

Repairing brain damage in MS

Gene therapy as treatment for MS

Neurological symptoms as a result of Multiple Sclerosis (MS), such as loss of cognitive or motor function, lead to a great many problems for over 2.5 million people worldwide. Nerve damage to the brain and the spinal cord cannot be repaired. Researchers of the Netherlands Institute for Neuroscience want to change this by means of the use of modern gene therapy technology. Funding from the Start2Cure Foundation will enable the Netherlands Institute for Neuroscience to embark on an ambitious project to repair the brain damage caused by MS.

The current treatments for MS are mainly geared towards inhibiting inflammatory cells from invading the brain. They reduce the number of MS attacks, but do not stop the disease from progressing. In the later stages of the disease they virtually cease to have an effect. "Unfortunately these therapies do not offer improvement of the symptoms for people whose nervous system is already damaged by MS. This damage is practically irreparable, and there are currently no therapies that repair damage to nerve cells. But repairing this damage is precisely what we aim to do with this project, by means of gene therapy," says Professor Inge Huitinga.

Gene therapy

Gene therapy involves introducing therapeutic genetic material into the body. For this a viral vector, a carrier of therapeutic genetic material, is used. It has recently become possible to reach the brain and the spinal cord by injecting the viral vector via the bloodstream. "This new form of gene therapy allows us, for the first time, to deliver a therapeutic gene to the widespread MS lesions in the brain," explains professor Joost Verhaagen.

Molecules that promote recovery

The aim of the new project is to start with identifying the molecules that stimulate the synthesis of new myelin and the repair of axons in brain tissue donated to the Netherlands Brain Bank by MS patients who recovered very well from MS attacks.

Myelin is the protective isolating fatty substance surrounding the nerve cell axons that transmit the signals in the brain and spinal cord. In people with MS this myelin layer degrades, which means that signals by nerve cells are no longer transmitted properly and loss of function ensues. The newly discovered molecules will then be introduced into viral vectors, together with a number of already known factors, and tested in an animal model for MS for their ability to produce myelin and restore neurological function.

"When this project delivers positive results, the translation to the clinic can be made, in collaboration with (an) industrial partner(s)," says Huitinga. "A drug that promotes functional recovery of the nervous system is very important and potentially very meaningful for a large group of people with advanced MS".

Starting point

Start2Cure is a new foundation which aims to cure MS within 50 years by enabling scientists to develop breakthrough treatments via its Venture Philanthropy Investment model. "Awarding our first *Therapeutic Innovation Research Grant* is the starting point of the Start2Cure Foundation and its *Global Research Grants and Awards program*. As such it will act as our *flagship project to outline the Foundation's specific research focus areas*", says Boaz Spermon Managing Director Start2Cure. "The gene therapy research project of the Netherlands Institute for Neuroscience is a very challenging project given the complexity and the uncertain outcome. As a result, funding opportunities for this type of research are often scarce. Should this innovative approach however turn out to be truly successful, the impact will be even more significant."

Note for editors

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The Start2Cure MS Initiative is a non-profit foundation which was founded by Aat van Herk, a successful private investor in Biotech and real estate. Its mission is to take a leadership role by supporting scientists tackling the toughest challenges in MS research on a global scale and invests in young talent, bold new ideas and scientific approaches to cure MS within 50 years. Ultimately it envisions a future in which the debilitating course of Multiple Sclerosis not only is halted, but also lost function caused by neurodegeneration is improved and finally restored. Start2Cure invests in scientists today to develop tomorrow's cures. (www.start2cure.org)



The Netherlands Institute for Neuroscience is a research institute aimed at the brain and the visual system. The Netherlands Institute for Neuroscience investigates how the brain enables cognitive functions such as consciousness, perception, movement, learning, and social interactions. Another line of research is how brain disorders affect these functions. Research takes place at different levels: at the genetic and molecular level, at the level of the nerve cell and the network, and at the system and behavioural level. The Netherlands Institute for Neuroscience is one of the institutes of the Royal Netherlands Academy of Arts and Sciences (KNAW).

The Netherlands Brain Bank (NBB) is part of the Netherlands Institute for Neuroscience and



collects human brain tissue of donors who have registered their wish to be a brain donor at the NBB. Brain material is collected of donors with a variety of neurological and psychiatric disorders, but also of non-diseased donors. This brain tissue is made available to scientific researchers worldwide. Scientific research done with this tissue results in a better understanding of the brain. This will lead to the development of therapies for the treatment of neurological and psychiatric diseases worldwide.