November 8, 2023

Dear Batten community,

This afternoon (link: REGENXBIO Press Release 8Nov2023), REGENXBIO announced a corporate restructuring that impacts our development of potential AAV gene therapies for CLN2, a form of Batten disease. We are disappointed to share that REGENXBIO will no longer be moving forward with the development of RGX-181 and RGX-381 for the treatment of late-infantile CLN2 and ocular manifestations of CLN2, respectively. This was a very difficult decision, but due to the challenging economic market facing biotechnology companies, REGENXBIO is no longer in a position to continue these programs on our own.

Ken Mills, President and Chief Executive Officer of REGENXBIO, said, "We believe that gene therapy has the potential to impact the course of Batten disease and we are committed to pursuing alternative paths forward for our Batten programs, including finding a potential partner."

Patients who have been dosed in the clinical trials of RGX-181 and RGX-381 will continue to be followed, but new patients will not be enrolled at this time. Families of patients currently enrolled in these trials will be contacted and receive further information through their study site and can reach out to their study team for more information. To reach the patient advocacy team, please write to cln2@regenxbio.com

It has been a privilege to be part of your community and to have experienced first-hand the amazing work of the Batten organizations and families to advocate for change for patients. Please know that we are committed to doing everything we can for the CLN2 Batten community to identify a partner to advance these investigational therapies to patients.

The CLN2 team at REGENXBIO