

Collaborations Pharmaceuticals, Inc. Awarded A Phase II SBIR From NIH/NINDS To Develop Batten Disease Treatment

A Phase II SBIR awarded to Collaborations Pharmaceuticals, Inc. by the National Institutes of Health / National Institute of Neurological Disorders and Stroke.

RALEIGH, NORTH CAROLINA, USA, June 9, 2022 /EINPresswire.com/ -- We are excited to be awarded this phase II SBIR grant for "Manufacture of an intracerebroventricular Enzyme Replacement Therapy for CLN1 [Batten Disease](#)" said [Collaborations Pharmaceuticals, Inc.](#) (CPI) CEO Dr. Sean Ekins.



Collaborations Pharmaceuticals, Inc staff

The neuronal ceroid lipofuscinoses (NCLs) are a group of incurable neurodegenerative storage disorders primarily affecting the brain and the retina of children and young adults, leading to dementia, blindness, epilepsy, and early death. There are currently no treatments available (other than palliative therapies) for this fatal disease.

“

While we realize we have a long way to go, we hope that on International Batten Disease Day our news brings some hope to those families waiting for a treatment for CLN1.”

Dr. Sean Ekins, CEO, CPI

With substantial funding from NINDS with academic collaborators we have been able to demonstrate how monthly intracerebroventricular administration of human PPT1 produced statistically significant treatment effects in enzyme palmitoyl-protein thioesterase-1 (PPT1) deficient mice (Ppt1^{-/-}), such as rescue of PPT1 enzyme activity, decreased secondary enzyme levels, decreased the loss of neurons in all regions of brain and spinal cord and improved gait and rotarod results. We have also been

supported by NINDS to participate in NIH I-Corps which provided valuable experience in

commercialization. In addition, a diversity supplement has also assisted us in hiring personnel and further work on this disease.

“We now propose in this Phase II SBIR to manufacture recombinant human PPT1 and conduct IND enabling toxicology studies. This work marks key steps in the further development of this enzyme replacement therapy (ERT) that is critical for CLN1 patients, setting the stage for future clinical studies and the development of a potential treatment. CPI already holds the Orphan Drug and rare disease designations from the Food and Drug Administration for this approach. This project fulfils our goal to pursue treatments for ultra-rare diseases and this new funding will catalyze the development of this ERT. We are now well positioned to continue the development of this potential treatment for a devastating disease in children. We welcome discussions with other rare disease companies and potential partners to help bring this to the families and children awaiting a treatment”.

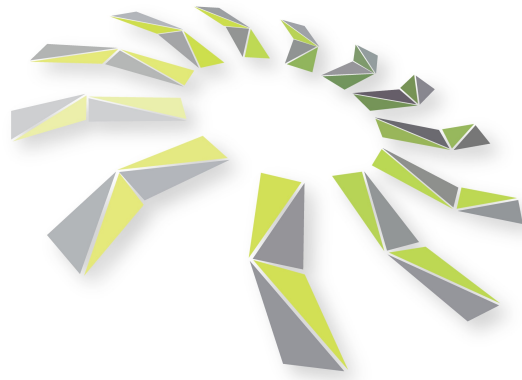
This grant was awarded on the same day as International Batten Awareness Day which aims to honor and support all those affected by Batten disease and to raise global awareness.

About Batten Disease

The infantile onset form CLN1 disease is caused by mutations in the CLN1/PPT1 gene, which codes for the lysosomal enzyme palmitoyl-protein thioesterase-1 (PPT1) resulting in a reduction or absence of enzyme activity. CLN1 disease usually presents between 6 and 24 months of age and there are 2-3 children with this form identified each year and there are over 20 known children with CLN1 in the US and 11 in Brazil (with likely many more undiagnosed globally).

About Collaborations Pharmaceuticals, Inc.

Collaborations Pharmaceuticals, Inc.[®] (CPI) performs research and development on innovative therapeutics and has a preclinical pipeline of treatments for rare and neglected diseases. In addition, CPI has developed software for data curation and machine learning called Assay Central[®] (www.assaycentral.org) as well as curated model bundles in MegaTox[®], MegaTrans[®] and MegaPredict[®]. Most recently we have developed generative software called MegaSyn and UV-adVISor for predicting UV-Vis spectra for small molecules. CPI is located in laboratories in the NC State Incubator at the Centennial campus. We have considerable experience with preclinical and computational approaches to drug discovery and toxicity prediction. For more information,



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Collaborations Pharmaceuticals logo

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