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NIH, Bill and Melinda Gates Foundation Collaborate to Develop Gene-Based HIV Treatment AJMC -25...

Kelly Davio, October 28, 2019

The National Institutes of Health (NIH) has announced plans to invest at least \$100 million over the next 4 years to develop gene-based therapies for 2 diseases: HIV and sickle cell disease (SCD). The Bill and Melinda Gates Foundation (Gates Foundation) will also contribute \$100 million to the goal of advancing these potential cures, with an aim toward providing affordable, globally available treatment that will be accessible to patients in low-resource settings.

According to the NIH, the collaboration between the organizations will focus on 2 key areas. First, it will identify potential candidate cures for preclinical and clinical evaluation, and second, it will define long-term opportunities to partner with organizations in Africa, a region particularly affected both by HIV and SCD and by a lack of affordable treatments.

The HIV therapies of interest to the organizations will target the <u>latent reservoir</u> of proviral DNA in CD4-positive T cells that persists despite antiretroviral therapy (ART). The organizations noted that the treatments they hope to develop will be in vivo, which they <u>said</u> in a statement would represent "a major step forward from current treatments which apply genetic therapies to cells taken outside the body" for reinfusion.

One approach to addressing the latent reservoir, according to the organizations, is to identify the location of the reservoir in HIV-infected cells that still harbor HIV genomes after ART and to target those DNA sequences with gene editing.

The Gates Foundation and the National Institute of Allergy and Infectious Diseases (NIAID), a part of the NIH, are both currently exploring gene-based treatments for HIV, together with other therapies, and they noted that this collaboration will allow them to intensify their research, coordinate on their efforts, and accelerate studies into early clinical trials to test promising interventions.

"This collaboration is an ambitious step forward, harnessing the most cutting-edge scientific tools and NIH's sizable global HIV research infrastructure to one day deliver a cure and end the global HIV pandemic," said NIAID director Anthony S.

Fauci, MD, in a statement announcing the initiative. "We are taking into account those with the greatest need at the foundation of this effort, to ensure that, if realized, this exceptional public health achievement will be made accessible to all."

Trevor Mundel, MD, PhD, president of the global health program at the Gates Foundation, added that although gene therapies in recent years have led to groundbreaking treatments for patients with rare genetic disorders and infectious diseases, people in low-resource settings have not had access to these innovations. "By working with the NIH and scientists across Africa, we aim to ensure these approaches will improve the lives of those most in need and bring the incredible promise of gene-based treatments to the world of public health," said Mundel.