History of BBDF 101

2008

- Beyond Batten Disease Foundation (BBDF) opens its doors and begins looking for a solution to fix what goes wrong in Batten-affected cells. Children with Batten have cellular waste centers (lysosomes) that work, but they work inefficiently. Over time, brain cells fill with toxic waste material and die.

- Transcription Factor –EB (TFEB), a master controller of lysosome production is discovered. If researchers can activate TFEB in Batten-affected cells, maybe each cell could clear itself, staving off its own death and slowing disease.

Finding a solution to the problem

2012

- BBDF grants TFEB discovery team $2.5 million to join the Jan and Dan Duncan Neurological Research Institute (NRI) at Texas Children's Hospital to apply TFEB activation to juvenile Batten (CLN3 disease). Housed on the largest medical center campus in the world, researchers have access to unprecedented resources.

- NRI investigators, together with international collaborators, demonstrate TFEB activated lysosome production is safe and effective at clearing accumulated waste material across multiple healthy and diseased animals throughout their development.

- Three years and an additional $1.8 million in BBDF-funding for labor-intensive searches and 100s of experiments lead to the identification of 6 potential drugs to treat CLN3 disease via TFEB activation.

Identifying potential drugs for treatment

2014

- $2.2 million in BBDF-funding to German pharmaceutical-grade contract research organization, Evotec, leads to the prioritization of Drug #1, the most promising compound with little to no side-effects.

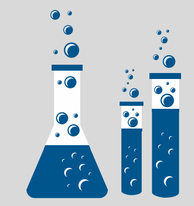
- Evotec and others conduct 125 experiments to determine that the best way for Drug #1 to reach the brain is through intravenous administration.

2016

- Leveraging BBDF’s investments with over $3 million in nonprofit and NIH-funding leads to the addition of Drug #2. Drug #2 enhances the ability of Drug #1 to reach the brain, therefore, creating the combination therapy BBDF 101.

- NRI researchers learn that, independent of Drug #1, Drug #2 clears a subset of waste material and inhibits the harmful effects of chronic inflammation in the brain, resulting in additional unexpected benefits of the drug combination.

- Evotec scientists conduct hundreds of experiments to determine the correct dosage for each drug independently, and in-combination with one another.



100s of experiments for dosing, safety and efficacy

2018

- Camargo Regulatory Strategists join BBDF to bring BBDF-101 to the U.S. Federal Drug Administration (FDA) for permission to enter into a clinical trial.

- After almost 9 years and over $23 million invested in overall research, the teams from BBDF and Camargo, along with key opinion leaders in Batten disease from University of Rochester, University Medical Center Hamburg-Eppendorf, Texas Children's Hospital and BDSRA, meet with the FDA for BBDF 101’s Pre Investigational New Drug (PreIND) meeting. This is the first formal step in clinical trial development.

Preparing for clinical trial success

BEYOND BATTEN DISEASE FOUNDATION