

Servier enrolls first US patient in rare paediatric epilepsy study

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The Phase Ib/II trial is evaluating an investigational KCNT1-targeted antisense oligonucleotide in children with developmental and epileptic encephalopathy.



Servier has enrolled the first U.S. patient in a Phase Ib/II first-in-human clinical study evaluating an investigational antisense oligonucleotide for KCNT1-related developmental and epileptic encephalopathy.

The study is designed to assess the safety, tolerability, pharmacokinetic and pharmacodynamic profile of the candidate in children with KCNT1-DEE. Global clinical trial sites are open and enrolling in the United States, Europe and Japan.

This is relevant because KCNT1-related developmental and epileptic encephalopathy is a severe early-onset epilepsy syndrome caused by pathogenic variants in the KCNT1 gene. Affected children can develop seizures within the first days or months of life and often experience profound developmental impairment.

The disease carries high mortality and currently has no curative or disease-modifying treatment. Existing treatment approaches are mainly supportive or symptom-directed, creating a major unmet need for therapies that target the genetic driver of the condition.

Servier's investigational therapy is an antisense oligonucleotide designed to target the underlying genetic mechanism of KCNT1-DEE. The candidate acts by degrading KCNT1 mRNA, with the goal of modifying disease biology rather than only controlling symptoms.

The company said the asset was developed by Servier scientists and has been shown to be well tolerated in preclinical studies. Its entry into first-in-human testing marks an important translational step for Servier's rare neurology strategy.

The programme also reflects the growing role of RNA-targeted approaches in rare neurological disorders. Antisense oligonucleotides are increasingly being used to address diseases where a defined genetic mechanism can be targeted at the RNA level.

For families affected by KCNT1-DEE, clinical development is especially time-sensitive. The disease can progress rapidly, and developmental impact may accumulate early in life. This makes early diagnosis, genetic testing and trial access important components of future adoption if the therapy advances.

The development strengthens Servier's neurology franchise, which focuses on rare neurological disorders associated with genetic mechanisms, including refractory epilepsy, rare movement disorders and neuromuscular diseases.

The broader industry implication is that rare paediatric neurological diseases are becoming more tractable as genetic diagnosis and RNA-targeted modalities mature. If the study generates encouraging early clinical data, it could support a

more targeted treatment pathway for a devastating epilepsy syndrome with few options today.