

THE CHARLOTTE & GWENYTH GRAY FOUNDATION TO CURE BATTEN DISEASE ANNOUNCES NEW PARTNERSHIP

New Partnership with Children's Hospital of Orange County (CHOC) where Non-Profit Relaunches CLN6 Gene Therapy Program

LOS ANGELES, CA, UNITED STATES, May 19, 2025 /EINPresswire.com/ -- The Charlotte & Gwenyth Gray Foundation to Cure Batten Disease is celebrating its ten-year milestone by joining forces with Children's Hospital of Orange

County (CHOC), now part of Rady Children's Health, to revitalize and enhance the CLN6 gene therapy program. This partnership builds on the findings from a Phase 1/2 clinical trial conducted from 2016-2020 at Nationwide Children's Hospital in Columbus, Ohio, reaffirming the commitment to progress in treating CLN6 Batten disease, an agonizing condition with no existing approved treatments.



"CLN6 Batten disease is a devastating disorder with no approved therapies, and families deserve hope," said Dr. Raymond Wang, Director of the Campbell Foundation of Caring Lysosomal Program at CHOC. "This reinvigorated program brings new scientific insights and renewed optimism for developing a meaningful treatment."

Since its inception in 2016, [the Charlotte and Gwenyth Gray Foundation](#) has been instrumental in furthering research for Batten disease CLN6. A pioneering Phase 1/2 clinical trial assessed a gene therapy designed to rectify the genetic defect causing the disease. This therapy, administered as a one-time injection into the spinal canal, showed encouraging results by preserving motor and language abilities in multiple children. The initial success captured the interest of Amicus Therapeutics, which pursued the treatment's development. However, in 2022, Amicus halted the program after long-term data indicated that initial benefits were not sustained beyond two years. Despite this disappointment, the trial was a vital advancement in understanding the potential of gene therapy for neurodegenerative disorders.

This new collaboration signifies a critical advancement toward developing effective treatments

for CLN6 Batten disease, a rare and severe condition leaving children cognitively impaired, blind and immobile and ultimately gone between the ages of 6 and 10 years old . By uniting the expertise of researchers, clinicians, and families, the initiative aims to enhance rare disease therapeutics and instill hope in affected children. The relaunch of this program is a pivotal move toward refining and enhancing gene therapy for CLN6 Batten disease.

"The opportunity to re-launch this gene therapy program is a testament to the unwavering commitment of the Gray Foundation, our research team at Sanford, and our clinical partners at CHOC," said Dr. Jill Weimer of Sanford Research. " We are more determined than ever to advance a treatment for children battling CLN6 Batten disease."

This partnership highlights the remarkable synergy between scientific research and advocacy. By uniting researchers, clinicians, and families, the initiative is expanding the horizons of what can be achieved in rare disease therapeutics. The combined expertise and commitment of these contributors will fuel progress and foster hope for a brighter tomorrow.

Even after the profound loss of their daughter Charlotte in 2024, the Gray family remains passionately committed to combating Batten disease. Through the Charlotte & Gwenyth Gray Foundation, they persist in advocating for research progress, supporting affected families, and striving for a cure. Their mission honors Charlotte's legacy and aims to prevent other families from experiencing the same heartache. By sharing their narrative and raising awareness, they are transforming their sorrow into action, nurturing hope for those still facing this devastating disease.

"Our objective is to create a more effective gene therapy strategy that will lead to a cure and ensure treatment accessibility for all individuals diagnosed with Batten CLN6," expressed Kristen Gray, Founder/Director of The Gray Academy and the Charlotte & Gwenyth Gray Foundation to Cure Batten Disease.

The return of this program to clinical settings signifies more than just a scientific achievement—it is a beacon of hope for families who have been clinging to optimism. With renewed vigor and determination, the team is eager to advance the program, united by the common goal of making a meaningful impact on the lives of those affected by CLN6 Batten disease.

The study is supported by The Charlotte and Gwenyth Gray Foundation to Cure Batten Disease (www.curebatten.org (<http://www.curebatten.org>)), which was established in June 2015 by film producer Gordon Gray and his wife Kristen after their two young daughters were diagnosed with this life-threatening childhood illness.

ABOUT THE CHARLOTTE AND GWENYTH GRAY FOUNDATION

Since 2016, the Charlotte & Gwenyth Gray Foundation has been at the forefront of the fight against CLN6 Batten disease. One of its most significant milestones was supporting a groundbreaking Phase 1/2 clinical trial aimed at correcting the genetic mutation responsible for

the disease. The therapy, a one-time injection into the spinal canal, initially showed promise in slowing or stabilizing disease progression.

This success attracted the attention of Amicus Therapeutics, a biotech company specializing in rare diseases, which took over the program for further development. By 2019, data suggested that the therapy helped preserve motor and language function in several treated children, with noticeable benefits lasting up to two years. In total, 15 children received the treatment. However, in 2022, Amicus discontinued the program after long-term data showed the improvements did not hold beyond two years.

While the program did not lead to a long-term cure, it was a crucial step in understanding how gene therapy can treat neurodegenerative diseases. The Gray Foundation's efforts paved the way for future research and reinforced the importance of continued investment in Batten disease treatments.

Beyond research, the foundation also supports children with neurological disorders through The Gray Academy, a non-profit, non-public school in Los Angeles, California. The school provides individualized instruction and integrates traditional therapeutic interventions. In 2023, the Gray Academy OC opened in Laguna Niguel, expanding this mission to families in Orange County.

For more information and to support this cause, please visit www.curebatten.org.

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