Livmarli® (maralixibat) – Expanded indication, new strength

- On July 25, 2024, Mirum Pharmaceuticals announced the FDA approval of Livmarli (maralixibat), for the treatment of cholestatic pruritus in patients 12 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
    - Livmarli was previously approved for this indication in patients 5 years of age and older.
    - Livmarli is not recommended in a subgroup of PFIC type 2 patients with specific ABCB11 variants resulting in non-functional or complete absence of bile salt export pump (BSEP) protein.
- In addition to the expanded indication, the FDA approved a new 19 mg/mL oral solution strength of Livmarli.
    - Livmarli was previously only approved in a 9.5 mg/mL strength.
- Livmarli is also approved for the treatment of cholestatic pruritus in patients 3 months of age and older with Alagille syndrome (ALGS).
- The approval of Livmarli for the expanded indication was supported by evidence in a trial in patients 1 to < 18 years of age that included 26 weeks of placebo-controlled safety and efficacy data.
    - The 19 mg/mL formulation of Livmarli should be used in patients with PFIC in order to minimize exposure to excipients, including propylene glycol. Patients less than 5 years of age are at highest risk for propylene glycol toxicity. The total daily intake of propylene glycol from all sources should be considered for managing the risk of propylene glycol toxicity
- The recommended dosage of Livmarli for PFIC is 570 mcg/kg twice daily 30 minutes before a meal. The starting dose is 285 mcg/kg orally once daily in the morning, and should be increased to 285 mcg/kg twice daily, 428 mcg/kg twice daily, and then to 570 mcg/kg twice daily, as tolerated. The maximum daily dose should not exceed 38 mg (2 mL) per day.
    - Refer to the drug label for complete dosing recommendations for PFIC and ALGS.
- The two strengths of Livmarli, 9.5 mg/mL and 19 mg/mL, should not be substituted for one another when treating PFIC patients. Special attention should be given to the accurate calculation of the dose volume of Livmarli. This is especially important for pediatric patients less than 5 years old as Livmarli oral solution contains the excipient propylene glycol (364.5 mg/mL).
- Mirum Pharmaceuticals' launch plans for Livmarli 19 mg/mL oral solution are pending.