BDSRA foundation
Batten Disease Support + Research + Advocacy

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Joint Statement to the Global Batten Disease Community

Dear Batten community,

Over the past few years, Lexeo Therapeutics has been involved in developing an AAV-mediated gene therapy program for treating CLN2 Batten disease. However, additional funding and resources are required for further studies, and Lexeo is now looking to identify "strategic partners or alternatives" to enable the ongoing development of the program.

For a brief background on Lexeo's CLN2 program: A Phase 1 clinical study was published by Sondhi and colleagues in 2020, showing that gene therapy via intraparenchymal (IPC, direct to brain tissue) administration slowed but did not stop CLN2 disease progression.

More recently, further preclinical work in nonhuman primates demonstrated an alternate route of delivery directly into the brain cerebrospinal fluid or CSF (via the intracisterna magna, or ICM), was safe, had better distribution, and increased TPP1 enzyme activity in brain and CSF at potentially therapeutic levels (Sondi et al, 2023). In subsequent meetings with the FDA, Lexeo was advised that further preclinical animal studies were required before they could continue with further Phase 1/2 human clinical trials.

Lexeo prepared a statement to share this update with our global Batten disease community. You can read a copy of Lexeo's letter here.

If you have any further questions on this program, we encourage you to reach out to the Lexeo team at clinicaltrials@lexeotx.com, or to your local patient advocacy group for support.

Warm Regards,