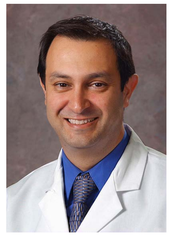
Bone Marrow Stem Cell Therapy for Vision Loss

UCDAVIS EYE CENTER

Susanna S. Park, MD, Ph.D.
Professor of Ophthalmology



Mehrdad Abedi, MD
Associate Professor Hematology / Oncology

Gerhard Bauer
Director, UC Davis GMP Laboratory Stem Cell Isolation

Jan Noltra, Ph.D.
Director, UC Davis Stem Cell Program

SUMMARY STATEMENT

An 8-patient clinical trial investigating the safety and efficacy of autologous stem cell transplant to treat vision loss from the hereditary retinal degenerative condition, retinitis pigmentosa (RP)

DISEASE/CONDITION

RP is a hereditary form of retinal degeneration that affects the entire retina. It is a complicated disease, with over 100 associated genes. RP severely affects the quality of life of affected individuals and leads to disability.

RP is progressive, disrupting night and peripheral vision and leading to severe vision loss. Eventual blindness can occur in in both eyes, happening as early as young adulthood. Most individuals with RP are legally blind by age 60. There is a great unmet need to develop a treatment that can limit or reverse vision loss associated with RP.

CURRENT TREATMENT

Gene therapy has been approved only for a rare and severe form of RP. However, the multiplicity of genes associated with RP poses challenges in developing effective gene therapy for more common forms of RP.

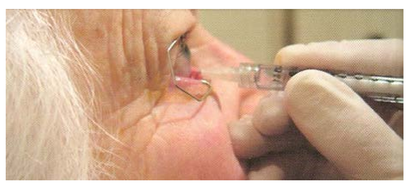
Currently, there is no effective treatment to reverse or limit the progression of vision loss associated with more common forms of RP except nutritional supplementation, which has only a modest effect on disease progression.

PROPOSED TREATMENT

Repurposing CD34+ autologous stem cells from the bone marrow of patients with vision loss from retinitis pigmentosa (RP) and injecting the cells directly into the eye.

Allogenic (from a donor) bone marrow stem cell transplants have been used to treat leukemias for many years. Autologous (from the patient) bone marrow stem cells also have been used in clinical trials for heart disease.

Adult human bone marrow contains CD34+ stem cells that play an important role in tissue repair and maintenance. They target the injured tissue and promote regeneration via secretion of trophic factors. By injecting the stem cells into the eye, the regenerative potential of these cells may be optimized. Previously, the regenerative effect of intravitreal injection of these stem cells in eyes with retinal degeneration has been demonstrated mice and rats.



The information obtained in this prospective study will help access the further safety and efficacy of this stem cell therapy as a treatment for vision loss associated with RP.

PROJECT

A phase I/II open-labeled prospective clinical trial to explore the safety and efficacy of intravitreal injection of autologous CD34+ stem cells from bone marrow as treatment for vision loss associated with RP.

We have received clearance from the FDA for this trial, and we have enrolled three patients to date. With additional funding, we will enroll five more patients with moderately advanced vision loss in both eyes from RP. Only one eye per subject will be treated. A comprehensive eye examination and diagnostic tests will be performed in both eyes at baseline and at study follow-up to month 6, and results from the treated eye will be compared to the untreated eye.

Specific Aims

- To verify the **safety and feasibility** of the treatment
- To observe whether the treatment **reduces vision loss** associated with RP
- To test whether the treatment **decreases the rate of progression** of RP

This study is an important step in determining whether autologous stem cell therapy can reduce the symptoms and rate of progression of RP and restore visual function. If successful, this research could lead to improved vision and quality of life for RP patients.

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