SMG 1218A.2

FDA Staff Manual Guides, Volume I – Organizations and Functions

Department of Health and Human Services

Food and Drug Administration

Center for Biologics Evaluation and Research

Office of Therapeutic Products

Office of Gene Therapy CMC

Effective Date: September 16, 2022

1. Office of Gene Therapy CMC (DCBGF).

A. Plans and conducts research related to the development, manufacturing, and testing of cellular, gene therapy (including those utilizing naturally occurring viral vectors and those prepared by genetic engineering and synthetic procedures), and therapeutic vaccines to develop and maintain a scientific base for establishing standards for safety, purity, potency, and effectiveness.

B. Develops policy and procedures to ensure the continued safety of human cells, tissues, and cellular and tissue-based products (HCT/Ps) for risk of communicable diseases.

C. Develops policy and procedures governing the pre-market approval review and evaluation of gene therapy, therapeutic vaccines, and products in keeping with the provisions of the Public Health Service (PHS) Act applicable provisions of the Food Drugs & Cosmetics Act (FD&C) Act, Center policies and procedures.

D. Reviews, evaluates, and takes appropriate action on Investigational New Drug Applications (INDs), Investigational Device Exemptions (IDEs), Pre-Market Approvals (PMAs), Biologics License Applications (BLAs), New Drug Applications (NDAs), and other regulatory submissions related to therapeutic products and amendments or supplements to these applications.

E. Reviews, evaluates, and takes appropriate action on product applications submitted by manufacturers of cellular, gene therapy, therapeutic vaccines, plasma-derived, and coagulation products, and proposes written and reference standards for cellular, tissue, gene therapy, plasma-derived, and coagulation products.

F. Coordinates with the Office of Compliance and Biologics Quality and the Office of Biostatistics and Epidemiology, evaluates clinical experience and reports of adverse events as necessary.

G. Cooperates with other Center components, as appropriate, tests products submitted for release by manufacturers.

H. Participates in inspections of manufacturing facilities for compliance with applicable standards.

I. Administers applicable provisions of the FD&C Act as they pertain to certain devices and drugs that are under the jurisdiction of the Office and cooperates with other Food and Drug Administration components and outside organizations on issues related to these products.

J. Develops, organizes, and maintains quality assurance and quality control for reviews of INDs, IDEs, PMAs, NDAs, BLAs, and other regulatory activities for products regulated by the Office.

K. Develops, organizes, and maintains quality assurance and quality control for the conduction of research in support of standards development to assure the continued safety, purity, and potency of products regulated by the Office.

2. Authority and Effective Date.

The functional statements for the Office of Gene Therapy CMC were approved by the Secretary of Health and Human Services on August 8, 2022 and effective on September 16, 2022.

Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
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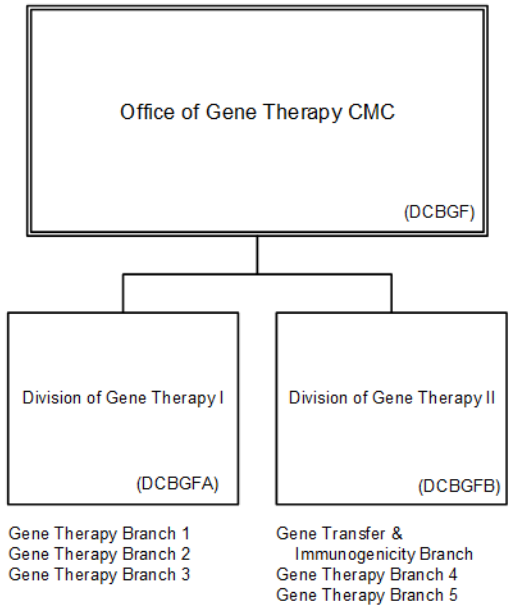
Office of Gene Therapy CMC



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The following is the Department of Health and Human Services, Food and Drug Administration, Center for Biologics Evaluation and Research, Office of Therapeutic Products, Office of Gene Therapy CMC organization structure depicting all the organizational structures reporting to the Director:

Office of Gene Therapy CMC (DCBFA):

- Division of Gene Therapy I (DCBGFA)
- Division of Gene Therapy II (DCBGFB)