



October 5, 2023

Dear CLN2 Batten Community,

We are writing to share a recent update on RGX-181, REGENXBIO's investigational gene therapy for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease.

REGENXBIO announced that initial interim data from a first-in-human, single-patient, investigator-initiated trial of RGX-181 were presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) annual Symposium in Jerusalem, Israel and at the 18th International NCL Congress in Hamburg, Germany. Dr Carolina Fischinger de Souza, the clinical trial investigator from the Hospital de Clinicas de Porto Alegre, Brazil, presented data from the five-year-old child who received a one-time dose of RGX-181 given by intracisternal injection. The data presented were as of June 30, 2023, and represented six months after the administration of the gene therapy and showed the following:

- RGX-181 was well tolerated with no serious adverse events
- Key efficacy measures
 - Sustained levels of TPP1
 - Increased time between enzyme replacement therapy (ERT) infusions
 - 86% reduction in the frequency of seizure through six months, leading to withdrawal of two anti-epileptic medications
 - Improvements in fine motor and expressive language skills

RGX-181 is investigational and is not approved for use by any regulatory agency. RGX-181 utilizes the NAV[®] AAV9 vector to deliver the gene encoding for TPP1, the enzyme deficient in children with CLN2 disease. Following administration of a single intracisternal injection, RGX-181 treatment is designed to provide a durable source of TPP1 potentially leading to long-term correction of cells throughout the central nervous system (CNS).

For more information on our announcement, here is a link to the press release:

[REGENXBIO CLN2 press release 30 August 2023](#)

We are grateful for the community's continued support and remain dedicated to improving the lives of those living with CLN2 disease. We will continue to share updates on our Batten disease programs as they become available.

Please contact the Patient Advocacy team with any questions at CLN2@regenxbio.com.

Warm regards,

CLN2 team at REGENXBIO