

## **Stem cell treatment**

Stem cells are special types of cells that are able to transform into other types of cell.

The main purpose of stem cell treatment will be to replace damaged or dead retinal cells in people who have already lost vision. Human trials have already started using RPE cells derived from stem cells. RPE cells provide support for the overlying photoreceptors which are also typically damaged in late stage MD. It is likely that ultimately, both RPE and photoreceptors will need to be replaced to provide significant restoration of vision. The development of photoreceptors from stem cells is many years behind the development of RPE cells, however in 2013, for the first time, scientists in the United Kingdom have implanted photoreceptors derived from stem cells into blind mice and restored vision.

### **Sources of stem cells:**

#### **1. Human embryonic stem cells (hESCs).**

One or two cells are removed from an embryo. These cells are then cultured in the laboratory to produce many millions of stem cells which can then be transformed into the desired celltype. hESCs are generally considered to be the most adaptable type of stem cell as they can be converted into almost any type of cell.

**2. Adult stem cells.** These are usually obtained from either umbilical cord blood, or from bone marrow. These cells are more limited in the types of other cells they can produce.

**3. Induced pluripotent stem cell (iPSC).** Certain types of adult cells such as skin or retinal cells can be re-programmed to revert back to being a type of stem cell, although they are more limited as to the type of new cell that can be formed, when compared to hESCs.

### **Some current trials on stem cells**

#### **1. Advanced Cell Technologies (ACT), USA**

ACT has been able to culture huge quantities of stem cells from a single hESC. They have then coaxed large numbers of stem cells into becoming RPE cells. Human trials with RPE cells implanted under the retina started in 2011, in small numbers of patients with very poor vision from advanced dry MD or Stargardt's disease. Initial studies are primarily testing the safety of treatment. New patients are receiving progressively larger numbers of implanted cells. To date, no safety issues have been identified. The implanted cells are stable, and are remaining in place. In May 2013, the company reported that one patient has shown an improvement in vision from 6/120 (legal blindness) to 6/12 (driving vision), a remarkable and unexpected result. It is not yet known if other patients will experience such dramatic improvement.

#### **2. StemCells Inc, USA**

This company is now assessing the safety and signs of visual benefit of human neural stem cells (HuCNS-SC) implanted into the retina of 16 patients with dry AMD. Previous research with rats has shown that these cells are able to survive for long periods, protect photoreceptors and preserve vision. Patients will be followed for 5 years to ensure long term safety and efficacy.

### **3. Riken Research, Japan**

The Riken group received approval in 2013 to commence human trials of retinal cells derived from a type of stem cell found in the patient's own skin. This approach should avoid the potential issue of rejection that could occur with stem cells from another source. The Japanese government is providing substantial funding for this research.

Other human stem cell trials are also underway in Brazil and South Korea.

### **4. Centre for Eye Research Australia (CERA), Melbourne**

Although there are presently no Australian human trials of stem cell-derived treatments for MD, CERA is currently taking skin cells from humans who have various eye diseases, including MD, and are turning these into stem cells which are then coaxed to become retinal cells grown in a dish. These cells are then being used as a "live" laboratory model of the human retina to gain a more accurate understanding of the causes of eye diseases. These cells could also be used as an early "test bed" for potential treatments before entering clinical trials in actual humans.

## **IMPORTANT NOTE ON STEM CELL TREATMENT**

The Foundation respects different points of view concerning stem cell research. The Foundation's role is simply to report on key research for your information.

- There are currently no commercially available, registered stem cell-derived treatments for MD available anywhere in the world.
- Please take heed of the Foundation's warning that in countries with poor regulatory controls, there are unscrupulous companies that are selling unproven and unregistered 'treatments' using products that they claim to be stem cells.
- The Foundation strongly advises all patients to discuss any treatment considerations with their eye specialist.