Collaborations Pharmaceuticals, Inc. demonstrates a treatment for CLN1 Batten disease crosses the blood-brain-barrier

rhPPT1 is a recombinant protein used to treat an ultra-rare disease called Batten disease CLN1 for which there is currently no FDA approved treatment available.

Collaborations Pharmaceuticals logo

RALEIGH, NC, UNITED STATES, January 16, 2026 /EINPresswire.com/ -- CLN1 Batten disease is an ultra-rare disease caused by mutations in the CLN1 gene which codes for the lysosomal enzyme palmitoyl-protein thioesterase-1 (PPT1). Disease progression is marked by intellectual and motor deterioration, seizures, vision loss, and early mortality. This disease affects approximately 20 children in the US and likely several hundred globally. There are no approved treatments for this severe pediatric condition and Collaborations Pharmaceuticals, Inc (CPI) is developing an enzyme replacement therapy).

In a new paper published in Molecular Genetics and Metabolism with collaborators at STC Biologics, Inc., the Veterans Affairs Puget Sound Health Care System as well as the University of Washington School of Medicine, Seattle, CPI describes the large scale development and characterization of recombinant human PPT1 (rhPPT1, CPI-601) suitable for use as a clinical enzyme replacement therapy in CLN1 Batten patients. This enzyme displays similar mannose-6-phosphate receptor (M6PR)-dependent uptake kinetics in neuronal cell lines from human, rat and non-human primate (but not in mouse cells). Importantly rhPPT1 was also identified to cross the blood-brain-barrier (BBB) in adult mice which is unusual for unmodified lysosomal enzymes and is independent of the M6PR and sialic acid receptors. Analytical characterization of rhPPT1 shows complex M6P and sialic acid containing glycans. Our findings suggest for the first time that intravenous dosing of rhPPT1 may be complementary to other dosing strategies in CLN1 patients and could be used to expand its therapeutic applications.

“This work provides valuable insights that addresses several questions from the FDA and could

> These findings suggest intravenous dosing of rhPPT1 may be complementary to other dosing strategies and could be used to expand CPI-601's therapeutic applications.”
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> *Sean Ekins, PhD, DSc, CEO*

broaden the commercial opportunities for CPI-601 in larger disease populations. We have recently completed our IND enabling toxicology study, manufactured the GMP material and are preparing the IND for the CLN1 indication. We are now readying ourselves for a clinical trial and hence funding this is a priority for us” said Dr. Sean Ekins, CEO, Collaborations Pharmaceuticals, Inc. “CPI will also be presenting the preliminary findings from the IND enabling toxicology study at the WORLDsymposium in February as well as describing new research assessing ocular delivery of CPI-601. Our work on CLN1 would not have been possible without the support of small business grant funding from NINDS/NIH” said Dr. Sean Ekins.

About this work

Renuka Raman, Ben Horst, Zahra Shahrokh, Nader Hatambeygi, Maryam Zare, Magdalena Leszczyniecka, Joshua S. Harris, William A. Banks, Kim M. Hansen, Michelle A. Erickson, Sean Ekins, Enzyme replacement therapy for CLN1 Batten disease that crosses the blood-brain-barrier, Molecular Genetics and Metabolism, 2026, 109733, https://doi.org/10.1016/j.ymgme.2026.109733.

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