

Medicine

Curing multiple sclerosis

Stem cells are starting to prove their value as medical treatments

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THIRTY years ago a young haematologist called Richard Burt was training at Johns Hopkins University, in Baltimore. He noticed that after leukaemia patients had received a treatment to wipe out their immune systems, they needed to be re-immunised against diseases such as measles and mumps. Although the patients in question had been vaccinated as children, the therapy for their blood cancer had erased this cellular memory. Dr Burt turned to his teacher, William Burns, and ask whether the same might be possible in autoimmune diseases. “I could see a light go on in his eyes. ‘You should try it in multiple sclerosis’ he said.” Thus began decades of painstaking work.



Multiple sclerosis (MS) happens when the body's immune system learns to attack its own nerve fibres in the same way that it learns to attack invading pathogens. Nobody really understands what causes this misplaced learning. But Dr Burt's idea did not depend on knowing that. He just wanted to wipe the memory out, in the way that the memory of a vaccination is wiped out by chemotherapy. By 2009 Dr Burt, now at Northwestern University, in Chicago, had proved that his treatment worked in patients with the most common form of the disease, relapsing remitting MS. The treatment involves using lower-dose chemotherapy to kill the white blood cells that are responsible for attacking nerve fibres, and then rebooting the immune system using stem cells collected from the patient before treatment began.

Stem cells are the source from which more specialised cells develop. Those found in bone marrow, known as hematopoietic stem cells, produce the many different cells found in blood, including the white cells implicated in MS. In Dr Burt's therapy such stem cells are extracted from a patient, stored until after the chemotherapy, and then infused back into him. Ten days later, he can go home.

It is effective. Although there is a relapse rate of around 10% within five years, many who have been treated in randomised trials in Brazil, Britain and Sweden feel as though they have been cured. Proving they actually have been means waiting for the results of the trials, and watching how participants fare over many years. Already patients have been seen to improve for two years after treatment.

This work should give drug companies some pause for thought. They are already facing criticism for the high prices of MS drugs. Moreover, though such drugs can slow the progression of the disease, they cannot do what the stem-cell therapy seems able to, which is to reverse it and improve patients' quality of life—for example by allowing them to walk again.

Last year a study published in *Neurology* found that the cost of MS drugs had risen five to seven times faster than the general inflation rate for prescription drugs. Medicines that cost \$8,000 to \$11,000 20 years ago were between \$59,000 and \$62,000 in 2013. On top of this come bills for doctors, MRI scans, blood draws and lab work. By contrast, Dr Burt reckons, a stem-cell transplant in America costs, all in, \$120,000—and that sum would be lower in countries with less-expensive health-care systems. Indeed, doctors in Britain think the treatment should cost hospitals about £30,000 (just over \$40,000). At those sorts of prices, stem cells are set to give MS drugs a real run for their money.

More broadly, this is good news for proponents of stem-cell therapies in general. Leukaemia is mostly treated these days with hematopoietic stem cells. Delete Blood Cancer, a British charity, is encouraging people around the world to register to donate stem cells. Registration can be done with a cheek swab, to show the genetic make-up of the potential donor's immune system. The cells themselves, if needed, are collected via a blood donation. And other conditions, too, seem susceptible to the stem-cell approach. Mesoblast, an Australian company, received approval last September for a stem-cell treatment for graft-versus-host disease, in which the immune system rejects a transplanted tissue. It has a number of other products in advanced trials, in areas such as chronic heart failure and chronic low-back pain. Stem-cell therapy, so long promised, is starting to become a reality.

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