

A study of T19 in subjects with spinal muscular atrophy

Submission date 03/01/2023	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered <input checked="" type="checkbox"/> Protocol
Registration date 08/01/2023	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
Last Edited 15/07/2025	Condition category Nervous System Diseases	<input type="checkbox"/> Individual participant data <input checked="" type="checkbox"/> Record updated in last year

Plain English summary of protocol

Background and study aims

Spinal muscular atrophy (SMA) is the most common genetic cause of death for children below two years old. Previous studies by us and others have suggested that the fibrinolysis system is involved in nerve degeneration and regeneration and in respiratory failure. In this study, the clinical effects of T19, the key substrate of the fibrinolysis system, were investigated in SMA patients.

Who can participate?

Type I and 1 non-5q (IGHMBP2 gene deficiency) SMA patients

What does the study involve?

Patients were given an intravenous injection of T19 1 time per 1-3 days, for two weeks as one treatment course, with 2-week intervals between courses. Trained clinical evaluators will assess the patients' motor function according to the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND). Respiratory function is assessed by the value of blood oxygen saturation.

What are the possible benefits and risks of participating?

The possible benefits of participating in the trial are improvement of the patient's motor function, respiration function and survival. Patients also get free medication. Considering the properties of T19, there may be a risk of bleeding, hypersensitivity reactions and infection after receiving a T19 injection.

Where is the study run from?

Beijing Chang'an Chinese and Western Integrated Medicine Hospital (China)

When is the study starting and how long is it expected to run for?

September 2018 to September 2021

Who is funding the study?

Talengen Institute of Life Sciences (China)

Who is the main contact?
Dr Jinan Li, jnl@talengen-pharma.com

Contact information

Type(s)

Principal Investigator

Contact name

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Additional identifiers

EudraCT/CTIS number

Nil known

IRAS number

ClinicalTrials.gov number

Nil known

Secondary identifying numbers

CA-18-10

Study information

Scientific Title

T19 shows rapid efficacy in treating patients with type I spinal muscular atrophy

Study objectives

Spinal muscular atrophy (SMA) is the most common genetic cause of death for children aged below two years old. Previous studies by us and others have suggested that the fibrinolysis system is involved in nerve degeneration and regeneration and in respiratory failure. In the present study, we investigated the clinical effects of T19.

Ethics approval required

Old ethics approval format

Ethics approval(s)

Approved 05/09/2018, Ethics Committee of Beijing Chang'an Chinese and Western Integrated Medicine Hospital (19 Zaolinqian St, Xicheng District, Beijing, China; +86-13522667371; 421337949@qq.com), ref: CA-18-10

Study design

Open-label one-arm non-randomized study

Primary study design

Interventional

Secondary study design

Non randomised study

Study setting(s)

Hospital

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use the contact details to request a participant information sheet

Health condition(s) or problem(s) studied

Spinal muscular atrophy

Interventions

Spinal muscular atrophy (SMA) is the most common genetic cause of death for children below two years old. Previous studies by us and others have suggested that the fibrinolysis system is involved in nerve degeneration and regeneration and in respiratory failure. Clinical doctors or nursing staff with more than 5 years of clinical work experience will administer the intervention face-to-face. Based on the condition of the patients, the intervention is performed at the home of patients or at Beijing Chang'an Chinese and Western Integrated Medicine Hospital. The clinical study is an open-label, one-arm, and non-randomized study with a treatment duration of 72 weeks. Freeze-dried T19 (5 or 50 mg per vial), purified from human plasma fraction III at GMP-compliant facilities, was provided by the Talengen Institute of Life Sciences. T19 was dissolved in sterile water to produce 5 mg/ml solutions for use in the study. Patients were given an intravenous injection, at doses of 50-200 mg each time, 1 time per 1-3 days, with two weeks as one treatment course, and 2-week intervals between courses. Trained clinical evaluators assessed the patients' motor function according to the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND). Respiratory function is assessed by the value of blood oxygen saturation.

Intervention Type

Drug

Pharmaceutical study type(s)

Not Applicable

Phase

Not Applicable

Drug/device/biological/vaccine name(s)

Plasminogen

Primary outcome measure

Motor function scores measured using the CHOP INTEND scoring system at baseline and weeks 2, 6, 10, 22 and 46

Secondary outcome measures

1. Respiratory function measured by the value of blood oxygen saturation without Oxygen inhalation in pulse oximetry at baseline and weeks 2, 6, 10, 22 and 46
2. Anthropometric nutritional status measured using the proportion of high body weight, body fat, and growth parameters at baseline and weeks 10, 22 and 46
3. Adverse events assessed by routine blood test, blood biochemistry, coagulation function, hemolysis function, urine routine test, 12 lead ECG, physical examination, vital signs, etc measured using standard procedures at baseline, and weeks 22 and 46

Overall study start date

05/09/2018

Completion date

05/09/2021

Eligibility**Key inclusion criteria**

The subjects were diagnosed with type I SMA with SMN gene mutation or non-5q SMA with mutation in the gene encoding immunoglobulin-binding protein 2 (IGHMBP2), according to genetic tests and clinical symptoms

Participant type(s)

Patient

Age group

Child

Sex

Both

Target number of participants

30

Total final enrolment

20

Key exclusion criteria

Patients receiving more than 16 hours of invasive ventilation per day

Date of first enrolment

05/10/2018

Date of final enrolment

05/08/2020

Locations

Countries of recruitment

China

Study participating centre

Beijing Chang'an Chinese and Western Integrated Medicine Hospital

19 Zaolinqian St

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Sponsor information

Organisation

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Sponsor type

Industry

Funder(s)

Funder type

Industry

Funder Name

Talengen Institute of Life Sciences

Results and Publications

Publication and dissemination plan

Planned publication in a high-impact peer reviewed publication

Intention to publish date

10/05/2023

Individual participant data (IPD) sharing plan

The datasets generated during and/or analysed during the current study are/will be available upon request from Dr Jinan Li, jnl@talengen-pharma.com. The type of data that will be shared is the table of the scoring record, clinical observations in record form, images, videotape, and detection data. Data will be available from 05/10/2023 to 05/10/2033. Consent from participants was required and obtained. Except for the initial record, all patients use unique numbers in favour of anonymity in the experiment. The patient's data, pictures and other relevant information must be approved by the patient before being published.

IPD sharing plan summary

Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol file			06/01/2023	No	No