Amsterdam, 24 July 2025
EMADOC-1700519818-2064733
Committee for Medicinal Products for Human Use (CHMP)

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

Rezzayo

International non-proprietary name: Rezafungin

Procedure no.: EMA/PAM/0000266096

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	**Description**	**Planned date**	**Actual Date**
☐	CHMP Rapporteur AR	30 June 2025	30 June 2025
☐	CHMP comments	14 July 2025	14 July 2025
☐	Updated CHMP Rapporteur AR	17 July 2025	n/a
☒	CHMP outcome	24 July 2025	24 July 2025

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1. Introduction

On 11 April 2025, the MAH submitted a completed paediatric study for Rezzayo, in accordance with Article 46 of Regulation (EC) No1901/2006, as amended.

A short critical expert overview was also provided.

2. Scientific discussion

2.1. Information on the development program

The MAH stated that study MR907-1501 is part of a clinical development programme.

Study MR907-1501 was part of the clinical studies required to comply with the EMA's Paediatric Investigation Plan (PIP), as per the Decision of the EMA dated 3 January 2019 (EMEA-002319-PIP01-17). The PIP was subsequently modified on 8 Sep 2021 (EMEA-002319-PIP01-17-M01), 10 Aug 2022 (EMEA-002319-PIP01-17-M02) and 13 Sep 2024 (EMEA-002319-PIP01-17-M03). Enrolment in the study was halted early prior to completion of full enrolment in Part 1, due to significant problems with recruitment.

2.2. Information on the pharmaceutical formulation used in the study

The dose for Group 1 was 3 mg/kg.

2.3. Clinical aspects

2.3.1. Introduction

The MAH submitted a final report(s) for:

- study 2, MR907-1501, "A Phase 1, Multicentre, Open-Label Study to Evaluate the Pharmacokinetics, Safety, and Tolerability of a Single IV Dose of Rezafungin Acetate in Paediatric Subjects from Birth to <18 Years of Age, Receiving Systemic Antifungals as Prophylaxis for Invasive Fungal Infection or to Treat a Suspected or Confirmed Fungal Infection".

2.3.2. Clinical study

MR907-1501, Study 2

Description

Study MR907-1501 was a Phase 1, multicentre, open-label study to evaluate the PK, safety, and tolerability of a single IV dose of rezafungin in paediatric subjects from birth to <18 years of age, receiving concomitant systemic antifungals as prophylaxis (excluding echinocandins) for IFI or to treat a suspected or confirmed fungal infection.

This PK study was part of the clinical studies required to comply with the EMA's Paediatric Investigation Plan (PIP), in accordance with the EMA Decision on the original PIP dated 03 Jan 2019 (EMEA-002319-PIP01-17). The PIP was subsequently modified on 08 Sep 2021 (EMEA-002319-PIP01-17-M01), 10 Aug

2022 (EMEA-002319-PIP01-17-M02), and further revised on 13 Sep 2024 (EMEA-002319-PIP01-17-M03).

Methods

Study participants

Eligible participants were male or female aged birth to < 18 years receiving concomitant systemic antifungals as prophylaxis (excluding echinocandins) for IFI or to treat a suspected or confirmed fungal infection.

Treatments

Subjects received a single IV dose of rezafungin as a 2-hour (±5 minutes) IV infusion.

Objectives

Primary objective

To evaluate the PK of a single IV dose of rezafungin in paediatric subjects from birth to <18 years, receiving concomitant systemic antifungals as prophylaxis for IFI or to treat a suspected or confirmed fungal infection.

Secondary Objective

To assess the safety and tolerability of a single IV dose of rezafungin in paediatric subjects from birth to <18 years, receiving concomitant systemic antifungals as prophylaxis for IFI or to treat a suspected or confirmed fungal infection.

Outcomes/endpoints

Primary Endpoint

Parts 1 and 2: The following PK parameters will be assessed: C_{max}, T_{max}, AUC_{0-t}, $AUC_{0-\infty}$, CL, V_{ss}, V_z, $t_{1/2}$

Part 3: Rezafungin plasma concentrations

Secondary Endpoint

The safety evaluation was based on clinical review of the following parameters:

- Incidence of TEAEs
- Clinical laboratory evaluations (including haematology, blood chemistry and urinalysis)
- Vital signs
- 12-lead ECGs: clinically significant abnormalities
- Physical examination findings

Sample size

The study aimed to enrol 32 subjects, with a maximum of 40 subjects (10 per age group) to achieve 32 evaluable subjects for primary analysis. Subjects were to be divided into 4 groups based on age:

- Group 1 (12 to < 18 years): 8 subjects
- Group 2 (6 to < 12 years): 8 subjects
- Group 3 (2 to < 6 years): 8 subjects
- Group 4 (birth to < 2 years): 8 subjects

Randomisation and blinding (masking)

Not applicable as this is an open-label, single-arm study.

Statistical Methods

In general, continuous data will be summarised by group using descriptive statistics. Categorical data will be summarised by group as the number and percentage of subjects in each category.

Results

Participant flow

Two subjects were enrolled. Both were receiving concomitant systemic antifungals (oral or IV) as prophylaxis for IFI. Both provided informed consent, completed the study and performed all planned study visits. Both were enrolled and included in the analysis.

Recruitment

Study MR907-1501 (Phase 1 PK) was initiated in 10 sites across 3 countries (UK, Spain and Germany) and started recruiting the first cohort of participants (12 to <18-year-olds) on 11 July 2023.

Baseline data

Both subjects were included in Group 1 (12 to <18 years):

During the study, rezafungin was administered under the supervision of the investigator and the exact administration times were recorded in the case report form.

Number analysed

A total of 3 subjects provided informed consent. Of these, 2 subjects were enrolled and included in the analysis.

Pharmacokinetic Results

Single dose PK parameters of rezafungin were determined in 2 subjects.

Efficacy results

There were no efficacy results to report for study MR907-1501.

Discussion on clinical aspects

Preliminary data from the 2 enrolled subjects suggest that rezafungin exhibits a low clearance, a long terminal half-life, and a moderate volume of distribution (V_z and V_{ss}) in subjects aged 12 to < 18 years.

No significant safety concern was identified. The conclusions drawn from this study are limited by the small sample size, which restricts the generalizability of the findings.

3. Overall conclusion and recommendation

The MAH reported the findings from paediatric study MR907-1501 (Study 2 in EMEA-002319-PIP01-17-M02), which was terminated on 14 October 2024. This study aimed to assess the PK, safety, and tolerability of rezafungin in subjects from birth to < 18 years of age receiving concomitant systemic antifungal treatment as a standard-of-care as prophylaxis for IFI or to treat a suspected or confirmed fungal infection. However, enrolment in the study was halted early prior to completion of full enrolment in Part 1 of the study due to significant problems with recruitment. Following this, the MAH, complying with Article 46 of Regulation (EC) No 1901/2006, submitted the results obtained with the two subjects that were included in the study. These data can only be seen as preliminary and no further regulatory action based on them is required.

☒**- PAM Fulfilled:**

No regulatory action required.