



# STEM CELLS FOR EYESIGHT

## 5

### Questions you should have the answers to

The following is a summary of the Royal Australian and New Zealand College of Ophthalmologists' position statement on the role of stem cells in treating ocular disease. This resource was developed in collaboration with Stem Cells Australia.

#### 1 What are stem cells and how can they help?

Stem cells are special cells that have the unique ability to create copies of themselves (self-renew) and develop into different types of cells. There are many types of stem cells, each known by their function and location. They can be obtained from the patient that is being treated (autologous) or from living or deceased donors (allogeneic).

Stem cells are being investigated to treat a range of conditions affecting different parts of the eye. For example, clinical trials have recently shown that stem cells taken from the eye's surface, known as limbal stem cells, can help restore vision and reduce pain in patients suffering from conditions affecting the eye's window, the cornea. A different type of stem cell, referred to as pluripotent stem cells and obtained from human embryos or made in a dish in the laboratory from a patient or donor's cells, are being investigated as they can be grown into the cells that are damaged or absent in age-related macular degeneration (a condition that typically results in blurry central vision) and inherited disorders that affect the back of the eye, the retina. If these replacement cells, made from stem cells, can be successfully introduced into the back of the eye, they may be able to restore vision in retinal disease.

#### 2 What are the safety concerns?

The long-term safety of stem cell treatments for eye disease is not known. Factors such as the stem cell type and how the cells are prepared, stored and given can all significantly change your exposure to risk. Even if the cells are obtained from your own body (so called autologous cells), it does not mean that they are guaranteed to be safe. Many of the ways that the cells are delivered are highly invasive and carry a risk of causing damage to the delicate architecture of the eye.

#### 3 Is there evidence that stem cell therapies work?

While results from clinical trials using limbal stem cells to renew the eye's surface have shown promise, stem cell therapies for other eye conditions are still in the early stages

of investigation. Harnessing the potential of stem cells is complex, and although progress has been made in advancing our understanding, we are yet to have all the answers. There is currently no 'gold-standard' stem cell treatment for eye diseases. More research, including properly designed clinical trials, is required to determine whether the promise of new effective stem cell-based treatments can be realised.

#### 4 Stem cell treatments are still under investigation, so what does that mean for me?

Although many clinical trials are underway, stem cell treatments remain experimental. Initial promising studies need to be further explored in clinical trials over the long term and in a larger number of patients to establish their safety and effectiveness. Such efforts to advance the field of stem cell treatments need to be distinguished from services offered by unregulated stem cell clinics that claim they can already use stem cells to treat a range of conditions associated with vision loss. These expensive and non-evidence based practices can be very risky for patients.

#### 5 How can I make an informed decision about my options?

If you are thinking about stem cell treatments, it is important to be fully informed of what is on offer and the possible risks. Speak to your doctor for advice. Be wary of unsubstantiated marketing claims from unregulated stem cell clinics, including where patient testimonials are used to endorse commercial services. We recommend you only take part in clinical trials that have ethics approval and meet the standards of Australia's regulatory body, the Therapeutic Goods Administration. Approved clinical trials will be registered on the Australian and New Zealand Clinical Trials Registry and will monitor patients over time to assess the long-term safety and outcomes of treatment. You are not usually expected to pay to participate in an approved clinical trial.

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- 2 What are the safety concerns?
- 3 Is there evidence that stem cell therapies work?
- 4 Stem cell treatments are still under investigation, so what does that mean for me?
- 5 How can I make an informed decision about my options?

### Supporting Organisations

#### Royal Australian and New Zealand College of Ophthalmologists (RANZCO)

RANZCO is the leading body responsible for the professional development and training of ophthalmologists in Australia and New Zealand. It aims to ensure equitable access to high quality eye health across the region. RANZCO is committed to improving eye healthcare through education, advocacy, research and quality training. <https://ranzco.edu/>

#### Stem Cells Australia

Stem Cells Australia brings together experts in bioengineering, nanotechnology, stem cell biology, advanced molecular analysis and clinical research to harness the potential of stem cells for diagnostic, therapeutic and biotechnological purposes. Established with the support of the Australian Government through the Australian Research Council's Special Research Initiatives scheme, this collaboration not only supports excellence in stem cell research but also aims to lead public debate and discussion about the important ethical, legal and societal issues associated with stem cell science. <http://www.stemcellsaustralia.edu.au/About-Stem-Cells/For-Patients.aspx>

### Additional Resources

#### A Closer Look at Stem Cells website

<http://www.closerlookatstemcells.org/>

#### American Academy of Ophthalmology website

<https://www.aao.org/eye-health/tips-prevention/stem-cell-therapy-eye-disease-facts>

#### A review of recent clinical trials of stem cells in the eye

<http://www.stembook.org/node/716>

#### Australian New Zealand Clinical Trial Registry

<http://www.anzctr.org.au/GetInvolved.aspx>

#### Royal National Institute of Blind People, UK

<http://www.rnib.org.uk/Pages/Home.aspx>

#### Stem Cell Revolutions - a documentary with a chapter on macular degeneration

<http://www.stemcellrevolutions.com/>

#### The Australian Stem Cell Handbook

<http://www.stemcellfoundation.net.au/about-stem-cells/stem-cell-treatment-information/handbook>

#### The European Blind Union

<http://www.euroblind.org/>

#### The eye and stem cells: the path to treating blindness

<https://www.eurostemcell.org/eye-and-stem-cells-path-treating-blindness>

#### The London Project to Cure Blindness

<https://www.moorfields.nhs.uk/londonproject>