

Understanding NICE guidance

Information for people who use NHS services

Treating limbal stem cell deficiency with cultivated stem cell transplant

NICE 'interventional procedures guidance' advises the NHS on when and how new surgical procedures or procedures that use electromagnetic radiation (such as X-rays, lasers and gamma rays) can be used.

This leaflet is about when and how cultivated stem cell transplant can be used to treat people with limbal stem cell deficiency in the NHS in England, Wales, Scotland and Northern Ireland. It explains guidance (advice) from NICE (the National Institute for Health and Clinical Excellence).

NICE has produced this guidance because the procedure is quite new. This means that there is not a lot of information yet about how well it works, how safe it is and which patients will benefit most from it.

This leaflet is written to help people who have been offered this procedure to decide whether to agree (consent) to it or not. It does not describe the procedure in detail – a member of your healthcare team should also give you full information and advice about these. The leaflet includes some questions you may want to ask your doctor to help you reach a decision. Some sources of further information and support are on the back page.

Interventional procedures guidance makes recommendations on the safety of a procedure and how well it works. The guidance does not cover whether or not the NHS should fund a procedure. Decisions about funding are taken by local NHS bodies (primary care trusts and hospital trusts) after considering how well the procedure works and whether it represents value for money for the NHS.



What has NICE said?

NICE has said that there is not enough evidence about the safety of this procedure and how well it works for cultivated stem cell transplant to be offered routinely. There were five studies available, and the studies only looked at a small number of procedures.

If a doctor wants to use cultivated stem cell transplant, he or she should make sure that extra steps are taken to explain the uncertainty and the likely benefits and potential risks of the procedure. This should happen before the patient agrees (or doesn't agree) to the procedure. The patient should be given this leaflet and other written information as part of the discussion. There should also be special arrangements for monitoring what happens after the procedure.

NICE has also said that further information about how well cultivated stem cell transplant works in the long term would be helpful. In addition, further information about the risks and benefits of taking anti-rejection drugs in the long term would also be useful. NICE may look at this procedure again if more information becomes available.

This procedure may not be the only possible treatment for limbal stem cell deficiency. Your healthcare team should talk to you about whether it is suitable for you and about any other treatment options available.

Cultivated stem cell transplant

The procedure is not described in detail here – please talk to your consultant for a full description.

The limbus is part of the outermost layer of the eye. It contains special cells called limbal stem cells. The limbus protects the cornea (the transparent part of the eye covering the iris and the pupil). Sometimes, the body is no longer able to produce any or enough limbal stem cells. This is usually as a result of disease or chemical injury to the eye. Doctors call this limbal stem cell deficiency and it can result in a loss of vision.

The aim of treatment is to restore the healthy outer layer of the eye.

Cultivated stem cell transplant is one type of transplant surgery which is possible when both eyes are affected. It involves taking limbal stem cells from a living or deceased donor, growing them in the laboratory (the medical word for this is cultivation) and then transplanting them onto the patient's affected eye(s). The full name of the operation is tissue-cultured limbal stem cell allograft transplantation for regrowth of corneal epithelium. Tissue-cultured means that the cells are grown (or cultivated) in the laboratory. Allograft means that cells from one person are used in another person. The corneal epithelium is the outer layer covering the surface of the cornea.

The transplant is carried out under local or general anaesthesia.

Immediately after the transplant, a protective soft contact lens and artificial tears may be used to protect the eye and help the healing process. Sometimes, the procedure has to be repeated.

After the procedure, patients have to take tablets called immunosuppressants. The immunosuppressant medicine reduces the risk of the patient's immune system rejecting the transplanted cells. There are different types and doses of immunosuppressant medicines. They may be needed long term.

What does this mean for me?

If your doctor has offered you cultivated stem cell transplant for limbal stem cell deficiency, he or she should tell you that NICE has decided that the benefits and risks are uncertain. This does not mean that the procedure should not be done, but that your doctor should fully explain what is involved in having the procedure and discuss the possible benefits and risks with you. You should only be asked if you want to agree to this procedure after this discussion has taken place. You should be given written information, including this leaflet, and have the opportunity to discuss it with your doctor before making your decision.

You may want to ask the questions below

- What does the procedure involve?
- What are the benefits I might get?
- How good are my chances of getting those benefits? Could having the procedure make my vision worse?
- Are there alternative procedures?
- What are the risks of the procedure?
- Are the risks minor or serious? How likely are they to happen?
- What care will I need after the operation?
- How long will I be in hospital?
- What happens if something goes wrong?
- What may happen if I don't have the procedure?

You might decide to have this procedure, to have a different procedure, or not to have a procedure at all.

Summary of possible benefits and risks

Some of the benefits and risks seen in the studies considered by NICE are briefly described here. NICE looked at five studies on this procedure.

How well does the procedure work?

In four studies that NICE looked at, cultivated stem cell transplant was successful in 7 out of 10 eyes in one study, and in 4 out of 4, 7 out of 7 and 13 out of 13 eyes in the other studies. A fifth study showed that longer-term results may be poorer: in this study of 13 eyes, the transplant was successful in only 6 out of 13 eyes when the patients were followed up some time after the operation.

The studies also looked at sharpness and clarity of vision. Doctors call this visual acuity. After the procedure, visual acuity was reported in 4 out of 10 eyes in the first study, 10 out of 13 eyes in another study and 7 out of 7 eyes in another; however, some patients had a separate procedure to improve their vision at the same time as the transplantation.

The expert advisers said that if the transplant works well, the procedure is very good at improving vision.

Risks and possible problems

In one study of 13 eyes, 1 eye developed a bacterial infection after the procedure. In a second study of 13 eyes, 2 eyes developed a bacterial infection and in a third study of 4 eyes, 1 eye developed an infection. In addition, out of 13 eyes treated 1 eye developed glaucoma which needed surgery, and there were 4 cases of damage to the cornea after the procedure.

The expert advisers said that in theory, other potential risks are an infection transmitted from the donated cells, rejection of the transplanted cells and the small chance that the donor's limbal stem cells may fail in the future which would mean that they would need treatment themselves.

More information about limbal stem cell deficiency

Your local Patient Advice and Liaison Service (PALS) may be able to give you further advice and support.

About NICE

NICE produces guidance (advice) for the NHS about preventing, diagnosing and treating different medical conditions. The guidance is written by independent experts including healthcare professionals and people representing patients and carers. They consider how well an interventional procedure works and how safe it is, and ask the opinions of expert advisers. Staff working in the NHS are expected to follow this guidance.

To find out more about NICE, its work and how it reaches decisions, see www.nice.org.uk/aboutguidance

This leaflet and the full guidance aimed at healthcare professionals are available at www.nice.org.uk/IIPG216

You can order printed copies of this leaflet from the NHS Response Line (phone 0870 1555 455 and quote reference N1240).