

FDA Staff Manual Guides, Volume I - Organizations and Functions

Department of Health and Human Services

Food and Drug Administration

Center for Drug Evaluation and Research

Office of New Drugs

Office of Rare Diseases, Pediatrics, Urology & Reproductive Medicine

Division of Rare Diseases & Medical Genetics

Effective Date: September 25, 2019

1. Division of Rare Diseases & Medical Genetics (DCDGEE).

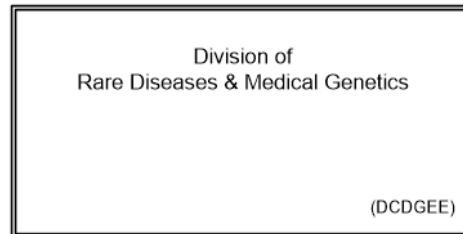
- A. Reviews Investigational New Drugs (INDs) and requests for claimed investigational exemption regulated by this Division and decides on appropriate regulatory action. Develops policies and procedures pertinent to particular aspects of investigation of drugs and biologics.
- B. Evaluates New Drug Applications (NDAs) and Biologics License Applications (BLAs) for safety and effectiveness and formulates decisions or recommendations regarding approvability in accord with applicable regulations/statutes. Also evaluates supplements that propose changes in the conditions upon which NDA/BLA approvals are based. Develops regulatory and scientific policies and procedures applicable to the review and evaluation of drugs and biologics for rare diseases and medical genetic drug products.
- C. Evaluates adequacy of information in proposed labeling for rare diseases and medical genetic drug products.
- D. Evaluates and takes appropriate action on recommendations concerning withdrawal of approval of NDAs and BLAs for rare diseases and medical genetic drug products.
- E. Provides advice and information to other components of the Center and to the Food and Drug Administration (FDA) on rare diseases and inborn errors of metabolism drug products with regard to medical and scientific issues, status of processing of drug and biologics applications, appropriate policy, and proposed regulatory actions.

- F. Utilizes the advisory committee process to obtain advice on product safety and effectiveness. Also participates in FDA sponsored consumer and professional educational programs on drug standards.
- G. Develops and disseminates guidances and policies related to rare disease drug development.
- H. Supports Office of New Drugs divisions in the review of rare disease product applications. Promotes consistency and innovation in the review of treatments for rare diseases, including organizing and providing rare disease education and training programs for internal and external audiences.
- I. Communicates with rare disease external stakeholders through formal and informal meetings to educate about FDA's policies and processes and to facilitate rare disease drug development.
- J. Administers the FDA European Medicines Agency Rare Disease Cluster which works to achieve a common understanding of each FDA's regulatory approaches to rare disease drug development as based on internal policies, guidance documents and regulations. Also provides a forum for discussion of candidate drugs and drug classes for the treatment of rare diseases including the scientific evaluation of these products.
- K. Coordinates rare disease regulatory programs and activities with the Center for Drug Evaluation and Research and other FDA stakeholder groups. Promotes the development of regulatory science for rare diseases including issue identification, data tracking and analysis, and advice generation.
- L. Performs medical and scientific evaluations of submissions on generic drugs, drugs under monograph, and Over-the-Counter drug products regulated by other offices in the Center, as applicable.

2. Authority and Effective Date.

The functional statements for the Division of Rare Diseases & Medical Genetics were approved by the Secretary of Health and Human Services on September 25, 2019.

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The following is the Department of Health and Human Services, Food and Drug Administration, Center for Drug Evaluation and Research, Office of New Drugs, Office of Rare Diseases, Pediatrics, Urology & Reproductive Medicine, Division of Rare Diseases & Medical Genetics organization structure depicting all the organizational structures reporting to the Director.

Division of Rare Diseases & Medical Genetics (DCDGEE).