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SCIENCE MEDICINES HEALTH

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Human Medicines Division

Assessment report for paediatric studies submitted according to Article 46 of the Regulation (EC) No 1901/2006

TAKHZYRO

Lanadelumab

Procedure no: EMA/PAM/0000326082

Note

Assessment report as adopted by the CHMP with all information of a commercially confidential nature deleted.

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Status of this report and steps taken for the assessment			
Current step[1]	**Description**	**Planned date**	**Actual Date**
☐	Start of Procedure	23 February 2026	23 February 2026
☐	CHMP Rapporteur AR	30 March 2026	30 March 2026
☐	CHMP comments	13 April 2026	n/a
☐	Updated CHMP Rapporteur AR	16 April 2026	n/a
☒	CHMP outcome	23 April 2026	23 April 2026

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1. Introduction

On 28 January 2026, the MAH submitted a completed paediatric study for TAKHZYRO, in accordance with Article 46 of Regulation (EC) No1901/2006, as amended.

A short critical expert overview has also been provided.

2. Scientific discussion

2.1. Information on the development program

The MAH stated that "***Study TAK-743-4012****: The Real-World Effectiveness and Safety of Lanadelumab (Takhzyro) and Icatibant (Firazyr®) for Hereditary Angioedema: An Observational Study in China"* is a stand-alone study.

2.2. Information on the pharmaceutical formulation used in the study

Commercial Takhzyro and Firazyr were used.

2.3. Clinical aspects

2.3.1. Introduction

Lanadelumab is a fully human monoclonal antibody against plasma kallikrein that prevents the cleavage of high-molecular-weight kininogen and generation of bradykinin. Lanadelumab has been approved in several countries and regions for routine prevention of attacks of hereditary angioedema (HAE).

The approvals of lanadelumab and icatibant in China were based on global trials that did not include Chinese participants; thus, the collection of real-world clinical outcomes of Chinese adult and paediatric participants was essential to better understand the effectiveness and safety of lanadelumab and icatibant in routine clinical practice in China.

Subjects of all ages for which lanadelumab and icatibant are approved in China were eligible to the study.

Assessor's comment

As this is a p46 procedure for a Phase 4 observational study that is not part of the EU RMP, only the paediatric data are assessed.

Data for the icatibant part of the study are presented separately in procedure Firazyr EMA/PAM/0000326083 (EMEA/H/C/000899)

2.3.2. Clinical study

Clinical study number and title

Study TAK-743-4012*: The Real-World Effectiveness and Safety of Lanadelumab (Takhzyro) and Icatibant (Firazyr®) for Hereditary Angioedema: An Observational Study in China*

Description

TAK-743-4012 was a retrospective, multicentre, observational trial to evaluate the effectiveness and safety of lanadelumab (Takhzyro) and icatibant (Firazyr) for HAE in the real-world settings in China.

Methods

Participants who used at least 1 dose of lanadelumab or icatibant from the lanadelumab National Medical Products Administration approval date (02 December 2020) to the date of the day before the first trial site initiation date in the real-world settings were included.

At patient-level, each participant is observed from the start date of the patient-level overall baseline period (i.e., the start of six-month period preceding the overall index date) until the end of patient-level retrospective observation period (i.e., the date of the day before the 1st site initiation date).

The data collection started from 20 July 2024 (first trial site initiated on 18 July 2024) and ended on 31 July 2025.

The participants included in this trial were categorized into one of the following mutually exclusive groups:

- Group L included participants who only use lanadelumab during the trial-level retrospective observation period.
- Group I included participants who only use icatibant during the trial-level retrospective observation period.
- Group B included participants who use both lanadelumab and icatibant during the trial-level retrospective observation period.

Study participants

Chinese patients who have received at least one dose of lanadelumab or icatibant from the lanadelumab national approval date to the date of the day before the first site initiation date were eligible. No age limits applied.

Treatments

This trial had no treatments provided nor any imposed visit schedules. Real-world treatment patterns (including dosage and frequency of administration, reason for discontinuation, and median time to discontinuation) were abstracted retrospectively from eligible participants' medical records and HAE diaries. Participant HAE diaries were administered as part of the participant's routine clinical care.

Objectives

- Primary objectives
    - Lanadelumab: Monthly attack rate during exposure
    - Icatibant: Time to complete HAE attack resolution
- Secondary objectives
    - Lanadelumab adverse events (AEs) and serious adverse events (SAEs)
    - Icatibant adverse events (AEs) and serious adverse events (SAEs)

    - Lanadelumab real-world treatment patterns
- Exploratory objectives
    - Lanadelumab treatment satisfaction (Treatment Satisfaction Questionnaire for Medication-9 items)

Sample size

The sample size was driven by study feasibility and other non-statistical considerations. Approximately 130 Chinese from about 10 sites were expected to be included into the study.

Randomisation and blinding (masking)

N/A

Statistical Methods

Only descriptive statistics applied.

Results

Participant flow

A total of 116 participants signed the informed consent form for screening, with 115 participants successfully enrolled in this trial. All 115 enrolled participants completed the trial and were included in the full analysis set, of which 44 participants were in Group L (lanadelumab).

Two participants from Group L were enrolled at the age of >18 years but received lanadelumab first injection at the age of less than 18 years.

Baseline data

One paediatric patient was diagnosed with type I HAE, and with a family history. The participant had no medical history or comorbidity.

The other paediatric patient was diagnosed with type I HAE, and without family history. The participant had no medical history or comorbidity

Efficacy results

The first paediatric participant had a treatment duration of 177 days during the retrospective period with 11 injections of lanadelumab without treatment discontinuation. The injection interval was from 13 to 22 days for the total 11 injections.

The other paediatric participant had a treatment duration of 65 days during the retrospective period with 5 injections of lanadelumab without treatment discontinuation. The injection interval was from 14 to 18 days for the total 5 injections.

None of the two paediatric patients experience any HAE attack after injection of lanadelumab.

Safety results

No TEAE was observed from any of the two paediatric participants.

2.3.3. Discussion on clinical aspects

Study TAK-743-4012 was a retrospective, multicentre, observational trial to evaluate the effectiveness and safety of lanadelumab (Takhzyro) and icatibant (Firazyr) for HAE in the real-world settings in China.

Two participants were enrolled at the age of >18 years but received lanadelumab first injection at the age of less than 18 years. The subjects received 11 and five injections, respectively, during the observational period. None of the two subjects experience any HAE attack after injection of lanadelumab. In addition, no TEAE was reported from any of the two paediatric participants.

3. Rapporteur's overall conclusion and recommendation

The effectiveness of lanadelumab in the paediatric subpopulation of study TAK-743-4012 is largely in line with the previous experience of lanadelumab. No TEAE was reported by any of the twio paediatric participants.

No additional actions are considered warranted based on the data in the paediatric subpopulation of the study.

The benefit/risk ratio for Takhzyro remains unchanged.

☒ **Fulfilled:**

No regulatory action required.