Safety and Tolerability of Maralixibat in Infants From 2 Months of Age With Alagille Syndrome or Progressive Familial Intrahepatic Cholestasis: Results From the RISE Study


Introduction
- Alagille syndrome (ALGS) and Progressive Familial Intrahepatic Cholestasis (PFIC) are rare childhood liver diseases associated with high disease burden due to chronic cholestasis and pruritus.

- Maralixibat (AMX103) is a selectively absorbed bile acid transport inhibitor (BATA) that inhibits pruritus caused by abnormal bile acid solubility and elimination.

- The RISE study is a Phase 2/3, randomized, placebo-controlled trial to evaluate the safety and tolerability of maralixibat in infants with ALGS or PFIC aged 2 months to 18 years.

Objective
- To evaluate the safety and tolerability of maralixibat in infants with ALGS or PFIC aged 2 months to 18 years in the open-label, Phase 2/3 RISE (Maralixibat Infant Safety Evaluation) study.

Methods

Results
- The median duration of exposure to maralixibat was 128 days.
- All 14 participants who were randomized to maralixibat completed the study.

Table 1. Baseline Characteristics

Table 2. Overview of TEAEs

Table 3. Incidence of TEAEs Occurring in ≥2 Participants

Conclusions
- Maralixibat was well tolerated and reduced pruritus in infants with ALGS or PFIC aged 2 months to 18 years.
- No serious adverse events occurred.
- Five participants (35.7%) had TEAEs related to maralixibat, most common were diarrhea and abdominal discomfort.

Outcomes
- No serious adverse events were observed.
- No deaths occurred.
- No new or unexpected adverse events occurred in this population.

Plasma PK Analysis
- All plasma drug levels were below the level of quantification for all tested doses in both ALGS and PFIC cohorts.
- These results suggest that maralixibat is safe and well tolerated in infants with ALGS or PFIC aged 2 months to 18 years.

References
- Each table in the text is accompanied by its respective reference.
- The RISE study is a Phase 2/3, randomized, placebo-controlled trial to evaluate the safety and tolerability of maralixibat in infants with ALGS or PFIC aged 2 months to 18 years.
- The study was conducted in the United States, Canada, and Europe.

Disclosures
- No disclosure information is available for this study.

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