Medical Xpress

OHSU Doernbecher Children's Hospital conducts second phase of landmark Batten study

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Oregon Health & Science University Doernbecher Children's Hospital will lead the next phase of a landmark clinical trial to further assess the safety and preliminary effectiveness of purified human neural stem cells (HuCNS-SC) as a potential treatment for infantile or late-infantile neuronal ceroid lipofuscinosis (NCL), a rare and currently fatal neurodegenerative disorder that affects infants and children.

The Phase Ib trial, sponsored by StemCells, Inc., is designed to further assess the safety and preliminary effectiveness of StemCells' HuCNS-SC as a potential treatment for NCL, also referred to as Batten disease. This second trial is expected to enroll six children whose NCL is less advanced than those enrolled in the initial Phase I trial, also carried out at OHSU Doernbecher, and, in addition to safety, is also designed to evaluate the impact of HuCNS-SC on disease progression.

"The hard work of testing neural stem cells for their safety and effectiveness in clinical use began here at OHSU Doernbecher Children's Hospital. We are very pleased to carry on this important effort with a second major trial. Our great hope is that this work will eventually yield significant benefits for patients who suffer from devastating nervous system diseases," said Nathan Selden, M.D., Ph.D., FACS, FAAP, Campagna Professor of Pediatric Neurosurgery and head of the Division of Pediatric Neurological Surgery at OHSU Doernbecher Children's Hospital, OHSU School of Medicine.

Selden was co-principal investigator on the groundbreaking initial Phase I trial and will lead the Ib study.

The initial trial was the first-ever FDA-authorized clinical trial of purified human neural stem cells as a potential therapeutic agent. Begun in March 2006, its aim was to test the safety and preliminary efficacy of transplanting HuCNS-SC in six children with advanced stages of NCL. Once transplanted, the children were followed for 12 months. Data from the Phase I trial, completed in January 2009, demonstrated that high doses of HuCNS-SC transplanted directly into multiple sites within the brain followed by 12 months of immunosuppression were well tolerated. The data also included evidence of engraftment and long-term survival of the donor cells.

Participants in the new study will be transplanted with HuCNS-SC via a neurosurgical procedure and their immune systems will be suppressed for nine months. Following transplantation, the children will be monitored and regularly evaluated for 12 months to assess the safety and tolerability of the HuCNS-SC, the surgery and the immunosuppression. In addition, participants will be evaluated and assessed at regular intervals using a comprehensive range of clinical and X-ray tests, both before transplantation to establish a baseline, and over the course of 12 months following transplantation.

StemCells, Inc. will initiate a separate four-year observational study at the conclusion of this trial.

Provided by Oregon Health & Science University