

## July 2021 Newsletter

### A Note from Karen & David

We can't believe that it is already halfway through 2021! Mackenzie and Amelia are now 11 years old and have completed 5th grade. Our summer plans include pool time, bike rides, and relaxing with friends and family. Summer will also include monthly palliative care meetings and navigating the growing dementia symptoms along with reduced mobility both girls are experiencing.

As always, we are so appreciative to our friends and family that have so passionately followed our journey with Batten. We welcome the opportunity to share updates with you through this newsletter.

With Hope,



### Fore the Cure

#### Janssen Cares Grant

We are thrilled to announce that ForeBatten is a recipient of the [Janssen Cares Contributions Fund](#) grant from Janssen Pharmaceutical Companies of Johnson & Johnson. We were nominated by Gabrielle Moriarty with help from her daughter Taylor, a friend of the girls since Kindergarten. Not only did ForeBatten receive valuable funds for research, the award also raises awareness of Batten disease within the pharmaceutical company. The informative and moving video submission was watched by all employees participating in the program. Thank you to Gabi & Tay for choosing to make a difference!

#### Study on Juvenile Batten Disease Published

Karen's sister, Kathryn Koenig Niederer, is a Physician Assistant and has put her professional expertise and love for her nieces to work raising awareness of Batten disease within the medical community. Her article, '[Juvenile Batten Disease: A Diagnostic Conundrum](#)' was recently published in the Journal of the American Academy of Physician Assistants, JAAPA. Kathryn has tracked the progression of the disease in Amelia and Makenzie since diagnosis.



#### New Research Funded!

We are excited to announce sponsorship of a new research study, supplementing our current Sanford Research project, to identify small molecule therapies for the treatment of CLN3-Batten disease. This project with May Khanna Lab at the University of Arizona will map protein interactions and identify druggable sites on CLN3 and its key cellular partners.

### Fore The Journey

#### Beat the Heat/Beat Batten

We were overwhelmed by the enthusiasm at the [Beat the Heat, Beat Batten](#) events held on International Batten Disease Awareness Day. In 11 neighborhoods and parks across the country, friends joined together to enjoy Kona Ice and learn about Batten disease. We gave out over 2,200 cool treats! Our hope is that with more awareness of this disease, the more likely a family will receive an earlier diagnosis.

#### Update: Fore The Journey Fund

Since [Fore The Journey Fund](#) was launched in conjunction with BDSRA in 2020, it has provided over a dozen Batten families items and experiences to brighten their day. The grants are as unique as each family and include a weekend escape to Great Wolf Lodge, an iPad, a trampoline, and even a family photoshoot. Karen and David's vision is coming to life and we hope to provide more similes to Batten families soon!

### Fore The Cure

#### Update: New Research Funded

With the help of our kind donors, we have been able to fund 3 new research projects in addition to what we shared in our last newsletter. Below is an update from our scientific team. Thank you for making continued research possible!

#### *Development of Translatable Biomarkers for CLN3 Disease, Jon Brudvig, PhD, Weimer Lab, Pediatrics and Rare Diseases Group; Sanford Research*

Finding sensitive outcome measures to use in clinical trials for Batten Disease has been challenging. This work will perform deep profiling of blood samples from a CLN3 pig model to identify new markers that be used to track disease progression and responses to experimental therapies.



#### About ForeBatten Foundation

[ForeBatten Foundation](#) is a 501(c)3 with a mission to fund pioneering research and support the Batten community. We work with researchers to develop gene therapies to detect, treat and potentially cure Batten disease. Rare diseases like Batten do not receive the same government assistance as other, more prevalent diseases, so we must join together as parents and caring individuals who are not willing to accept the fate of our children.

#### About Batten Disease

Juvenile Batten disease (CLN3) is a rare, fatal, inherited disorder of the nervous system that typically begins in childhood. The first noticeable sign of juvenile Batten disease is often loss of vision, which begins between the ages of 5 and 10 years in previously healthy children and tends to worsen rapidly. Eventually, young adults become blind, bedridden, and physically and mentally incapacitated, requiring 24-hour care until premature death.