

January 30, 2024

Dear Batten community,

We are excited to announce that The Abigail Wexner Research Institute at Nationwide Children's Hospital (NCH) has received the Investigational New Drug (IND) for the Batten clinical trial programs. NCH has been a global leader in researching and treating devastating neurological disorders like SMA and DMD. We remain committed to moving the CLN3 and CLN6 programs forward.

We are planning to submit a new protocol to our Institutional Review Board (IRB) updating them with these changes. We will also advise them of our plans to continue the post transfer safety monitoring as required by the Food and Drug Administration (FDA). We look forward to exploring partnerships and funding opportunities to support any future clinical trials.

I would like to personally thank the families who have participated in our clinical trials. Their efforts and hard work have allowed us to move the needle forward for our children and it is a privilege to work for our families. We hope that this communication reduces any concerns you may have. I am happy to personally discuss these changes with you. Please call Clinical Research Services at 614-722-2650 or the Neurology Division at 614-722-4625.

With best regards,

Emily de los Reyes, MD

Primary investigator of Batten disease programs

Clinical Professor of Pediatrics and Neurology

Nationwide Children's Hospital