ORPHAN DRUG ACT [1]

[As Amended Through P.L. 117–328, Enacted December 29, 2022]

[Currency: This publication is a compilation of the text of Public Law 97–414. It was last amended by the public law listed in the As Amended Through note above and below at the bottom of each page of the pdf version and reflects current law through the date of the enactment of the public law listed at https://www.govinfo.gov/app/collection/comps/]

[Note: While this publication does not represent an official version of any Federal statute, substantial efforts have been made to ensure the accuracy of its contents. The official version of Federal law is found in the United States Statutes at Large and in the United States Code. The legal effect to be given to the Statutes at Large and the United States Code is established by statute (1 U.S.C. 112, 204).]

GRANTS AND CONTRACTS FOR DEVELOPMENT OF DRUGS FOR RARE DISEASES AND CONDITIONS

SEC. 5. (a) [21 U.S.C. 360ee] The Secretary may make grants to and enter into contracts with public and private entities and individuals to assist in (1) defraying the costs of developing drugs for rare diseases or conditions, including qualified testing expenses, (2) defraying the costs of developing medical devices for rare diseases or conditions, (3) defraying the costs of developing medical foods for rare diseases or conditions, and (4) developing regulatory science pertaining to the chemistry, manufacturing, and controls of individualized medical products to treat individuals with rare diseases or conditions.

(b) For purposes of subsection (a):

(1) The term "qualified testing" means—

(A) human clinical testing—

(i) which is carried out under an exemption for a drug for a rare disease or condition under section 505(i) of the Federal Food, Drug, and Cosmetic Act (or regulations issued under such section); and

(ii) which occurs before the date on which an application with respect to such drug is submitted under section 505(b) of such Act or under section 351 of the Public Health Service Act;

(B) preclinical testing involving a drug for a rare disease or condition which occurs after the date such drug is designated under section 526 of such Act and before the date on which an application with respect to such drug is submitted under section 505(b) of such Act or under section 351 of the Public Health Service Act; and

(C) prospectively planned and designed observational studies and other analyses conducted to assist in the understanding of the natural history of a rare disease or con-

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dition and in the development of a therapy, including studies and analyses to—

(i) develop or validate a drug development tool related to a rare disease or condition; or

(ii) understand the full spectrum of the disease manifestations, including describing genotypic and phenotypic variability and identifying and defining distinct subpopulations affected by a rare disease or condition.

(2) The term "rare disease or condition" means (1)[2] in the case of a drug, any disease or condition which (A)[2] affects less than 200,000 persons in the United States, or (B)[2] affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drugs, (2)[2] in the case of a medical device, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical device for such disease or condition will be developed without assistance under subsection (a), and (3)[2] in the case of a medical food, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical food for such disease or condition will be developed without assistance under subsection (a). Determinations under the preceding sentence with respect to any drug shall be made on the basis of the facts and circumstances as of the date the request for designation of the drug under section 526 of the Federal Food, Drug, and Cosmetic Act is made.

(3) The term "medical food" means a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.

(c) AUTHORIZATION OF APPROPRIATIONS.—For grants and contracts under subsection (a), there is authorized to be appropriated $6,904,110 for the period beginning on October 1, 2022 and ending on December 23, 2022[3].

[2] So in law. See section 3 of Public Law 100–290 (102 Stat. 90). In subsection (b)(2) above, the organizational units (1), (2), and (3) probably should be (A), (B), and (C) (and in the unit (1), (A) and (B) probably should be (i) and (ii)).

[3] Section 3107(2) of division FF of Public Law 117-328 attempts to amend subsection (c) by striking "$6,328,767 for the period beginning on October 1, 2022, and ending on December 23, 2022" and inserting "$30,000,000 for each of fiscal years 2023 through 2027". This amendment does not execute due to a prior amendment made by section 307 of division C of Public Law 117-229.