Information for Families with Children Affected by Alport Syndrome

Travere Therapeutics is now enrolling children with rare kidney diseases in the EPPIK clinical study, including Alport syndrome patients ages 2-7.

The EPPIK study will evaluate the study medication, sparsentan, for the treatment of selected rare kidney diseases. The rare kidney diseases included in the EPPIK study are characterized by gradual loss of kidney function and increased protein in the urine (proteinuria). Proteinuria is seen as a marker of kidney function and lowering its level is associated with slowing down the loss of kidney function and better kidney outcomes.

The EPPIK Study is a pediatric study that is evaluating sparsentan, Travere’s investigational approach is to lower proteinuria levels with sparsentan and slow the loss of kidney function in children with:

- Alport syndrome (AS)
- Focal segmental glomerulosclerosis (FSGS)
- Minimal change disease (MCD)
- IgA nephropathy (IgAN), also known as Berger’s disease
- IgA vasculitis (IgAV), also known as Henoch-Schönlein purpura

This study is a European Union regulatory requirement to support approval submissions for IgAN and FSGS. It is not designed to ultimately support an approval in MCD, IgAV or Alport syndrome.

FAQs:

Q: What are the aims/goals/objectives of EPPIK?
A: This study aims to evaluate whether sparsentan helps in the treatment of kidney diseases and if it is safe to use in children.

Q: How is sparsentan administered?
A: Sparsentan is administered as an oral medication, taken in liquid form by mouth.

Q: How many patients will you be enrolled in this study?
A: Approximately 57 children (including all disease groups) will participate in the EPPIK Study.

Q: Where are the clinical sites located?
A: Clinical study sites are located in the US, UK and various countries in the EU.
Q: What range of Alport patients are eligible to participate in the EPPIK Study?
A: Alport syndrome patients with confirmed diagnosis ages 2 – 7 at screening. For the other disease cohorts, visit clinicaltrials.gov. Note: Children who are 18 during screening are not eligible for the EPPIK Study as they are considered adults on Day 1.

Q: What is the eGFR that is eligible for this study?
A: The child should have an eGFR greater than or equal to 30 at screening.

Q: What are the permitted UP/C (urine protein to creatinine ratio – a measure of protein loss in the urine or proteinuria) ranges?
A: For Alport syndrome the UP/C must be greater than or equal to 1.0 g/g.

Q: Are children who are on dialysis or have had a transplant eligible for EPPIK?
A: No, children who are on dialysis or have had a transplant are not eligible for EPPIK.

Q: What is the timeframe for the patient to receive sparsentan?
A: Patients will participate in the EPPIK Study for approximately 2 years and 3 months.

Q: Will telehealth or homecare be available to reduce the amount of office visits?
A: Depending on the reason for homecare or telehealth, both would be available to the patient. The study center will provide this information.

Q: Is reimbursement for transportation and lodging available?
A: Yes, reimbursement for your expenses related to the clinical study such as transportation, lodging and meals is available. Please contact the study center staff for more information.

Q: Where can I find sites near me?
A: To find a site near you, visit clinicaltrials.gov (NCT05003986), clinicaltrialsregister.eu (EudraCT: 2021-000621-27), or contact medinfo@travere.com.

More information about the EPPIK Study and its inclusion of children affected by Alport syndrome is available at: https://www.alportsyndrome.org/for-patients/clinical-trials/current-past-clinical-trials/