

## STEM CELL THERAPY – ATAXIA UK POSITION STATEMENT

There has been considerable emphasis in the last few years on the potential for using stem cells to treat a wide variety of medical conditions. Stem cells are cells that have the ability to become many different types of cell found in the body—a characteristic that can be harnessed to treat medical conditions. However, clinical work with stem cells is currently at a very early stage and has not been proven to have benefits in treating ataxia.

### What is stem cell therapy?

Stem cells are used in medicine and research in many different ways. One type of stem cell therapy has been used in the clinic for many years to treat some blood cancers. This treatment involves matching the patient to a stem cell donor, and replacing the stem cells of the patient with stem cells from the donor to treat the cancer. In recent years, a similar type of stem cell therapy which uses a patient's own stem cells has successfully been used to treat conditions which involve the immune system, such as multiple sclerosis (MS).

### Stem cell therapy and ataxia

Possible benefits of stem cell therapies have been highly publicised in the press, and there are clinics around the world who claim to offer stem cell therapy to treat a number of conditions, including ataxia. We believe that any treatment should be offered on the basis of the results of rigorous trials in patients that assess both the risks and benefits of the intervention. However, unlike the treatments described above, **stem cell therapies have not yet been tested in this way in people with ataxia.**

Clinics offering stem cell therapy for ataxia often do not provide important details such as the source of the stem cells, but will use positive testimonials from patients to sell their treatment. It is important to note that patients that have reported benefit following a visit to one of these clinics often find that the improvement in symptoms is short-lived (lasts for days/weeks), and the treatment is often given alongside intense physiotherapy, which could be the real cause of any short-lived improvement in symptoms. Importantly, there have been **no clinical trials proving the effectiveness of stem cell therapy for Friedreich's ataxia or any of the other ataxias.** The potential risks have also not been fully evaluated. Many countries are now tightening their regulations to prevent unproven stem cell products from being offered as therapies in clinics – but this is not always successful.

As the potential risks of stem cell therapy have not been thoroughly studied, we advise against pursuing stem cell therapy. We urge anyone who wants to know more to speak to their neurologist.

### The future of stem cell therapy

Although stem cell therapy is not yet at a stage where it can be used in the clinic for ataxia, we believe that it offers hope for future treatment for the ataxias. Ataxia UK fully supports ethical research into stem cells in the UK. We have awarded a number of grants to study their potential as future treatments for the ataxias; the first step in bringing stem cell-based therapies for the ataxias to clinical trials.

This statement was reviewed by Ataxia UK's Medical Advisory Panel

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Ataxia UK, 12 Broadbent Close, London, N6 5JW

[www.ataxia.org.uk](http://www.ataxia.org.uk)

Helpline: 0800 995 6037 Tel: +44 (0)20 7582 1444 email [helpline@ataxia.org.uk](mailto:helpline@ataxia.org.uk)