Clinical Trial Protocol
Iranian Registry of Clinical Trials

28 May 2026

A phase III, single-arm, cross-over, multicenter clinical trial to compare efficacy and safety of YW17 (laronidase; CinnaGen) versus laronidase (Aldurazyme®; Genzyme, BioMarin) in patients with Mucopolysaccharidosis type I (MPS I)

Protocol summary

Study aim

The Primary objective of this clinical trial is to assess efficacy of YW17 (Laronidase produced by CinnaGen Co.) in comparison with Aldurazyme® (Laronidase produced by Genzyme, BioMarin) in patients with mucopolysaccharidosis type I (MPS I).

Design

Phase III, single arm, open-label, cross-over, and multicenter clinical trial

Settings and conduct

.Phase III, open labeled, one armed, cross over, active controlled, with 1:1 allocation and sample size 12, in Tehran - Iran.

Participants/Inclusion and exclusion criteria

Inclusion criteria: Mucopolysaccharidosis type I patients, aged between 5 to 18 years; exclusion criteria: Prior bone marrow transplantation or being a candidate for receiving HSCT, prior Tracheotomy, pregnancy or lactation, circumstance that could significantly interfere with study compliance, severe organic disease not associated with mucopolysaccharidosis type I (MPS I), known hypersensitivity to laronidase or components of the laronidase solution, Abnormal renal function, acute hydrocephalus, patients who are naïve to laronidase.

Intervention groups

All patients will be in the intervention group. Patients will receive 0.58 milligram per kilogram of body weight of Aldurazyme® (laronidase produced by Genzyme, BioMarin) for 12 weeks and then will receive the same amount of YW17 (laronidase produced by Cinnagen Co.) for the next 12 weeks by intravenous infusion. In addition, to minimize possible infusion-related reactions, all patients will receive acetaminophen and diphenhydramine one hour before each injection.

Main outcome variables

Mean level of urinary Glycosaminoglycan adjusted by the level of urinary creatinine; Distance traveled (based on meters) in 6 minutes (6-minute walk test); Forced Vital Capacity percentage; Adverse events; Enzyme assay

General information

Reason for update

Manufacture code change

Acronym

IRCT registration information

IRCT registration number: **IRCT20150303021315N30**
Registration date: **2022-05-02, 1401/02/12**
Registration timing: **prospective**

Last update: **2024-08-11, 1403/05/21**
Update count: **2**

Registration date

2022-05-02, 1401/02/12

Registrant information

Name
Nassim Anjidani
Name of organization / entity
Orchid Pharmed
Country
Iran (Islamic Republic of)
Phone
+98 21 4347 3000
Email address
amini@orchidpharmed.com

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2022-05-22, 1401/03/01

Expected recruitment end date
2023-05-22, 1402/03/01
Actual recruitment start date
empty
Actual recruitment end date
empty
Trial completion date
empty

Scientific title
A phase III, single-arm, cross-over, multicenter clinical trial to compare efficacy and safety of YW17 (laronidase; CinnaGen) versus laronidase (Aldurazyme®; Genzyme, BioMarin) in patients with Mucopolysaccharidosis type I (MPS I)

Public title
A phase III clinical trial to assess efficacy and safety of YW17 (laronidase; CinnaGen)
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Patients Aged Between 5 to 18 years
Mucopolysaccharidosis type I confirmed Willingness and having informed consent (patient or his/her parents/ legal guardian) to participate in this study
Exclusion criteria:
Prior bone marrow transplantation Being a candidate for receiving hematopoietic stem cell transplantation Prior Tracheotomy Pregnancy Lactation Administration of any investigational drug within 30 days before study enrollment Medical condition or other circumstance that could significantly interfere with study compliance The severe organic disease that is not associated with mucopolysaccharidosis type 1 Known hypersensitivity to laronidase or components of the laronidase solution Abnormal renal function determined by measuring serum creatinine and blood urea nitrogen (BUN) levels Acute hydrocephalus Prescribing laronidase for the first time for the patient (naive patient)
Age
From **5 years** old to **18 years** old
Gender
Both

Phase
3
Groups that have been masked
No information
Sample size
Target sample size: **12**
More than 1 sample in each individual
Number of samples in each individual: **2**
In this study, each person will take drug twice and compare with each other.
Randomization (investigator's opinion)
N/A
Randomization description
Blinding (investigator's opinion)
Not blinded
Blinding description
Placebo
Not used
Assignment
Crossover
Other design features
This clinical trial consists of screening visit, visits 1 to 24, and final visit. After screening, the interval between visits is one week.

Secondary Ids

empty

Ethics committees

1

Ethics committee
Name of ethics committee
Ethics Committee of the Institute of Pharmaceutical Sciences (TIPS)
Street address
The Institute of Pharmaceutical Sciences (TIPS), Faculty of Pharmacy, Tehran University of Medical Sciences, Poursina Avenue
City
Tehran
Province
Tehran
Postal code
14176-13151
Approval date
2022-03-17, 1400/12/26
Ethics committee reference number
IR.TUMS.TIPS.REC.1401.001

Health conditions studied

1

Description of health condition studied
Patients with Mucopolysaccharidosis type I (MPS I)
ICD-10 code
E76.0
ICD-10 code description
Mucopolysaccharidosis, type I

Primary outcomes

1

Description
Mean level of urinary Glycosaminoglycan adjusted by the level of urinary creatinine
Timepoint
Visits 9, 11, and 13 (related to the treatment with Aldurazyme®) and visits 21, 23, and final visit (related to the treatment with YW17)
Method of measurement
Liquid chromatography tandem mass spectrometry

Secondary outcomes

1

Description
Distance traveled (based on meters) in 6 minutes (6-minute walk test)
Timepoint
Visits 1, 13, and Final
Method of measurement
Timer and meter

2

Description
Forced Vital Capacity percentage
Timepoint
Visits 1, 13, and Final
Method of measurement
Spirometer

3

Description
Adverse events
Timepoint
At all visits
Method of measurement
All adverse events are assessed through patient reporting, physician diagnosis, and are then classified by severity (based on common terminology criteria for adverse events (CTCAE)), seriousness and relationship to the study drug.

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Description
Enzyme assay (α-L-iduronidase activity measurement)
Timepoint
Immediately after, half an hour after, and 1.5 hours after the infusion of medicine at visits 12 and 24
Method of measurement
Liquid chromatography tandem mass spectrometry or High-throughput mass spectrometry

Intervention groups

1

Description
Intervention group: Patients will receive Aldurazyme® (laronidase produced by Genzyme, BioMarin) from visits 1 to 12 (weekly) and will receive YW17 (laronidase produced by Cinnagen Co.) from visit 13 to 24. The Dosing of laronidase is 0.58 milligram per kilogram of patient’s body weight. Laronidase is administered by intravenous infusion in 3 to 4 hours. The dosage form of laronidase (Both products) is vial consisting of a solution with a concentration of 2.9 milligrams per 5 milliliters to be diluted in normal saline. To minimize possible infusion-related reactions, all patients will receive acetaminophen and diphenhydramine one hour before each injection.
Category
Treatment - Drugs

Recruitment centers

1

Recruitment center
Name of recruitment center
Growth and Development Research Center, Iran Metabolic Laboratory
Full name of responsible person
Dr. Ali Rabani - Dr. Aria Setoodeh
Street address
Metabolic Laboratory of Pediatric Medical Center, Building No. 2,No. 62, Pediatric Medical Center, Dr. Gharib St, End of Keshavarz Boulevard
City
Tehran
Province
Tehran
Postal code
14197333141
Phone
+98 21 6657 5730
Fax
+98 21 6694 9662
Email
gdrc@tums.ac.ir

2

Recruitment center
Name of recruitment center
Loqmane Hakim Hospital
Full name of responsible person
Dr. Shadab Salehpour
Street address
Tehran, South Kargar St., Kamali St., Loqmane Hakim Hospital
City
Tehran
Province
Tehran
Postal code
1333635445
Phone
+98 21 5541 9005
Fax
+98 21 5541 9009
Email
loghman.hospital@sbmu.ac.ir

3

Recruitment center
Name of recruitment center
Mofid Children's Hospital
Full name of responsible person
Dr. Mohammad Reza Alaei - Dr. Marjan Shakiba
Street address

Tehran, Shariati St., above Hosseinieh Irshad, Mofid Children's Hospital

City
Tehran

Province
Tehran

Postal code
15514 – 15468

Phone
+98 21 2222 7021

Email
info-mch@sbmu.ac.ir

4

Recruitment center

Name of recruitment center
Akbar Children's Hospital

Full name of responsible person
Dr. Samaneh Nowrozi Asl - Dr. Peyman Eshraghi

Street address
Mashhad, Shahid Kaveh Boulevard, in front of Shahid Kaveh 14, Akbar Children's Hospital

City
Tehran

Province
Tehran

Postal code
9177897157

Phone
+98 51 3871 3801

Email
ak.pr@mums.ac.ir

Sponsors / Funding sources

1

Sponsor

Name of organization / entity
CinnaGen company

Full name of responsible person
Dr. Haleh Hamedifar

Street address
CinnaGen research and production Company, Simin Dasht Industrial Park, Karaj, Alborz, Iran

City
Karaj

Province
Alborz

Postal code
3165933155

Phone
+98 26 3667 0980

Email
cinnagen@cinnagen.com

Grant name

Grant code / Reference number

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

Title of funding source
CinnaGen 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
Industry

Person responsible for general inquiries

Contact

Name of organization / entity
Orchid Pharmed Co.

Full name of responsible person
Dr. Nasim Anjidani

Position
Medical Department Manager

Latest degree
Medical doctor

Other areas of specialty/work
Medical Pharmacy

Street address
No 42. Attar sq, Attar st, Valiasr st, Vanak sq, Tehran, Iran

City
Tehran

Province
Tehran

Postal code
1994766411

Phone
+98 21 4347 3000

Email
anjidani.n@orchidpharmed.com

Person responsible for scientific inquiries

Contact

Name of organization / entity
Orchid Pharmed Company

Full name of responsible person
Dr. Nasim Anjidani

Position
Pharmacist, Clinical Trial Manager

Latest degree
Medical doctor

Other areas of specialty/work
Medical Pharmacy

Street address
No 42. Attar sq, Attar st, Valiasr st, Vanak sq, Tehran, Iran

City
Tehran

Province
Tehran

Postal code

1994766411

Phone

+98 21 4347 3000

Email

anjidani.N@orchidpharmed.com

Person responsible for updating data

Contact

Name of organization / entity

Orchid Pharmed Co.

Full name of responsible person

Dr. Nasim Anjidani

Position

Medical Department Manager

Latest degree

Medical doctor

Other areas of specialty/work

Medical Pharmacy

Street address

• No 42. Attar sq, Attar st, Valiasr st, Vanak sq, Tehran, Iran

City

Tehran

Province

Tehran

Postal code

1994766411

Phone

+98 21 4347 3000

Email

anjidani.n@orchidpharmed.com

Sharing plan

Deidentified Individual Participant Data Set (IPD)

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

Study Protocol

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

Statistical Analysis Plan

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

Informed Consent Form

Undecided - It is not yet known if there will be 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