What is the purpose of transplanting retinal cells?
The goal of transplant therapies is to save and restore vision by replacing damaged cells in the retina with new, healthy cells.

Currently researchers are working to find ways of replacing two different kinds of cells:
- Retinal pigmented epithelial (RPE) cells
- Photoreceptor cells

Why transplant cells not the whole retina?
Unfortunately, mature cells in a transplant retina do not grow and make the nerve connections necessary to carry light signals to the brain. Research has shown that less mature cells can make these connections, so scientists began to study ways to use stem cells to grow cells for transplant.

What is a stem cell?
A stem cell is a very basic, unspecialized type of cell. An early human embryo is made up entirely of such cells.

Most of the cells in our body reproduce by dividing. So, for example, as we grow a skin cell on the surface of our body will divide, creating two skin cells. Stem cells are unique. When they divide, the two new daughter cells may be come stem cells like their parent, or they may become more specialized cells. It is this remarkable property that allows a human embryo to grow from a blob of unspecialized cells to a baby with many different specialized cells and organs.

Are stem cells only found in embryos?
No. Stem cells are an important part of how the body grows and replenishes itself throughout life. For example, our bone marrow contains stem cells that divide and produce different blood cells as needed. Stem cells in other areas help generate new tissues if our body is damaged by disease or injury. Stem cells in the adult body often become partially specialized. So for example, stem cells in the liver specialize to only make liver cells. We call these partially specialized stem cells, progenitor cells.
Are there stem cells in the retina?
Yes. In the early 1990s, Dr. Derek van der Kooy at the University of Toronto, showed that there are stem cells in the human retina. This discovery was made with support from Foundation Fighting Blindness donors.

Unfortunately, the stem cells and retinal progenitor cells in the human retina, are not functional. They cannot produce new cells when the retina has been damaged. However, Dr. van der Kooy and his team have shown that when these cells are extracted from a human retina, they can be used to produce new retinal cells in a dish in the lab. He and his team study how we might use these cells grown in the lab for transplants into the diseased or damaged eye.

Would it ever be possible to mobilize the retinal stem cells already in the eye so that they produce new retinal cells?
We hope so. Some types of fish and frogs can repair a damaged retina using stem cells in their eyes. If we can understand the genes that control retinal stem cells in these other animals, we might be able to manipulate these cells in humans, enabling them to repair damage to the eye. Foundation donors currently support Dr. Vince Tropepe’s studies on the genes that mobilize stem cells in fish.

How are stem cells used to make transplant cells?
Stem cells can be grown in the laboratory and then prompted to become the type of retinal cells needed for transplants by using different growth factors and genetic techniques. Cells created in this way can be injected into the retina. In animal studies, such cells have been able to make complex connections including connections to nerve cells that restore vision.

Different types of cells are needed to treat different conditions. For example, replacing damaged RPE cells might improve vision in people with Stargardt disease or age-related macular degeneration. For people with late stage retinal disease, photoreceptors will be needed to restore sight. Cone photoreceptors will be the cells needed by people with age-related macular degeneration and other macular degenerations; for people with retinitis pigmentosa, rod photoreceptors (or both types of photoreceptors) will be needed.

If stem cells can be used to restore sight to animals why haven’t human trials started?
Some human trials have now begun to transplant RPE cells, but not yet photoreceptors. Photoreceptors are more difficult to produce in the laboratory and more difficult to transplant effectively.

There are three significant challenges to effective transplant treatments:

To produce large numbers of cells for treatment – Right now most cells used in transplants are produced in research labs. The process is tremendously complicated, with different technical requirements for each type of cell. Many “recipes” to produce cells are being proposed and patented, but efficiency is still low; we do not yet know the recipes that will be most successful. Many research teams are working on this challenge including several funded by the Foundation Fighting Blindness. Facilities like the Centre for Commercialization of Regenerative Medicine in Ontario are emerging to take the recipes developed in research labs and produce the large volumes needed for treatments.
To produce completely pure cultures of cells – If retinal cells, either RPE cells or photoreceptors are to be injected into people, we must be confident that the cells injected do not include stem cells or other unwanted cell types. Unwanted cells might grow abnormally. Scientists must be sure that the transplant cells will not increase cancer risk.

To enable newly transplanted cells to integrate with existing cells and make connections – Once the new cells are injected into the eye, they must avoid the body’s defense systems and connect to other retinal cells. This is particularly challenging for photoreceptors cells, which must not only make connections with other cells for nourishment, but must also make connections to nerve cells in order to send light signals to the brain. Unless they can make these connections, the transplanted cells will not be able to restore vision. We know that this is possible in animal studies, but unfortunately such connections are not easily made. Less than 0.01% of transplanted photoreceptor cells survived in the initial animal studies. This is a busy area of research, with scientific teams using a variety of biomaterials to support these cells at the time of transplant, protecting them and holding them in the correct area of the retina while they make connections. For one example, see this video on Dr. van der Kooy’s work at

Where do the stem cells used in retinal research come from?
The earliest studies of stem cell therapies used cells collected from human embryos. These cells are used in some research because they have been studied for longer and more is known about how to grow them.

Since Dr. van der Kooy’s discovery of retinal stem cells, he and others around the world have begun using cells gathered from human eyes donated to eye banks. The advantage of these cells in that they are already partly specialized to produce retinal cells; however less is known about the optimal way to grow them.

In 2006, a group of scientists in Japan produced induced pluripotent stem cells (iPSC). These are normal adult cells gathered from the skin or the inside of the cheek that are manipulated in the laboratory to become stem cells. This amazing technology could potentially allow patients to be treated with cells derived from their own body. However, the technology is currently complex and expensive, and it is possible that there might be increased risks of using cells that have been manipulated so extensively.

In 2011, iPSCs were used by a team at Harvard University to make photoreceptor cells, which were then shown to restore some sight to blind mice - proving that cells derived from skin can be used in this way. Dr. Budd Tucker, a post-doctoral fellow from Newfoundland funded by the Foundation Fighting Blindness was part of this ground-breaking team. Recently a team of scientists in Japan received approval to begin a human clinical trial of RPE cells made from iPSCs to treat age-related macular degeneration.

Could iPSCs be used to create transplant photoreceptors for inherited retinal conditions?
Inherited retinal diseases occur due to a gene defect. Since all of the cells in our body have the same genes, new photoreceptors grown from the skin cells
of a person with inherited disease would have the same mutation that caused the damage. Over time it is likely that these cells would degenerate and vision loss would recur.

If iPS cells prove safe and useful, there are potential solutions to this problem. For example, other types of therapies, such as gene therapies (see our fact sheet) might be used to repair the new cells before they are transplanted. For people whose retinal disease developed late in life, unrepaired cells might extend vision for a sufficient time.

Even if it does not prove practical to treat inherited retinal degenerative diseases with a person’s own cells, cells could be gathered from closely-related relatives who do not have the disease. Unlike other transplants, this would be easy and painless for the donor.

What human trials are underway testing retinal transplants?

In addition to the previously mentioned Japanese trial, a few other human trials are now testing RPE cell transplants to improve vision in people with Stargardt disease and age-related macular degeneration (AMD). These conditions were chosen because toxins build up in the RPE in people with these diseases. Some types of retinitis pigmentosa which also damage the RPE could be the focus of future trials.

Currently these trials are only recruiting small numbers of people in early safety trials. Advanced Cell Technology is conducting trials of RPE transplants in London, UK and in several American centres in Los Angeles, Miami, Boston and Philadelphia. Another company, CHA Bio & Diostech is doing similar studies in Korea.

Are any human clinical trials transplanting photoreceptors?

No, not yet. Two groups, iCyte Inc, led by Dr Henry Klassen in California and the UK company, ReNeuron, have said publicly that they intend to make applications for approval of such trials in 2014.

What about centres that inject stem cells as treatment?

The idea of stem cells as a potent therapy for incurable diseases has become a powerful one and clearly therapies derived from stem cells have great potential. However many unscrupulous “treatment centres” have been created to take advantage of people’s eagerness for these therapies. These centres offer to inject stem cells at significant cost. These clinics have no evidence that this is effective. You should never have to pay to be part of a clinical trial!

However it is possible that direct treatment with some types of stem cells might have therapeutic value, if the product is carefully controlled and the objectives of the study are clear. Some scientists do continue to explore these questions including the company, Stem Cells Inc, which is currently conducting a clinical trial for AMD at several US sites.

Updated October 15, 2013: Reviewed by Dr. Valerie Wallace, Toronto Western Hospital and Dr. Carol Schuurmans, University of Calgary.