGA [from the MDAAT], PtGA, MMT, HAQ-DI, muscle enzymes, and extramuscular global assessment) associated with the change in each core set measure. A total improvement score of ≥20 represented minimal improvement, a score of ≥40 represented moderate improvement, and a score of ≥60 represented major improvement.

End point type	Secondary

End point timeframe:

Week 4, Week 8 and Week 12

Notes:

[20] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This secondary endpoint is only for arms in Stage 3.

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Units on a scale				
least squares mean (confidence interval 90%)				
Week 4	25.83 (13.15 to 38.52)	36.67 (23.98 to 49.35)		
Week 8	36.67 (23.38 to 49.95)	49.17 (35.88 to 62.45)		
Week 12	36.94 (21.93 to 51.96)	56.39 (41.38 to 71.40)		

Statistical analyses

Statistical analysis title	PF-06823859 600 mg vs Placebo at Week 4
Statistical analysis description:	
Difference of the active treatment from Placebo at Week 4	
Comparison groups	Placebo then PF-06823859 600 mg IV (Stage 3) v PF-06823859 600 mg IV then placebo (Stage 3)
Number of subjects included in analysis	18
Analysis specification	Pre-specified
Analysis type	other[21]
P-value	= 0.1537
Method	Mixed models analysis
Parameter estimate	Least Squares Mean Difference
Point estimate	10.83
Confidence interval	
level	90 %
sides	2-sided
lower limit	-7.1
upper limit	28.77
Variability estimate	Standard error of the mean
Dispersion value	10.274

Notes:

[21] - Subjects in the arm of "Placebo then PF-06823859 600 mg IV (Stage 3)" received placebo on Day 1, Weeks 4, 8 and 12 with a treatment switch to PF-06823859 at Week 12. Subjects in the arm of "PF-06823859 600 mg IV then placebo (Stage 3)" received PF-06823859 on Day 1, Weeks 4, 8 and 12 with a treatment switch to placebo at Week 12.

Statistical analysis title	PF-06823859 600 mg vs Placebo at Week 12
Statistical analysis description:	
Difference of the active treatment from Placebo at Week 12	
Comparison groups	Placebo then PF-06823859 600 mg IV (Stage 3) v PF-06823859 600 mg IV then placebo (Stage 3)
Number of subjects included in analysis	18
Analysis specification	Pre-specified
Analysis type	
P-value	= 0.0647
Method	Mixed models analysis
Parameter estimate	Least Squares Mean Difference
Point estimate	19.44
Confidence interval	
level	90 %
sides	2-sided
lower limit	-1.79
upper limit	40.68
Variability estimate	Standard error of the mean
Dispersion value	12.161

Statistical analysis title	PF-06823859 600 mg vs Placebo at Week 8
Statistical analysis description:	
Difference of the active treatment from Placebo at Week 8	
Comparison groups	Placebo then PF-06823859 600 mg IV (Stage 3) v PF-

	06823859 600 mg IV then placebo (Stage 3)
Number of subjects included in analysis	18
Analysis specification	Pre-specified
Analysis type	other[22]
P-value	= 0.1312
Method	Mixed models analysis
Parameter estimate	Least Squares Mean Difference
Point estimate	12.5
Confidence interval	
level	90 %
sides	2-sided
lower limit	-6.29
upper limit	31.29
Variability estimate	Standard error of the mean
Dispersion value	10.761

Notes:

[22] - Subjects in the arm of "Placebo then PF-06823859 600 mg IV (Stage 3)" received placebo on Day 1, Weeks 4, 8 and 12 with a treatment switch to PF-06823859 at Week 12. Subjects in the arm of "PF-06823859 600 mg IV then placebo (Stage 3)" received PF-06823859 on Day 1, Weeks 4, 8 and 12 with a treatment switch to placebo at Week 12.

Secondary: Change From Baseline in the Core Set Measures (CSM) of the TIS (Stage 3)

End point title	Change From Baseline in the Core Set Measures (CSM) of the TIS (Stage 3)

End point description:

The TIS was the sum of all 6 improvement scores (PhGA [from the MDAAT], PtGA, MMT, HAQ-DI, muscle enzymes, and extramuscular global assessment) associated with the change in each core set measure. A total improvement score of ≥20 represented minimal improvement, a score of ≥40 represented moderate improvement, and a score of ≥60 represented major improvement.

End point type	Secondary

End point timeframe:

Week 4, Week 8 and Week 12

End point values	Placebo then PF-06823859 600 mg IV (Stage 3)	PF-06823859 600 mg IV then placebo (Stage 3)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	9		
Units: Units on a scale				
least squares mean (confidence interval 90%)				
PhGA (CM) Week 4	-1.00 (-1.89 to -0.11)	-1.68 (-2.57 to -0.79)		
PhGA (CM) Week 8	-1.65 (-2.80 to -0.51)	-2.76 (-3.90 to -1.61)		
PhGA (CM) Week 12	-2.05 (-3.56 to -0.55)	-3.40 (-4.90 to -1.90)		
PtGA Week 4	-18.29 (-31.78 to -4.79)	-17.88 (-30.75 to -5.00)		
PtGA Week 8	-16.81 (-29.88 to -3.73)	-32.60 (-45.85 to -19.35)		

PtGA Week 12	-14.04 (-25.91 to -2.17)	-43.81 (-55.68 to -31.95)		
MMT8 Total Score - Derived Week 4	7.76 (1.37 to 14.16)	8.24 (1.84 to 14.63)		
MMT8 Total Score - Derived Week 8	12.14 (4.60 to 19.68)	15.24 (7.70 to 22.78)		
MMT8 Total Score - Derived Week 12	11.65 (2.29 to 21.01)	21.24 (11.87 to 30.60)		
HAQ01-HAQ-DI Score Week 4	-0.03 (-0.24 to 0.18)	-0.20 (-0.39 to 0.00)		
HAQ01-HAQ-DI Score Week 8	0.00 (-0.28 to 0.28)	-0.38 (-0.64 to -0.11)		
HAQ01-HAQ-DI Score Week 12	-0.06 (-0.40 to 0.28)	-0.52 (-0.84 to -0.19)		
Extramuscular Global Assessment Week 4	-1.02 (-2.07 to 0.03)	-1.55 (-2.60 to -0.49)		
Extramuscular Global Assessment Week 8	-1.91 (-2.80 to -1.01)	-2.48 (-3.38 to -1.58)		
Extramuscular Global Assessment Week 12	-1.59 (-2.64 to -0.53)	-2.81 (-3.87 to -1.76)		
Aldolase (U/L) Week 4	-0.19 (-1.34 to 0.96)	-3.09 (-4.25 to -1.92)		
Aldolase (U/L) Week 8	-1.31 (-2.45 to -0.16)	-3.57 (-4.74 to -2.40)		
Aldolase (U/L) Week 12	-0.66 (-2.18 to 0.86)	-3.20 (-4.76 to -1.64)		
Creatine Kinase (U/L) Week 4	-37.96 (-119.16 to 43.24)	-157.48 (-238.68 to -76.28)		
Creatine Kinase (U/L) Week 8	-70.96 (-137.03 to -4.90)	-175.93 (-241.99 to -109.86)		
Creatine Kinase (U/L) Week 12	-39.85 (-125.71 to 46.01)	-185.77 (-273.92 to -97.62)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Up to Week 40	
Adverse event reporting additional description:	
The safety analysis set includes all participants who received at least one dose of randomized treatment in any stage. The AEs reported were treatment emergent.	
Assessment type	Non-systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	25.1

Reporting groups	
Reporting group title	Placebo
Reporting group description:	
Participants who received placebo in any stage.	
Reporting group title	Total
Reporting group description:	
Treatment Group Description TBD	
Reporting group title	PF-06823859 600 mg IV
Reporting group description:	
Participants who received PF-06823859 600 mg IV in any stage.	
Reporting group title	PF-06823859 150 mg IV
Reporting group description:	
Participants who received PF-06823859 150 mg IV in any stage.	

Serious adverse events	Placebo	Total	PF-06823859 600 mg IV
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 45 (6.67%)	5 / 75 (6.67%)	4 / 47 (8.51%)
number of deaths (all causes)	1	1	1
number of deaths resulting from adverse events	1	1	0
Investigations			
Blood bilirubin increased			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatic enzyme increased			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			

Humerus fracture			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	1 / 47 (2.13%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Central nervous system lesion			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Cytopenia			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Leukopenia			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	1 / 47 (2.13%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune system disorders			
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 1	0 / 0
Gastrointestinal disorders			
Colitis microscopic			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	1 / 47 (2.13%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Ocular icterus			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			

Osteoarthritis			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	1 / 47 (2.13%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	PF-06823859 150 mg IV		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 17 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Investigations			
Blood bilirubin increased			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatic enzyme increased			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Humerus fracture			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Central nervous system lesion			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Cytopenia			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Leukopenia			

subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Immune system disorders			
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Colitis microscopic			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Ocular icterus			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Osteoarthritis			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Placebo	Total	PF-06823859 600 mg IV
Total subjects affected by non-serious adverse events			
subjects affected / exposed	22 / 45 (48.89%)	45 / 75 (60.00%)	18 / 47 (38.30%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Parathyroid tumour benign			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
General disorders and administration site conditions			

Fatigue			
subjects affected / exposed	4 / 45 (8.89%)	6 / 75 (8.00%)	1 / 47 (2.13%)
occurrences (all)	4	6	1
Reproductive system and breast disorders			
Vaginal haemorrhage			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Respiratory, thoracic and mediastinal disorders			
Upper-airway cough syndrome			
subjects affected / exposed	0 / 45 (0.00%)	2 / 75 (2.67%)	1 / 47 (2.13%)
occurrences (all)	0	2	1
Investigations			
Blood potassium decreased			
subjects affected / exposed	0 / 45 (0.00%)	2 / 75 (2.67%)	1 / 47 (2.13%)
occurrences (all)	0	2	1
SARS-CoV-2 test positive			
subjects affected / exposed	4 / 45 (8.89%)	4 / 75 (5.33%)	1 / 47 (2.13%)
occurrences (all)	4	4	1
Weight increased			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	1	1	0
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	0 / 45 (0.00%)	3 / 75 (4.00%)	2 / 47 (4.26%)
occurrences (all)	0	3	2
Infusion related reaction			
subjects affected / exposed	2 / 45 (4.44%)	2 / 75 (2.67%)	0 / 47 (0.00%)
occurrences (all)	2	2	0
Skin laceration			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	1	1	0
Nervous system disorders			

Headache			
subjects affected / exposed	7 / 45 (15.56%)	15 / 75 (20.00%)	6 / 47 (12.77%)
occurrences (all)	8	20	9
Hypoaesthesia			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Mental impairment			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	2 / 45 (4.44%)	5 / 75 (6.67%)	3 / 47 (6.38%)
occurrences (all)	2	5	3
Diarrhoea			
subjects affected / exposed	0 / 45 (0.00%)	4 / 75 (5.33%)	3 / 47 (6.38%)
occurrences (all)	0	4	3
Abdominal pain			
subjects affected / exposed	0 / 45 (0.00%)	2 / 75 (2.67%)	1 / 47 (2.13%)
occurrences (all)	0	2	1
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	5 / 45 (11.11%)	6 / 75 (8.00%)	1 / 47 (2.13%)
occurrences (all)	5	6	1
Rosacea			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Alopecia			
subjects affected / exposed	1 / 45 (2.22%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	1	1	0
Dermatomyositis			
subjects affected / exposed	1 / 45 (2.22%)	2 / 75 (2.67%)	0 / 47 (0.00%)
occurrences (all)	1	2	0
Musculoskeletal and connective tissue			

disorders			
Neck pain			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Myalgia			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Arthralgia			
subjects affected / exposed	1 / 45 (2.22%)	5 / 75 (6.67%)	3 / 47 (6.38%)
occurrences (all)	1	5	3
Infections and infestations			
Sinusitis			
subjects affected / exposed	3 / 45 (6.67%)	4 / 75 (5.33%)	0 / 47 (0.00%)
occurrences (all)	4	5	0
Tooth abscess			
subjects affected / exposed	0 / 45 (0.00%)	1 / 75 (1.33%)	0 / 47 (0.00%)
occurrences (all)	0	1	0
Upper respiratory tract infection			
subjects affected / exposed	1 / 45 (2.22%)	5 / 75 (6.67%)	4 / 47 (8.51%)
occurrences (all)	1	6	5

Non-serious adverse events	PF-06823859 150 mg IV		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	13 / 17 (76.47%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Parathyroid tumour benign			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Reproductive system and breast disorders			
Vaginal haemorrhage			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		

Respiratory, thoracic and mediastinal disorders			
Upper-airway cough syndrome			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Investigations			
Blood potassium decreased			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
SARS-CoV-2 test positive			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences (all)	0		
Weight increased			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Infusion related reaction			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Skin laceration			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Cardiac disorders			
Tachycardia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Nervous system disorders			
Headache			
subjects affected / exposed	3 / 17 (17.65%)		
occurrences (all)	3		
Hypoaesthesia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Mental impairment			

subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	0 / 17 (0.00%)		
occurrences (all)	0		
Diarrhoea			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Abdominal pain			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Rosacea			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Alopecia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Dermatomyositis			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
Neck pain			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Myalgia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		

Arthralgia subjects affected / exposed occurrences (all)	2 / 17 (11.76%) 2		
Infections and infestations Sinusitis subjects affected / exposed occurrences (all) Tooth abscess subjects affected / exposed occurrences (all) Upper respiratory tract infection subjects affected / exposed occurrences (all)	 1 / 17 (5.88%) 1 1 / 17 (5.88%) 1 0 / 17 (0.00%) 0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
20 September 2021	The overall rationale for the amendment is to permit participants actively enrolled in the C0251002 study to have the option to continue active treatment into a long term open label extension study, known as protocol C0251008.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported