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FDA Staff Manual Guides, Volume I – Organizations and Functions

Department of Health and Human Services

Food and Drug Administration

Office of the Commissioner

Office of the Chief Medical Officer

Office of Orphan Products Development

Effective Date: May 13, 2024

1. Office of Orphan Products Development (DCJC).

A. Manages extramural programs of clinical research and consortia to evaluate safety and effectiveness of orphan products by funding grants and contracts to promote product development for rare diseases and children as specified in the following statutory provisions:

   a. Orphan Drug Act, per Section 526 and 527 of the Food Drug and Cosmetic Act (FDCA) and federal regulations at 21 CFR 316;

   b. Rare Pediatric Disease Designation Program per Section 529 of FDCA; and

   c. Humanitarian Use Device Designation as required in 21 CFR 814.3(n).

B. Manages implementation of designated programs to promote product development for rare diseases via the listed programs below:

   a. Orphan Products Grants Program (OPGP);

   b. Pediatric Device Consortia Program (PDCP); and

   c. FDA Rare Neurodegenerative Disease Grant Program (FDA RNDGP).

C. Develops and communicates Food and Drug Administration (FDA) policy and makes decisions on approval of sponsor requests and incentives under the Federal FDCA, including orphan drug designations per section 526, orphan drug exclusivity per section 527, rare pediatric disease designation per

section 529, orphan drug grants and contracts to support clinical research, humanitarian devices, pediatric device consortia grants and other areas of FDA policy related to the development of products for rare disorders.

D. Represents the Commissioner or serves as one of FDA's principal authorities and spokespersons to governmental committees, industry, foreign regulatory bodies, professional, patient advocates and consumer associations requesting FDA participation in orphan product development activities.

2. Authority and Effective Date.

The functional statements for the Office of Orphan Products Development were approved by the Secretary of Health and Human Services on March 5, 2024 and effective on May 13, 2024.

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The following is the Department of Health and Human Services, Food and Drug Administration, Office of the Chief Medical Officer, Office of Orphan Products Development organization structure depicting all the organizational structures reporting to the Director: