

About NCL

Neuronal ceroid lipofuscinosis (NCL) — also known as Batten Disease — is a neurodegenerative lysosomal storage disorder in children, caused by inheritance of a recessive genetic mutation. The defective gene results in the deficiency of an important “housekeeping” enzyme that processes cellular waste substances. Without this enzyme, the cellular waste accumulates in the lysosome of the central nervous system (CNS) cells, causing the neurons to cease functioning and eventually die.



The infantile and late-infantile forms of NCL are estimated occur in 2 to 4 out of every 100,000 live births in the U.S. It is more frequently found in northern Europe, Canada and Newfoundland.

Infants born with NCL initially appear healthy. Onset of symptoms typically begins later in infancy or early childhood. Those afflicted with NCL usually suffer from seizures and blindness and all endure progressive loss of motor skills and diminishing mental capacity. Children ultimately become bedridden and unable to communicate or function independently. There is currently no treatment for the relentless degenerative effects of the disease. While available drugs may reduce seizures and physical therapy may temporarily facilitate mobility, NCL remains a fatal disease.

The Bigger Picture

NCL is just one of more than 50 lysosomal storage disorders (LSDs). LSDs affect approximately one in every 5,000 individuals worldwide; a child is born with a lysosomal disorder approximately every half hour. While there is currently no known cure, certain LSDs can be treated with enzyme replacement therapies. However, such an approach is not a practical treatment option for the more than 20 CNS-mediated LSDs, as enzymes are too large to cross the blood-brain barrier. For these LSDs, neural stem cell transplantation directly into the CNS may hold promise as a future therapy.

Milestones

StemCells, Inc. has shown in preclinical studies that its patented highly purified human neural stem cells survive, migrate throughout the brain, produce the enzyme necessary to reduce cellular waste buildup and protect the host neurons.

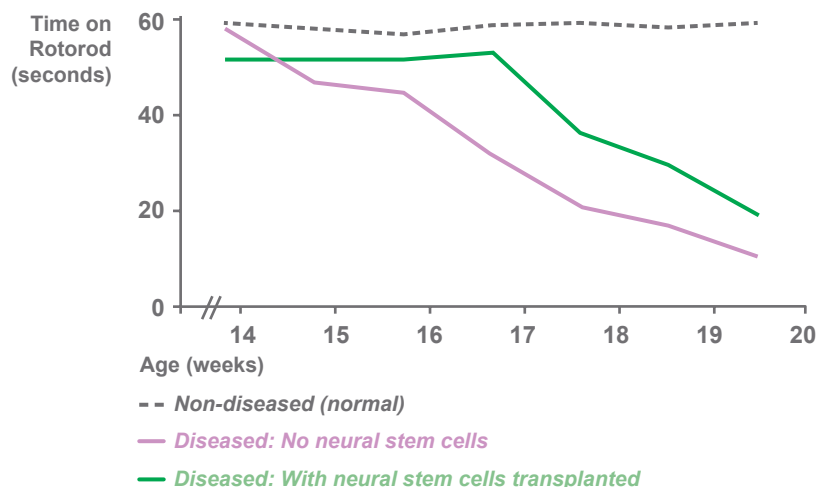
- ▶ **October 2005:** Received clearance from the U.S. Food and Drug Administration (FDA) to initiate a Phase I clinical trial to evaluate the safety and preliminary efficacy of our HuCNS-SC[®] product candidate as a treatment for NCL
- ▶ **November 2006:** Announced the first transplantation of HuCNS-SC neural stem cells in an NCL patient
- ▶ **June 2009:** Reported positive safety results from the Phase I trial
- ▶ **October 2010:** Initiated a Phase Ib trial in NCL targeting enrollment of patients in earlier stages of the disease
- ▶ **April 2011:** Discontinued Phase Ib trial due to a lack of eligible patients identified within a reasonable timeframe
- ▶ **June 2011:** Evidence of long-term survival of HuCNS-SC cells following completion of immunosuppression regimen presented at ISSCR 9th Annual Meeting

Preclinical Proof of Concept

Published data from preclinical studies in NCL highlights the novel neuroprotective approach that StemCells is pursuing to treat neurodegenerative diseases, and supports the Company's clinical development of its HuCNS-SC product candidate.

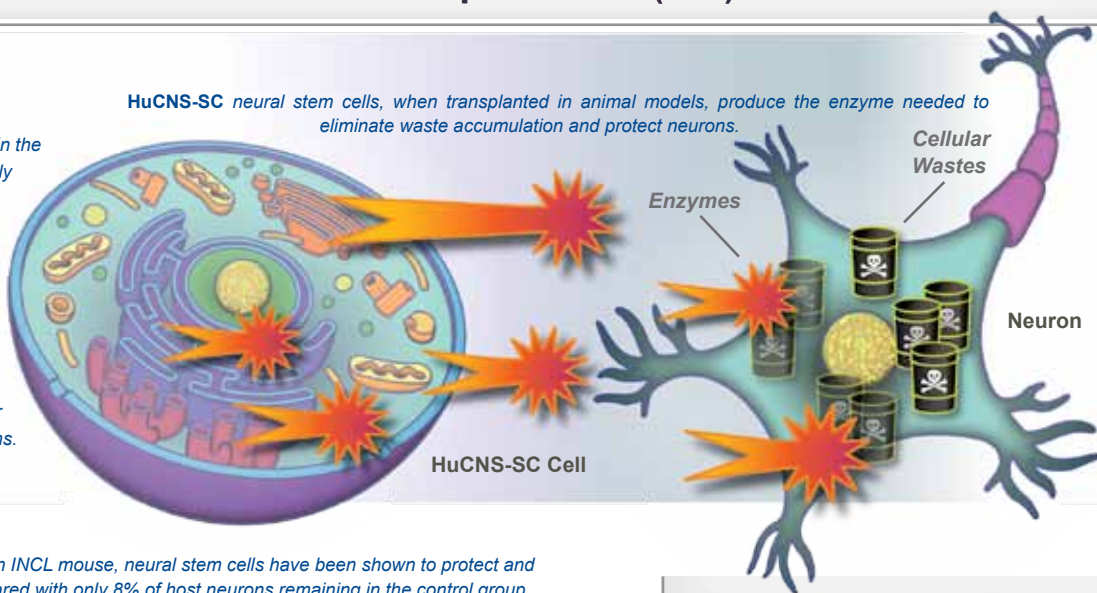
StemCells has shown that when transplanted in a mouse model of infantile NCL (INCL), its neural stem cells engraft, migrate throughout the brain and continuously secrete the missing lysosomal enzyme characteristic of NCL, which is needed to process cellular waste and keep neurons functioning and healthy. When compared with a control (non-transplanted) group, the mice that received the transplanted neural stem cells showed a statistically significant reduction in cellular waste build-up, protection of critical host neurons and delayed loss of motor function. Preclinical studies have demonstrated that these neural stem cells also produce the enzyme missing in late infantile NCL (LINCL), thereby providing the scientific rationale for enzyme replacement via transplantation of these cells in this subtype, as well as in INCL.

Transplanted neural stem cells delay loss of motor coordination in INCL mice.

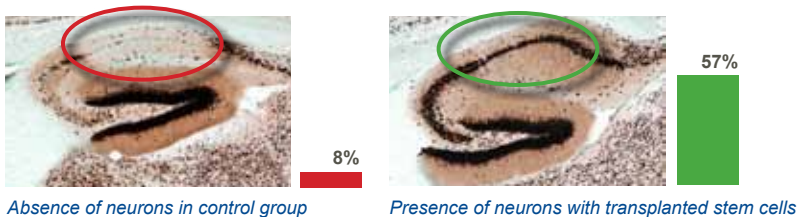


Batten Disease / Neuronal Ceroid Lipofuscinosis (NCL)

Lysosomes are small organelles in the neurons of the brain that normally process wastes. In NCL, lack of a "housekeeping" enzyme results in cellular waste buildup, which is harmful to neurons. HuCNS-SC neural stem cells, when transplanted into the brain, migrate to areas where the neurons are affected and produce the deficient enzyme for uptake by the patient's neurons.



When transplanted into the brain of an INCL mouse, neural stem cells have been shown to protect and maintain 57% of host neurons, compared with only 8% of host neurons remaining in the control group.



About HuCNS-SC

(Human Neural Stem Cells)

Preclinical research has shown that our purified human neural stem cells can be directly transplanted into the central nervous system (CNS), after which they engraft, migrate and differentiate into neurons, astrocytes and oligodendrocytes, surviving long-term with no sign of tumor formation or adverse effects. This suggests the possibility of a durable clinical benefit following a single transplantation. In 2009, data from our first clinical trial demonstrated the safety and tolerability of our HuCNS-SC product candidate and the transplantation process. Additional data reported in 2011 provides evidence that HuCNS-SC cells persist long after immunosuppression is discontinued. We are currently developing our HuCNS-SC product candidate for the treatment of several indications including:

- Spinal cord injury (Ph. I/II trial underway)
- PMD (Ph. I trial underway)
- Retinal disorders (Ph. I trial targeted for 2012)
- Alzheimer's disease and stroke (preclinical)

Processed in compliance with cGMP standards, our HuCNS-SC cells can be expanded, cryopreserved and then stored in banks for future use as "stem cells in a bottle."

About StemCells, Inc.

Driven by nearly 20 years of pioneering research and innovation, StemCells, Inc. is applying its scientific and industry leadership in stem cell biology to discover, develop and commercialize novel therapeutics and enabling tools and technologies for use in stem cell-based research and drug discovery.

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Clinical Development in NCL

Phase I Trial – Completed January 2009

The Company's Phase I trial in NCL, initiated in 2006, was the first ever FDA-authorized clinical trial of human neural stem cells. This landmark study, which was conducted at OHSU Doernbecher Children's Hospital (OHSU) was primarily focused on assessing the safety of HuCNS-SC cells as a potential treatment for NCL. Data from this first trial, reported in June 2009, and from an ongoing follow-on observational study initiated following completion of the trial, demonstrated the clinical safety and tolerability of HuCNS-SC cells and the transplantation procedure. Additional data gathered in 2011 has shown that transplanted HuCNS-SC cells can persist even after immunosuppression has been discontinued.

Phase Ib Trial – Initiated October 2010 / Discontinued April 2011

Based on favorable safety data from the Company's first NCL trial, StemCells initiated in October 2010 a second clinical trial at OHSU to further assess the safety and preliminary efficacy of its HuCNS-SC product candidate as a potential treatment for NCL. The Phase Ib trial was designed to enroll patients with less advanced stages of the disease than those enrolled in the first NCL trial, with the goal of enhancing the prospects for clinical benefit.

In April 2011, StemCells discontinued the Phase Ib study due to a lack of eligible identified within a reasonable timeframe, and made the decision to shelve its NCL program.