A Note from Karen and David,

In some ways, our 2020 is likely similar to what everyone is experiencing; a complete upheaval in everyday life. But as time marches on, so does this disease. Our days are now spent engrossed in dementia-filled conversations about fairies and birds and why Jane Goodall can't live with us. Behavior outbursts are all too common, sometimes lasting over 10 hours a day. The nights are filled with fear and anxiety as the disease doesn't allow the brain to rest, creating perpetual sleepless nights.

As parents, we try to keep our daughters safe from harm, constantly playing the role of 'bumpers' as they attempt to blindly navigate the house. We pick them up when their bodies fail or their brains seize. They are, unfortunately, still aware enough to realize what they are missing - all that Batten is robbing from them - which makes coping extremely difficult. This new "norm" is difficult, not only for our daughters but for our entire extended family. But we march on, finding the happy moments and enjoying the smiles.

This foundation is our way of trying to make a difference - not only to Amelia and Makenzie but to all others impacted by this awful disease. Our mission is to provide funding for critical research while compassionately supporting the batten community. We cannot fight this fight alone. Amelia, Makenzie, and all other Batten children need the support of empathetic people in this world. Without that support, hope is evaporated. Hope is what we need. Hope is all we have.

Thank you from the bottom of our hearts for following along as we fight for a treatment and a cure. You provide our hope!

Winners Announced: Butterfly Art Contest

The support for our first annual Butterfly Art Contest was overwhelming! We had over 80 creative submissions ranging from paintings to beadwork, crafts to sketches. We even had a butterfly made from Cheerios! Our judges had the impossible task of picking just 5 winners and while it was difficult to narrow it down, we are happy to announce the following winners and honorable mentions.

Update: Take a Swing Fore Batten IV

Look for an announcement soon about our 2021 Take A Swing ForeBatten event. As with everything nowadays, it may look different than in the past. Stay tuned!

Fore The Journey

The Fore The Journey Fund, a joint collaboration with the Batten Disease Support and Research Association (BDSRA), is aimed at providing grants to Batten families. This fund will further ForeBatten Foundation's mission to support the Batten community and enhance day to day life, bringing much-needed smiles to all. More details can be found at https://www.bdsra.org/fore-the-journey-fund/

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.

Supplement to Identifying Potential Molecular Therapies for the Treatment of CLN3-Batten Disease, Jill Weimer, PhD, *Senior Director of Therapeutic Development Scientist, Pediatrics and Rare Disease Group, Sanford Research.* This project will expand on current drug screening in the Weimer lab to include a new Kinematic Gait Analysis system. This system tracks fine movement patterns and is incredibly sensitive for detecting benefits from drug treatments.

Antisense Oligonucleotides for the Treatment of CLN3 Batten disease, Michelle L. Hastings, PhD, *Director, Center for Genetic Diseases; Professor, Chicago Medical School; Vice-Chair, Cell Biology and Anatomy; Rosalind Franklin University of Medicine and Science.* A major roadblock in the study of CLN3 Batten disease and the development of therapeutics is the lack of a method to detect the protein encoded by the CLN3 gene and mutated in CLN3 Batten disease. If the protein cannot be detected, it is difficult to determine whether a therapeutic is having the desired effect on the disease target, a critical measurement of drug efficacy. To address this hurdle, we are creating a mouse that has a genetic sequence inserted into the mouse CLN3 gene. This sequence tags the gene's protein product such that it can be easily detected and measured. This mouse will not only aid drug development but will also allow researchers to study the protein and its function and activity in its native environment.

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.