Collaborations Pharmaceuticals, Inc. Introduce Batten Disease CLN1 Registry In Order to Find Patients

Collaborations Pharmaceuticals, Inc. has developed a registry for Batten Disease CLN1 In order to find patients with this rare disease and gather information.

Collaborations Pharmaceuticals logo

RALEIGH, NC, USA, April 11, 2024 /EINPresswire.com/ -- “We are proud to announce the release of a registry for Batten Disease CLN1 Registry at https://cln1registry.collaborationspharma.com to help us identify CLN1 families currently living with this ultra-rare disease” said Collaborations Pharmaceuticals, Inc. (CPI) CEO Dr. Sean Ekins.

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.

Collaborations Pharmaceuticals, Inc. is developing an enzyme replacement therapy to replace the enzyme palmitoyl-protein thioesterase-1 (PPT1) that is missing or mutated in neurons in all regions of brain and spinal cord. This treatment has progressed to the preIND stage and is currently awaiting toxicology assessment.

In order to rapidly bring a new treatment to CLN1 patients, we need to find them so that we can contact them in future and share details on natural history and clinical trials. Parents, Guardians or carers can visit the website and once they have provided their informed consent they may choose to provide answers to a small number of disease related questions to provide more details on the patient. Their data is deidentified and will help us understand where CLN1 families are located so this information can help us decide on future clinical sites.

The objectives of the Batten Disease CLN1 registry is to:
1. Identify families with children currently living with CLN1.
2. Obtain basic contact information on the primary caregiver and disease history.
3. Assist in recruitment for future natural history and clinical studies.
4. Analysis of the deidentified questions for future publication.

This Batten Disease CLN1 Registry will not only connect us to patients it will serve to raise awareness for this disease and demonstrate that No Disease is Too Small.

A logo for a CLN1 (Batten disease) registry

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 likely over 20 known children with CLN1 in the US, 11 in Brazil, 10 in the UK (with likely many more undiagnosed globally).

The study protocol has been approved by WCG IRB - IRB Tracking Number: 20241428

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