Maralixibat Impact on Concomitant Medication Use for the Treatment of Cholestatic Pruritus in Alagille Syndrome: Real-World Experience in the United States

Robin Howard, MD, Douglas B. Mogul, MD, Julian M. Terner-Rosenthal, MD, Wiktor Stopka, MD, Ida Goldstein

Marlixin Pharmaceuticals, Inc., Foster City, California; Regulis Consulting LLC, San Diego, California

Introduction

- Alagille syndrome (AGS) is a rare, disabling, autosomal dominant disorder that presents with a broad range of clinical manifestations.1
- Key clinical manifestations include cholestasis, pruritus, failure to thrive, xanthomata, and progressive liver disease, all of which can lead to liver transplantation on death.2
- The majority of patients studied in clinical trials of maralixibat for AGS received 3 medications to help alleviate cholestatic pruritus.3
- Maralixibat (MRX) is a minimally absorbed oral bile acid transporter (BAT) inhibitor that prevents enterohepatic bile acid recirculation and is approved for the treatment of cholestatic pruritus in patients with AGS 3 months of age in the US and 2 months of age in the EU.4,5
- Because the ICONIC trial permitted continuation of preexisting antipruritic medications at stable dosages,6 note are available to support how concomitant medication use may evolve after initiation of maralixibat treatment.

Objective

- To assess real-world concomitant medication usage at the initiation of maralixibat treatment and changes in usage over time.

Methods

- Pharmacy data from the Mirun Access Plus program in the US were used to evaluate concomitant medication trends for 31 year of potential use.
- The analysis included patients in the US who received their first commercial shipment of maralixibat by April 1, 2022, and have not discontinued therapy.
- Concomitant medications were confirmed by the Mirun Access Plus pharmacy prior to each maralixibat shipment, which typically occurs monthly.

Results

- The analysis included 116 patients with a median age of 6 years at the start of maralixibat treatment.
- Over half (53.4%) of the patient cohort were male.
- All initiation of maralixibat treatment, patients were taking a median of 2 antipruritic medications and 1 nutritional or vitamin supplement.
- The median time on maralixibat treatment was 480 days.

Figure 3. Proportion of Patients With Different Primary Payers of Maralixibat

- 46% patients were covered by commercial insurance.
- 50% patients were covered by Medicaid.
- 4% patients were covered by other.

Figure 4. Number of Concomitant Medications Discontinued During Maralixibat Treatment

- Approximately 1 in 3 Patients Discontinued 1 Concomitant Medication and 1 in 5 Discontinued 2 Concomitant Medications During Maralixibat Treatment

Figure 5. Impact of Maralixibat on Time to Discontinuation of Concomitant Antipruritic Medications

- Time on concomitant medication until discontinuation

Figure 6. Concomitant Medication Usage at Baseline and During Maralixibat Treatment

- Reductions in Concomitant Medication Usage Were Seen Across All Medication Types

- Consistent with the natural history of AGS, the majority of patients in this analysis were taking multiple antipruritic medications prior to starting treatment with maralixibat.
- More than one-third of patients were able to discontinue at least one antipruritic medication during maralixibat treatment.
- Nearly 1 in 5 patients decreased their use of nutritional/vitamins supplements.
- These real-world data demonstrate the potential of maralixibat to reduce the polypharmacy burden within the first year of treatment.

Conclusions

References


Presented at North American Society for Pediatric Gastroenterology, Hepatology and Nutrition (NAPGHAN) Annual Meeting: October 4-7, 2012; San Diego, California

Abbreviations

AGS, Alagille syndrome; BAI, bile acid sequestrant; BAT, bile acid transporter; MUX, maralixibat; PXR, pregnane X receptor; SPSI, selective photomontage respanse inhibitor

Keywords

To contact us, please call 888-852-8699 or visit www.marlixin.com

updated 6/16/21