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Human Medicines Division

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

Firazyr

Icatibant

Procedure no: EMA/PAM/0000326083

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 Firazyr, 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

Icatibant is a selective antagonist of the B2 receptor and can alleviate the symptoms of an acute release of bradykinin. Icatibant has been approved for the symptomatic treatment of acute attacks of HAE in 63 countries. The indications for use may vary in different countries.

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 lanadelumab part of the study are presented separately in procedure Takhzyro EMA/PAM/0000326082 (EMEA/H/C/004806).

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 hereditary angioedema (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 28 participants were in Group I (icatibant only).

The trial enrolled 1 participant <18 years old at enrolment. The paediatric participant only received icatibant and was included in Group I.

Baseline data

The paediatric participant was a patient with type I HAE, with a family history. The participant had no medical histories or comorbidities.

Efficacy results

The paediatric participant experienced 2 mild attacks during the retrospective period. The two attacks both affected abdominal-gastrointestinal site, and each attack completely resolved 10 minutes after icatibant injection, thus the median complete resolution time was 10.0 (NE, NE) minutes.

Safety results

No TEAE was reported from the paediatric participant.

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.

The trial enrolled one icatibant-treated participant <18 years old at enrolment. The paediatric participant experienced two mild HAE attacks during the retrospective period. Each attack completely resolved 10 minutes after icatibant injection.

No TEAE was reported from the paediatric participant

3. Rapporteur's overall conclusion and recommendation

The effectiveness of icatibant in the single icatibant-treated paediatric patient in study TAK-743-4012 is largely in line with the previous experience of icatibant. No TEAE was reported by the paediatric participant.

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

The benefit/risk ratio for Firazyr remains unchanged.

☒ **Fulfilled:**

No regulatory action required.