‘Miracle’ stem cell therapy reverses multiple sclerosis

**By**[**Sarah Knapton**](http://www.telegraph.co.uk/journalists/sarah-knapton/)**, Science Editor** 1:16PM GMT 01 Mar 2015

## The treatment, is the first to reverse the symptoms of MS, which has no cure, and affects around 100,000 people in Britain.

The first stem cell treatment has been approved by the European Medicines Agency Photo: ALAMY

A pioneering new stem cell treatment is allowing multiple sclerosis sufferers to walk, run and even dance again, in results branded ‘miraculous’ by doctors.

Patients who have been wheelchair-bound for 10 years have regained the use of their legs in the groundbreaking therapy, while others who were blind can now see again.

The treatment, is the first to reverse the symptoms of MS, which has no cure, and affects around 100,000 people in Britain.

The two dozen patients who are taking part in the trials at the Royal Hallamshire Hospital in Sheffield and Kings College Hospital, London, have effectively had their immune systems ‘rebooted’.

Although it is unclear what causes MS, some doctors believe that it is the immune system itself which attacks the brain and spinal cord, leading to inflammation and pain, disability and in severe cases, death.

In the new treatment, specialists use a high dose of chemotherapy to knock out the immune system before rebuilding it with stem cells taken from the patient’s own blood.

Stem cells are so effective because they can become any cell in the body based on their environment.

"Since we started treating patients three years ago, some of the results we have seen have been miraculous," Professor Basil Sharrack, a consultant neurologist at Sheffield Teaching Hospitals NHS Foundation Trust, told The Sunday Times.

"This is not a word I would use lightly, but we have seen profound neurological improvements."

During the treatment, the patient's stem cells are harvested and stored. Then doctors use aggressive drugs which are usually given to cancer patients to completely destroy the immune system.

The harvested stem cells are then infused back into the body where they start to grow new red and white blood cells within just two weeks.

Within a month the immune system is back up and running fully and that is when patients begin to notice that they are recovering.

Holly Drewry, 25, of Sheffield, was wheelchair bound after the birth of her daughter Isla, now two.

But she claims the new treatment has transformed her life.

“It worked wonders,” she said. “I remember being in the hospital... after three weeks, I called my mum and said: 'I can stand'. We were all crying.

"I can run a little bit, I can dance. I love dancing, it is silly but I do. I enjoy walking my daughter around the park in her pram. It is a miracle but I can do it all."

However specialists warn that patients need to be fit to benefit from the new treatment.

"This is not a treatment that is suitable for everybody because it is very aggressive and patients need to be quite fit to withstand the effects of the chemotherapy," warned Prof Sharrack.

Charities welcomed the research but also urged caution.

Dr Sorrel Bickley, Research Communications Manager at the MS Society said: “This new study reports very encouraging findings, which add to a growing body of research into stem cell transplantation in MS. However, there are limitations to how we can interpret these results because there was no control group used, which means we can’t be sure the results are robust.

"Momentum in this area of research is building rapidly and we're eagerly awaiting the results of larger, randomised trials and longer term follow up data.

“New treatments for MS are urgently needed, but as yet there are no stem cell therapies licensed for MS anywhere in the world. This means they aren't yet established as being both safe and effective. This type of stem cell therapy is very aggressive and does carry significant risks, so we would strongly urge caution in seeking this treatment outside of a properly regulated clinical trial."

MS stem cell treatment only a 'miracle' for some

Monday March 2 2015

The Daily Telegraph reports a “miracle” stem cell therapy that reverses multiple sclerosis and which, according to The Sunday Times, gets “wheelchair-bound” people dancing.



**Stem cells were injected into MS patients after chemotherapy**

Multiple sclerosis (MS) affects nerves in the brain and spinal cord, causing problems with muscle movement, balance and vision. It’s an autoimmune disease, where the body’s immune system attacks its own nerve cells. There is currently no cure, but many different treatments are available to help with symptoms.

This study was mainly about relapsing remitting MS, the most common type, where people have distinct attacks of symptoms, which then fade away either partially or completely.

The new treatment was very aggressive. It used high doses of chemotherapy to “knock out” the existing faulty cells of the immune system, before rebuilding it using stem cells taken from the patient’s own blood. This, in effect, gave the immune system a chance to reboot from scratch.

The therapy was tested in 145 patients and led to significant reductions in their levels of disability in almost 64% of people up to four years after treatment. Improvements were seen in quality of life and other ratings of symptoms and disability. Because of the difficulty in treating MS effectively, any improvements are good news.

The downside is that there was no control group. We don’t know if some people would have improved on their own, or whether the improvements are any better than best available care. It’s also worth noting that not everyone will be able to tolerate the aggressive chemotherapy used, and that the technique did not work for people with more severe or longstanding MS (over 10 years).

Find out more about [multiple sclerosis](http://www.nhs.uk/Conditions/Multiple-sclerosis/Pages/Introduction.aspx).

## Where did the story come from?

The study was led by researchers from Northwestern University Feinberg School of Medicine, Chicago, US, and was funded by the Danhakl family, the Cumming Foundation, the Zakat Foundation, the McNamara Purcell Foundation, and Morgan Stanley and Company.

Two study authors declared financial conflicts of interest by serving as consultants to pharmaceutical companies including Biogen Idec, which makes treatments for patients with “neurological, autoimmune and hematological disorders”.

The study was published in the [peer-reviewed](http://www.nhs.uk/news/Pages/Newsglossary.aspx#peerreview) Journal of the American Medical Association.

Generally, the papers reported the story accurately. It is always difficult to justify the use of a “miracle” cure, because it means different things to different people – and the improvements cited for some people do seem worthy of the tag. However, while the treatment looks promising, it’s at an early stage of development. The treatment is very aggressive, and also tested in specific types of MS, so may not be suitable for all people with MS. Similarly, the treatment has not yet been proven effective or safe in large enough groups for the results to be reliable.

We have not been able to independently assess if the truth of the claims that “wheelchair-bound” MS patients who were treated in this way are now able to dance.

## What kind of research was this?

This was a [case-series](http://www.nhs.uk/news/Pages/Newsglossary.aspx#caseseries) testing a new stem cell treatment in people with relapsing remitting MS or secondary progressive MS.

Most people in the study had relapsing-remitting MS, which tends to have distinct attacks of symptoms, which then fade away either partially or completely. This is the most common type of MS, affecting around 85% of people with the condition, according to the [MS Society](http://www.mssociety.org.uk/).

The research also included a smaller group of people with secondary progressive MS. Many people with relapsing remitting MS progress to this form, where there is a sustained build-up of disability that no longer fades away. There is no cure for MS, but many different treatments are available to help with symptoms.

Case series are useful to test new treatments, but they have several limitations, meaning they can’t prove the treatments are effective very accurately or reliably. The big downside is a lack of a comparison group, called a [control group](http://www.nhs.uk/news/Pages/Newsglossary.aspx#controlgroup). This means that you never know how much better or worse the new treatment is compared with an existing treatment, or doing nothing. This limitation applies to this study.

## What did the research involve?

The study gave 123 patients with relapsing remitting MS and 28 with secondary progressive MS a stem cell transplant. The transplants were carried out at a single US institution between 2003 and 2014, and researchers followed the patients for up to five years to see how they did.

The average age of participants was 36 (ranging from 18 to 60) and most were women (85%). The average follow-up after treatment was 2.5 years.

The new treatment used chemotherapy drugs cyclophosphamide and alemtuzumab or cyclophosphamide and thymoglobulin, followed by infusion of stem cells isolated from the patients’ blood.

Before treatment, participants were subjected to a range of questionnaires and assessments to rate their symptoms, level of disability and quality of life. These were repeated at regular intervals after to measure any changes.

The main measure of interest was an improvement in score of 1.0 or more on the [Expanded Disability Status Scale (EDSS)](http://www.mstrust.org.uk/atoz/edss.jsp). The EDSS is a way of quantifying disability in MS and monitoring changes over time. It is widely used in clinical trials and in the assessment of people with MS.

The main analysis compared EDSS ratings before and after treatment, looking for statistically significant improvements. Similar comparisons were done for other measures of MS-related disability and quality of life.

## What were the basic results?

### Change in disability scores

There was significant improvement in disability up to four years after treatment. Reductions in the EDSS (disability) score of 1.0 or more were seen in half of patients at two years (50%, 95% [confidence interval (CI)](http://www.nhs.uk/news/Pages/Newsglossary.aspx#confidenceinterval) 39% to 61%) and almost two out of every three people at four years (64%, 95% CI 46% to 79%).

Scores from the EDSS improved significantly. Before treatment, the average (median) EDSS score was 4.0, which improved to 3.0 at two years and to 2.5 at four years. Both were statistically significant reductions.

Taking the reduction of 4.0 to 2.5, this means the person went from having “Significant disability, but self-sufficient and up and about some 12 hours a day. Able to walk without aid or rest for 500m” to “Mild disability in one functional system or minimal disability in two functional systems”.

However, some people did not improve. The EDSS score did not improve in people with secondary progressive MS or in those with disease duration longer than 10 years.

### Ratings of disability and quality of life

Many other measures also improved, including neurological function, walking function, hand function and self-reported quality of life. The tests also involved a brain scan assessing the size of inflammation at a specific part of the spinal cord in the upper back (the T2 vertebra), which is said to correlate with disease severity. After treatment, the size of the damage reduced, and stayed lower up to two years longer.

### Improvements quoted in the news

Some of the more miraculous findings were reported in the news, but not in the study publication itself. The Telegraph, for example, reported: “Patients who have been wheelchair-bound for 10 years have regained the use of their legs … while others who were blind can now see again.”

We couldn’t confirm these biblical results, based on the publication alone. They may have come from interviews with the study team or case studies provided by the research institutions.

## How did the researchers interpret the results?

The researchers summed up that, “Among patients with relapsing remitting MS, nonmyeloablative hematopoietic stem cell transplantation was associated with improvement in neurological disability and other clinical outcomes.”

To their knowledge: “this [was] the first report of significant and sustained improvement in the EDSS score following any treatment for MS”.

## Conclusion

This case-series showed that a new stem cell treatment reduced the disability in people with relapsing remitting MS up to four years after treatment. It worked in more than half of those given treatment. The authors claim that this was the first time this had been achieved, and is important because there is currently no cure for MS.

Given the relative lack of alternative treatments for MS, these results are encouraging. However, there are issues to bear in mind.

The average EDSS score before treatment in the group was 4.0. The scale goes from 10 (death due to MS), to 1.0 (no disability). Ratings above 5.0 involve impairment with walking. An average of 4.0 suggests most people didn’t have the more severe forms of MS. A small number of people with more severe MS were included in the study, but too few to reach any reliable conclusions about this subgroup. Therefore, the results are most applicable to those with non-severe relapsing remitting MS.

The treatment only improved the main outcome (EDSS improvement of 1.0 or more) in 50% of people after two years, meaning it didn’t work in the other half. It worked for slightly more people after four years. It also didn’t work in people with MS over 10 years or those with secondary progressive MS. This suggests that selecting the most appropriate patient group for this treatment will be important. It doesn’t look like it would work for everyone.

These preliminary findings are from an uncontrolled study. This means we don’t know if, or how much, the new treatment is better than any existing treatment, or doing nothing. The treatment was described by a study author in the Telegraph as being very aggressive, and only suitable for people who were fit enough to withstand the effects of chemotherapy. Chemotherapy is not without risk. Further studies will need to pay careful attention to weighing up the benefits and risks of this therapy.

Dr Sorrel Bickley, from the MS Society, cautiously welcomed the results in the Telegraph, saying: "Momentum in this area of research is building rapidly and we're eagerly awaiting the results of larger, randomised trials and longer-term follow-up data.

“New treatments for MS are urgently needed, but as yet there are no stem cell therapies licensed for MS anywhere in the world. This means they aren't yet established as being both safe and effective. This type of stem cell therapy is very aggressive and does carry significant risks, so we would strongly urge caution in seeking this treatment outside of a properly regulated clinical trial."

## Pioneering use of adult stem cells is making huge improvement in the condition of MS patients

By Dave Andrusko, March 3, 2015

If we’ve written once about the successful uses of adult stem cells, we’ve probably written 50 times, demonstrating that ethically unobjectionable adult stem cells are far superior in practice to stem cells taken from embryonic human beings.

When it comes to making a difference in people’s lives, adult stem cells– isolated from many different tissues, including bone marrow, blood, muscle, fat, and umbilical cord blood–are the gold standard.

Recovery: MS sufferer Holly Drewery became wheelchair-bound after the birth of daughter Isla, but thanks to the stem cell transplant she can dance, run and chase after Isla in the park

The latest evidence comes courtesy of a story in the Daily Mail. In typical Daily Mail fashion the headline is anything but understated: “Dancing, walking and running again, the wheelchair-bound MS patients after they were given ‘miraculous’ stem cell treatment.”

But while the headline over Fiona Macrae’s story seems a bit overstated, her story quietly demonstrates that real progress is being made in treating multiple sclerosis, a devastating disease. I asked David Prentice, an expert on stem cells of all kinds, what he thought of the study, lead by Professor Basil Sharrack, of the Royal Hallamshire Hospital in Sheffield. He told NRL News Today

**This is another gratifying example of the successful application of adult stem cells. Prof. Sharrack and colleagues are to be congratulated for undertaking this study, which has shown extremely promising results. Their publication of the data in the Journal of the American Medical Association adds even more evidence to the already-substantial successes for adult stem cell treatments for patients. The protocol they use, pioneered by Dr. Richard Burt at Northwestern University (who is also a co-author on the JAMA paper), involves harvest and purification of some of the patient’s bone marrow adult stem cells, then chemotherapy to kill the rogue immune cells that are causing the autoimmune problem of multiple sclerosis, followed by re-infusion of the patient’s adult stem cells. The patient’s adult stem cells “reboot” their immune and blood system, and also seem to facilitate some repair of the nervous system.**

There are also two hurtles that are bypassed. By using the patient’s own cells, you’ve precluded transplant rejection. Moreover, unlike embryonic stem cells, adult stem cells carry no concern of tumors.

Macrae’s story provides two encouraging examples.

Holly Drewery was plagued with numbness and blurred vision. She was diagnosed with MS. Her health grew worse, as Macrae writes.

**She became wheelchair-bound after her health worsened on Isla’s [her daughter’s] birth. She needed help with basic tasks and couldn’t even wiggle her toes.**

**Three weeks after the stem cell transplant she was able to walk out of hospital.**

**Now, more than 18 months on, she is almost back to normal. She has a part-time office job and, although she still gets tired, can dance, run and chase after Isla, two, in the park.**

**She said: ‘All I wanted to be able to do is take Isla out. It worked wonders. I am more or less back to normal.’**

Another patient, Sam Ramsey, was paralysed from the neck down by MS after she collapsed when out celebrating her 22nd birthday.

Nothing worked until the adult stem cell treatment. Now she can walk on crutches and can drive.

“This treatment has given me my life back,” she told the Sunday Times. “’This is not a word I would use lightly but we have seen profound neurological improvements.”

Of course more follow-up must be done to ensure that the amazing progress is not short-lived. But toward the very end of the story comes this very encouraging secondary improvement:

**As well as stopping the disease in its tracks the treatment, known as autologous haematopoietic stem cell transplantation, seems to heal damage that has already been done.**