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FDA Announces New Framework for Gene Therapies to Treat Rare Diseases

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Gene therapies have the potential to treat and cure even some of the most intractable of diseases, and the FDA recently released a new framework for the development, review, and approval of gene therapies.

FDA Commissioner Scott Gottlieb, MD, announced the framework, which is intended to be complementary to the comprehensive policy framework for regenerative medicine that was announced last year. Over the course of the past year, 3 different gene therapy products have been approved by the FDA, reflecting the advancements in the area.

"Today, we're taking a step toward shaping this modern structure for the regulation of gene therapy," Gottlieb said in a statement. "The agency is issuing a suite of [6] scientific guidance documents intended to serve as the building blocks of a modern, comprehensive framework for how we'll help advance the field of gene therapy while making sure new products meet the FDA's gold standard for safety and effectiveness."

The 3 new draft guidance documents are the following:

- Human gene therapy for hemophilia guidance, which will provide recommendations on the FDA's thinking on clinical trial design and preclinical considerations to support the development of gene therapy products for treating hemophilia.
- Human gene therapy for retinal disorders guidance, which will help those developing gene therapy products for a variety of retinal disorders affecting adult and pediatric patients by focusing on issues that are specific to retinal disorders and providing recommendations related to product development, preclinical testing, and clinical trial design.
- Human gene therapy for rare diseases guidance, which will include recommendations of preclinical, manufacturing, and clinical trial design for all phases of the clinical development program for rare disease gene therapies.

The FDA also made updates to 3 existing guidance documents that address manufacturing issues related to gene therapy:

- "Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs)" provides sponsors with recommendations on how to provide sufficient CMC information to assure safety, identity, quality, and strength of investigational gene therapy products.
- "Testing of Retroviral Vector-Based Gene Therapy Products for Replication Competent Retrovirus (RCR) during Product Manufacture and Patient Follow-up" provides recommendations for the proper testing for RCR during the manufacture of retroviral vector-based gene therapy products and followup monitoring of patients who received retroviral vector-based gene therapy products.

- “Long Term Follow-Up After Administration of Human Gene Therapy Products” provides recommendations for the design of long-term follow-up observational studies for collecting data on delayed adverse events following the administration of a gene therapy product.

When these updated guidance documents are finalized, they will replace previous versions issued by the FDA.

“Gene therapy represents one of the most promising opportunities for developing highly effective and even curative treatments for many vexing disorders,” Gottlieb concluded. “Some of these products are almost certainly going to change the contours of medical practice, and the destiny of patients with some debilitating diseases.”