Drugs

PEER-REVIEWED FEATURE

Trofinetide: Adis Evaluation

Key Points

- An oral, small molecule, synthetic analog of GPE (the N-terminal tripeptide derivative of IGF-1) is being developed by Neuren Pharmaceuticals and Acadia Pharmaceuticals for the treatment of rare childhood neurodevelopmental disorders
- Received its first approval on 10 March 2023 in the USA
- Approved for use in Rett syndrome in adults and pediatric patients 2 years of age and older

Summary

Trofinetide (DAYBUE™), an oral, small molecule, synthetic analog of glycine-proline-glutamate [GPE; the N-terminal tripeptide derivative of insulin like growth factor-1 (IGF-1)], is being developed by Neuren Pharmaceuticals and Acadia Pharmaceuticals for the treatment of rare childhood neurodevelopmental disorders.

Trofinetide was approved in March 2023 in the USA for the treatment of Rett syndrome in adult and pediatric patients 2 years of age and older.

Keam S. J. Drugs. 2023. https://doi.org/10.1007/s40265-023-01883-8