Age-related Macular Degeneration (AMD)

hNSC Therapeutic Development Program
(Human Neural Stem Cells)

About AMD
Currently afflicting 25-30 million people worldwide, 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
Preclinical and early clinical studies of purified human neural stem cells for the treatment of retinal degenerative diseases such as dry AMD have been completed by the StemCells, Inc. BOCO ReGen Med now owns the technology and will continue clinical development of hNSC for AMD.

- **February 2012:** Published data showing the retinal protective effects of transplanted human neural stem cells into the RCS model of retinal degeneration. Studies support hNSC transplants as a potential treatment for leading causes of vision loss and blindness
- **June 2015:** Completed a Phase I/II clinical study in geographic atrophy of AMD demonstrating safety and preliminary efficacy with a trend toward slowing the progression of geographic atrophy

Preclinical Results
Preclinical data demonstrates the therapeutic potential of the human neural stem cell, hNSC product candidate to treat retinal degenerative diseases such as AMD.

Our published studies (McGill 2012 EJN) 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, 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 other retinal degenerative disorders.

Human neural stem cells (hNSC) preserve visual acuity in RCS rats as shown by optokinetic tests measuring visual function over time.
The Human Eye

The retina is the light-sensitive inner lining at the back of the eye. The retina contains millions of photoreceptors – light sensing nerve cells called rods and cones. Rods are extremely sensitive to light and dark changes, general shapes and movement, while cones are responsible 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.

hNSC 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. Multiple mechanisms of action of the hNSC appear to contribute to retinal protection.

Pathway to the Clinic

BOCO ReGen Med hopes to build upon the promising results of the research and early clinical development performed by StemCells, Inc. through the re-initiation of a Phase II clinical trial for patients with dry AMD.

In the future, BOCO ReGen Med hopes to develop hNSC human neural stem cells as a potential therapeutic product to treat other disorders of the central nervous system (CNS). These cells were previously tested in early clinical studies for the treatment of spinal cord injury and two fatal neurodegenerative diseases in children. The human safety data that accumulated for the hNSC 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.

References:


About hNSC (Human Neural Stem Cells)

Purified human neural stem cells are culture expanded as neurospheres in a defined serum-free culture formulation to create cell banks that are cryopreserved for later use in clinical applications. Preclinical research has shown that our purified and expanded 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. Because the transplanted cells engraft and survive long-term, this suggests the possibility of a durable clinical benefit following a single transplantation. Data from four early clinical studies of the hNSC product candidate demonstrated a favorable safety profile, along with evidence of engraftment and long-term survival of the transplanted cells. The hNSC product was tested for the treatment of several indications including:

- Spinal cord injury (Ph. I/II trial completed)
- PMD (Ph I trial completed)
- Retinal disorders (Ph I trial completed)
- Alzheimer’s disease & Stroke (preclinical)

About BOCO ReGen Med.

BOCO ReGen Med was formed in 2016 upon the acquisition of the human neural stem cell technology from StemCells, Inc. About 20 years of pioneering research and innovation that identified and characterized this unique stem cell and the promising early clinical data compels BOCO ReGen Med to pursue the clinical development of the hNSC cells first in retinal degenerative disorders followed other neurodegenerative disorders.

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