FDA Approves Early Human Trial of Potential HIV Functional Cure

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The U.S. Food and Drug Administration (FDA) has given the green light to an early human trial of an HIV gene therapy that may lead to a functional cure, Medical Daily reports. The treatment involves drawing blood-producing stem cells from HIV-positive study participants who have had a poor response to antiretroviral (ARV) therapy. Next, the researchers will splice in genetic instructions to immune cells to make them resistant to the virus.

In order to infect an immune cell, HIV must first latch onto a receptor on the surface of that cell. HIV is mostly drawn to the CCR5 receptor. (There is another receptor, known as CXCR4, which HIV tends to start to use in the event of advanced HIV disease.) This genetic therapy would lead to the production of immune cells that lack a functional CCR5 receptor, thus blocking HIV from making the necessary initial contact with the cell. Ideally, these immune cells would persist indefinitely.

A similar take on this approach to gene therapy showed promise in a Phase II study, the results of which were announced in September 2013. One participant was able to spend a significant amount of time off of ARVs while maintaining an undetectable viral load. The treatment also led to a long-term increase in CD4 levels among the study participants and a corresponding drop in the size of the viral reservoir.

This new study is a Phase I trial, meaning it is only intended to establish the safety of the therapy. In order for a new treatment to receive FDA approval, it must first pass through three phases of clinical trials, which become progressively larger and more complex. The process can take many years.

To read the Medical Daily article, click here.