The REPLACE Registry

Status: Recruiting

Eligibility Criteria

Sex: All
Age: Not specified
This study is NOT accepting healthy volunteers

Inclusion Criteria:

1. Male and female patients, of any age. 2. The patient and/or the patient's parent/legal guardian is willing and able to provide signed informed consent, and the patient, if less than 18 years of age, is willing to provide assent as appropriate and in accordance with local regulatory, IRB, and EC requirements. 3. The patient has a diagnosis for which Cholbam is indicated. 4. The patient is or will be treated with Cholbam at the time of signing the informed consent form (ICF) (enrollment).

Exclusion Criteria:

1. Patients who, by judgement of the Investigator, will not be able to comply with the requirements of the protocol will be excluded

Conditions & Interventions

Interventions:
Drug: Cholbam
Conditions:
Bile Acid Synthesis Disorders
Keywords:
Bile Acid Synthesis Disorder, Zellweger Spectrum Disorder, Peroxisomal Disorder, Cholic Acid, Cholbam, The REPLACE Registry

More Information

Description: This is a prospective, observational, non-interventional patient registry designed to document product safety and clinical outcomes for 10 years in patients treated with Cholbam/Kolbam, including those who have been using Cholbam/Kolbam for at least one month (existing users) and those who start Cholbam/Kolbam treatment at enrollment (new users). Patients who have been using Cholbam/Kolbam for less than one month and those who had previously been treated and who restart treatment will also be included but will not be counted in the existing or new users groups.
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