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For Industry

Developing Products for Rare Diseases & Conditions

The FDA Office of Orphan Products Development (OOPD) mission is to advance the evaluation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. In fulfilling that task, OOPD evaluates scientific and clinical data submissions from sponsors to identify and designate products as promising for rare diseases and to further advance scientific development of such promising medical products. The office also works on rare disease issues with the medical and research communities, professional organizations, academia, governmental agencies, industry, and rare disease patient groups.

OOPD provides incentives for sponsors to develop products for rare diseases. The program has successfully enabled the development and marketing of more than 400 drugs and biologic products for rare diseases since 1983. In contrast, fewer than 10 such products supported by industry came to market between 1973 and 1983. The Orphan Grants Program has been used to bring more than 45 products to marketing approval. The Humanitarian Use Device Program has been the first step in approval of more than 50 Humanitarian Device Exemption approvals.

The [Orphan Drug Designation program](#)¹ provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected to recover the cost of developing and marketing a treatment drug.

The [Humanitarian Use Device \(HUD\) program](#)² designates a device that is intended to benefit patients by treating or diagnosing a disease or condition that affects fewer than 4,000 individuals in the United States per year as per 21 CFR 814.3(n).

The OOPD administers two extramural grant programs. The [Orphan Products Grants Program](#)³ provides funding for clinical research that tests the safety and efficacy of drugs, biologics, medical devices and medical foods in rare diseases or conditions. The [Pediatric Device Consortia \(PDC\) Grant Program](#)⁴ provides funding to develop nonprofit consortia to facilitate pediatric medical device development.

For more information on any of our programs, please visit the programs' web pages.

Please note our mailing address, telephone number, FAX number and email address:

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