UNITED STATES DEPARTMENT OF HEALTH AND HUMAN SERVICES

AND THE FOOD AND DRUG ADMINSTRATION

PETITION FOR APPROVAL OF	**:**	
CLINICAL TRIALS FOR	**:**	
GENE THERAPY CLN2	**:**	**Docket No. 2013-S-0610**
BATTENS DISEASE	**:**	

CITIZEN PETITION

The undersigned submits this petition pursuant to 21 CFR § 10.30 and related relevant provisions of the Federal Food, Drug, and Cosmetic Act and Public Health Service Act; the Public Health and Welfare at, inter alia, 42 U.S.C. § 262(a)(2)(A)-(C) and 42 U.S.C. § 262(j); and 42 U.S.C. § 300aa-10 et seq. to request the Commissioner of Food and Drugs (the "Commissioner") approve Clinical trials of Gene Therapy for CLN2- Battens Disease.

A. Action Requested

1. It is hereby requested that the Commissioner:

    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.

B. Statement of Grounds

2. Raising awareness on this rare disease is especially important. To conclude further data in reference to a potentially better treatment. Further research is most definitely required to address any current knowledge, and any evidence gaps that may be presented, when concluding and gathering evidence for the Gene Therapy clinical trial. Investigational research is implemented to accrue new data experiments must be conducted. Under regulations it may be difficult to share information, go for a process of approval with the FDA. Animal models are used to evaluate the virus in the drug. Toxicology studies would be in the best interest to complete clinical trials for gene therapy. Administer these dosing's to the monkeys, and trace for serious side-effects. All viruses are the same, just using different genetic materials, it will not have much significant different. Clinical trials can eb conducted in phases. All safety concerns can be discussed with REGENXBIO, the FDA along with parents as well. To determine if the AAV9 Vector is safe with Brineura patients, trials will have to be conducted to conclude this. Safety data must be conducted in testing. We are aware of the logistical concerns related to all rare diseases and would like to be able to discuss all these topics.

C. Environmental Impact

"Although the positive impacts of this field could be enormous, there are many questions raised that needs to be answered. New organisms created by genetic engineering could present an ecological problem. One cannot predict the changes that a genetically engineered species would make on the environment. The release of a new genetically engineered species would also have the possibility of causing an imbalance in the ecology of a region just exotic species would do. An accident or an unknown result could cause several problems. An accident in engineering the genetics of a virus or bacteria for example could result in a stronger type, which could cause a serious epidemic when released. This could be fatal in human genetic engineering creating problems ranging from minor medical problems"[1] (Patra S, A., 2015). As we are aware of these known factors, the final thesis can not be concluded without proper investigations being conducted.

[1] Patra S, A. (2015). *Human, Social, and Environmental Impacts of Human Genetic Engineering*. Journal of Biomedical Sciences. https://www.jbiomeds.com/biomedical-sciences/human-social-and-environmental-impacts-of-human-genetic-engineering.php?aid=7264

D. Economic Impact

The economic impact that are dealt with CLN2 patients are as follows:

a. The financial burdens delt with infusions, such as increasing insurance premiums.
b. There's constant co-pays and co-insurance as well.
c. Insurance may not cover the current infusions for enzyme replacement therapy, which leaves some kids to get denied treatment. This is most definitely life threatening.
d. Many parents must miss work every other week due to infusions, if our children were offered gene therapy, this would only be a one-time administration.
e. Many families must relocate for the child's infusions, or travel bi-weekly, the costs can most definitely cause a financial burden.
f. Parents spends hours upon hours of working with insurance companies, and lack thereof.
g. Many children have had to miss infusions, due to discrepancies with their insurance companies.

E. Certification

The undersigned certifies, that, to the best knowledge and belief of the undersigned, this petition includes all information and views on which the petition relies, and that it includes representative data and information known to the petitioner which are unfavorable to the petition.

_____ (Signature)

Cristina Maria Rosa _____ (Name of petitioner)

(b) (6) _____ (Mailing address)

(b) (6) _____(Telephone Number)

Enc.

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* Attached to this Citizen Petition are copies of my Petition Campaign.

* Photos of my child, along with other children who are battling CLN2.

* A copy of all the signatures obtained from my campaign on Change.org

* A copy of all the comments obtained from my campaign on Change.org

* A copy of my comment originally submitted for the Citizen Petition.