Early results of CRISPR gene-editing treatment shows promise in first human trials

November 20 2019, by Bob Yirka

Credit: CC0 Public Domain

Officials from Vertex Pharmaceuticals and CRISPR Therapeutics have announced that preliminary results from testing CRISPR gene-editing treatment in human patients with blood disorders show promise thus far. The joint project between the two firms is taking place at one location in Europe and another in the United States. The results have been posted on

the Vertex Pharmaceuticals web site.

There has been a lot of excitement surrounding the possibility of using the CRISPR gene-editing technique to cure people of genetic diseases. There has also been a constant stream of results from scientists testing the technique under various circumstances—some of which involved treating live animals to see if CRISPR might be used to "cure" them of diseases. Now, scientists have taken the next step—editing the genes of human patients with blood disorders to see if it might cure them.

Officials from the Boston-based Vertex Pharmaceuticals and the Swiss based CRISPR Therapeutics have recently given some details regarding two clinical trials they are running—one in Germany, the other in the U.S. In the trial running in Germany, patients are being treated for a disease called beta thalassemia, in which a genetic defect prevents the body from producing enough hemoglobin. In the trial running in the U.S, patients are being treated for sickle cell disease, in which a genetic defect causes sickle-shaped red blood cells.

The researchers collected stem cells from patients in both trials. The cells were then edited using a CRISPR gene-editing technique called CTX001. Meanwhile, the patients undergo a procedure in which mutant bone marrow is removed from their body. Then the newly edited cells are reinfused into the bone marrow, where they take hold and begin generating properly formed blood cells.

Officials with the two companies report that thus far, the results are promising. One patient with beta thalassemia has not needed a transfusion since undergoing the procedure nine months ago—previously, she was reported to have needed transfusions on a regular basis. They also report that a sickle cell patient has had no vaso-occlusive crises—a painful condition that occurs when misshapen blood cells clog small blood vessels—since undergoing treatment seven months

ago.

More information: ir.crisprtx.com/news-releases/ … -positive-safety-and