Maralixibat Persistency and Adherence for the Treatment of Cholestatic Pruritus in Alagille Syndrome: Real-World Experience in the United States

Robin Hoekstra, MD; Jake M. Temin-Rosenstem, MD; William Stoecker, MD (Presenting)

Mirum Pharmaceuticals, Inc., Irvine, California; MychTI Consulting, LLC, Irvine, California

Introduction

- Alagille syndrome (AGS) is a rare, debilitating, autosomal dominant disorder that presents with a broad range of clinical manifestations.1
- The key clinical manifestations include cholestasis, pruritus, failure to thrive, xanthomas, and progressive liver disease, all of which can lead to liver transplantation or death.1
- Maralixibat (MRLX) is a minimally absorbed bile acid sequestrant (BAS) inhibitor that prevents enterohepatic recycling and is approved for the treatment of cholestatic pruritus in patients with AGS at 3 months of age in the US and 6 months of age in the EU.2,3
- The recommended dose of maralixibat is 380 μg/kg once daily.4

The Mirum Access Plus program is a single-source specialty pharmacy and education program for patients receiving maralixibat that provides insurance coverage assistance, financial support, medication delivery, refill reminders, and educational resources.5

Methods

- Discretized data from the Mirum Access Plus program were analyzed to evaluate trends for 21 year of potential use.
- The analyses included patients in the US who received their first commercial shipment of maralixibat by April 1, 2022.
- Adherence was measured via medication possession ratio (MPR).
- MPR = Number of data points for each patient
- Number of days patient has medication on hand
- Number of days patient should have medication on hand to not have gap in treatment
- Utilization data regarding the Mirum Access Plus program’s disease state and product education programs were also analyzed.
- Maralixibat dosing was analyzed compared with the 380-μg/kg dose recommended in the prescribing information (PI).6

Results

- The study included 161 patients with AGS who received maralixibat through the Mirum Access Plus program.
- The median age of patients was 7 years (40% Male, 9% Unknown).

Figure 1. Mirum Access Plus Program

Patients can opt to enroll in the educational support program

Figure 2. Types of Insurance Coverage (A) and Insurance Approval Rate (B) in the Mirum Access Plus Cohort

- MRLX = Maralixibat

Figure 3. Proportion of Patients Who Titrated Per Day and Who Had Weight-Based Dose Increases

Figure 4. MPR in Overall Cohort (A) and Stratified by Number of Educational Calls (B)

Figure 5. Number of Educational Calls for Patients or Caregivers Who Consented to Using the Mirum Access Plus Educational Nursing Support Program

Figure 6. Percent of Patients or Caregivers Who Consented to Use the Mirum Access Plus Educational Nursing Support Program in Overall and Stratified by Number of Educational Calls

Conclusions

- This is the first study analyzing the real-world use of maralixibat in the US.
- These real-world data demonstrate that most patients with AGS received insurance coverage for maralixibat treatment.
- Adherence to maralixibat treatment was strong, with a markedly higher adherence rate than typically seen for other chronic conditions.
- The Mirum Access Plus educational program had a positive impact on medication adherence, highlighting the importance of disease state and producer-related education.
- These data suggest that maralixibat treatment is well tolerated and easy to adhere to for chronic treatment.

Disclosures

- MRLX, Alagille syndrome, BART, bile acid sequestrant, MPR, medication possession ratio, PI, prescribing information.

References


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