Clinical trial results:
Effect of adipose tissue derived mesenchymal stromal cells on autism and
leaky gut syndrome. A phase I pilot study.

Summary

EudraCT number	2022-002940-27
Trial protocol	DK
Global end of trial date	20 September 2024

Results information

Result version number	v1 (current)
This version publication date	09 July 2025
First version publication date	09 July 2025

Trial information

Trial identification

Sponsor protocol code	C2C1-1

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05602116
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Cell2Cure ApS
Sponsor organisation address	Kajerødgård 9, Birkerød, Denmark, 3460
Public contact	Jens Kastrup, Cell2Cure ApS, 45 21202994, jk@cell2cure.com
Scientific contact	Jens Kastrup, Cell2Cure ApS, 45 21202994, jk@cell2cure.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	22 April 2025
Is this the analysis of the primary completion data?	Yes
Primary completion date	20 September 2024
Global end of trial reached?	Yes
Global end of trial date	20 September 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The purpose of this dose titrating study was to evaluate the safety and efficacy of an allogeneic adipose tissue-derived mesenchymal stromal/stem cell product (C2C_ASC) in children with autism spectrum disorder (ASD) and gastrointestinal symptoms. Results from this study will be used to support the feasibility and safety of C2C_ASC treatment in children. And further to support the hypothesis of a connection between gastrointestinal symptoms, increased local gastrointestinal and systemic elevated immunological and inflammatory activity, bacterial toxins in the blood and symptoms of autism spectrum disorder that can be reduced or normalized by modulating the immunological activity and inflammation by treatment with mesenchymal stromal/stem cells.

Protection of trial subjects:

Treatment visits was planned at least one week apart, to observe for serious adverse events related to the cell treatment and procedure, before treatment of a new trial subject.
Data monitoring was done for all enrolled trial subjects.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	09 June 2023
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	Denmark: 10
Worldwide total number of subjects	10
EEA total number of subjects	10

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)	3
Adolescents (12-17 years)	7

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

Subject disposition

Recruitment

Recruitment details:

The trial subjects was recruited through the Department of Child and Adolescent Psychiatry, Psychiatry Clinic South, Aalborg University Hospital, Denmark.
A total number of 10 subjects was planned and enrolled. All 10 subjects received the study intervention and completed the study follow-up.

Pre-assignment

Screening details:

Date of enrolment (first participant's first visit): 05 September 2023
Date of completion (last participant's last visit): 20 September 2024

Key inclusion criteria were:
• Children aged 6 – 14 years
• Diagnosis of autism spectrum disorder (ASD)
• Gastrointestinal symptoms or previous gastrointestinal symptoms

Period 1

Period 1 title	Baseline (overall period)
Is this the baseline period?	Yes
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	1 x 1 million ASCs/kg body weight

Arm description:

Trial subject 1-5 was allocated to this arm with one treatment of 1 x 1 million ASCs/kg body weight.

Arm type	Experimental
Investigational medicinal product name	CSCC_ASC5010
Investigational medicinal product code	CSCC_ASC5010
Other name	Allogeneic adipose tissue-derived mesenchymal stromal/stem cells
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

The investigational product (IP) was an advanced therapy investigational medicinal product (ATIMP) manufactured from allogeneic adipose tissue-derived mesenchymal stromal/stem cells (ASCs). The active substance is in vitro expanded ASCs. The IP, CSCC_ASC5010, was manufactured as a cryopreserved suspension of 50 million ASCs per ml with a total volume of 1,3 ml per vial.
In chronological order subjects was randomized to a low dose or a high dose where the first 5 enrolled subjects received 1 x 1 million ASCs/kg body weight (low dose), and the last 5 enrolled subjects received 2 x
1 million ASCs/kg body weight (high dose). The IP dose was calculated from the subject's body weight. The calculated dose was extracted from one or more vials and was diluted in 50 ml isotonic saline and infused in a vein in the hand.

Arm title	2 x 1 million ASCs/kg body weight

Arm description:

Trial subject 6-10 was allocated to this arm with one treatment of 2 x 1 million ASCs/kg body weight.

Arm type	Experimental

Investigational medicinal product name	CSCC_ASC5010
Investigational medicinal product code	CSCC_ASC5010
Other name	Allogeneic adipose tissue-derived mesenchymal stromal/stem cells
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

The investigational product (IP) was an advanced therapy investigational medicinal product (ATIMP) manufactured from allogeneic adipose tissue-derived mesenchymal stromal/stem cells (ASCs). The active substance is in vitro expanded ASCs. The IP, CSCC_ASC5010, was manufactured as a cryopreserved suspension of 50 million ASCs per ml with a total volume of 1,3 ml per vial.
In chronological order subjects was randomized to a low dose or a high dose where the first 5 enrolled subjects received 1 x 1 million ASCs/kg body weight (low dose), and the last 5 enrolled subjects received 2 x
1 million ASCs/kg body weight (high dose). The IP dose was calculated from the subject's body weight. The calculated dose was extracted from one or more vials and was diluted in 50 ml isotonic saline and infused in a vein in the hand.

Number of subjects in period 1	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight
Started	5	5
Completed	5	5

Baseline characteristics

Reporting groups	
Reporting group title	Baseline

Reporting group description: -

Reporting group values	Baseline	Total	
Number of subjects	10	10	
Age categorical Units: Subjects			
Children (2-11 years)	3	3	
Adolescents (12-17 years)	7	7	
Age continuous Units: years			
arithmetic mean	12.6		
standard deviation	± 2.19	-	
Gender categorical Units: Subjects			
Female	1	1	
Male	9	9	
Reported gastrointestinal symptoms			
Reported gastrointestinal symptoms. Measurement: history of painful or hard stools.			
Units: Subjects			
History of painful or hard stools	10	10	
Body weight Units: kilogram(s)			
arithmetic mean	47.06		
standard deviation	± 22.72	-	
Childrens communication checklist - Generel communication			
Questionnaire Childrens communication checklist. Measurement: generel communication.			
Units: unit(s)			
arithmetic mean	44.6		
standard deviation	± 20.58	-	
Childrens communication checklist - Social interaction			
Questionnaire Childrens communication checklist. Measurement: Social interaction.			
Units: unit(s)			
arithmetic mean	-9.9		
standard deviation	± 8.84	-	
IBS-QOL			
Questionnaire IBS-QOL. Measurement: Overall			
Units: unit(s)			
arithmetic mean	82.87		
standard deviation	± 13.51	-	
CRP Units: milligram(s)/litre			

arithmetic mean	1.4		
standard deviation	± 0.68	-	

Subject analysis sets

Subject analysis set title	Baseline data
Subject analysis set type	Intention-to-treat

Subject analysis set description:
Age

Reporting group values	Baseline data		
Number of subjects	10		
Age categorical Units: Subjects			
Children (2-11 years)	3		
Adolescents (12-17 years)	7		
Age continuous Units: years			
arithmetic mean	12.6		
standard deviation	± 2.19		
Gender categorical Units: Subjects			
Female	1		
Male	9		
Reported gastrointestinal symptoms			
Reported gastrointestinal symptoms. Measurement: history of painful or hard stools.			
Units: Subjects			
History of painful or hard stools	9		
Body weight Units: kilogram(s)			
arithmetic mean	47.06		
standard deviation	± 22.72		
Childrens communication checklist - Generel communication			
Questionnaire Childrens communication checklist. Measurement: generel communication.			
Units: unit(s)			
arithmetic mean	44.6		
standard deviation	± 20.58		
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standard deviation	± 8.84		
IBS-QOL			
Questionnaire IBS-QOL. Measurement: Overall			
Units: unit(s)			
arithmetic mean	82.87		

standard deviation	± 13.51		
CRP			
Units: milligram(s)/litre			
arithmetic mean	1.4		
standard deviation	± 0.68		

End points

End points reporting groups

Reporting group title	1 x 1 million ASCs/kg body weight
Reporting group description:	
Trial subject 1-5 was allocated to this arm with one treatment of 1 x 1 million ASCs/kg body weight.	
Reporting group title	2 x 1 million ASCs/kg body weight
Reporting group description:	
Trial subject 6-10 was allocated to this arm with one treatment of 2 x 1 million ASCs/kg body weight.	
Subject analysis set title	Baseline data
Subject analysis set type	Intention-to-treat
Subject analysis set description:	
Age	

Primary: Safety

End point title	Safety[1]
End point description:	
To assess the safety and tolerability of intravenous administration of a single dose 1 x 1 million ASCs per kg body weight and 2 x 1 million ASCs per kg body weight in children with autism spectrum disorder and gastrointestinal symptoms. Endpoint defined by adverse events (AEs), serious adverse events (SARs), and suspected unexpected serious adverse events (SUSARs).	
End point type	Primary
End point timeframe:	
From treatment to the 12-week follow-up.	

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: The primary end point is safety registred as the number of safety events, thus no statistical analyses are needed.

End point values	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	5		
Units: Totals				
Adverse events (AEs)	8	1		
Adverse reactions (ARs)	2	0		
Serious adverse events (SAEs)	0	0		
suspected unexpected serious adverse events (SUSAR	0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Efficacy - changes in symptoms of autism spectrum disorder

End point title	Efficacy - changes in symptoms of autism spectrum disorder
End point description:	

To characterize changes in symptoms of autism spectrum disorder. Assessed by questionnaires:
• Children’s Communication Checklist (second edition, Pearson Clinical)

End point type	Secondary

End point timeframe:

From baseline to the 12-week follow-up.

End point values	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	5		
Units: unit(s)				
arithmetic mean (standard deviation)				
Generel communication	48.6 (± 21.31)	47.2 (± 24.51)		
Social interaction	-8.6 (± 6.11)	-4.2 (± 12.99)		

Statistical analyses

No statistical analyses for this end point

Secondary: Efficacy - changes in gastrointestinal symptoms

End point title	Efficacy - changes in gastrointestinal symptoms

End point description:

To characterize changes in gastrointestinal symptoms. Assessed by questionnaire:
• IBS-QOL (irritable bowel syndromequality of life) (ePROVIDE)

End point type	Secondary

End point timeframe:

From baseline to the 12-week follow-up.

End point values	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	5		
Units: unit(s)				
arithmetic mean (standard deviation)				
Overall	90.44 (± 8.76)	75.15 (± 27.1)		

Statistical analyses

No statistical analyses for this end point

Secondary: Efficacy - changes in inflammatory markers

End point title	Efficacy - changes in inflammatory markers

End point description:

To characterize changes in inflammatory markers. Assessed by plasma analysis of:
• CRP

End point type	Secondary

End point timeframe:

From baseline to the 12-week follow-up.

End point values	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	5		
Units: milligram(s)/litre				
arithmetic mean (standard deviation)				
CRP	1.52 (± 1.05)	1.74 (± 1.19)		

Statistical analyses

No statistical analyses for this end point

Secondary: Efficacy - changes in gastrointestinal symptoms

End point title	Efficacy - changes in gastrointestinal symptoms

End point description:

End point type	Secondary

End point timeframe:

Reported gastrointestinal symptoms.
Measurement: history of painful and hard stools.

End point values	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	5		
Units: Subjects				
History of painful or hard stools	1	0		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: From treatment to the 12-week follow-up.	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	18
Reporting groups	
Reporting group title	1 x 1 million ASCs/kg body weight
Reporting group description: -	
Reporting group title	2 x 1 million ASCs/kg body weight
Reporting group description: -	

Serious adverse events	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 5 (0.00%)	0 / 5 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

Frequency threshold for reporting non-serious adverse events: 1 %

Non-serious adverse events	1 x 1 million ASCs/kg body weight	2 x 1 million ASCs/kg body weight	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 5 (60.00%)	2 / 5 (40.00%)	
Gastrointestinal disorders			
Diarrhea			
subjects affected / exposed	1 / 5 (20.00%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Vomitting			
subjects affected / exposed	1 / 5 (20.00%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Increased fecal calprotectin			
subjects affected / exposed	1 / 5 (20.00%)	0 / 5 (0.00%)	
occurrences (all)	1	0	

Skin and subcutaneous tissue disorders Atopic eczema subjects affected / exposed occurrences (all)	 1 / 5 (20.00%) 1	 0 / 5 (0.00%) 0	
Renal and urinary disorders Pain in testicles subjects affected / exposed occurrences (all)	 1 / 5 (20.00%) 1	 0 / 5 (0.00%) 0	
Musculoskeletal and connective tissue disorders Headache subjects affected / exposed occurrences (all)	 1 / 5 (20.00%) 1	 0 / 5 (0.00%) 0	
Infections and infestations Fever subjects affected / exposed occurrences (all) Increased C-reactive protein subjects affected / exposed occurrences (all) Common cold subjects affected / exposed occurrences (all)	 1 / 5 (20.00%) 1 1 / 5 (20.00%) 1 0 / 5 (0.00%) 0	 0 / 5 (0.00%) 0 0 / 5 (0.00%) 0 2 / 5 (40.00%) 2	

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

None reported