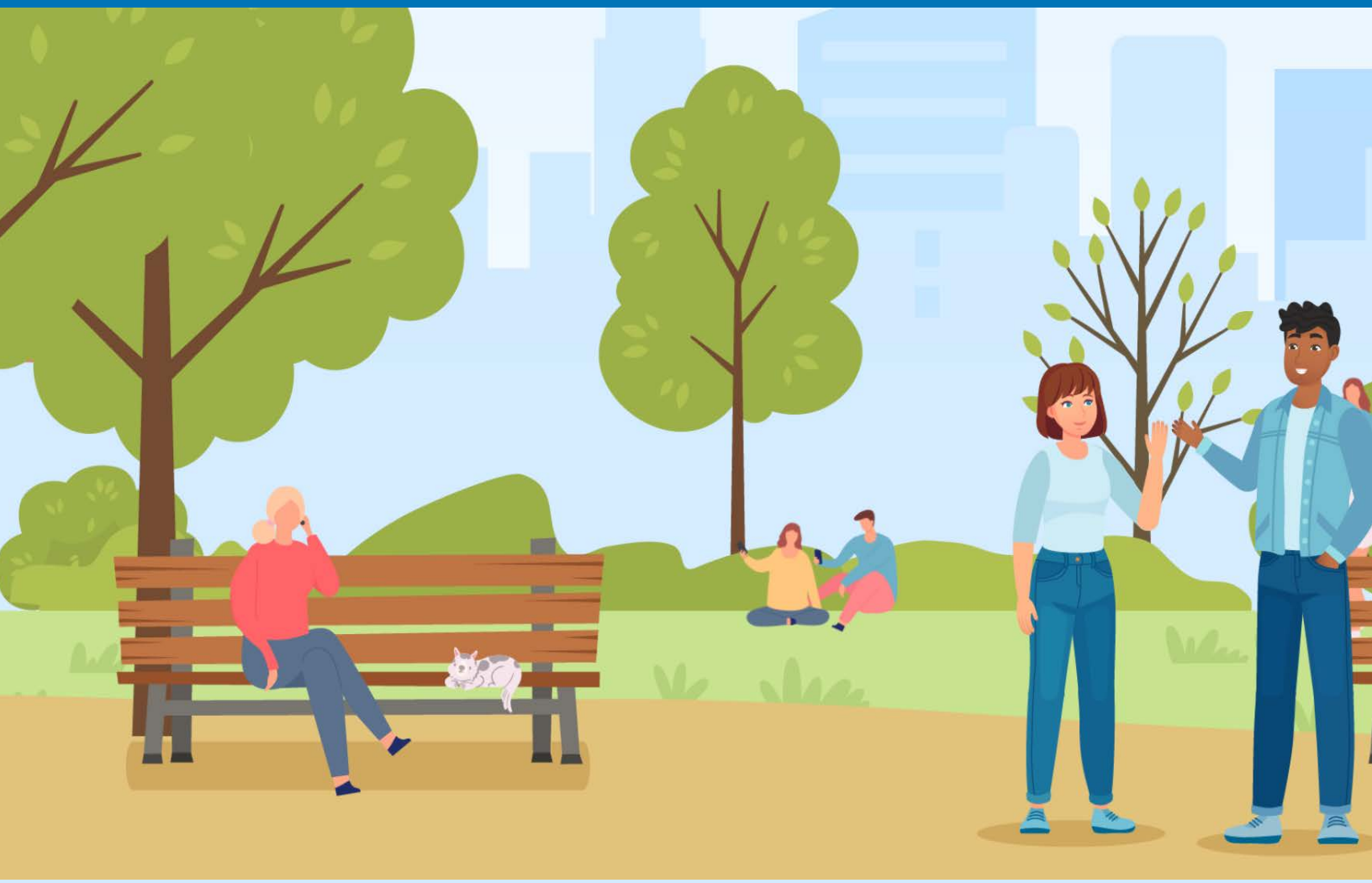
Gene Therapy Clinical Trial

For Hemophilia A



This brochure describes an investigational second-generation gene therapy cleared exclusively for clinical trials in the U.S. by the FDA, Food and Drug Administration

ASC Therapeutics

The Challenge

Hemophilia A is the most common form of hemophilia; this rare genetic disorder is caused by defects in a gene that is essential for the production of a coagulation protein called factor VIII. Lack of factor VIII leads to uncontrolled bleeding.

Current therapies replacing the missing coagulation factor are not a long-term fix, and in fact, people with hemophilia must receive these treatments during their entire lives, requiring regular therapeutic interventions, often burdensome, painful, expensive and time-consuming.

First-generation gene therapies for hemophilia have been investigated in the past few years on hundreds of patients; one of these therapies for Hemophilia A is under review by the U.S Food and Drug Administration and conditionally approved for clinical use in the European Union.

The Solution

Gene therapy, a one-and-done infusion, can incorporate a corrected version of the defective gene into the patient's liver, which becomes a biofactory that produces the missing coagulation factor, potentially for life.

Once the corrective gene is transported into the liver cell, the process of producing functional factor VIII takes place in multiple compartments of the cell. Maintaining a healthy lifestyle is critical to ensure that people with hemophilia can both produce and secrete factor VIII from the liver biofactory in the long run.

ASC Therapeutics has developed a second-generation gene therapy for hemophilia A that, in extensive preclinical studies, showed a significant increase in the secretion of factor VIII from the liver into the bloodstream, potentially increasing durability and requiring a lower dose of factor VIII production.

Gene therapy is a promising therapy that brings new hope to people with hemophilia A. Patients, together with their families and doctors, will potentially be able to live a normal life without all the burdens of current hemophilia therapies.

Speak to your doctor about participating in this clinical trial:

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www.asctherapeutics.com
www.clinicaltrials.gov/ct2/show/NCT04676048

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