

Collaborations Pharmaceuticals, Inc. and Collaborative Team Progress Development of a Treatment for Batten Disease CLN1

Collaborations Pharmaceuticals is collaborating with STC Biologics, RTI International, Northern Bio, CTI Clinical Trial & Consulting, and Long Scientific, LLC.

RALEIGH, NC, UNITED STATES, February 27, 2025 /EINPresswire.com/ -- 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. The infantile or juvenile forms called CLN1 disease are caused by mutations in the CLN1/PPT1 gene, which codes for a

lysosomal enzyme palmitoyl-protein thioesterase-1 (PPT1) and this affects approximately 20 children in the US and likely several hundred globally. There is currently no treatment for CLN1. Collaborations Pharmaceuticals, Inc (CPI) is developing an enzyme replacement therapy ([ERT](#)) for this disease and has produced a registry for finding patients (<https://cln1registry.collaborationspharma.com>).

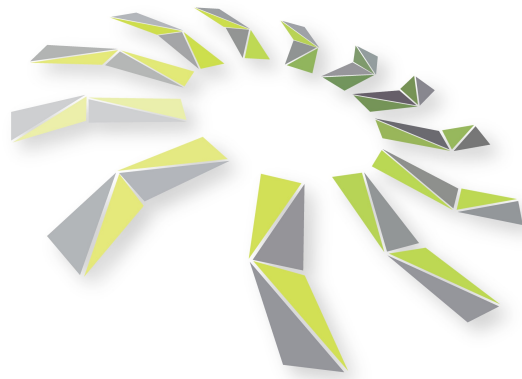


We are currently at the IND enabling toxicology study stage, following that we will file the IND, manufacture the GMP material and ready ourselves for a clinical trial"

Dr. Renuka Raman

"To translate our enzyme replacement therapy for CLN1 we have built up an array of collaborations with various companies, and not-for-profit organizations in the USA to translate this protein to a clinical product. This is critically important because CPI is a small company, and it does not have the internal capabilities, equipment or skillsets to do everything itself. The most cost-effective approach we have

found is to assemble a team of experts to cover all the bases. We are currently at the IND



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Collaborations Pharmaceuticals logo

enabling toxicology study stage, following that we will file the IND, manufacture the GMP material and ready ourselves for a clinical trial, of which Nationwide Children's Hospital would be a site. So far, we have made remarkable progress on a shoe-string budget" said Dr. Renuka Raman, Associate Director, Collaborations Pharmaceuticals, Inc.

"STC Biologics has cloned and purified the recombinant human protein and prepared GLP quality material, next we will develop the GMP material for a future clinical trial. Our company combines decades of scientific expertise working on a wide array of biologics and rare diseases" - Dr. Magdalena Leszczyniecka, CEO, STC Biologics.

"In order to file an IND, we need to ensure that everyone has followed quality procedures from the outset, Long Scientific, LLC has set these up for CPI and provided training to new employees. This is an area which I have a decade of experience in and is important for the FDA filing." - Joshua Long, CEO Long Scientific, LLC.

"Our team of consultants at RTI International has assisted CPI in their preIND in 2023 with the FDA and will assist with the future IND filing. RTI has a team of experts who have spent their whole careers in this space which can be applied to what CPI need in order to answer their questions" - Dr Sita Somara, Regulatory Strategy and Product Development Program, RTI International.

"Northern Bio is performing the IND enabling toxicology studies for CPI to determine the safety of rhPPT1 in a large animal model to provide a go/no go for a future IND filing. Northern Bio has performed such studies for many pharmaceutical clients, and we pride ourselves in our attention to detail to deliver the highest quality data package possible." - Mark Johnson, Chief Scientific Officer, Northern Bio.

"CTI have assisted in drafting a clinical protocol for a future clinical trial for this project. We are experts on rare disease and for 25 years we have been moving medicines forward with our experienced staff who help create a lasting impact on healthcare. Our expert rare disease team can seamlessly support each stage from regulatory planning to marketing approval." - Mr. Corey



Miller, Sr. Director Business Development and Client Management, CTI Clinical Trial & Consulting.

“From the start of this project collaborations with numerous CLN1 families, scientists and experts have built a foundation for where we are today. We realized that to reach the clinic we would also require capabilities we could not expect to obtain in-house based on our company scale and with the funding available to us. Careful evaluation, selection and assembly of all the components of the team was a critical undertaking as part of our mission to bring this treatment to the clinic. We are excited and very thankful for the support of this incredible collaborative team. We believe that “no disease is too small” and bringing this ERT for CLN1 to the clinic is possible and important for these families. We still have a way to go but we have come a long way with the support of the NIH NINDS. By assembling this team, it will give us the best possible chance of success. The biggest challenge we have is not technological, it is purely financial to fund a small clinical trial of 5 children and to do this as quickly as possible. The clock is ticking for these patients, they don’t have time to wait a moment longer.” said Dr. Sean Ekins, CEO Collaborations Pharmaceuticals, Inc.

This work is funded by NIH/NINDS: 2R44NS107079, 1SB1NS135733-01

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 including CLN1 Batten disease.

About STC Biologics, Inc.

STC Biologics, Inc is a boutique turn-key CDMO located in Newton MA, USA that provides full CMC Services and Consulting to enable partners advance their biologic products from discovery to commercialization.

About Long Scientific, LLC.

Long Scientific offers a range of services including Quality System implementation (paper or e-system), SOP authoring, internal/external audits, training, supplier management, document control, change control, and investigation/CAPA.

About RTI International

RTI International is an independent, nonprofit research institute dedicated to improving the human condition. Our vision is to address the world's most critical problems with science-based solutions in pursuit of a better future.

About Northern Bio

Northern Bio is a non-clinical research organization located in West Michigan. Our knowledgeable team is experienced in providing industry-leading research for targeted-delivery of drugs, biologics, gene/cell therapies, and medical devices.

About CTI Clinical Trial & Consulting

Founded in 1999, CTI has grown from a startup into a leading force in clinical research, recognized for its commitment to excellence, innovation, and quality. To date, CTI has played a key role in securing more than 200 regulatory approvals.

Sean Ekins

Collaborations Pharmaceuticals, Inc.

+1 215-687-1320

[email us here](#)

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