

Administration of CPI-601

ICD-10 Coordination and Maintenance Committee Fall Update
September 2025

Collaborations

Pharmaceuticals, Inc.

Raleigh, NC, USA



Batten Disease - The Problem we Solve



Amelia, CLN1 patient

- There are over a dozen forms of Batten disease each requiring a separate treatment
- Batten disease CLN1, is characterized by progressive intellectual and motor deterioration, seizures, loss of vision and early death
- Caused by mutations in the *CLN1* gene, which codes for the lysosomal enzyme palmitoyl-protein thioesterase-1 (PPT1, resulting in a reduction or absence of enzyme activity
- Presents between 6 and 24 months of age; there are 2-3 children with this form identified each year
- ~24 known children with CLN1 in the US, 10 in the UK, > 20 in India and possibly hundreds globally (with likely many more undiagnosed)
- There is no treatment available.
- We have the support of CLN1 families

We are Developing an Enzyme Replacement Therapy for CLN1

ERT

Opportunities

Proven track record with FDA

Safety advantages over gene therapy

Stabilized disease outcomes

Improved quality of life

Potential for in utero treatment

Challenges

Delivery to the brain

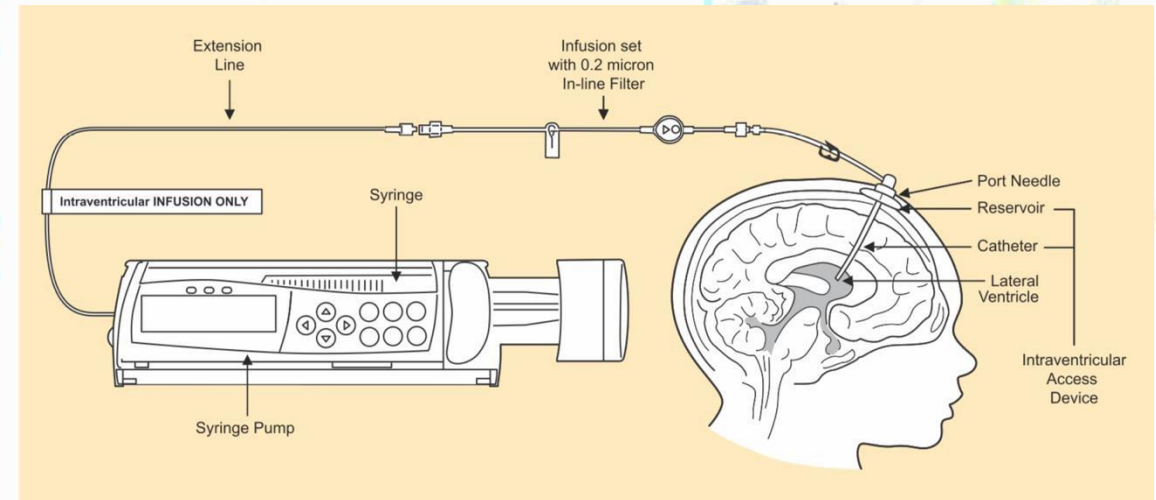
Frequent dosing

Cost of treatment

Hypersensitivity reactions

ERT = recombinant human PPT1 (rhPPT1) aka CPI-601

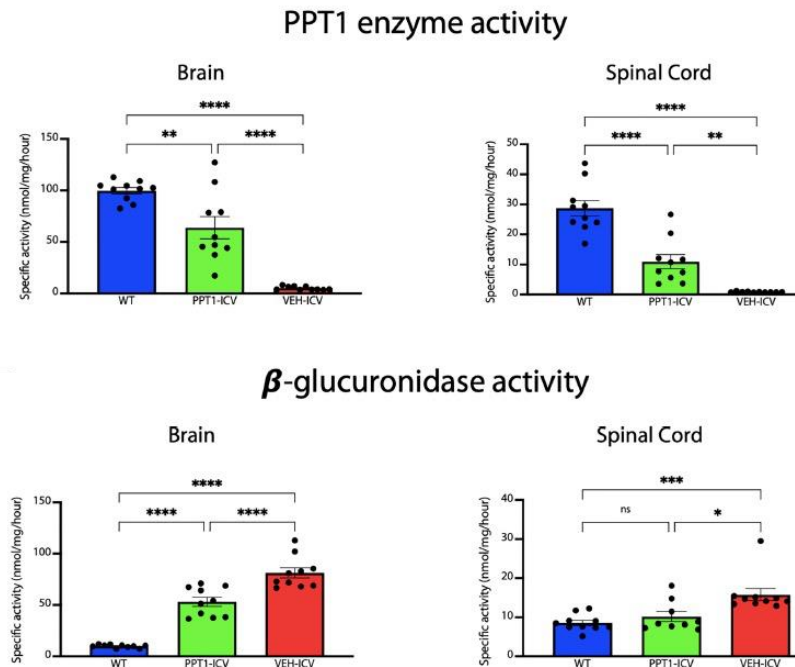
Exactly the same approach as Biomarin used for CLN2



An ERT - rhPPT1 for CLN1 (CPI-601)

We showed in CLN1 ^{-/-} mice that ICV rhPPT1 has statistically significant effects on:

- gait
- motor abnormalities
- neuron loss
- glial activation
- storage material accumulation



Nelvagal et al., J Clin Invest 2022 Oct 17;132(20):e163107

CPI-601: Progress and Collaborators

Process Development/Manufacturing



Sharp



Regulatory



Clinical



NATIONWIDE CHILDREN'S®
When your child needs a hospital, everything matters.

Toxicology



- **Regulatory:** pre-IND meeting held in May 2023
- **Manufacturing:** GLP production complete, have stable formulation, analytical assays developed, GMP next
- **Quality:** Started implementation of Quality Systems
- **Toxicology:** GLP Tox study in NHP – in life part completed - no significant toxicity seen to date
- **Clinical:** Preferred clinical site identified; built rare disease registry software and analyzed data



Patient Registry for CLN1 Launched April 2024

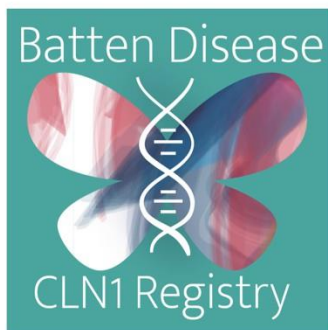
Batten Disease CLN1 Registry

Home

Consent Info

Logout

Registration Form



Welcome to the Batten Disease CLN1 Registry

Welcome to the Batten Disease CLN1 Registry from [Collaborations Pharmaceuticals, Inc.](#) We are a small pharmaceutical company that has is developing an [enzyme replacement therapy for this disease](#). The objectives of 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.

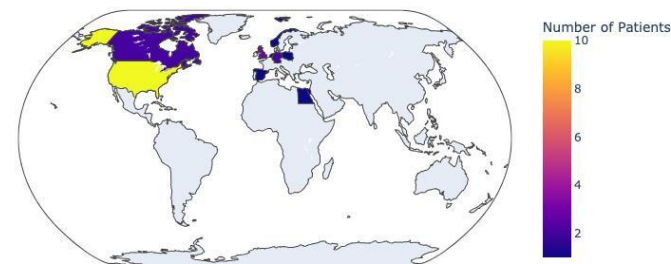
If you are the parent / guardian of a child diagnosed with CLN1, and you would like to opt in to register please register here. Your data will not be shared with anyone else without your permission. You can opt out at any time.

What is a registry?

A rare disease registry provides a means to capture information from patients and their families to help in preparation for a future clinical trial. In general, such registries are used to help connect patient/families with companies and clinicians, enable the understanding of the natural history (how the disease develops), identify outcomes of the disease, while also providing support to help

<https://cln1registry.collaborationspharma.com>

Countries Where CLN1 Patients Reside



| Class | N | Current age (Y) | Age of diagnosis (M) | Age of motor function decline (M)# | Age of language function decline (M)# | Ave number of seizures/ day# | Age of onset of seizures (Y)# |
|-------------------------------------|----|-----------------|----------------------|------------------------------------|---------------------------------------|------------------------------|-------------------------------|
| Infantile (< 18 months) | 6 | 6.66 ± 2.16 | 11.67 ± 7.17**** | 22.83 ± 18.99 | 28.25 ± 32.19 | 6.83 ± 6.31 | 2.60 ± 3.05 |
| Late Infantile (18 months - <4 yrs) | 11 | 5.54 ± 2.73 | 30.09 ± 8.94* | 16.63 ± 5.97* | 17.5 ± 4.90 | 9.27 ± 14.69 | 3.63 ± 4.99 |
| Juvenile (4 yr - < 18 yrs) | 6 | 11.33 ± 4.97 | 104.0 ± 39.77 | 104.0 ± 69.05 | 80.25 ± 74.50 | 0.8 ± 1.10 | 9 ± 3.61 |



CPI-601 Clinical Goals & Proposed Documentation

- *File a patent on formulation and process*
- *Develop USAN name and logo for product, file trademark*
- *Conduct research for additional indications, uses, biomarker development, and natural history study on CLN1*
- *Ongoing patient advocacy*
- *Presentation at the WORLD Symposium 2025; Publications on manufacturing and uptake in cells*

Proposed Terminology and Documentation

ERT = recombinant human PPT1 (rhPPT1)

CPI-601

The administration of CPI-601 is expected to be documented in the medication administration record, physician progress notes, or nurses notes.