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A study of T06 in patients with amyotrophic lateral sclerosis and Parkinson's disease

Submission date
22/01/2023

Recruitment status
No longer recruiting

Registration date
27/01/2023

Overall study status
Completed

Last Edited
07/07/2025

Condition category
Nervous System Diseases

[_] Prospectively registered
[X] Protocol
[_] Statistical analysis plan
[_] Results
[_] Individual participant data
[_] Record updated in last year

Plain English summary of protocol

Background and study aims
The plasminogen activator (PA) system, a general system that breaks proteins down into smaller peptides (proteolysis), has been suggested to be involved in neurodegeneration and regeneration processes in neurodegenerative diseases, including amyotrophic lateral sclerosis (ALS), a lethal motoneuron disease. During the development of neurodegenerative disorders, including, CNS pathological proteins (including conformationally abnormal proteins or CAPs), i. e., misfolded proteins, denatured proteins and protein aggregates, accumulate inside and between neurons. CNS pathological protein aggregates are toxic to neurons and have an 'infectivity' property, allowing them to spread and propagate between neurons, which causes the death of neurons and the rapid expression of tissue plasminogen activator (tPA) in local neurons and microglia. Upon administration, T06 rapidly passes through the blood-brain-barrier, possibly through tight junctions and transport by endothelial cells, and arrives in the injured extracellular area, where it colocalizes with tPA on CAP aggregates. The formed plasmin rapidly degrades intercellular CNS pathological protein aggregates, and the remaining soluble fragments, i.e., plasmin-generated protein fragments are taken up by microglial cells through endocytosis and subsequently degraded by lysosomes in these cells. In addition, T06 may directly degrade intracellular CNS pathological protein aggregates or indirectly interact with other components to further enhance intracellular degradation. T06 also enters the nucleus to directly or indirectly regulate gene transcription and protein translation, which may further contribute to a decrease in neurodegeneration and an increase in neuroregeneration. Importantly, through the mechanisms proposed above, increases in intracellular and extracellular TP06 levels effectively clear CNS pathological proteins both inside and outside neurons and inhibit the spread of propagative CNS pathological proteins between neurons, and further in combination with its other neuroprotective effects, alleviates neurodegeneration, promotes neuroregeneration and may even restore normal neural functions. Thus, this study will investigate whether T06 is an effective therapeutic strategy to inhibit ALS disease progression, reverse disease symptoms, or even treat the disease.

Who can participate?
Patients diagnosed with ALS or PD

What does the study involve?
This study involves the treatment of T06 in ALS or PD

What are the possible benefits and risks of participating?
Patients can get free medication, and the clinical symptoms of ALS such as disability will be improved after the administration of the drug. Considering the properties of plasminogen, there may be the risk of bleeding, hypersensitivity reactions and infection after receiving plasminogen.

The study involves the effects of plasminogen on ALS patients.

Where is the study run from?
The intervention was performed at the home of patients or Beijing Chang'an Chinese and Western Integrated Medicine Hospital

When is the study starting and how long is it expected to run for?
January 2018 to October 2021

Who is funding the study?
Talengen Institute of Life Sciences (China)

Who is the main contact?
1. Dr Jinan Li (Principal investigator), jnl@talengen-pharma.com
2. Ms Chunying Guo (public/scientific contact), guocy@talengen-pharma.com

Contact information

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Public

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Protocol serial number
CA-18-08

Study information

Scientific Title
A study of T06 in patients with amyotrophic lateral sclerosis and Parkinson's disease

Study objectives
The use of T06 effectively clears CNS pathological proteins, exerts neuroprotective effects, alleviates neurodegeneration, promotes neuroregeneration and may even restore normal neural functions in patients with amyotrophic lateral sclerosis and Parkinson's disease.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 05/09/2018, The 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: none provided

Primary study design

Interventional

Study design
One-arm open-label non-randomized interventional study

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Amyotrophic lateral sclerosis and Parkinson’s disease

Interventions
T06 is a protein being studied as a potential treatment for amyotrophic lateral sclerosis (ALS) and Parkinson’s disease (PD). Currently, there are only a few FDA-approved drugs to treat ALS or PD, but they do not have a significant impact on survival or disease progression. Therefore, researchers are looking for new and effective ways to treat ALS and PD.

The study was conducted by clinical doctors or nursing staff with more than 5 years of clinical work experience. They administered the treatment face-to-face, either at the patient's home or at the Beijing Chang’an Chinese and Western Integrated Medicine Hospital. The study was an open-label, one-arm, and non-randomized study. The treatment duration was 72 weeks.

The treatment was given through intravenous injection, at a dose of 50-200 mg each time, 1 time per 1-3 days, two weeks as one treatment course, and 2 weeks intervals between courses. Sometimes, the intravenous injection was combined with atomization inhalation, which was administered once a day, 10 mg each time, beginning on the third day of treatment.

The patients' clinical symptoms of ALS were measured using the Revised Amyotrophic Lateral Sclerosis Functional Rating (ALSFRS-R) or by trained clinical evaluators.

Intervention Type
Drug

Phase
Not Applicable

Drug/device/biological/vaccine name(s)
T06

Primary outcome(s)
Current primary outcome measure as of 09/11/2023:
Motor function measured using the ALS Functional Rating Scale-Revised (ALS-FRS-R) scale at baseline and 2, 6, 10, 22 and 46 weeks or the statement of patients were evaluated by Movement Disorder Society-Sponsored Revision of the Unified Parkinson’s Disease Rating Scale (MDS-UPDRS) at baseline and 2, 6, 10, 22 and 46 weeks

Previous primary outcome measure:
Motor function measured using the ALS Functional Rating Scale-Revised (ALS-FRS-R) scale at baseline and 2, 6, 10, 22 and 46 weeks

Key secondary outcome(s)

Adverse events measured by a routine blood tests, blood biochemistry, coagulation function, hemolysis function, routine urine tests, 12 lead ECG, physical examination, vital signs, etc at baseline and 22 and 46 weeks

Completion date
01/10/2021

Eligibility

Key inclusion criteria
1. Male and female patients ≥18 years of age with sporadic or familial ALS or PD
2. If taking riluzole and/or edaravone, must be on a stable dose prior to screening

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 Years

Sex
All

Key exclusion criteria
1. History of a clinically significant non-ALS or PD neurologic disorder
2. Inability to swallow capsules.
3. Human immunodeficiency virus (HIV) or current chronic/active infection with hepatitis C virus or hepatitis B virus
4. Women who are pregnant, planning to become pregnant, or are breastfeeding.
5. Use of non-invasive ventilation (NIV) or mechanical ventilation via tracheostomy, or any form of oxygen supplementation.
6. Current or anticipated need for a diaphragm pacing system (DPS).
7. Currently using glucocorticoids or have a history of regular systemic glucocorticoid use within the last 12 months.
8. Previous exposure or treatment with glucocorticoid receptor modulators or antagonists.

Date of first enrolment
05/01/2019

Date of final enrolment
01/10/2020

Locations

Countries of recruitment

China

Study participating centre
Beijing Chang'an Chinese and Western Integrated Medicine Hospital
19 Zaolinqian St, Xicheng District
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Sponsor information

Organisation
Talengen Institute of Life Sciences

Funder(s)

Funder type
Research organisation

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 Ms Chunying Guo, guocy@talengen-pharma.com.

The type of data that will be shared comprises a table showing the scoring records, clinical observation record forms, images, videotapes, and detection data. Dates of availability: 05/10 /2023 to 05/10/2033.

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol file			27/01/2023	No	No