Dear Batten disease community,

We are writing to provide an update on Lexeo's CLN2 disease gene therapy program.

As you may know, Lexeo conducted a Phase 1 study testing the investigational gene therapy, AAVrh10CLN2*, using the intraparenchymal (IPC) route of administration (i.e. direct to brain only). Initial results from this study showed that although AAVrh10CLN2 may have some impact on disease progression, the IPC administration may not be the most optimal route to distribute the therapy throughout the central nervous system.

A new study in monkeys testing the intracisternal magna (ICM) route of administration (i.e. into the cerebrospinal fluid) was also conducted, with results demonstrating improved distribution, indicating that ICM could be a potential alternative to IPC administration.

Lexeo met with FDA following the conclusion of these studies. During this meeting, the agency determined that multiple new animal studies would be required before proceeding further with clinical trials in humans, given ongoing discussion around the route of administration for the gene therapy. These new studies would require additional financial investment and resources.

Following the feedback from this meeting, Lexeo is looking to identify strategic partners or alternatives to help take the CLN2 program forward into additional clinical trials.

We are sharing this information with you as part of our commitment to ongoing, open communication with the community and we recognize that this news will have an impact on families in the Batten disease community. We hope to share more information with you if there are new developments with this program.

If you have any further questions, please reach out to: clinicaltrials@lexeotx.com

Sincerely,
The Lexeo Team

*AAVrh10 is a type of gene therapy vector in the AAV family