Clinical Drug Investigation

PEER-REVIEWED FEATURE

Olipudase Alfa: Adis Evaluation

Clinical Considerations

- First and currently the only disease-modifying therapy
- Improves lung function, reduces liver and spleen volume, and increases platelet counts; efficacy sustained through at least 2 years of treatment
- Generally well tolerated

Plain Language Summary

Background and rationale

- Sphingomyelin, a fatty substance found in mammalian cell membranes, is broken down by the enzyme acid sphingomyelinase in healthy individuals.
- Acid sphingomyelinase deficiency (ASMD) is a rare inherited genetic disorder, in which the patient's body does not produce enough of the acid sphingomyelinase enzyme, leading to accumulation of sphingomyelin in major organs such as lungs, liver and spleen.
- ASMD types A and A/B (but not type B) also involve brain cells.
- Olipudase alfa (Xenpozyme™) is an enzyme replacement therapy indicated to treat non-CNS manifestations of ASMD in adult and paediatric patients.

Clinical findings

- By reducing sphingomyelin accumulation, olipudase alfa improves lung function, reduces liver and spleen volume, and increases platelet counts, while also correcting other ASMD-related dysfunctions. These benefits are sustained through at least 24 months of treatment.
- Olipudase alfa is generally well tolerated.

Conclusion

Olipudase alfa is the first and currently the only disease-modifying treatment for ASMD.