Clinical Trial Protocol
Iranian Registry of Clinical Trials

18 Sep 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
Spirometery

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
City
Tehran
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Postal code
17117
Phone
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Email
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
City
Tehran
Province
Tehran
Postal code
1657163871
Phone
+98 21 7625 0661
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
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Person responsible for scientific inquiries

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Name of organization / entity
Islamic Azad University

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Khadijeh Hajinaghi Tehrani
Position
Neurologist
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Person responsible for updating data

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Name of organization / entity
Islamic Azad University
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1477899679
Phone
+98 21 4486 7262
Email
Khtehrani@iautmu.ac.ir

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