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Batten Disease Support + Research + Advocacy

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Joint Statement to the Global Batten Disease Community

On Thursday, February 15, 2024, important news broke regarding CLN1 clinical research that global Batten disease patient advocacy groups wish to share with the Batten community. A patient with CLN1 disease was treated with the investigational gene therapy candidate TSHA-118 under an individual-patient investigator-initiated Investigational New Drug (IND) at RUSH University Medical Center in Chicago, Illinois.

In a recent announcement, Taysha Gene Therapies stated that it provided investigational clinical trial material for TSHA-118 to support the individual-patient investigator-initiated IND request from RUSH University Medical Center. As disclosed in early 2022, Taysha is not currently evaluating TSHA-118 for CLN1 disease. The Company continues to evaluate potential partnerships and opportunities to further the CLN1 program development.

Dr. Elizabeth Berry-Kravis, the principal investigator and sponsor of the investigator-initiated IND at RUSH University Medical Center, has been leading clinical trials for more than 20 years with extensive experience in fragile X syndrome, Rett syndrome, Angelman syndrome, Niemann-Pick type C, Batten disease, other rare diseases, gene therapy, and investigator-initiated trials.

"As a physician who treats patients with this devastating disease, I am pleased to have helped enable the dosing of an individual patient with CLN1 disease with investigational gene therapy candidate TSHA-118. I remain committed to helping advance care for patients and families impacted by CLN1 disease," said Principal Investigator Elizabeth Berry-Kravis, M.D., Ph.D., Professor of Pediatrics, Neurology, and Anatomy/Cell Biology at RUSH University Medical Center.

The gene therapy research was funded in part by Taylor's Tale, a non-profit organization founded by Taylor King's family and friends to fund research for CLN1 Batten disease. It was transferred several times until Taysha acquired it in August 2020.

"More than a decade ago, Taylor's Tale and our dedicated volunteers and donors dared to believe in a novel gene therapy approach for CLN1 disease, catalyzing the study that led to today's exciting announcement," Taylor's mother Sharon King, President of Taylor's Tale, and Taylor's sister Laura King Edwards, Vice President of Taylor's Tale, said in a joint statement. "While the treatment came too late to save Taylor's life, it stands as her lasting legacy. We're thrilled for this patient and their family, and we'll continue to advocate so that others may benefit in the future."

Resources & Definitions:

- What is an individual-patient investigator-initiated trial?
    - While many clinical trials are initiated by industry sponsors, such as pharmaceutical or biotech companies, investigator-initiated trials are clinical trials in which the investigator initiates the research, develops the protocol, and serves as a sponsor.
- Who is the sponsor of the individual-patient investigator-initiated trial?
    - RUSH University Medical Center is the sponsor of the individual-patient investigator-initiated trial.
- For more information about investigational gene therapies, visit https://patienteducation.asgct.org/.