SMG 1410.901

FDA STAFF MANUAL GUIDES, VOLUME II - DELEGATIONS OF AUTHORITY

REGULATORY - ORPHAN PRODUCTS

ORPHAN PRODUCTS

Effective Date: January 6, 2011

1. AUTHORITY DELEGATED AND TO WHOM DELEGATED.

A. Director, Office of Orphan Products Development (OOPD), Office of Special Medical Programs (OSMP), Office of the Commissioner (OC), is authorized to issue notices, and amendments thereto, inviting sponsorship for orphan products (human and animal drugs, biological products, and medical devices) and submission of:

1. requests for designation of drugs for rare diseases or conditions;

2. requests for Humanitarian Use Device designations for devices intended to treat or diagnose a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year;

3. requests for grants for drugs, devices, and medical foods for rare diseases or conditions; and

4. requests for grants to support the development of medical devices designed for pediatric use through a consortia which in turn supports pediatric device development.

B. The Director, OOPD, OSMP, OC, is authorized:

1. to determine whether there is reason to believe that a drug is for a disease or condition that is rare in the United States under Section 526 of the Federal Food, Drug, and Cosmetic Act (the Act) (21 U.S.C. 360bb) and to designate such drug as a drug for a rare disease or condition under Section 526(a) of the Act (21 U.S.C. 360bb(a));

2. to revoke orphan-drug designation for a drug for the reasons described in 21 CFR 316.29;

3. to notify a sponsor that an approved or licensed drug designated as a drug for a disease or condition that is rare in the United States under

Section 526(a) of the Act (21 U.S.C. 360bb(a)) has orphan-drug exclusive approval as described in 21 CFR 316.31;

4. to permit, under the conditions described in Section 527 of the Act (21 U.S.C. 360cc), during the seven-year period beginning on the date of the application approval or the issuance of the license, approval of another application under Section 505 of the Act or issuance of a license under Section 351 of the Public Health Service Act, for such drug for such disease or condition for a person who is not the holder of such approved application or of such license;

5. to encourage sponsors of an investigational new drug designated under Section 526(a) of the Act (21 U.S.C. 360bb(a)) for a rare disease or condition to design protocols for clinical investigations of the drug to permit the addition to the investigation of persons with the disease or condition, as described under Section 528 of the Act (21 U.S.C. 360dd);

6. to approve, return, or disapprove Humanitarian Use Device designations as described in 21 CFR 814.102; and

7. to revoke a Humanitarian Use Device designation for the reasons described in 21 CFR 814.102(c).

C. The following Officials are authorized to provide sponsors, under Section 525(a) of the Act (21 U.S.C. 360aa(a)), with recommendations for non-clinical or clinical investigations believed to be necessary for a drug for a rare disease or condition to be approved or licensed:

1. For drugs under the jurisdiction:

   a. Director and Deputy Directors, Center for Drug Evaluation and Research (CDER)

   b. Directors and Deputy Directors, Office of New Drugs (OND) and Office of Pharmaceutical Science (OPS), CDER

   c. Directors and Deputy Directors, Offices of Drug Evaluation (ODE) I, II, III, and IV; and Office of Antimicrobial Products (OAP) and Office of Oncology Drug Products (OODP), OND, CDER

   d. Directors and Deputy Directors of Divisions, ODE I, II, III, and IV; and OAP and OODP, OND, CDER

2. For biological products under their jurisdiction:

a. Director and Deputy Director, Center for Biologics Evaluation and Research (CBER)

b. Directors and Deputy Directors, Office of Blood Research and Review (OBRR), Office of Vaccines Research and Review (OVRR), Office of Cellular, Tissue and Gene Therapies (OCTGT), CBER

c. Directors and Deputy Directors, Divisions in OBRR, OVRR, and OCTGT, CBER

2. REDELEGATION.

These officials may not further redelegate these authorities.

3. EFFECTIVE DATE.

The Commissioner of Food and Drugs approved this delegation, via memorandum on January 6, 2011.

STATUS (I, R, C)	DATE APPROVED	LOCATION OF CHANGE HISTORY	CONTACT	APPROVING OFFICIAL
Initial	06/04/2010	N/a	OC/OSMP	Margaret A. Hamburg, M.D., Commissioner of Food and Drugs
Revision	01/06/2011	N/a	OC/OSMP	Margaret A. Hamburg, M.D., Commissioner of Food and Drugs