Researchers Announce New HIV-Silencing Stem Cell Therapy

April 1, 2010

Researchers in the Netherlands hope to begin human studies of a novel treatment approach designed to silence HIV genes to prevent the virus from reproducing, according to a Society for General Microbiology (SGM) press release highlighting a talk given by Ben Berkhout, PhD, of the University of Amsterdam at the society's spring 2010 meeting concluding today in Edinburgh.

Researchers studying human disease have been excited about gene-silencing therapy—technically known as RNA interference (RNAi)—since it was first discovered in the early 1990s. Initial studies involved using fragments of RNA, a mirror image of DNA, to suppress a key enzyme responsible for giving petunias their vibrant color.

RNAi typically involves small strands of RNA that can bind to genes, like strips of Velcro, and shut down the gene's activity in plants, animals and humans. With HIV, RNAi has the potential to shut down the genes the virus uses to reproduce once inside cells.

Berkhout and his colleagues have been experimenting with RNAi specific to HIV for much of the last decade. Previous studies conducted by Berkhout's team suggest that it is possible to make and deliver RNAi-producing genes to immune cells capable of shutting down HIV replication.

In his recent Edinburgh talk, Berkhout said that his group has discovered 21 short strands of RNA that silence parts of the virus that don't mutate easily. This is critical, because the targets of all the existing antiretroviral (ARV) drugs can mutate into drug-resistant forms over the time, thus rendering the drugs ineffective.

Berkhout's group now plans to extract human stem cells and then infuse them with genes that will go on to produce the most effective RNAi strands upon being transplanted back into the body.

"This therapy would offer an alternative for HIV-infected patients that can no longer be treated with regular antivirals," he suggests.

It is the transplant process that may pose the greatest risk to patients. For genetically modified stem cells to be effective, they must produce HIV-fighting immune system cells that can flourish throughout the body. But first, all the older stem cells and their progeny must be killed off. This requires a process called conditioning, which involves notably high-dose chemotherapy and/or

radiation.

Until safer conditioning methods are developed, stem cell procedures—even if quite successful—may only be available to limited groups of people. As Bekhout states, this might include people for whom available ARVs clearly no longer work.

Bekhout hopes his lab will be ready to move from animal studies to human studies within the next three 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.”

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