HuCNS-SC® Therapeutic Development Program

(Human Neural Stem Cells)

About PMD

PMD (Pelizaeus-Merzbacher Disease) is a degenerative disorder caused by a mutation in the gene controlling the production of proteolipid protein (PLP), which is integral to the formation of myelin. Myelin is the substance that surrounds nerve fibers (axons) and provides the insulation necessary for proper transmission of electrical signals. Without myelin, nerve impulses are disrupted, resulting in deteriorating coordination, motor control and intellectual function.

The gene for producing PLP is found on the X chromosome. So males (who have just one X chromosome) are more likely to inherit the condition than females, who may inherit a normal X chromosome to offset the mutation in the other.

PMD symptoms typically appear in early childhood. Individuals with the milder form may have nearly normal life spans, but suffer from a decline in neurological function. The more severe form, connatal PMD, usually becomes apparent in the first few months of life. Early symptoms are often nystagmus (jerky side-to-side eye movement) and hypotonia (floppy muscle tone). Seizures and spasticity may develop as neurological function deteriorates. Severe neurological impairment, resulting in abnormal mental and physical development, is followed by premature death.

While symptoms may be mediated by medication for movement disorders, there is currently no standard course of treatment, nor is there a cure.

The Bigger Picture

The dysmyelinating neurodegenerative disorders known as leukodystrophies, of which PMD is just one, are relatively rare. Demyelinating disorders, in which once-healthy myelin is damaged or destroyed, are far more common, with multiple sclerosis and spinal cord injury each estimated to affect over 2 million people worldwide. Transverse myelitis and a certain type of cerebral palsy are other disorders in which disturbances of myelination play a key role. The potential of a cell-based therapy to treat myelin deficiencies represents hope for patients with these debilitating or fatal conditions.

Milestones

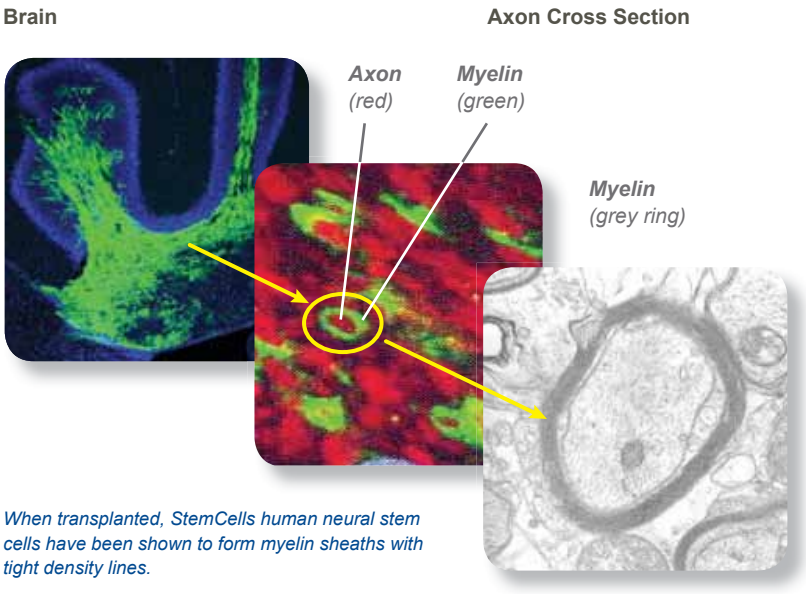
StemCells, Inc. is conducting a Phase I Clinical Trial in PMD — the first ever neural stem cell trial in a myelination disorder.

- **August 2005:** Initial preclinical myelination data published in the *Proceedings of the National Academy of Science* (Cummings, et al. 2005).
- **June 2008:** Additional preclinical myelination studies presented at the International Society of Stem Cell Research (ISSCR) *Annual Meeting*.
- **December 2008:** Clearance received from the U.S. Food and Drug Administration (FDA) for an Investigational New Drug application (IND) to conduct a clinical trial of the StemCells HuCNS-SC product candidate in patients with PMD.
- **November 2009:** StemCells initiates Phase I Clinical Trial at UCSF Children's Hospital to evaluate safety and preliminary efficacy of HuCNS-SC human neural stem cells as a treatment for PMD.
- **February 2010:** Enrolled and treated first patient in Phase I Clinical Trial.

Preclinical Proof of Concept

Preclinical studies performed by StemCells and its collaborators provide a rationale for potential therapeutic use of the Company's HuCNS-SC product candidate in myelination disorders. StemCells has demonstrated that, when transplanted into an animal model of hypomyelination (shiverer mouse), its neural stem cells engraft and differentiate into mature oligodendrocytes and form myelin sheaths around host nerve fibers. StemCells is using this same approach to treat spinal cord injury, which is often associated with neuron loss and demyelination. Preclinical studies have shown that when transplanted into the spinal cord of injured mice, StemCells neural stem cells form myelin around the damaged nerve axons and restore lost motor function.

Neural stem cells restore myelin in animal model of hypomyelineation (shiverer mouse).



When transplanted, StemCells human neural stem cells have been shown to form myelin sheaths with tight density lines.

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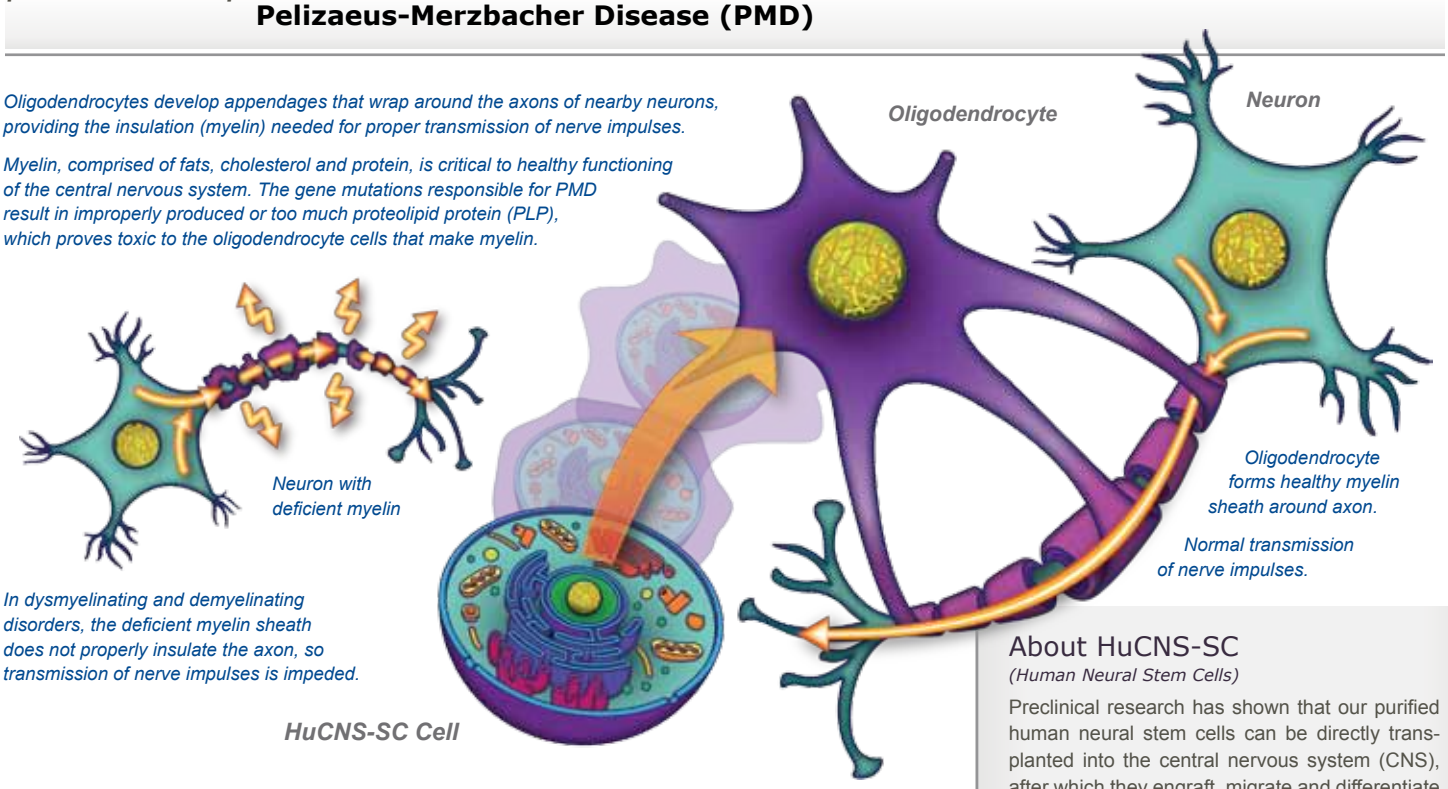
Pelizaeus-Merzbacher Disease (PMD)

Oligodendrocytes develop appendages that wrap around the axons of nearby neurons, providing the insulation (myelin) needed for proper transmission of nerve impulses.

Myelin, comprised of fats, cholesterol and protein, is critical to healthy functioning of the central nervous system. The gene mutations responsible for PMD result in improperly produced or too much proteolipid protein (PLP), which proves toxic to the oligodendrocyte cells that make myelin.



In dysmyelinating and demyelinating disorders, the deficient myelin sheath does not properly insulate the axon, so transmission of nerve impulses is impeded.

The StemCells Approach: Myelin Production to Protect Nerve Cells

When StemCells neural stem cells are transplanted, they migrate to the sites where myelin is deficient. They differentiate into oligodendrocytes and form healthy myelin sheaths to protect axons, helping nerve cells communicate with each other.

Phase I Trial in PMD

In November 2009, StemCells, Inc. initiated a Phase I Clinical Trial of its HuCNS-SC human neural stem cells in PMD at the University of California, San Francisco (UCSF) Children's Hospital, one of the leading medical centers in the United States for neonatology, pediatric neurology and neurosurgery. In February 2010, the HuCNS-SC product candidate was used to treat the first patient enrolled in the trial, marking the first time that human neural stem cells have been transplanted as a potential treatment for a myelination disorder. This is the second clinical trial of StemCells HuCNS-SC human neural stem cells. The first study, a Phase I Clinical Trial in NCL (neuronal ceroid lipofuscinosis), often referred to as Batten disease, was completed in January 2009. Data from the NCL trial demonstrated a favorable safety profile, along with evidence of engraftment and long-term survival of the HuCNS-SC cells.

This Phase I trial is designed to assess the safety and preliminary efficacy of the HuCNS-SC product candidate as a potential treatment for PMD, and is expected to enroll four patients with connatal PMD, the most severe form of the disease. While the primary focus in this first trial is safety, StemCells will also be looking for evidence of new myelin formation in the patients' brains following transplantation, as well as any signs of improved neurological function. All patients will be transplanted with HuCNS-SC cells, and will be immunosuppressed for nine months. Following transplantation, the patients will be evaluated regularly over a 12-month period in order to monitor and evaluate the safety and tolerability of the HuCNS-SC product candidate, the surgery and the immunosuppression. In addition, MRI examination of the brain post-transplant may enable the measurement of new myelin formation. StemCells plans to follow the effects of this therapy long-term so, as with its Phase I NCL trial, this trial will also be followed by a separate, four-year observational study.

About HuCNS-SC

(Human Neural Stem Cells)

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- NCL (Phase 1 Clinical Trial completed)
- PMD (Phase 1 Clinical Trial underway)
- Retinal disorders
- Spinal cord injury

Processed to exacting cGMP standards, our HuCNS-SC cells can be expanded, cryopreserved and banked for future use as "stem cells in a bottle."

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Batten Disease / Neuronal Ceroid Lipofuscinosis (NCL)

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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.

A rare disease, NCL is estimated to 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.

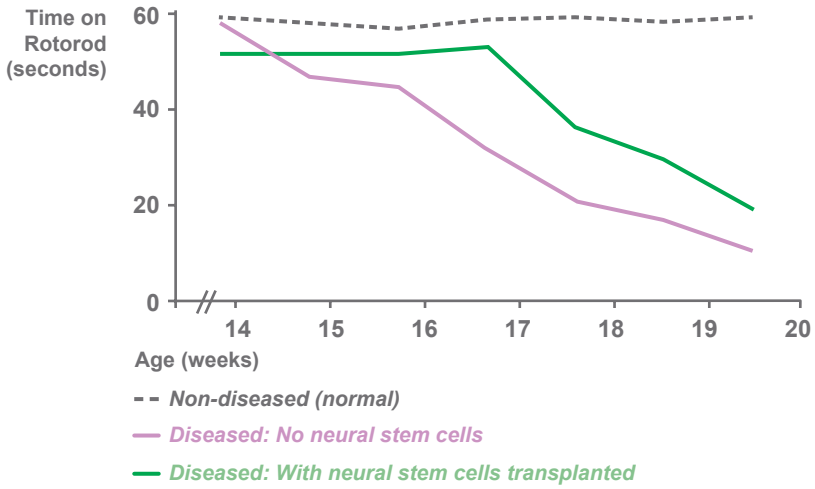
- **October 2005:** The Company received clearance from the U.S. Food and Drug Administration (FDA) to initiate a Phase I Clinical Trial to evaluate safety and preliminary efficacy of its HuCNS-SC product candidate as a treatment for NCL.
- **November 2006:** StemCells announced the first transplantation of HuCNS-SC neural stem cells in an NCL patient.
- **June 2009:** Positive safety results reported from the Phase I Trial.
- **May 2010:** Secured FDA authorization to proceed to a second NCL trial.

Preclinical Proof of Concept

Published data from preclinical studies highlights the novel neuro-protective 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 the control (non-transplanted) group, the mice that received the transplanted neural stem cells showed 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.



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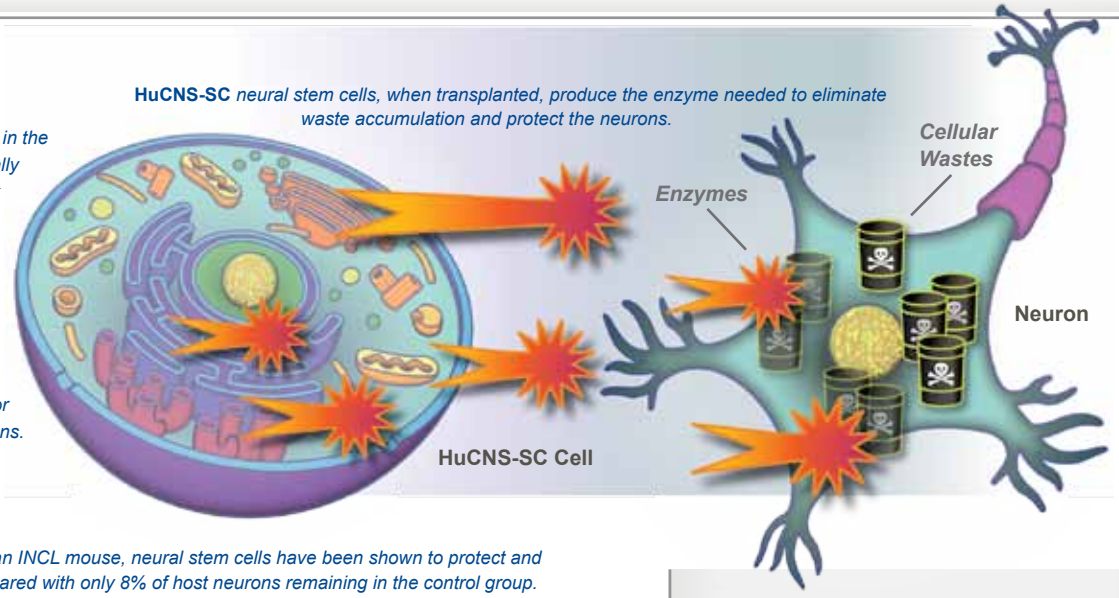
Batten Disease / Neuronal Ceroid Lipofuscinosis (NCL)

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.

HuCNS-SC *neural stem cells, when transplanted, produce the enzyme needed to eliminate waste accumulation and protect the 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.

Absence of neurons in control group

Presence of neurons with transplanted stem cells

Phase I Trial in NCL

StemCells HuCNS-SC product candidate was recently tested in the first-ever FDA-authorized clinical trial of human neural stem cells. The landmark Phase I study assessed the safety and preliminary efficacy of HuCNS-SC neural stem cells as a treatment for NCL, a fatal neurodegenerative disorder primarily afflicting children.

In this open-label, dose-escalating Phase I study, the HuCNS-SC product candidate was transplanted into six patients with either infantile or late infantile NCL. Enrollment in the trial was limited to patients in advanced stages of the disease with significant neurological and cognitive impairment (patients whose developmental age was demonstrated to be less than two-thirds of their chronological age). Two dose levels were administered, with the first three patients receiving a target dose of approximately 500 million cells; the other three patients receiving a target dose of approximately one billion cells. The HuCNS-SC cells were directly transplanted into each patient's brain via a neurosurgical procedure. Patients were immunosuppressed for 12 months following transplantation. The patients were evaluated and assessed at regular intervals using a comprehensive range of medical, neurological and neuropsychological tests, both before transplantation to establish a baseline, and over the course of 12 months following transplantation. Following completion of the Phase I trial, the patients were automatically enrolled in a separate four-year follow-up study.

In June 2009, the Company reported positive results from this trial including:

- **Favorable safety profile: cell transplantation and immunosuppression well tolerated**
- **Evidence of engraftment and long-term survival of the donor cells**

StemCells is now preparing for a second clinical trial in NCL, targeted for initiation in Fall 2010. This second trial will place an increased emphasis on the measurement of clinical benefit in patients with less neuronal degeneration and brain atrophy.

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About AMD

Currently afflicting 25-30 million people world-wide, AMD (age-related macular degeneration) results in a progressive and irreversible loss of vision. AMD is the number one cause of legal blindness for those over age 55, and the leading cause of vision loss in developed countries.

The eye contains photoreceptor cells known as rods and cones. These photoreceptors convert light into electrical impulses that are sent via the optic nerve into the brain, which then interprets what we see. Rods allow us to see under low light conditions, while cones, which require brighter light, distinguish fine detail and color. Cones are highly concentrated within the macula, a small area at the center of the retina. Because the macula is predominantly made up of cones, this area of the eye facilitates the sharp, straight-ahead vision required for such tasks as reading, driving and recognizing faces.

Patients with AMD progressively lose their clear, central vision when the cones within the macula degenerate. As of today, there is no cure for AMD.

The Bigger Picture

As the "baby boom" generation ages, the incidence of AMD is expected to increase dramatically, tripling by 2025. Photoreceptor protection through neural stem cell transplantation may be viable as a future therapy for AMD. This approach may also hold promise for treating other retinal degenerative diseases such as retinitis pigmentosa (RP), the most common inherited cause of blindness, affecting an estimated 1.5 million people worldwide and rendering many legally blind by the age of 40.

Milestones

StemCells, Inc. is conducting preclinical studies of its patented highly purified human neural stem cells for the treatment of retinal degenerative diseases such as AMD.

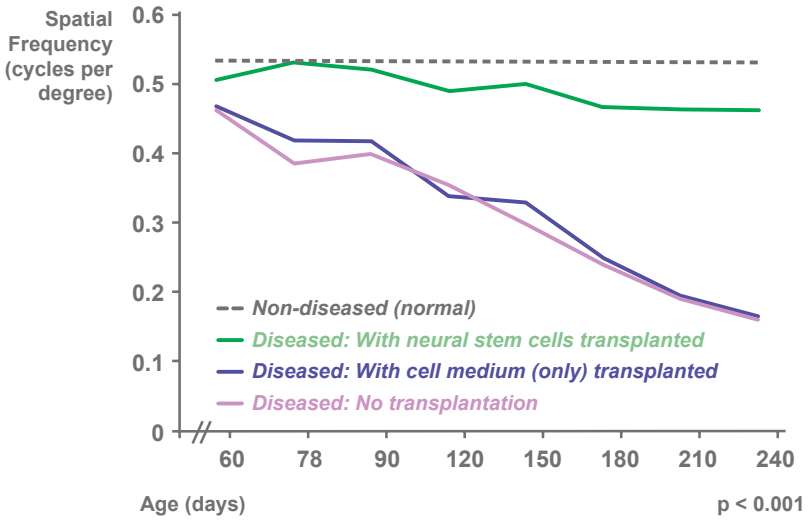
- **January 2008:** StemCells enters into research collaboration with the Casey Eye Institute at Oregon Health & Science University (OHSU) to evaluate its neural stem cells as a potential treatment for leading causes of vision loss and blindness.
- **May 2009:** Data showing ability to protect retina from progressive degeneration presented at Association for Research in Vision (ARVO) *Annual Meeting*.
- **October 2009:** Data showing photoreceptor protection and ability to preserve visual function long term presented at Society for Neuroscience *Annual Meeting*.

Preclinical Results

Preclinical data demonstrates the therapeutic potential of the StemCells HuCNS-SC product candidate to treat retinal degenerative diseases such as AMD.

Studies performed by StemCells and the Casey Eye Institute show that, when transplanted into the sub-retinal space of the RCS (Royal College of Surgeons) rat, a well-established animal model of retinal degeneration, the Company's human neural stem cells protect the retina from progressive degeneration and preserve visual function long term as measured by two separate visual tests. The transplanted cells also exhibit robust, long-term protection of both rod and cone photoreceptors. The ability to protect cones, in particular, is significant in regard to AMD, since it is the progressive deterioration of these specific cells that ultimately results in the devastating vision loss caused by this disease. The protection of both rods and cones is important in considering the potential of using human neural stem cells as a treatment for retinitis pigmentosa and other retinal degenerative disorders.

StemCells human neural stem cells preserve visual acuity in RCS rats as shown by optokinetic tests measuring visual function over time.



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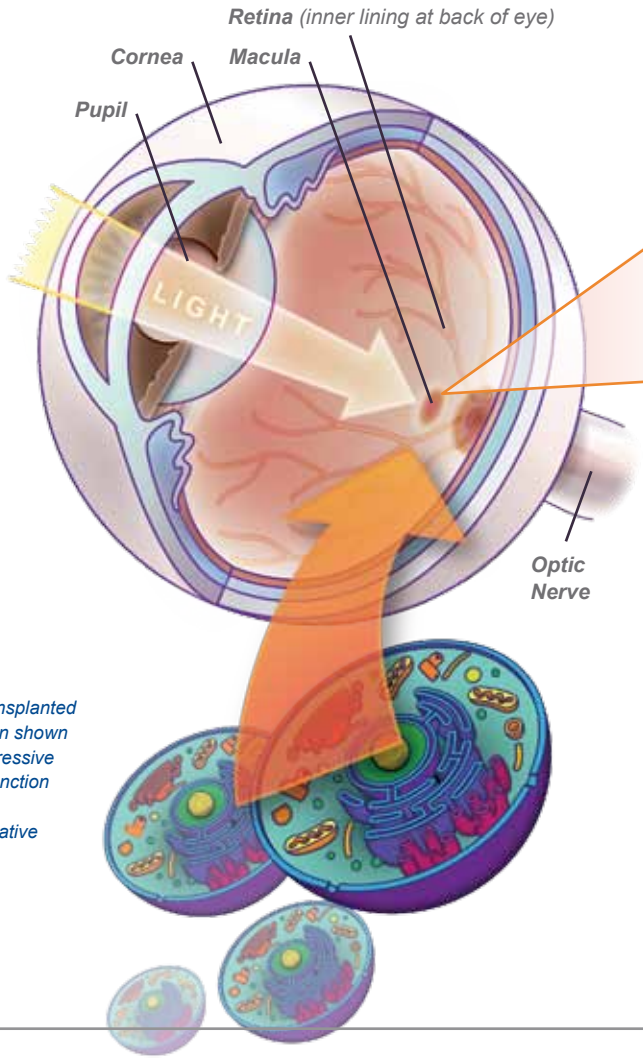
Age-related Macular Degeneration (AMD)

The Human Eye

The retina is the light-sensitive inner lining at the back of the eye. The retina contains millions of photo-receptors – light sensing nerve cells called rods and cones. Rods are extremely sensitive to light and dark changes, general shapes and move-ment, while cones are re-sponsible for color vision and acuity.

The macula is a small region within the retina where the greatest number of cones are located. At its center is the fovea, containing only cones. Because of this high concentration of cones, the macula is critical to our ability to see color and detail.



HuCNS-SC Cells

Human neural stem cells, when transplanted into the sub-retinal space, have been shown to protect photoreceptors from progressive degeneration and preserve visual function long-term, suggesting a promising approach to treating retinal degenerative disorders such as AMD.

AMD degenerates rods and cones

In AMD, central vision is lost when the cone cells within the macula deteriorate and eventually die.

When human neural stem cells are transplanted, photoreceptor cells – and vision – are preserved.

Stem cells protect rods and cones

Pathway to the Clinic

StemCells, Inc. hopes to build upon the promising results of its research through the initiation of clinical trials for patients with retinal degenerative diseases. The Company has already engaged the FDA in discussions regarding a pathway to clinical testing of its human neural stem cells for retinal indications and additional preclinical studies are underway in pursuit of that goal.

StemCells is developing HuCNS-SC human neural stem cells as a potential therapeutic product to treat several disorders of the central nervous system (CNS). These cells are currently in clinical development for two fatal neurodegenerative diseases in children. The human safety data that StemCells is accumulating for its HuCNS-SC product candidate through these clinical trials is expected to facilitate the pathway for future clinical testing in other CNS disorders including retinal degenerative diseases such as AMD and retinitis pigmentosa.

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