U.S. FOOD & DRUG ADMINISTRATION

June 29, 2022

Cristina Maria Rosa
(b) (6)

Sent via email to: (b) (6)

Re: Docket No. FDA-2022-P-0032

Dear Ms. Rosa,

I am writing to inform you that the Food and Drug Administration (FDA) has not yet reached resolution of the issues raised in your citizen petition received by the Dockets Management Staff on January 3, 2022. In your petition, you request that FDA:

1. "approve a meeting request to discuss the options of CLN2 Gene Therapy Clinical Trials;"
2. "[r]econsider the option to open clinical trials of Gene Therapy for CLN2-Battens Disease;"
3. "[r]econsider the medical benefits of Gene Therapy;"
4. "[r]econsider the long-term effects of Brineura, and the overruling outcome of the disease progressing;"
5. "[r]econsider the current treatment for CLN2, is not effective to prevent vision loss;" and,
6. "[r]econsider allowing REGENXBIO, to perform further testing on patients with CLN2."

Because of the existence of other FDA priorities, we have not been able to reach a decision on the petition at this time. This interim response is provided in accordance with FDA's regulations on citizen petitions (21 CFR 10.30(e)(2)). We will respond to your petition as soon as we have reached a decision on your request.

Sincerely,

Peter Marks

Peter Marks, MD, PhD
Director
Center for Biologics Evaluation and Research

cc: Dockets Management Staff

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