

## Beyond Batten Disease Foundation: June Research Update

---

We are in an exciting and hopeful place in Batten disease research history. Not very long ago, scientists were unsure of the genetic cause of juvenile Batten disease, why cellular processes go awry, and, because of this, were at a loss as to how to fix the problem. Today, we can identify mistakes in DNA that cause the disease. We understand much of what goes wrong and are even able to slow the disease in our animal models! With your support and funding from Beyond Batten Disease Foundation, researchers have identified three potential compounds that may be used to treat children with juvenile Batten disease and are looking for more.

With these discoveries in-hand, we are embarking on an aggressive, well-informed, strategic path to clinical trials. Together with Orphan Solutions,<sup>™</sup> BBDF will design drug development pathways in partnership with the Pharmaceutical Industry, major donors and pediatric, rare, neurodegenerative, and Batten disease foundations. By identifying the hurdles, pitfalls and must-haves to move promising discoveries forward across the [Valley of Death](#), the chasm between basic discovery and therapy development, we will achieve our goals in the least amount of time.

We don't want to get down the road, like so many others have, and discover that we skipped important steps and need to start over. We are in a race against time. To that end, BBDF has contracted with Orphan Solutions<sup>™</sup>, led by Marlene Haffner, MD, MPH and Lisa Beth Ferstenberg, MD, to create an efficient and expedited plan. Dr. Haffner is known best as the "Dean of Orphan Drugs." Marlene administered the United States Federal Drug Administration (US FDA) Office of Orphan Products Development for over 20 years, establishing not only the groundwork in orphan product regulation for the US, but also assisting in the development of Orphan Product programs in the Europe, Japan, Australia and beyond. She is the world's expert on what is necessary to take existing research findings and design a plan to develop approved treatments for rare diseases. Dr. Ferstenberg is a clinical research methodologist who has designed and managed clinical trials in all phases leading to the successful treatments in the US and abroad.

Drs. Haffner and Ferstenberg will bring researchers, clinicians and families together under strategies with the highest potential for success. Possible activities may include combining existing patient registries, supplementation of ongoing investigations, incentivizing investigators and clinicians to work together on group projects, and the identification of clinical measurement tools capable of telling us whether treatments are working; necessary for permission to conduct clinical trials, medical approval, marketing and health insurance coverage. This plan will not only create a pathway for today's discoveries, but lay the groundwork for accelerated development of future discoveries.

None of this would be possible without the support and encouragement of each of you. Thank you for continuing to help us fight Batten disease.