May 5, 2021

Dear Members of the Batten Disease Community,

REGENXBIO would like to share some information related to our CLN2 programs. Today, we provided an update on RGX-181 and RGX-381, our two investigational gene therapies for the treatment of CLN2 Batten disease:

RGX-181 for the Treatment of CNS manifestations of CLN2 disease

An Investigational New Drug (IND) application was submitted to the U.S. Food and Drug Administration (FDA), after which the FDA notified REGENXBIO in a letter that its proposed trial had been placed on clinical hold and the agency requested more information to support the initial dose selection and certain study drug administration procedures. REGENXBIO is evaluating the FDA's requests and plans to provide an update on the program in the second half of 2021.

RGX-381 for the Treatment of Ocular Manifestations of CLN2 disease

Based on communication with the FDA and the update from the RGX-181 program, REGENXBIO now expects to provide a program update for RGX-381 in the second half of 2021.

REGENXBIO values partnering with patients, caregivers, healthcare professionals and patient groups to help guide our work, and we will continue to engage the CLN2 community. We are grateful for your partnership and ongoing interest and support of our work.

We look forward to joining many of you at the annual BDSRA family conference this summer. While we wish we could meet in person, we are thankful to the BDSRA for hosting another virtual conference. We hope you will visit our company booth. We would love to connect with you!

Best wishes,

The REGENXBIO CLN2 team

CLN2@regenxbio.com
PatientAdvocacy@regenxbio.com