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Stem cell therapy for renal dysfunction

Submission date	Recruitment status	[X] Prospectively registered
29/12/2022	Recruiting	[X] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
07/01/2023	Ongoing	[_] Results
Last Edited	**Condition category**	[_] Individual participant data
18/03/2024	Urological and Genital Diseases	[_] Record updated in last year

Plain English summary of protocol

Background and study aims
Alport syndrome (AS) is one of the most common human fatal hereditary renal (kidney) diseases and is characterized by hematuria (blood in the urine), albuminuria (too much protein in the urine), and a progressive decline of kidney function. The risk is high for male patients with COL4A5 deficiency to develop into an end-stage renal disease without effective treatments. The main aim of this study is to find out whether intravenous infusion (into a vein) of human umbilical cord mesenchymal stem cells (hUC-MSCs) is safe and feasible with good tolerance for patients with AS.

Who can participate?
Patients aged 3-18 years with AS

What does the study involve?
Participants will receive four doses of hUC-MSC as scheduled. Safety and effectiveness evaluations are performed at baseline (Day 0), weekly in the treatment phase (Day 7, Day 14, Day 21), and monthly in a follow-up phase (Months 1-12 after treatment). The study lasts for 1 year in total.

What are the possible benefits and risks of participating?
The treatment could reverse albuminuria based on pre-clinical studies in mice. No serious adverse events were reported in previous studies in children with cerebral palsy. The main risk is that patients might experience a transient fever of no more than 38.5°C based on other studies of stem cell transfusion.

Where is the study run from?
Affiliated Taihe Hospital of Hubei University of Medicine (China)

When is the study starting and how long is it expected to run for?
January 2015 to December 2026

Who is funding the study?
1. Shenzhen Key Medical Discipline Construction Fund (China)
2. Science and Technology Research Project of Hubei Province (China)

Who is the main contact?
Prof. Che Zhang, prof.zh@163.com

Contact information

Type(s)
Principal investigator

Contact name
Prof Che Zhang

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Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

ClinicalTrials.gov (NCT)
Nil known

Protocol serial number
Nil known

Study information

Scientific Title
The safety and efficacy of human umbilical cord mesenchymal stem cell transfusion in patients with Alport syndrome

Study objectives
The intravenous infusion of human umbilical cord mesenchymal stem cell (hUC-MSC) is safe and feasible with good tolerance for patients with Alport syndrome

Ethics approval required
Old ethics approval format

Ethics approval(s)

Approved 08/03/2015, Institutional Review Board of the Affiliated Taihe Hospital of Hubei University of Medicine (No. 32 Southen Renmin Road, Shiyan, Hubei 422000, China; +86 (0)719 8801691; thyyllwyh@126.com), ref: 20150303

Study design
Multicenter interventional single-arm trial

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Albuminuria in the patients with Alport syndrome

Interventions
hUC-MSCs will be injected intravenously at a dose of 20 × 10e6 cells. The duration of infusion will last for 20 - 30 min. Patients will receive four doses of hUC-MSC at an interval of 7 days.

Intervention Type
Biological/Vaccine

Phase
Phase I

Drug/device/biological/vaccine name(s)
hUC-MSCs

Primary outcome(s)
1. Adverse event incidence is calculated at baseline (Day 0), weekly in the treatment phase (Day 7, Day 14, Day 21), and monthly in a follow-up phase (Month 1-12 post-treatment)
2. Urine protein is tested in the laboratory at baseline (Day 0), weekly in the treatment phase (Day 7, Day 14, Day 21), and monthly in a follow-up phase (Month 1-12 post-treatment)

Key secondary outcome(s))
1. Hematuria is tested in the laboratory at baseline (Day 0), weekly in the treatment phase (Day 7, Day 14, Day 21), and monthly in a follow-up phase (Month 1-12 post-treatment)
2. Creatinine clearance rate is tested in the laboratory at baseline (Day 0), and monthly in a follow-up phase (Month 1-12 post-treatment)

Completion date
31/12/2026

Eligibility

Key inclusion criteria
1. Diagnosed with Alport syndrome (AS) according to the diagnosis criteria
2. Aged 3-18 years

3. Hematuria or combination with albuminuria persisted without remission after routine treatments for 1 year
4. Chronic kidney disease stage I-III, glomerular filtration rate >60 ml/min.1.73 m^2
5. Written informed consent is obtained before study specific procedure

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Child

Lower age limit
3 years

Upper age limit
18 years

Sex
All

Key exclusion criteria
1. Aged >18 years
2. Creatinine clearance rate <30 ml/min, or chronic kidney disease stage IV-V
3. With one of the disease histories: immunological disease or autoimmune diseases; serious hematologic or coagulation disorder; urogenital abnormalities; malignancy history; congenital heart disease or serious cardiovascular, liver, or pulmonary dysfunction
4. Uncontrolled endocrine diseases (e.g. diabetes, hyperthyroidism)
5. Serious allergy history or known allergy to more than two kinds of foods or medications
6. Active systemic infection
7. Any other concerns that hampered compliance or safety as judged by the investigator

Date of first enrolment
15/01/2023

Date of final enrolment
31/12/2025

Locations

Countries of recruitment
China

Study participating centre
Affiliated Taihe Hospital of Hubei University of Medicine
No. 32 Southen Renmin Road
Shiyan

China
422000

Study participating centre
Shenzhen Baoan Maternal and Child Health Hospital Affiliated to Jinan University
No. 56 Yulv Road
Shenzhen
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518133

Study participating centre
Hainan Women and Children's Medical Center
No. 75 South Longkun Road
Haikou
China
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Sponsor information

Organisation
Science and Technology Department of Hubei Province

ROR
https://ror.org/00tbh0t11

Funder(s)

Funder type
Government

Funder Name
Science and Technology Research Project of Hubei Province

Funder Name
Shenzhen Key Medical Discipline Construction Fund

Results and Publications

Individual participant data (IPD) sharing plan

The datasets generated during the current study will be available upon request from the principal investigator Prof. Che Zhang (prof.zh@163.com).
The type of data that will be shared: de-identified demographic data, clinical characteristics, and outcomes of participants.
Dates of availability: currently unknown.
Whether consent from participants was required and obtained: written consent forms will be obtained before any study-specific procedure, and will be kept as the source documents at the investigational sites.
Comments on data anonymization: clinical data are de-identified before being collected for scientific research purposes to protect the privacy of patients.
Any ethical or legal restrictions: the current study has been approved by the ethical committee and follows the guidelines for Good Clinical Practice, as well as the requirements of the local Institution for New Drug Clinical Trials.

IPD sharing plan summary

Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol article		15/03/2024	18/03/2024	Yes	No
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes