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Stem cell therapy to tackle HIV

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A novel stem cell therapy that arms the immune system with an intrinsic defence against HIV could be a powerful strategy to tackle the disease.

Professor Ben Berkhout speaking at the Society for General Microbiology's spring meeting in Edinburgh today explains how this new approach could dramatically improve the quality of life and life expectancy for HIV sufferers in whom antiviral drugs are no longer effective.

In the absence of an effective vaccine, daily administration of anti-retroviral drugs is the most effective treatment for HIV. However, low patient compliance rates combined with the virus's ability to easily mutate has led to the emergence of drug-resistant strains that are difficult to treat.

Professor Berkhout from the University of Amsterdam is investigating a novel gene therapy that has long-lasting effects even after a single treatment. It involves delivering antiviral DNA to the patients' own immune cells that arms them against viral infection. "This therapy would offer an alternative for HIV-infected patients that can no longer be treated with regular antivirals," he suggested.

The therapy involves extracting and purifying blood stem cells from the patient's bone marrow. Antiviral DNA is transferred to the cells in the laboratory, after which the cells are re-injected into the body. The DNA encodes tiny molecules called small RNAs that are the mirror image of key viral genes used by HIV to cause disease. The small RNAs float

around inside the immune cell until they encounter viral genes which they can stick to like Velcro™. This mechanism, called 'RNA interference' can block the production of key viral components from these genes.

Transferring the antiviral DNA to stem cells would help to restore a large part of the patient's immune system. "Stem cells are the continually dividing 'master copy' cells from which all other immune cells are derived. By engineering the stem cells, the antiviral DNA is inherited by all the immune cells that are born from it," explained Professor Berkhout.

The group hopes to start clinical trials of the therapy within 3 years. "So far, very promising results have been obtained in the laboratory, and we are now testing the safety and efficacy in a pre-clinical mouse model," said Professor Berkhout.

Provided by Society for General Microbiology