Clinical trial results:
A Phase 2, Randomized, Double-blind, Placebo-controlled, 2-part Study to Evaluate EDP-938 Regimens In Subjects Aged 28 Days to 36 Months Infected with Respiratory Syncytial Virus (RSV)

Summary

EudraCT number	2020-001966-13
Trial protocol	DE PL ES RO
Global end of trial date	19 August 2024

Results information

Result version number	v1
This version publication date	06 March 2025
First version publication date	06 March 2025

Trial information

Trial identification

Sponsor protocol code	EDP 938-201

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04816721
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Enanta Pharmaceuticals, Inc.
Sponsor organisation address	4 Kingsbury Ave, Watertown, MA, United States, 02472
Public contact	Medical Monitor, Enanta Pharmaceuticals, Inc., +1 6176070800, enquiries@enanta.com
Scientific contact	Medical Monitor, Enanta Pharmaceuticals, Inc., +1 6176070800, enquiries@enanta.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-003609-PIP01-24
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	19 August 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	19 August 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objectives of Part 1 of the study were to evaluate the pharmacokinetics (PK) of EDP-938 and to assess the safety and tolerability of EDP-938. The main objective of Part 2 of the study was to evaluate the antiviral activity of EDP-938.

Protection of trial subjects:

The study was conducted in compliance with the protocol, principles of E6 Good Clinical Practice: Consolidated Guidance (ICH-GCP), Declaration of Helsinki, and all applicable local laws and regulations governing clinical studies.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	26 April 2022
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	United States: 29
Country: Number of subjects enrolled	Spain: 23
Country: Number of subjects enrolled	Poland: 3
Country: Number of subjects enrolled	Argentina: 8
Country: Number of subjects enrolled	South Africa: 8
Country: Number of subjects enrolled	Taiwan: 8
Country: Number of subjects enrolled	Mexico: 5
Country: Number of subjects enrolled	Israel: 7
Country: Number of subjects enrolled	Brazil: 4
Country: Number of subjects enrolled	Romania: 2
Country: Number of subjects enrolled	Australia: 2
Worldwide total number of subjects	99
EEA total number of subjects	28

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)	87
Children (2-11 years)	12
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

A total of 99 participants were enrolled at 78 sites across 15 countries between April 2022 and August 2024.

Pre-assignment

Screening details:

Out of the 99 participants that were enrolled, 52 participants received either EDP-938 or placebo in Part 1 and 44 participants received either EDP-938 or placebo in Part 2.

Period 1

Period 1 title	Overall Study (overall period)
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	Part 1: EDP-938

Arm description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 once daily (QD) from Day 1 to Day 5 of the study.

Arm type	Experimental
Investigational medicinal product name	EDP-938
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

EDP-938 was received orally by mouth, nasogastric tube, or orogastric tube.

Arm title	Part 1: Placebo

Arm description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received placebo matching EDP-938 QD from Day 1 to Day 5 of the study.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

Placebo matching EDP-938 was received orally by mouth, nasogastric tube, or orogastric tube.

Arm title	Part 2: EDP-938

Arm description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 QD from Day 1 to Day 5 of the study.

Arm type	Experimental

Investigational medicinal product name	EDP-938
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

EDP-938 was received orally by mouth, nasogastric tube, or orogastric tube.

Arm title	Part 2: Placebo

Arm description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received placebo matching EDP-938 QD on Day 1 to Day 5 of the study.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

Placebo matching EDP-938 was received orally by mouth, nasogastric tube, or orogastric tube.

Number of subjects in period 1	Part 1: EDP-938	Part 1: Placebo	Part 2: EDP-938
Started	36	17	36
Treated	35	17	34
Completed	34	15	34
Not completed	2	2	2
Consent withdrawn by subject	2	1	1
Miscellaneous	-	-	1
Lost to follow-up	-	1	-

Number of subjects in period 1	Part 2: Placebo
Started	10
Treated	10
Completed	10
Not completed	0
Consent withdrawn by subject	-
Miscellaneous	-
Lost to follow-up	-

Baseline characteristics

Reporting groups	
Reporting group title	Part 1: EDP-938
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 once daily (QD) from Day 1 to Day 5 of the study.	
Reporting group title	Part 1: Placebo
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received placebo matching EDP-938 QD from Day 1 to Day 5 of the study.	
Reporting group title	Part 2: EDP-938
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Reporting group title	Part 2: Placebo
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received placebo matching EDP-938 QD on Day 1 to Day 5 of the study.	

Reporting group values	Part 1: EDP-938	Part 1: Placebo	Part 2: EDP-938
Number of subjects	36	17	36
Age categorical Units: Subjects			
≥ 28 days to < 6 months	14	6	16
≥ 6 months to ≤ 36 months	22	11	20
Gender categorical Units: Subjects			
Female	17	9	19
Male	19	8	17
Race Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	4	0	4
Black or African American	4	7	5
Native Hawaiian or Other Pacific Islander	0	0	0
White	28	6	24
Other	0	2	2
Not Reported	0	2	0
Unknown	0	0	1
Ethnicity Units: Subjects			
Hispanic or Latino	14	6	12
Not Hispanic or Latino	22	11	23
Not Reported	0	0	1

Reporting group values	Part 2: Placebo	Total	
Number of subjects	10	99	
Age categorical Units: Subjects			
≥ 28 days to < 6 months	4	40	
≥ 6 months to ≤ 36 months	6	59	
Gender categorical Units: Subjects			
Female	6	51	
Male	4	48	
Race Units: Subjects			
American Indian or Alaska Native	0	0	
Asian	1	9	
Black or African American	0	16	
Native Hawaiian or Other Pacific Islander	0	0	
White	6	64	
Other	2	6	
Not Reported	1	3	
Unknown	0	1	
Ethnicity Units: Subjects			
Hispanic or Latino	6	38	
Not Hispanic or Latino	4	60	
Not Reported	0	1	

End points

End points reporting groups

Reporting group title	Part 1: EDP-938
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 once daily (QD) from Day 1 to Day 5 of the study.	
Reporting group title	Part 1: Placebo
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received placebo matching EDP-938 QD from Day 1 to Day 5 of the study.	
Reporting group title	Part 2: EDP-938
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Reporting group title	Part 2: Placebo
Reporting group description:	
Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received placebo matching EDP-938 QD on Day 1 to Day 5 of the study.	
Subject analysis set title	Part 1: Lower-dose EDP-938 (≥ 28 days to < 3 months)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received a lower dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Subject analysis set title	Part 1: Lower-dose EDP-938 (≥ 3 months to < 6 months)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received a lower dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Subject analysis set title	Part 1: Lower-dose EDP-938 (≥ 6 months to < 12 months)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received a lower dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Subject analysis set title	Part 1: Lower-dose EDP-938 (≥ 12 months to ≤ 36 months)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received a lower dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Subject analysis set title	Part 1: Higher-dose EDP-938 (≥ 12 months to ≤ 36 months)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received a higher dose of EDP-938 QD from Day 1 to Day 5 of the study.	
Subject analysis set title	Combined EDP-938
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants from Part 1 and Part 2 who received a lower or higher dose of EDP-938 QD from Day 1 to Day 5 of the study were pooled for analysis.	
Subject analysis set title	Combined Placebo
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants from Part 1 and Part 2 who received placebo matching EDP-938 QD from Day 1 to Day 5 of the study were pooled for analysis.	

Primary: Part 1: Concentrations of EDP-938 in Plasma

End point title	Part 1: Concentrations of EDP-938 in Plasma[1]

End point description:

Plasma concentrations of EDP-938 were assessed at the designated time points. 99999 = Data not available.

PK Population: Included all participants who received one full dose of study drug and had samples with quantifiable plasma levels to allow for estimation of PK parameters. Per protocol, data were analyzed per age group and dose received.

End point type	Primary

End point timeframe:

3 hours post-dose on Day 1 and pre-dose on Day 2 (hospitalized participants only), Day 3, and Day 5

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: No comparative statistical analyses were planned.

End point values	Part 1: Lower-dose EDP-938 (≥ 28 days to < 3 months)	Part 1: Lower-dose EDP-938 (≥ 3 months to < 6 months)	Part 1: Lower-dose EDP-938 (≥ 6 months to < 12 months)	Part 1: Lower-dose EDP-938 (≥ 12 months to ≤ 36 months)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	9[2]	4[3]	7[4]	7[5]
Units: ng/mL				
arithmetic mean (standard deviation)				
Day 1	1132.222 (± 397.4289)	1989.500 (± 1331.5646)	1819.286 (± 809.9508)	1420.000 (± 604.8140)
Day 2	368.400 (± 149.8741)	399.250 (± 423.9515)	502.350 (± 379.6487)	249.675 (± 294.5416)
Day 3	448.000 (± 229.1026)	99999 (± 99999)	346.000 (± 54.7449)	97.533 (± 64.9043)
Day 5	528.563 (± 284.6010)	201.750 (± 98.6623)	431.429 (± 637.5664)	252.043 (± 329.2155)

Notes:

[2] - Day 1 N = 9
Day 2 N = 7
Day 3 N = 2
Day 5 N = 7
[3] - Day 1 N = 4
Day 2 N = 4
Day 3 N = 0
Day 5 N = 4
[4] - Day 1 N = 7
Day 2 N = 4
Day 3 N = 3
Day 5 N = 7
[5] - Day 1 N = 6
Day 2 N = 4
Day 3 N = 3
Day 5 N = 7

End point values	Part 1: Higher-dose EDP-938 (≥ 12 months to ≤ 36 months)			
Subject group type	Subject analysis set			
Number of subjects analysed	9[6]			
Units: ng/mL				

arithmetic mean (standard deviation)				
Day 1	1428.250 (± 817.4801)			
Day 2	238.900 (± 239.0287)			
Day 3	116.250 (± 43.4871)			
Day 5	180.622 (± 158.3268)			

Notes:

[6] - Day 1 N = 8
Day 2 N = 7
Day 3 N = 2
Day 5 N = 9

Statistical analyses

No statistical analyses for this end point

Primary: Part 1: Number of Participants Who Experienced a Treatment-emergent Adverse Event (TEAE)

End point title	Part 1: Number of Participants Who Experienced a Treatment-emergent Adverse Event (TEAE)[7][8]

End point description:

TEAEs were defined as any event, side effect, or untoward medical occurrence in a participant enrolled in a clinical study whether or not it was considered to have a causal relationship to the study drug and first occurred or worsened during the post-baseline phase compared to baseline. Clinically significant changes from baseline in vital signs and clinical laboratory results were reported as TEAEs.

Safety Population: Included all participants who received any dose (including partial doses) of any study drug. Per Section 4.1 of the statistical analysis plan (SAP), analyses were planned to be grouped by treatment, rather than by specific dose.

End point type	Primary

End point timeframe:

Day 1 to Day 28

Notes:

[7] - 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 comparative statistical analyses were planned.

[8] - 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: 'Number of Participants Who Experienced a Treatment-emergent Adverse Event (TEAE)' was a primary endpoint for Part 1 only.

End point values	Part 1: EDP-938	Part 1: Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	36	16		
Units: participants	14	9		

Statistical analyses

No statistical analyses for this end point

Primary: Part 2: Daily Change From Baseline in RSV Shedding in Nasal Swab Samples

End point title	Part 2: Daily Change From Baseline in RSV Shedding in Nasal Swab Samples[9][10]

End point description:

Daily change from baseline in RSV shedding was defined as the daily change from baseline in RSV ribonucleic acid (RNA) viral load and was measured using reverse transcription-quantitative polymerase chain reaction (RT-qPCR) from nasal swabs.

Efficacy Population: Included all participants who received one full dose of study drug and had at least one evaluable measurement while on treatment. Per Section 4.1 of the SAP, analyses were planned to be grouped by treatment, rather than by specific dose.

End point type	Primary

End point timeframe:

Baseline and Days 3, 5, 9, and 14

Notes:

[9] - 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 comparative statistical analyses were planned.

[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: 'Daily Change From Baseline in RSV Shedding in Nasal Swab Samples' was a primary endpoint for Part 2 only.

End point values	Part 2: EDP-938	Part 2: Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	28[11]	9[12]		
Units: log10 copies/mL				
arithmetic mean (standard deviation)				
Day 3	-1.42 (± 1.437)	-0.10 (± 1.875)		
Day 5	-3.12 (± 2.007)	-1.36 (± 1.680)		
Day 9	-3.76 (± 1.793)	-2.77 (± 2.378)		
Day 14	-4.93 (± 2.485)	-5.44 (± 1.314)		

Notes:

[11] - Day 3 N = 28
Day 5 N = 27
Day 9 N = 25
Day 14 N = 27
[12] - Day 3 N = 9
Day 5 N = 9
Day 9 N = 8
Day 14 N = 9

Statistical analyses

No statistical analyses for this end point

Primary: Pooled Population: Percent Daily Change From Baseline in RSV Shedding in Nasal Swab Samples

End point title	Pooled Population: Percent Daily Change From Baseline in RSV Shedding in Nasal Swab Samples

End point description:

Daily change from baseline in RSV shedding in nasal swab samples was defined as the daily change from baseline in RSV RNA viral load and measured using RT-qPCR from nasal swabs.

Efficacy Population: Included all participants who received one full dose of study drug and had at least one evaluable measurement while on treatment. Per Section 4.1 of the SAP, analyses were planned to

be grouped by treatment, rather than by specific dose.

End point type	Primary

End point timeframe:

Baseline and Days 3, 5, 9, 14

End point values	Combined EDP-938	Combined Placebo		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	62[13]	21[14]		
Units: log10 copies/mL				
arithmetic mean (standard deviation)				
Day 3	-1.56 (± 1.327)	-1.29 (± 2.143)		
Day 5	-3.01 (± 1.798)	-2.55 (± 1.906)		
Day 9	-4.65 (± 2.244)	-3.72 (± 2.212)		
Day 14	-5.02 (± 2.408)	-5.27 (± 1.783)		

Notes:

[13] - Day 3 N = 62
Day 5 N = 60
Day 9 N = 57
Day 14 N = 58
[14] - Day 3 N = 21
Day 5 N = 21
Day 9 N = 19
Day 14 N = 21

Statistical analyses

Statistical analysis title	Day 3: EDP-938 Versus Placebo

Statistical analysis description:

The model includes treatment group (EDP-938, placebo) and Day (3, 5, 9, and 14) as fixed effect, associated baseline, and treatment group by Day interaction term as factors. An unstructured covariance matrix is imposed. The Satterthwaite approximation is used to estimate the denominator degrees of freedom.

Comparison groups	Combined EDP-938 v Combined Placebo
Number of subjects included in analysis	83
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6574
Method	Mixed-effect model for repeated measures
Parameter estimate	Least-squares mean difference
Point estimate	-0.17
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.93
upper limit	0.59

Statistical analysis title	Day 5: EDP-938 Versus Placebo

Statistical analysis description:

The model includes treatment group (EDP-938, placebo) and Day (3, 5, 9, and 14) as fixed effect, associated baseline, and treatment group by Day interaction term as factors. An unstructured covariance matrix is imposed. The Satterthwaite approximation is used to estimate the denominator degrees of freedom.

Comparison groups	Combined EDP-938 v Combined Placebo
Number of subjects included in analysis	83
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4728
Method	Mixed-effect model for repeated measures
Parameter estimate	Least-squares mean difference
Point estimate	-0.33
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.23
upper limit	0.58

Statistical analysis title	Day 9: EDP-938 Versus Placebo

Statistical analysis description:

The model includes treatment group (EDP-938, placebo) and Day (3, 5, 9, and 14) as fixed effect, associated baseline, and treatment group by Day interaction term as factors. An unstructured covariance matrix is imposed. The Satterthwaite approximation is used to estimate the denominator degrees of freedom.

Comparison groups	Combined EDP-938 v Combined Placebo
Number of subjects included in analysis	83
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2058
Method	Mixed-effect model for repeated measures
Parameter estimate	Least-squares mean difference
Point estimate	-0.7
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.79
upper limit	0.39

Statistical analysis title	Day 14: EDP-938 Versus Placebo

Statistical analysis description:

The model includes treatment group (EDP-938, placebo) and Day (3, 5, 9, and 14) as fixed effect, associated baseline, and treatment group by Day interaction term as factors. An unstructured covariance matrix is imposed. The Satterthwaite approximation is used to estimate the denominator degrees of freedom.

Comparison groups	Combined EDP-938 v Combined Placebo

Number of subjects included in analysis	83
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5391
Method	Mixed-effect model for repeated measures
Parameter estimate	Least-squares mean difference
Point estimate	0.33
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.74
upper limit	1.41

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Day 1 to Day 28

Adverse event reporting additional description:

Safety Population: Included all participants who received any dose (including partial doses) of any study drug. Per Section 4.1 of the SAP, analyses were planned to be grouped by treatment, rather than by specific dose.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	26.1

Reporting groups

Reporting group title	Part 1: EDP-938

Reporting group description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 once daily (QD) from Day 1 to Day 5 of the study.

Reporting group title	Part 1: Placebo

Reporting group description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and hospitalized participants aged between ≥ 28 days and < 6 months received placebo matching EDP-938 QD from Day 1 to Day 5 of the study.

Reporting group title	Part 2: EDP-938

Reporting group description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received a lower or higher dose of EDP-938 QD from Day 1 to Day 5 of the study.

Reporting group title	Part 2: Placebo

Reporting group description:

Hospitalized and non-hospitalized participants aged between ≥ 6 months and ≤ 36 months, and between ≥ 28 days and < 6 months received placebo matching EDP-938 QD on Day 1 to Day 5 of the study.

Serious adverse events	Part 1: EDP-938	Part 1: Placebo	Part 2: EDP-938
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 36 (0.00%)	2 / 16 (12.50%)	1 / 34 (2.94%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Respiratory, thoracic and mediastinal disorders			
Pleural effusion			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			

Bronchiolitis			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	0 / 36 (0.00%)	0 / 16 (0.00%)	1 / 34 (2.94%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Part 2: Placebo		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 10 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Respiratory, thoracic and mediastinal disorders			
Pleural effusion			
subjects affected / exposed	0 / 10 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Bronchiolitis			
subjects affected / exposed	0 / 10 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	0 / 10 (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	Part 1: EDP-938	Part 1: Placebo	Part 2: EDP-938
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 36 (19.44%)	7 / 16 (43.75%)	9 / 34 (26.47%)
Investigations			

Myocardial necrosis marker increased			
subjects affected / exposed	0 / 36 (0.00%)	0 / 16 (0.00%)	0 / 34 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 36 (0.00%)	0 / 16 (0.00%)	0 / 34 (0.00%)
occurrences (all)	0	0	0
Thrombocytosis			
subjects affected / exposed	0 / 36 (0.00%)	0 / 16 (0.00%)	2 / 34 (5.88%)
occurrences (all)	0	0	2
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	1 / 36 (2.78%)	0 / 16 (0.00%)	0 / 34 (0.00%)
occurrences (all)	1	0	0
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	4 / 36 (11.11%)	1 / 16 (6.25%)	3 / 34 (8.82%)
occurrences (all)	4	1	3
Vomiting			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	0	1	0
Respiratory, thoracic and mediastinal disorders			
Nasal congestion			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	1 / 34 (2.94%)
occurrences (all)	0	1	1
Skin and subcutaneous tissue disorders			
Dermatitis diaper			
subjects affected / exposed	1 / 36 (2.78%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	1	1	0
Eczema			
subjects affected / exposed	1 / 36 (2.78%)	1 / 16 (6.25%)	1 / 34 (2.94%)
occurrences (all)	1	1	1
Papule			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	0	1	0
Rash			

subjects affected / exposed	1 / 36 (2.78%)	0 / 16 (0.00%)	2 / 34 (5.88%)
occurrences (all)	1	0	2
Infections and infestations			
Acarodermatitis			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	0	1	0
Nasopharyngitis			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	1 / 34 (2.94%)
occurrences (all)	0	1	1
Otitis media acute			
subjects affected / exposed	1 / 36 (2.78%)	1 / 16 (6.25%)	1 / 34 (2.94%)
occurrences (all)	1	1	1
Pneumonia bacterial			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	0	1	0
Respiratory tract infection			
subjects affected / exposed	0 / 36 (0.00%)	1 / 16 (6.25%)	0 / 34 (0.00%)
occurrences (all)	0	1	0

Non-serious adverse events	Part 2: Placebo		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	4 / 10 (40.00%)		
Investigations			
Myocardial necrosis marker increased			
subjects affected / exposed	1 / 10 (10.00%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 10 (10.00%)		
occurrences (all)	1		
Thrombocytosis			
subjects affected / exposed	0 / 10 (0.00%)		
occurrences (all)	0		
General disorders and administration site conditions			
Pyrexia			

subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1		
Gastrointestinal disorders Diarrhoea subjects affected / exposed occurrences (all) Vomiting subjects affected / exposed occurrences (all)	 0 / 10 (0.00%) 0 0 / 10 (0.00%) 0		
Respiratory, thoracic and mediastinal disorders Nasal congestion subjects affected / exposed occurrences (all)	 0 / 10 (0.00%) 0		
Skin and subcutaneous tissue disorders Dermatitis diaper subjects affected / exposed occurrences (all) Eczema subjects affected / exposed occurrences (all) Papule subjects affected / exposed occurrences (all) Rash subjects affected / exposed occurrences (all)	 0 / 10 (0.00%) 0 0 / 10 (0.00%) 0 0 / 10 (0.00%) 0 1 / 10 (10.00%) 1		
Infections and infestations Acarodermatitis subjects affected / exposed occurrences (all) Nasopharyngitis subjects affected / exposed occurrences (all) Otitis media acute subjects affected / exposed occurrences (all)	 0 / 10 (0.00%) 0 1 / 10 (10.00%) 1 0 / 10 (0.00%) 0		

Pneumonia bacterial subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0		
Respiratory tract infection subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
13 August 2020	Protocol Version 2.0 (global amendment)
01 June 2021	Protocol Version 4.0 (global amendment)
13 September 2021	Protocol Version 6.0 (global amendment)
28 February 2022	Protocol Version 8.0 (global amendment)
23 August 2022	Protocol Version 10.0 (global amendment)
03 October 2022	Protocol Version 11.0 (global amendment)
03 January 2023	Protocol Version 13.0 (global amendment)
22 September 2023	Protocol Version 15.0 (global amendment; submitted to the United States Food and Drug Administration only)
04 December 2023	Protocol Version 16.0 (global amendment)
09 July 2024	Protocol Version 18.0 (global amendment)

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported