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A study of T19 in subjects with spinal muscular atrophy

Submission date
03/01/2023

Recruitment status
No longer recruiting

Registration date
08/01/2023

Overall study status
Completed

Last Edited
15/07/2025

Condition category
Nervous System Diseases

[_] Prospectively registered
[X] Protocol
[_] Statistical analysis plan
[_] Results
[_] Individual participant data
[X] 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
Dr Jinan Li

ORCID ID
https://orcid.org/0000-0001-6746-967X

Contact details
Room C602G
289 Digital Peninsula
Shunfeng Industrial Park
No.2 Red Willow Road
Futian District
Shenzhen
China
518000
+86 15919440001
jnl@talengen-pharma.com

Type(s)
Public

Contact name
Dr Chunying Guo

ORCID ID
https://orcid.org/0000-0001-5679-4389

Contact details
Department of Applied Research
Talengen Institute of Life Sciences
Room C602G
289 Digital Peninsula
Shunfeng Industrial Park
No.2 Red Willow Road
Futian District
Shenzhen
China
518000
+86 15167735556
guocy@talengen-pharma.com

Type(s)

Scientific

Contact name
Dr Chunying Guo

Contact details
Department of Applied Research
Talengen Institute of Life Sciences
Room C602G
289 Digital Peninsula
Shunfeng Industrial Park
No.2 Red Willow Road
Futian District
Shenzhen
China
518000
+86 15167735556
guocy@talengen-pharma.com

Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

Protocol serial number
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

Study type(s)
Treatment

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

Phase
Not Applicable

Drug/device/biological/vaccine name(s)
Plasminogen

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

Key secondary outcome(s))
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

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

Healthy volunteers allowed
No

Age group
Child

Sex
All

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
Xicheng District
Beijing
China
100010

Sponsor information

Organisation
Talengen Institute of Life Sciences

Funder(s)

Funder type
Industry

Funder Name
Talengen Institute of Life Sciences

Results and Publications

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