DEPARTMENT OF HEALTH & HUMAN SERVICES Public Health Service

Food and Drug Administration
Silver Spring MD 20993

January 3, 2022

Cristina Maria Rosa
(b) (6)

Sent via email to: (b) (6)

Dear Petitioner:

Your submission requesting the Commissioner of Food and Drug Administration:

A. Will approve a meeting request to discuss the options of CLN2 Gene Therapy Clinical Trials.

B. Reconsider the option to open clinical trials of Gene Therapy for CLN2- Battens Disease.

C. Reconsider the medical benefits of Gene Therapy.

D. Reconsider the long-term effects of Brineura, and the overruling outcome of the disease progressing.

E. Reconsider the current treatment for CLN2, is not effective to prevent vision loss.

F. Reconsider allowing REGENXBIO, to perform further testing on patients with CLN2.

Your petition was received and processed under CFR 10.30 by this office on 01/03/2022 and was assigned docket number FDA-2022-P-0032. Please refer to this docket number in future correspondence on this subject with the Agency.

Also, note that the acceptance of the petition for filing is a procedural matter and in no way reflects the agency's decision on the substantive merits of the petition.

Sincerely,

Dynna Gorham-Bigby
Digitally signed by Dynna Gorham-Bigby
Date: 2022.01.05 12:33:09 -05'00'

Dynna Bigby
Supervisory Administrative Proceedings Officer
Dockets Management Staff
FDA/Office of Operations (OO)