FDA Pathway for BBDF 101

A Treatment for CLN3 Disease

Pre IND → Juvenile Toxicity → Phase III → Pre Market Studies → FDA New Drug Approval (NDA)

Pre Investigational New Drug (Pre IND) Meeting at the FDA on May 10, 2018

This spring BBDF had its Pre IND meeting with the FDA to get input and direction on our plan for clinical trials. This meeting was a huge milestone and an important first step in the process to develop BBDF's drug combination, BBDF 101. (Pictured above: key opinion leaders and BBDF team at the FDA). The FDA gave very positive feedback and it was clear they want to help us reach our goal. The FDA guidelines provide a clear path forward for our program and do include some action items for us to address which will help set us up for success when we submit BBDF 101 for New Drug Approval. Read on for more detailed information about each step in the process:

Juvenile Toxicity Study

A Juvenile Toxicity Study is required by the FDA prior to testing BBDF 101 in children. Even though there is a great deal of safety data on the two drugs, the FDA would like to see more on the combination to determine safety in a younger population. This study will be conducted in animals and should take approximately 6 weeks to complete.

Phase III *

Because of the volume of safety/toxicity data available on these compounds, the FDA will not require Phase I and Phase II and will allow us to start at Phase III. This enables the program to advance more quickly by immediately administering the drugs at therapeutic levels to measure effectiveness in patients under 18. Additionally, the FDA will not require a placebo to be administered, therefore, every child enrolled will receive BBDF 101.

Pre Market Studies

The FDA will require a safety study in healthy adults prior to New Drug Approval.

A Mouse Factorial Study will be required by the FDA for New Drug Approval to better correlate animal endpoints to humans. Both studies will be run in parallel with Phase III.

FDA New Drug Approval (NDA)

After completing the steps outlined above, BBDF can apply for NDA by the FDA. Once approved this treatment will be made available to the public and should be eligible for coverage by insurance.

* Expanded Access/Compassionate Use

Based on the FDA's feedback, BBDF is pursuing Expanded Access, also known as Compassionate Use, in patients over 18 years of age. This could start prior to Phase III.

ODD and RDPRV

The FDA encouraged the application for Orphan Drug Designation (ODD) and Rare Disease Pediatric Review Voucher (RDPRV). These two special drug designations could make BBDF 101 attractive for pharmaceutical investment.

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