The Stem Cell Ocular Regenerative Medicine—STORM—Center is pioneering the use of regenerative medicine and stem cells to restore vision.
The **STORM Center** is harnessing the awe-inspiring **regenerative potential of stem cells** to overcome many devastating eye diseases and reverse blindness.

We are pursuing visionary—but achievable—**goals**:

1. **To slow, and ultimately prevent**, the loss of sight.
2. **To restore vision** for patients who have already lost sight.
Our window into the world

The human eye is a remarkably sophisticated organ. To provide the experience of sight, it must successfully perform two tasks: (1) capture images, using special light-sensing cells called photoreceptors, and (2) transmit those images to the brain, via the optic nerve.

Certain eye diseases cause dysfunction and death of photoreceptors.

These retinal degenerative diseases include age-related macular degeneration, retinitis pigmentosa, macular dystrophy and Stargaardt disease.

Other eye diseases cause dysfunction and death of retinal ganglion cells (RGCs), the cells of the optic nerve that connect the eye to the brain.

These RGC diseases include glaucoma and other forms of optic nerve damage such as trauma, tumors and toxin exposure.

Patients lose vision when significant numbers of their photoreceptors or RGCs are damaged or die.

Regenerative medicine holds promise for many diseases, spanning almost all body systems, and the eye is an ideal organ to use in service of this research because it is:

• **Well understood.** Medical scientists have already developed a model of the retina that is used in studies to better understand the brain. A robust body of existing knowledge about the eye is expediting stem cell research.

• **Low Risk.** The immune system in the eye is more forgiving than in other organs. Genetically dissimilar cells can be transplanted into the eye with limited chance of immune rejection.

• **Accessible.** The eye is the only part of the central nervous system that is externally visible and accessible.
At the STORM Center, our scientists have achieved several key successes—setting us on the path to develop effective regenerative medicine therapies.

Our **breakthroughs** include the:

- Creation in the lab of **three-dimensional human retinas**, referred to as retinal cups, composed of photoreceptors that actually sense light.
- Growth of **new retinal ganglion cells** (RGCs) in the lab from human stem cells.
- Use of **stem cell-derived retinal cells** for improved drug discovery.

With these building blocks, we are pressing forward to develop new treatment strategies for blinding eye diseases.

Our current work focuses on:

- **Optimizing** regenerative medicine for the eye, specifically, honing technologies to (a) correct genetic mutations, and (b) grow healthy photoreceptors, RGCs and other retinal cells from human stem cells.
- **Identifying** molecules that help the new cells survive and creating the best environment to keep transplanted cells alive in the eye.
- **Building** and testing surgical devices and approaches to deliver stem cell-derived retinal cells into the eye.
- **Facilitating** assimilation of the transplanted photoreceptors and RGCs to connect them with other cells in the retina and the brain, which will enable the patient to see.
- **Developing** real-time imaging technology that will allow us to monitor the performance of the transplanted cells.
When possible, we will use specialized gene editing technology to correct the DNA mutation in the patient's cells that is responsible for the disease.

We are already developing techniques for implanting the new personalized stem cells in ways that help them survive, thrive and function like the cells in healthy eyes.

We will first derive the patient's own stem cells from blood, skin or other easily obtained tissue.

With specialized cell engineering, we will re-program these stem cells to develop into the specific type of eye cell required for the patient's treatment.

When possible, we will use specialized gene editing technology to correct the DNA mutation in the patient's cells that is responsible for the disease.

We will follow the patient to track the outcomes of STORM treatment and refine our methods.

Over time, the patient's personalized stem cells will regenerate the eye tissue, restoring vision.

Personalized regenerative medicine

This work entails all stages of research—from the laboratory “bench” to pre-clinical testing and ultimately to human clinical trials.
Motivated by a desire to help and inspired by the possibilities for restoring vision, you can partner with us to realize the goals of the STORM Center.

Together, we can identify a giving opportunity that will have major impact for patients and that will be the most meaningful for you.

Johns Hopkins Wilmer Eye Institute
Wilmer Development Office
Email: wildev@jhmi.edu
Phone: 410-955-2020
www.hopkinsmedicine.org/wilmer/research/storm