EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMADOC-1700519818-2067426
Human Medicines Division

Assessment report for paediatric studies submitted in accordance with article 46 of regulation (EC) No 1901/2006, as amended

Hizentra

Human normal immunoglobulin

Procedure no.: EMA/PAM/0000266310

Note

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

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1. Introduction

On 14 April 2025, the MAH submitted a completed paediatric study for Hizentra, 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 Hizentra 20% S.C.Injection 1g/5mL, 2g/10mL, 4g/20mL General Drug Use-Results Survey (IgPro20_5007) is a stand-alone study.

2.2. Information on the pharmaceutical formulation used in the study

The study was performed in a post-marketing setting using commercial Hizentra.

2.3. Clinical aspects

2.3.1. Introduction

The MAH submitted a final report for:

- IgPro30_5007: Hizentra 20% S.C.Injection 1g/5mL, 2g/10mL, 4g/20mL General Drug Use-Results Survey

2.3.2. Clinical study IgPro30_5007

CHMP comment

This is a p46 procedure for a non-interventional study that was conducted in Japan only. Therefore, only the paediatric data will be assessed.

Description

Study IgPro20_5007 was conducted to examine the incidence of infusion site reactions as adverse drug reactions related to Hizentra under the routine clinical practice of chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) patients, and any differences in the incidence associated with the dose increase compared with that of previously approved indications of agammaglobulinemia and hypogammaglobulinemia. The study was conducted in Japan.

The observation/treatment period was set to 6 months.

The MAH stated that no changes to the European SmPC are proposed based on the study results.

Methods

Study participants

The survey enrolled CIDP patients treated with Hizentra after the approval date for the indication "preventing the progress of decreased motor function by CIDP (those who showed improvement in

muscular weakness)", as well as patients who gave the consent for personal data handling and use of this survey results in the contracted medical institutions.

Treatments

Treatment with Hizentra according to label

A treatment period of 6 months was set, which was the same as the observation period.

Objective(s)

Objectives of this survey were to examine the incidence of infusion site reactions including ulceration like-infusion site reaction as adverse drug reactions which related Hizentra (hereinafter, ADRs) of Hizentra 20% S.C. infusion 1g/5mL, 2g/10mL, and 4g/20mL (hereinafter, Hizentra) under the routine clinical practice of chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) patients, and any differences in the incidence associated with the dose increase compared with that of previously approved indications of agammaglobulinemia and hypogammaglobulinemia. The risk factors associated with the incidence of infusion site reactions were also investigated.

Outcomes/endpoints

"Infusion site reactions including ulceration like-infusion site reactions" was set as a safety assessment in this survey.

Table 1: Survey Items

Patient characteristics	Date of birth or age, sex, start date of Hizentra, reason for use of Hizentra, onset date of CIDP, diagnosis date of CIDP, clinical pathological type, CIDP pre-treatment drugs, medical history/complication, height
Details on Hizentra treatment	Administration date, total dose, weight, administration duration, number of infusion site, administration site, administration location, person who administered Hizentra
Hizentra treatment at the end of observation period	Treatment continued/discontinued (date of discontinuation)
Concomitant drugs related to CIDP treatment	Drug name, daily dose, administration route, start date, end date
Concurrent therapy	Details on concurrent therapies
Infusion site reactions including ulceration like-infusion site reactions	Adverse event term of infusion site reactions including ulceration like-infusion site reactions, administration site at the onset of infusion site reactions, onset date, batch No. of Hizentra, duration of the event, treatment details, seriousness, action taken with Hizentra, outcome date, outcome, causal relationship to Hizentra, factors other than Hizentra

Sample size

Target sample size: 80

Randomisation and blinding (masking)

N/A

Statistical Methods

N/A

Results

Participant flow

A total of 108 patients were registered from 38 sites in Japan.

Only one patient under 18 years old was enrolled.

Recruitment

Survey period: October 15, 2019 to September 30, 2022

Baseline data

The paediatric patient was 17 years old female. No complications or concomitant medications for CIDP treatment were observed. Hizentra was administered at an initial dose of 100mL and average dose of 100 mL. Hizentra was administered 26 times during the 6-month observation period.

Efficacy results

Not applicable

Safety results

In the paediatric patient, no injection site reactions, which are a safety consideration, were observed during the 6-month observation period.

CHMP comment

No additional information has been provided by the MAH. The adolescent patient was not described separately in the CSR. While this information is sparse, it is not expected that a request for additional information on this patient would lead to a change in the known safety profile of Hizentra.

2.3.3. Discussion on clinical aspects

Study IgPro20_5007 was a non-interventional survey conducted in Japan to examine the incidence of infusion site reactions in CIDP patients under routine clinical practice. Only one subject under the age of 18 was enrolled.

The only information on safety in the paediatric population provided by the MAH is limited to noting that the adolescent patient experienced no injection site reactions. While this information is sparse, it

is not expected that a request for additional information on this patient would lead to a change in the known safety profile of Hizentra.

3. CHMP overall conclusion and recommendation

No injection site reactions were observed in the adolescent patient with CIDP. Thus, the safety profile in the paediatric/adolescent population of the Study IgPro20_5007 can be considered consistent with the known safety profile of Hizentra.

It is agreed that no changes to the Product information are required.

The benefit/risk ratio for Hizentra remains unchanged.

☒ **Fulfilled:**

No regulatory action required.