



Clinical trial results:

A Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy, Safety, and Tolerability of Luvadaxistat in Subjects With Cognitive Impairment Associated With Schizophrenia, Followed by Open-Label Treatment

Summary

EudraCT number	2021-003834-34
Trial protocol	CZ ES
Global end of trial date	10 October 2024

Results information

Result version number	v1 (current)
This version publication date	26 October 2025
First version publication date	26 October 2025

Trial information

Trial identification

Sponsor protocol code	NBI-1065844-CIAS2023
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05182476
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Neurocrine Biosciences, Inc.
Sponsor organisation address	6027 Edgewood Bend Ct, San Diego, CA, United States, 92130
Public contact	Medical Information Call Center, Neurocrine Biosciences, Inc., +34 932483137, medinfo@neurocrine.com
Scientific contact	Medical Information Call Center, Neurocrine Biosciences, Inc., +34 932483137, medinfo@neurocrine.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	10 October 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	10 October 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and efficacy of luvadaxistat compared with placebo on improving cognitive performance in participants with schizophrenia.

Protection of trial subjects:

The Sponsor personnel and the investigators ensured that the study was conducted in full compliance with International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) Good Clinical Practice (GCP) guidelines, and with the laws and regulations of the country in which the study was conducted.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	07 December 2021
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 110
Country: Number of subjects enrolled	Bulgaria: 59
Country: Number of subjects enrolled	Czechia: 12
Country: Number of subjects enrolled	Serbia: 20
Country: Number of subjects enrolled	Spain: 2
Worldwide total number of subjects	203
EEA total number of subjects	73

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)	203
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants were randomized to receive luvadaxistat lower-dose, higher-dose, or placebo once daily (QD) during the Double-blind Treatment Period. Participants who completed the Double-blind Treatment Period and remained on treatment entered the Open-label Period and received luvadaxistat high-dose QD for 6–12 months.

Pre-assignment

Screening details:

A total of 203 participants were randomized, of which 202 participants received at least 1 dose of study drug.

Period 1

Period 1 title	Double-blind Treatment Period
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Carer, Assessor

Arms

Are arms mutually exclusive?	Yes
Arm title	Luvadaxistat Lower-dose

Arm description:

Participants received luvadaxistat at a lower-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Arm type	Experimental
Investigational medicinal product name	Luvadaxistat
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Luvadaxistat was administered per schedule specified in the arm description.

Arm title	Luvadaxistat Higher-dose
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Arm description:

Participants received luvadaxistat at a higher-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Arm type	Experimental
Investigational medicinal product name	Luvadaxistat
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Luvadaxistat was administered per schedule specified in the arm description.

Arm title	Placebo
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Arm description:

Participants received placebo matched to luvadaxistat as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Arm type	Placebo
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Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Placebo was administered per schedule specified in the arm description.

Number of subjects in period 1^[1]	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo
Started	49	52	101
Received at Least 1 Dose of Study Drug	49	52	101
Completed	44	46	88
Not completed	5	6	13
Consent withdrawn by subject	5	3	7
Physician decision	-	1	-
Adverse event, non-fatal	-	1	4
Other than specified	-	1	-
Protocol-specified withdrawal criteria met	-	-	1
Lost to follow-up	-	-	1

Notes:

[1] - The number of subjects reported to be in the baseline period are not the same as the worldwide number enrolled in the trial. It is expected that these numbers will be the same.

Justification: A total of 203 participants were randomized, of which 202 participants received at least 1 dose of study drug.

Period 2

Period 2 title	Open-label Treatment Period
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	Luvadaxistat Lower to Higher-dose

Arm description:

Participants who received luvadaxistat at a lower-dose during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.

Arm type	Experimental
Investigational medicinal product name	Luvadaxistat
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Luvadaxistat was administered per schedule specified in the arm description.

Arm title	Luvadaxistat High to High-dose
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Arm description:

Participants who received luvadaxistat at a higher-dose during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.

Arm type	Experimental
Investigational medicinal product name	Luvadaxistat
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Luvadaxistat was administered per schedule specified in the arm description.

Arm title	Placebo to Luvadaxistat Higher-dose
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Arm description:

Participants who received placebo matched to luvadaxistat during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Placebo was administered per schedule specified in the arm description.

Number of subjects in period 2	Luvadaxistat Lower to Higher-dose	Luvadaxistat High to High-dose	Placebo to Luvadaxistat Higher-dose
Started	44	46	88
Received Luvadaxistat High-dose	44	46	88
Completed	28	26	48
Not completed	16	20	40
Consent withdrawn by subject	2	1	6
Adverse event, non-fatal	-	2	5
Study terminated by sponsor	13	16	24
Other than specified	1	-	-
Lost to follow-up	-	1	4
Pregnancy	-	-	1

Baseline characteristics

Reporting groups

Reporting group title	Luvadaxistat Lower-dose
Reporting group description: Participants received luvadaxistat at a lower-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	
Reporting group title	Luvadaxistat Higher-dose
Reporting group description: Participants received luvadaxistat at a higher-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	
Reporting group title	Placebo
Reporting group description: Participants received placebo matched to luvadaxistat as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	

Reporting group values	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo
Number of subjects	49	52	101
Age categorical Units: Subjects			
18 to <35 Years	19	20	37
≥35 to 50 Years	30	32	64
Gender categorical Units: Subjects			
Female	14	20	57
Male	35	32	44
Race Units: Subjects			
American Indian or Alaska Native	0	0	1
Asian	1	1	2
Black or African American	15	9	24
White	32	39	69
Native Hawaiian or Other Pacific Islander	0	0	0
Other	1	2	5
Multiple	0	1	0
Ethnicity Units: Subjects			
Hispanic or Latino	8	12	21
Not Hispanic or Latino	41	40	80

Reporting group values	Total		
Number of subjects	202		
Age categorical Units: Subjects			
18 to <35 Years	76		
≥35 to 50 Years	126		

Gender categorical			
Units: Subjects			
Female	91		
Male	111		
Race			
Units: Subjects			
American Indian or Alaska Native	1		
Asian	4		
Black or African American	48		
White	140		
Native Hawaiian or Other Pacific Islander	0		
Other	8		
Multiple	1		
Ethnicity			
Units: Subjects			
Hispanic or Latino	41		
Not Hispanic or Latino	161		

End points

End points reporting groups

Reporting group title	Luvadaxistat Lower-dose
Reporting group description: Participants received luvadaxistat at a lower-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	
Reporting group title	Luvadaxistat Higher-dose
Reporting group description: Participants received luvadaxistat at a higher-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	
Reporting group title	Placebo
Reporting group description: Participants received placebo matched to luvadaxistat as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.	
Reporting group title	Luvadaxistat Lower to Higher-dose
Reporting group description: Participants who received luvadaxistat at a lower-dose during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.	
Reporting group title	Luvadaxistat High to High-dose
Reporting group description: Participants who received luvadaxistat at a higher-dose during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.	
Reporting group title	Placebo to Luvadaxistat Higher-dose
Reporting group description: Participants who received placebo matched to luvadaxistat during the Double-blind Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.	

Primary: Change from Baseline in the Brief Assessment of Cognition in Schizophrenia (BACS) Composite Score at Day 98

End point title	Change from Baseline in the Brief Assessment of Cognition in Schizophrenia (BACS) Composite Score at Day 98
End point description: The BACS is a performance-based assessment that measures treatment-related changes in cognition and assesses 6 domains including verbal memory and learning, working memory, motor function, verbal fluency, attention and speed of processing, and executive function. For each domain, higher scores represent better cognition, and raw scores were converted to age and gender corrected normalized scores. The composite score was calculated as the mean of the normalized scores from the 6 domains. The BACS composite score was normalized based on z-scores transformed to T-scores (mean=50, SD=10 in normative data). There is no theoretical maximum score. Higher scores represent better outcomes. Efficacy Analysis Set: Included all randomized participants who demonstrated study treatment compliance and demonstrated symptoms stability between Visits 1, 2 and 3. Number of participants analysed = participants evaluable for this outcome measure.	
End point type	Primary
End point timeframe: Baseline, Day 98	

End point values	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	38	40	83	
Units: score on a scale				
least squares mean (confidence interval 95%)	1.9 (0.1 to 3.7)	2.1 (0.3 to 3.8)	2.6 (1.4 to 3.8)	

Statistical analyses

Statistical analysis title	Luvadaxistat Lower-dose versus Placebo
Comparison groups	Placebo v Luvadaxistat Lower-dose
Number of subjects included in analysis	121
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5023
Method	ANCOVA
Parameter estimate	Least-Squares Mean Difference
Point estimate	-0.7
Confidence interval	
level	95 %
sides	2-sided
lower limit	-2.8
upper limit	1.4
Variability estimate	Standard error of the mean
Dispersion value	1.1

Statistical analysis title	Luvadaxistat Higher-dose versus Placebo
Comparison groups	Placebo v Luvadaxistat Higher-dose
Number of subjects included in analysis	123
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6123
Method	ANCOVA
Parameter estimate	Least-Squares Mean Difference
Point estimate	-0.5
Confidence interval	
level	95 %
sides	2-sided
lower limit	-2.7
upper limit	1.6
Variability estimate	Standard error of the mean
Dispersion value	1.1

Secondary: Change from Baseline in Schizophrenia Cognition Rating Scale (SCoRS)

Interviewer Score at Day 98

End point title	Change from Baseline in Schizophrenia Cognition Rating Scale (SCoRS) Interviewer Score at Day 98
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End point description:

The SCoRS includes 20 items focusing on cognitive impairment and the degree to which it affects day-to-day functioning, as well as a global functioning scale. Number of participants analysed = participants evaluable for this outcome measure. The scores ranged from 20 to 80, with lower scores indicating a better outcome.

Efficacy Analysis Set: Included all randomized participants who demonstrated study treatment compliance and demonstrated symptoms stability between Visits 1, 2 and 3.

End point type	Secondary
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End point timeframe:

Baseline, Day 98

End point values	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	37	40	82	
Units: score on a scale				
least squares mean (confidence interval 95%)	-2.3 (-4.0 to -0.7)	-2.7 (-4.3 to -1.2)	-2.1 (-3.2 to -1.0)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in the Virtual Reality Functional Capacity Assessment Tool (VRFCAT) Score at Day 98

End point title	Change from Baseline in the Virtual Reality Functional Capacity Assessment Tool (VRFCAT) Score at Day 98
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End point description:

The VRFCAT is an immersive, virtual-reality-based computerized assessment that evaluates functional capacity across the following 4 domains: transportation, finances, household management, and planning. Briefly, the VRFCAT consists of 4 mini scenarios, which include checking the kitchen for the availability of items to complete a recipe and planning a trip to the grocery store, taking a bus and paying the correct fare, shopping for the items in a store, and returning home. VRFCAT T-scores were calculated from standardized task performance (mean=50, SD=10). There was no theoretical maximum score. Higher scores indicated faster completion, fewer errors, and fewer forced progressions, reflecting better functional capacity.

Efficacy Analysis Set: Included all randomized participants who demonstrated study treatment compliance and demonstrated symptoms stability between Visits 1, 2 and 3. Number of participants analysed = participants evaluable for this outcome measure.

End point type	Secondary
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End point timeframe:

Baseline, Day 98

End point values	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	37	40	83	
Units: T-score				
least squares mean (confidence interval 95%)				
VRFCAT-Adjusted Total Time T Score	1.633 (-2.409 to 5.674)	3.983 (0.075 to 7.892)	1.844 (-0.859 to 4.548)	
VRFCAT-Total Error Count T Score	-1.194 (-6.248 to 3.860)	3.954 (-0.977 to 8.885)	0.038 (-3.362 to 3.437)	
VRFCAT-Total Forced Progressions T Score	-4.533 (-12.136 to 3.070)	3.939 (-3.453 to 11.330)	-0.891 (-6.015 to 4.232)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline on the Clinical Global Impression – Severity (CGI-S) Score at Day 98

End point title	Change from Baseline on the Clinical Global Impression – Severity (CGI-S) Score at Day 98
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End point description:

The CGI-S scale was based on a 7-point scale (range: 1=normal to 7=among the most extremely ill patients), was used to rate the overall global severity of schizophrenia.

Efficacy Analysis Set: Included all randomized participants who demonstrated study treatment compliance and demonstrated symptoms stability between Visits 1, 2 and 3. n=number of participants analyzed at specified time points. Number of participants analysed = participants evaluable for this outcome measure.

End point type	Secondary
End point timeframe:	
Baseline, Day 98	

End point values	Luvadaxistat Lower-dose	Luvadaxistat Higher-dose	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	41 ^[1]	43 ^[2]	88 ^[3]	
Units: participants				
Baseline: 1=Normal, not at all ill	0	0	1	
Baseline: 2=Borderline ill	3	3	6	
Baseline: 3=Mildly ill	11	17	38	
Baseline: 4=Moderately ill	24	20	39	
Baseline: 5=Markedly ill	3	3	3	
Baseline: 6=Severely ill	0	0	1	

Baseline: 7=Among the most extremely ill patients	0	0	0	
Day 98: 1=Normal, not at all ill	0	0	0	
Day 98: 2=Borderline ill	2	5	15	
Day 98: 3=Mildly ill	18	20	35	
Day 98: 4=Moderately ill	15	14	32	
Day 98: 5=Markedly ill	2	0	2	
Day 98: 6=Severely ill	0	0	0	
Day 98: 7=Among the most extremely ill patients	0	0	0	

Notes:

[1] - Baseline: n=41

Day 98: n=37

[2] - Baseline: n=43

Day 98: n=39

[3] - Baseline: n=88

Day 98: n=84

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Up to Week 68

Adverse event reporting additional description:

Safety Analysis Set: Included all randomized participants who received at least 1 dose of study treatment.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.0
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Reporting groups

Reporting group title	Double-blind Treatment Period: Luvadaxistat Lower-dose
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Reporting group description:

Participants received luvadaxistat at a lower-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Reporting group title	Double-blind Treatment Period: Luvadaxistat Higher-dose
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Reporting group description:

Participants received luvadaxistat at a higher-dose as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Reporting group title	Double-blind Treatment Period: Placebo
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Reporting group description:

Participants received placebo matched to luvadaxistat as oral tablets QD for up to 14 weeks during the Double-blind Treatment Period.

Reporting group title	Open-label Treatment Period: Luvadaxistat Higher-dose
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Reporting group description:

All participants who entered the Open-label Treatment Period continued to receive luvadaxistat at a higher-dose as oral tablets QD for up to either 6 or 12 months.

Serious adverse events	Double-blind Treatment Period: Luvadaxistat Lower-dose	Double-blind Treatment Period: Luvadaxistat Higher-dose	Double-blind Treatment Period: Placebo
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 49 (2.04%)	1 / 52 (1.92%)	3 / 101 (2.97%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Intentional overdose			
subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Seizure			

subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	1 / 101 (0.99%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Abdominal discomfort			
subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	0 / 49 (0.00%)	1 / 52 (1.92%)	0 / 101 (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
Psychiatric disorders			
Schizophrenia			
subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Suicide attempt			
subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Cellulitis			
subjects affected / exposed	1 / 49 (2.04%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	0 / 101 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urinary tract infection			

subjects affected / exposed	0 / 49 (0.00%)	0 / 52 (0.00%)	1 / 101 (0.99%)
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	Open-label Treatment Period: Luvadaxistat Higher-dose		
Total subjects affected by serious adverse events			
subjects affected / exposed	4 / 178 (2.25%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Injury, poisoning and procedural complications			
Intentional overdose			
subjects affected / exposed	1 / 178 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Seizure			
subjects affected / exposed	0 / 178 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Abdominal discomfort			
subjects affected / exposed	1 / 178 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	0 / 178 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Psychiatric disorders			
Schizophrenia			
subjects affected / exposed	1 / 178 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Suicide attempt			
subjects affected / exposed	1 / 178 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Cellulitis			
subjects affected / exposed	0 / 178 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	1 / 178 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Urinary tract infection			
subjects affected / exposed	0 / 178 (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	Double-blind Treatment Period: Luvadaxistat Lower- dose	Double-blind Treatment Period: Luvadaxistat Higher- dose	Double-blind Treatment Period: Placebo
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 49 (0.00%)	3 / 52 (5.77%)	1 / 101 (0.99%)
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 49 (0.00%)	3 / 52 (5.77%)	1 / 101 (0.99%)
occurrences (all)	0	3	1

Non-serious adverse events	Open-label Treatment Period: Luvadaxistat Higher- dose		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	5 / 178 (2.81%)		
Nervous system disorders			

Headache			
subjects affected / exposed	5 / 178 (2.81%)		
occurrences (all)	5		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
24 September 2021	Global Amendment 1.0
12 September 2022	Global Amendment 2.0
20 March 2023	Global Amendment 3.0

Notes:

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 as the study failed to meet the primary endpoint.

Notes: