

Clinical Trial Protocol

Iranian Registry of Clinical Trials

10 Jun 2026

Evaluation of safety and efficacy of focal intramuscular injection of wharton's gelly-derived mesenchymal stem cell in Duchenne muscular dystrophy pediatrics between 5 to 12 years old

Protocol summary

Study aim

Evaluation of therapeutic effects and complications of treatment of Duchenne muscular dystrophy with stem cells

Design

The clinical trial is performed on 5 patients who have referred for treatment and are followed up for one year.

Settings and conduct

Two injections of 1 million/kg stem cells derived from Wharton gel in 4 muscles of 5 children with Duchenne dystrophy

Participants/Inclusion and exclusion criteria

Inclusion criteria: age 5 to 12 years old; genetic evidence; the patient's ability to walk is not completely lost and the use of assistive devices is not permanent; the patient's muscle strength is decreasing; willingness and satisfaction to participate in the study; weight between 15 to 35. Exclusion criteria: advanced motor impairment leading to permanent use of crutches, walkers and wheelchairs; history of known malignancy; history of associated genetic and non-genetic degenerative diseases; history of any other acute or chronic illness that, at the discretion of the clinician responsible for implementing the plan, prevents the patient from entering the study.

Intervention groups

Intervention group: In each injection, 1 x 10⁶ mesenchymal cell derived from Wharton's gelly is injected per kilogram of body weight. The injection volume of 25 cc includes a suspension containing one million mesenchymal cells, two local injections are performed in 4 to 5 areas under the muscle fascia at an interval of 45 days

Main outcome variables

Respiratory Function(FVC); Muscular Function(physical examination and lab study); Cardiac Function(EF)

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20150206020981N4**

Registration date: **2022-12-19, 1401/09/28**

Registration timing: **registered_while_recruiting**

Last update: **2022-12-19, 1401/09/28**

Update count: **0**

Registration date

2022-12-19, 1401/09/28

Registrant information

Name

Khadijeh Hajinaghitehrani

Name of organization / entity

Azad Medical University

Country

Iran (Islamic Republic of)

Phone

+98 21 2257 1228

Email address

khtehrani@iautmu.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2022-12-19, 1401/09/28

Expected recruitment end date

2023-12-19, 1402/09/28

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Evaluation of safety and efficacy of focal intramuscular injection of wharton's gelly-derived mesenchymal stem cell in Duchenne muscular dystrophy pediatrics between 5 to 12 years old

Public title

Evaluation of safety and efficacy of focal intramuscular injection of wharton's gelly-derived mesenchymal stem cell in Duchenne muscular dystrophy pediatrics between 5 to 12 years old

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Age between 5 to 12 Genetic evidence of Duchenne disease as a cause of myopathy The patient's ability to walk is not completely lost and the use of assistive devices such as canes and walkers is not permanent. The patient's muscle strength is decreasing Willingness and satisfaction to participate in the study by the patient's legal guardian in decision making and willingness to participate regularly in follow-up courses Weight between 15 to 35

Exclusion criteria:

Advanced motor impairment leading to permanent use of crutches, walkers and wheelchairs A history of known malignancy History of associated genetic and non-genetic degenerative diseases A history of any other acute or chronic illness that, at the discretion of the clinician responsible for implementing the plan, prevents the patient from entering the study.

Age

From **5 years** old to **12 years** old

Gender

Male

Phase

2

Groups that have been masked

No information

Sample size

Target sample size: **5**

Randomization (investigator's opinion)

N/A

Randomization description

Blinding (investigator's opinion)

Not blinded

Blinding description

Placebo

Not used

Assignment

Single

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Ethics Committee on Research Islamic Azad University Pharmaceutical Sciences Branch

Street address

Tehran Province, Tehran, District 1, Yakhchal Street, Yasaman Alley

City

Tehran

Province

Tehran

Postal code

1941933111

Approval date

2022-12-04, 1401/09/13

Ethics committee reference number

IR.IAU.PS.REC.1401.334

Health conditions studied

1

Description of health condition studied

Duchenne muscular dystrophy

ICD-10 code

G71.0

ICD-10 code description

Muscular dystrophy

Primary outcomes

1

Description

Respiratory Function

Timepoint

1, 3, 6, 9 and 12 month after injection

Method of measurement

Spirometry

2

Description

Muscular Force

Timepoint

First every 14 days to 1 month, then Every 1 month to 1 year

Method of measurement

Physical Exam

3

Description

Cardiac Function

Timepoint

1, 3, 6, 9 and 12 month after injection

Method of measurement

Echocardiography

Secondary outcomes

1

Description

Muscular Enzymes

Timepoint

1, 3, 6, 9 and 12 month after injection

Method of measurement

Lab Data

Intervention groups

1

Description

Intervention group: In each injection, 1million mesenchymal cell derived from Wharton's gelly is injected per kilogram of body weight. The injection volume of 25 cc includes a suspension containing one million mesenchymal cells, two local injections are performed in 4 to 5 areas under the muscle fascia at an interval of 45 days

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Iranian Muscular Dystrophy Association

Full name of responsible person

Dr. Khadijeh Hajinaghi Tehrani

Street address

No 27, Montazeri St. Piroozi St. Shohada Square

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17117

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+98 21 3660 7093

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dr_tehrani10@yahoo.com

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Sinacell Company

Full name of responsible person

Ehsan Seyed Jafari

Street address

No. 162, No. 162, 16th Innovation Street,Pardis
Technology Park, Jajroud Road,Kilometer 16

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1657163871

Phone

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Fax

Email

Seyedjafari@ut.ac.ir

Grant name

Grant code / Reference number

Is the source of funding the same sponsor organization/entity?

Yes

Title of funding source

Sinacell Company

Proportion provided by this source

100

Public or private sector

Private

Domestic or foreign origin

Domestic

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

Academic

Person responsible for general inquiries

Contact

Name of organization / entity

Islamic Azad University

Full name of responsible person

Khadijeh Hajinaghi Tehrani

Position

Neurologist / Plan Designer

Latest degree

Specialist

Other areas of specialty/work

Neurology

Street address

Farhikhtegan Hospital,Tehran Province, Tehran,
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Person responsible for scientific inquiries

Contact

Name of organization / entity

Islamic Azad University

Full name of responsible person

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Person responsible for updating data**Contact****Name of organization / entity**

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Sharing plan**Deidentified Individual Participant Data Set (IPD)**

Yes - There is a plan to make this available

Study Protocol

Yes - There is a plan to make this available

Statistical Analysis Plan

Yes - There is a plan to make this available

Informed Consent Form

Yes - There is a plan to make this available

Clinical Study Report

Undecided - It is not yet known if there will be a plan to make this available

Analytic Code

Undecided - It is not yet known if there will be a plan to make this available

Data Dictionary

Undecided - It is not yet known if there will be a plan to make this available

Title and more details about the data/document

The only some part of data will be shared.

When the data will become available and for how long

5 months after publish

To whom data/document is available

Only academic researcher

Under which criteria data/document could be used

By official permission

From where data/document is obtainable

By the corresponding author

What processes are involved for a request to access data/document

By the official request to the corresponding author, if she/he agrees to it.

Comments