Clinical trial results:
Phase 3 Extension Study of Ataluren (PTC124) in Patients with Nonsense Mutation Cystic Fibrosis

Summary

EudraCT number	2014-005355-83
Trial protocol	BE IT ES NL FR BG GR GB
Global end of trial date	02 June 2017

Results information

Result version number	v1 (current)
This version publication date	30 April 2020
First version publication date	30 April 2020

Trial information

Trial identification

Sponsor protocol code	PTC124-GD-021e-CF

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02456103
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	PTC Therapeutics, Inc.
Sponsor organisation address	100 Corporate Court, South Plainfield, United States, NJ 07080
Public contact	Medical Information, PTC Therapeutics, Inc., +011 44 1-866-562-4620, medinfo@ptcbio.com
Scientific contact	Medical Information, PTC Therapeutics, Inc., +353 19068700, medinfo@ptcbio.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-000115-PIP02-09
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	28 July 2017
Is this the analysis of the primary completion data?	Yes
Primary completion date	02 June 2017
Global end of trial reached?	Yes
Global end of trial date	02 June 2017
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this Phase 3 extension study was to obtain long-term safety data to augment the overall safety database. The secondary objectives was to augment the efficacy data collected in the double-blind study (PTC124-GD-021-CF; NCT02139306).

Protection of trial subjects:

The study was conducted in accordance with the ethical principles that have their origins in the Declaration of Helsinki (revised version of Edinburgh, Scotland, 2000) and in conformance with the International Council for Harmonisation (ICH) Good Clinical Practice (GCP) guidance documents.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	31 August 2015
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	Argentina: 6
Country: Number of subjects enrolled	Australia: 14
Country: Number of subjects enrolled	Belgium: 6
Country: Number of subjects enrolled	Brazil: 1
Country: Number of subjects enrolled	Bulgaria: 6
Country: Number of subjects enrolled	Canada: 6
Country: Number of subjects enrolled	France: 19
Country: Number of subjects enrolled	Germany: 19
Country: Number of subjects enrolled	Greece: 5
Country: Number of subjects enrolled	Israel: 16
Country: Number of subjects enrolled	Italy: 33
Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Poland: 14
Country: Number of subjects enrolled	Spain: 23
Country: Number of subjects enrolled	United Kingdom: 2
Country: Number of subjects enrolled	United States: 73
Worldwide total number of subjects	246
EEA total number of subjects	130

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)	28
Adolescents (12-17 years)	68
Adults (18-64 years)	150
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

All eligible participants, including those who received placebo in the double-blind study (PTC124-GD-021-CF; NCT02139306), were enrolled to this open-label extension study. To avoid interruption in treatment, when possible, Screening/Baseline for this extension study was to occur on the same day as the End-of-Study visit for the double-blind study.

Period 1

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

Arms

Arm title	Ataluren

Arm description:

Participants were administered ataluren orally at a dose of 10 milligrams/grams (mg/kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening for up to 96 weeks.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	PTC124
Other name	Translarna
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

Ataluren will be provided as a vanilla-flavored powder to be mixed with water or milk.

Number of subjects in period 1	Ataluren
Started	246
As-Treated Population	245
Intent-to-treat (ITT) Population	244
Completed	0
Not completed	246
Adverse event, serious fatal	1
Physician decision	1
Consent withdrawn by subject	24
Study Closure	207
Adverse event, non-fatal	4
Abnormal Laboratory Value	2
Required prohibited medications	5
Lost to follow-up	1

Protocol deviation	1

Baseline characteristics

Reporting groups

Reporting group title	Overall Study

Reporting group description: -

Reporting group values	Overall Study	Total	
Number of subjects	246	246	
Age categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	28	28	
Adolescents (12-17 years)	68	68	
Adults (18-64 years)	150	150	
From 65-84 years	0	0	
85 years and over	0	0	
Age Continuous Units: years			
arithmetic mean	23.12		
standard deviation	± 10.86	-	
Sex: Female, Male Units: participants			
Female	113	113	
Male	133	133	
Race/Ethnicity, Customized Units: Subjects			
Asian	4	4	
White	236	236	
White-Arabic/North African Heritage	1	1	
Non-White	5	5	

Subject analysis sets

Subject analysis set title	Ataluren (As-Treated Population)
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants who received at least 1 dose of ataluren.

Subject analysis set title	Ataluren (ITT Population)
Subject analysis set type	Intention-to-treat

Subject analysis set description:

Participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment.

Reporting group values	Ataluren (As-Treated Population)	Ataluren (ITT Population)	
Number of subjects	245	244	
Age categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	28	28	
Adolescents (12-17 years)	67	67	
Adults (18-64 years)	150	149	
From 65-84 years	0	0	
85 years and over	0	0	
Age Continuous Units: years			
arithmetic mean	23.1	23.2	
standard deviation	± 10.88	± 10.9	
Sex: Female, Male Units: participants			
Female	113	112	
Male	132	132	
Race/Ethnicity, Customized Units: Subjects			
Asian	4	4	
White	235	234	
White-Arabic/North African Heritage	1	1	
Non-White	5	5	

End points

End points reporting groups

Reporting group title	Ataluren
Reporting group description:	
Participants were administered ataluren orally at a dose of 10 milligrams/grams (mg/kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening for up to 96 weeks.	
Subject analysis set title	Ataluren (As-Treated Population)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants who received at least 1 dose of ataluren.	
Subject analysis set title	Ataluren (ITT Population)
Subject analysis set type	Intention-to-treat
Subject analysis set description:	
Participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment.	

Primary: Number of Participants With Treatment Emergent Adverse Events (TEAEs)

End point title	Number of Participants With Treatment Emergent Adverse Events (TEAEs)[1]
End point description:	
TEAE: any untoward medical occurrence or undesirable event that begins or worsens following administration of study drug, whether or not considered related to study drug by Investigator. Serious adverse event (SAE): an adverse event (AE) resulting in any of following outcomes or deemed significant for any other reason: death, initial or prolonged inpatient hospitalization, life-threatening experience (immediate risk of dying) or persistent or significant disability/incapacity. Except for cystic fibrosis (CF) pulmonary exacerbations, an event wasn't reported as an SAE, if event was exclusively a relapse or an expected change or progression of baseline CF. AEs included both SAEs and nonserious AEs. AEs classified according to NCI CTCAE v3.0 and coded using MedDRA. Population included participants who received at least 1 dose of ataluren (As-Treated Population). A summary of SAEs and all nonserious AEs, regardless of causality, is located in the Reported Adverse Events section.	
End point type	Primary
End point timeframe:	
Baseline up to Week 100	

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Statistical analyses not applicable for this endpoint.

End point values	Ataluren (As-Treated Population)			
Subject group type	Subject analysis set			
Number of subjects analysed	245			
Units: participants				
At least 1 TEAE	222			
Mild TEAE	28			
Moderate TEAE	147			
Severe TEAE	46			
Life-Threatening TEAE	1			
Fatal TEAE	0			
Serious TEAE	89			
TEAE Unrelated to Study Drug	140			
TEAE Unlikely Related to Study Drug	55			
TEAE Possibly Related to Study Drug	23			

TEAE Probably Related to Study Drug	4			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With a Clinically Meaningful Abnormal Clinical Laboratory (Serum Biochemistry, Hematology, and Urinalysis) Parameter

End point title	Number of Participants With a Clinically Meaningful Abnormal Clinical Laboratory (Serum Biochemistry, Hematology, and Urinalysis) Parameter[2]

End point description:

Clinical laboratory results considered clinically meaningful were determined by Investigator. Serum biochemistry parameters: sodium, potassium, chloride, bicarbonate, blood urea nitrogen, creatinine, magnesium, calcium, phosphorus, uric acid, glucose, total protein, albumin, globulin, bilirubin, creatine kinase, lactate dehydrogenase, ALT, AST, gamma glutamyl transferase, ALP, total cholesterol, high-density lipoprotein, low-density lipoprotein, triglycerides, cystatin C. Hematology parameters: WBC count, hemoglobin, hematocrit, mean corpuscular volume, mean corpuscular hemoglobin, red cell count with morphology, platelet count. Urinalysis parameters: pH, specific gravity, glucose, ketones, blood, protein, creatinine, urobilinogen, bilirubin, nitrite, leukocyte esterase. Population included participants who received at least 1 dose of ataluren (As-Treated Population). A summary of all SAEs/nonserious AEs, regardless of causality, is located in the Reported Adverse Events section.

End point type	Primary

End point timeframe:

Baseline up to Week 100

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: Statistical analyses not applicable for this endpoint.

End point values	Ataluren (As-Treated Population)			
Subject group type	Subject analysis set			
Number of subjects analysed	245			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Percent-Predicted Forced Expiratory Volume in 1 Second (FEV1) as Measured by Spirometry at Week 24

End point title	Change From Baseline in Percent-Predicted Forced Expiratory Volume in 1 Second (FEV1) as Measured by Spirometry at Week 24

End point description:

Pulmonary function of percent-predicted FEV1 was measured using a spirometer. FEV1 is the volume of air that can forcibly be blown out in 1 second. Each percent-predicted FEV1 was based gender, age, and the height value obtained at the same study visit. The percentage of change in percent-predicted of

FEV1 was calculated as follows: (percent-predicted FEV1 - Baseline percent-predicted FEV1/Baseline percent-predicted FEV1)*100. Population included participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment (ITT Population) and had evaluable FEV1 data.

End point type	Secondary

End point timeframe:

Baseline, Week 24

End point values	Ataluren (ITT Population)			
Subject group type	Subject analysis set			
Number of subjects analysed	233			
Units: percentage of predicted FEV1				
arithmetic mean (standard deviation)				
Baseline (n=233)	60.219 (± 17.6163)			
Change from Baseline at Week 24 (n=205)	0.015 (± 6.7180)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Percent-Predicted of Forced Vital Capacity (FVC) as Measured by Spirometry at Week 24

End point title	Change From Baseline in Percent-Predicted of Forced Vital Capacity (FVC) as Measured by Spirometry at Week 24

End point description:

Pulmonary function of FVC was measured using a spirometer. FVC is the volume of air that can forcibly be blown out. Each percent-predicted FVC was based gender, age, and the height value obtained at the same study visit. The percentage of change in percent-predicted of FVC was calculated as follows: (percent-predicted FVC - Baseline percent-predicted FVC/Baseline percent-predicted FVC)*100. Population included participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment (ITT Population) and had evaluable FVC data.

End point type	Secondary

End point timeframe:

Baseline, Week 24

End point values	Ataluren (ITT Population)			
Subject group type	Subject analysis set			
Number of subjects analysed	233			
Units: percentage of predicted FVC				
arithmetic mean (standard deviation)				
Baseline (n=233)	75.249 (± 15.8508)			
Change from Baseline at Week 24 (n=205)	0.166 (± 6.5276)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Forced Expiratory Flow Between 25% and 75% of Expiration (FEF25-75) as Measured by Spirometry at Week 24

End point title	Change From Baseline in Forced Expiratory Flow Between 25% and 75% of Expiration (FEF25-75) as Measured by Spirometry at Week 24

End point description:

Pulmonary function of FEF25-75 was measured using a spirometer. FEF25-75 is the forced expiratory flow between 25% and 75% of vital capacity. Each percent-predicted FEF25-75 was based gender, age, and the height value obtained at the same study visit. The percentage of change in percent-predicted of FEF25-75 was calculated as follows: (percent-predicted FEF25-75 - Baseline percent-predicted FEF25-75/Baseline percent-predicted FEF25-75)*100. Participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment (ITT Population) and had evaluable FEF25-75 data.

End point type	Secondary

End point timeframe:

Baseline, Week 24

End point values	Ataluren (ITT Population)			
Subject group type	Subject analysis set			
Number of subjects analysed	233			
Units: percentage of FEF25-75				
arithmetic mean (standard deviation)				
Baseline (n=233)	38.099 (± 22.0980)			
Change from Baseline at Week 24 (n=205)	0.698 (± 13.1241)			

Statistical analyses

No statistical analyses for this end point

Secondary: Rate of Pulmonary Exacerbations as Defined by Modified Fuch's Criteria Over 48 Weeks

End point title	Rate of Pulmonary Exacerbations as Defined by Modified Fuch's Criteria Over 48 Weeks

End point description:

A modified Fuchs' exacerbation was defined as an event requiring treatment with or without intravenous antibiotics for any 4 of the following 12 symptoms: change in sputum; new or increased hemoptysis; increased cough; increased dyspnea; fatigue; temperature >38°C; anorexia; sinus pain; change in sinus discharge; change in physical examination of the chest; decrease in pulmonary function by 10 percent

or more from a previously recorded value; or radiographic changes indicative of pulmonary function. The 48-week rate = (the total number of events/ treatment duration by week)*48. Population included participants who received at least 1 dose of ataluren and have at least 1 postbaseline efficacy assessment (ITT Population).

End point type	Secondary

End point timeframe:

Baseline up to Week 48

End point values	Ataluren (ITT Population)			
Subject group type	Subject analysis set			
Number of subjects analysed				
Units: exacerbations				
arithmetic mean (standard deviation)	1.051 (± 2.1654)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Baseline up to Week 100	
Adverse event reporting additional description:	
Adverse events collected from participants who received at least 1 dose of ataluren (As-Treated Population).	
Assessment type	Systematic

Dictionary used	
Dictionary name	17.0
Dictionary version	17.0

Reporting groups	
Reporting group title	Ataluren
Reporting group description:	
Participants were administered ataluren orally at a dose of 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening for up to 96 weeks.	

Serious adverse events	Ataluren		
Total subjects affected by serious adverse events			
subjects affected / exposed	89 / 245 (36.33%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events	1		
Investigations			
Forced expiratory volume decreased			
subjects affected / exposed	2 / 245 (0.82%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Protein urine present			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pseudomonas test positive			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pulmonary function test decreased			

subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Squamous cell carcinoma of the cervix	Additional description: This is a sex-specific adverse event that only affects female participants.		
subjects affected / exposed[1]	1 / 113 (0.88%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Congenital, familial and genetic disorders			
Cystic fibrosis lung			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Depressed level of consciousness			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pregnancy, puerperium and perinatal conditions			
Pregnancy	Additional description: This is a sex-specific adverse event that only affects female participants.		
subjects affected / exposed[2]	1 / 113 (0.88%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Abdominal pain lower			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Distal intestinal obstruction syndrome			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Duodenal ulcer			

subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Enteritis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Intestinal obstruction				
subjects affected / exposed	3 / 245 (1.22%)			
occurrences causally related to treatment / all	0 / 3			
deaths causally related to treatment / all	0 / 0			
Pancreatitis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	1 / 1			
deaths causally related to treatment / all	0 / 0			
Respiratory, thoracic and mediastinal disorders				
Acute respiratory failure				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 1			
Aspiration				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Asthma				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Dyspnoea exertional				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Haemoptysis				

subjects affected / exposed	4 / 245 (1.63%)			
occurrences causally related to treatment / all	0 / 4			
deaths causally related to treatment / all	0 / 0			
Renal and urinary disorders				
Nephrolithiasis				
subjects affected / exposed	2 / 245 (0.82%)			
occurrences causally related to treatment / all	1 / 2			
deaths causally related to treatment / all	0 / 0			
Renal colic				
subjects affected / exposed	2 / 245 (0.82%)			
occurrences causally related to treatment / all	2 / 2			
deaths causally related to treatment / all	0 / 0			
Renal failure acute				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Infections and infestations				
Bronchopulmonary aspergillosis allergic				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Device related infection				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Gastroenteritis viral				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Infective pulmonary exacerbation of cystic fibrosis				
subjects affected / exposed	63 / 245 (25.71%)			
occurrences causally related to treatment / all	0 / 85			
deaths causally related to treatment / all	0 / 0			

Mycobacterium abscessus infection				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Peritonitis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Pneumonia				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Pseudomonas infection				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Respiratory syncytial virus infection				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Sinusitis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Viral pericarditis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Appendicitis				
subjects affected / exposed	1 / 245 (0.41%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Metabolism and nutrition disorders				

Dehydration			
subjects affected / exposed	1 / 245 (0.41%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Notes:

[1] - The number of subjects exposed to this adverse event is less than the total number of subjects exposed to this adverse event. These numbers are expected to be equal.
Justification: This is a sex-specific adverse event that only affects female participants.

[2] - The number of subjects exposed to this adverse event is less than the total number of subjects exposed to this adverse event. These numbers are expected to be equal.
Justification: This is a sex-specific adverse event that only affects female participants.

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

Non-serious adverse events	Ataluren		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	191 / 245 (77.96%)		
Investigations			
Forced expiratory volume decreased			
subjects affected / exposed	16 / 245 (6.53%)		
occurrences (all)	18		
Nervous system disorders			
Headache			
subjects affected / exposed	16 / 245 (6.53%)		
occurrences (all)	28		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	13 / 245 (5.31%)		
occurrences (all)	14		
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	14 / 245 (5.71%)		
occurrences (all)	18		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	32 / 245 (13.06%)		
occurrences (all)	47		
Haemoptysis			
subjects affected / exposed	14 / 245 (5.71%)		
occurrences (all)	35		
Infections and infestations			

Infective pulmonary exacerbation of cystic fibrosis			
subjects affected / exposed	136 / 245 (55.51%)		
occurrences (all)	224		
Influenza			
subjects affected / exposed	13 / 245 (5.31%)		
occurrences (all)	16		
Sinusitis			
subjects affected / exposed	25 / 245 (10.20%)		
occurrences (all)	29		
Upper respiratory tract infection			
subjects affected / exposed	21 / 245 (8.57%)		
occurrences (all)	26		
Viral upper respiratory tract infection			
subjects affected / exposed	30 / 245 (12.24%)		
occurrences (all)	40		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

This study was terminated early because the CF data from the double-blind CF Study PTC124-GD-021-CF (NCT02139306) did not meet endpoints.

Notes: