Prevention of Rare Diseases and Orphan Drug Act

Promulgated by Presidential Order Hua Tsung (1) Yi Tzu No. 8900031600 on February 9, 2000.
Deleted Article 12 and Article 37; and amended Article 1, Article 3, Article 5, Article 7 through Article 11, Article 13, Article 15, Article 19, Article 25, Article 32, Article 34 and Article 36, and promulgated by Presidential Order Hua Tsung (1) Yi Tzu No. 09400004921 on January 19, 2005.
Amended Article 6 and Article 33, and promulgated by Presidential Order Hua Tsung (1) Yi Tzu No. 09900331421 on December 8, 2010.
Deleted Article 5; and amended Article 2, Article 3, Article 4, Article 8, Article 10, Article 11, Article 13, Article 15-1, Article 17, Article 22, Article 26, Article 27-1, Article 33 and Article 34-1, and promulgated by Presidential Order Hua Tsung (1) Yi Tzu No. 10400002311 on January 14, 2015.

Article 1 This Act is enacted for the prevention of the occurrence of rare diseases; for the early diagnosis of rare diseases; for the intensive care of rare disease patients; for assisting patients in gaining access to specific drugs for the treatment of rare diseases and special nutritional foods essential for the maintenance of life; and for promoting and ensuring the supply, manufacturing, research and development of such drugs and foods.

For matters not regulated in this Act, regulations of relevant laws shall apply.

Article 2 The competent authorities mentioned in this Act: at central level is under Ministry of Health and Welfare; at municipality level is under municipal governments and at counties and cities level is under county and city governments.

Article 3 The term "rare diseases" as used in this Act shall refer to diseases with prevalence rate lower than the standard announced by the central competent authority; or diseases recognized through review by the Review Committee specified in Article 4 of this Act, and designated and publicly announced by the central competent authority under special circumstances.

The term orphan drugs as used in this Act shall refer to drugs with major indications for the prevention, diagnosis and treatment of rare diseases that have been submitted in accordance with this Act for application, recognized through review by the Review Committee specified in Article 4, and announced by the central competent authority.

The term "special nutritional foods essential for the maintenance of life" as used in this Act shall refer to foods that are primarily suited for providing nutrients to rare disease patients, and have been recognized through review by the Review Committee specified in Article 4 and announced by the central competent authority.

Article 4 The Review Committee for Rare Diseases and Orphan Drugs (hereafter referred to as the Review Committee) is charged with the following duties:

1.Reviewing the designation of rare diseases, and counseling on their prevention and control;

2.Reviewing the designation of orphan drugs and special nutritional foods essential for the maintenance of life;

3.Reviewing the registration and market approval of orphan drugs;

4.Reviewing the subsidies and research and development of orphan drugs and special nutritional foods essential for the maintenance of life;

5.Reviewing and providing assistance and counseling on international medical cooperation projects for rare diseases;

6.Reviewing non-orphan drugs for the treatment of specific diseases;

7.Providing counseling on other matters related to rare diseases.

The Review Committee shall be called by the central competent authority and members shall consist of representatives of government organizations, medical scholars and experts and impartial citizens; at least half of the members shall be medical scholars and experts with experience in providing clinical treatment to or care for patients with rare diseases or those conducting rare diseases research; each gender shall constitute no less than one third of the committee members.

For the implementation of the matters stipulated in the first paragraph of this article, the Review Committee shall consult advice from other scholars with relevant expertise, industry representatives or advocates of patients with rare diseases.

Article 5 (Deleted)

Article 6 The central competent authority shall perform tasks related to the prevention, control and research of rare diseases.

Article 7 Medical personnel, upon detection of patients who have contracted a rare disease or victims who have died as a result of a rare disease, shall report this matter to the central competent authority.

Article 8 Upon receiving a report as specified in the preceding Article, or on detecting a patient with anomalies associated with rare genetic diseases, the central competent authority shall, with the consent of the patient or the patients legal guardian, dispatch specialized personnel to meet with the patient and advise on the impact of the disease and provide psychological support, maternity attentiveness, care consultation services,

etc. to the patient and their families.

The regulations governing the content, implementation methods and other compliance matters of the services specified in the preceding paragraph shall be stipulated by the central competent authority.

Article 9 Organizations, institutions, groups and the personnel of the said bodies who carry out the tasks specified in the preceding two Articles shall be attentive to their attitude and method of execution, respect the will and dignity of the patients, and safeguard patients' privacy and normal social functioning.

The personnel referred to in the preceding Paragraph may not disclose or hand over confidential information learned or obtained in the course of duties without cause.

Article 10 The central competent authority shall encourage and reward medical care institutions at all levels, research institutions and groups related to rare diseases to carry out prevention of rare diseases and provide funding for manpower development, research and facilities.

The items, scope and amount of incentives and funding specified in the preceding paragraph shall be stipulated by the central competent authority; such stipulation may be adopted by the municipal and county (city) competent authorities.

Article 11 The competent authorities shall organize educational and public awareness activities on rare diseases; said activities shall be conducted with the assistance of organizations, schools, civic groups and mass media.

When rare disease patients encounter problems in school enrollment, employment or life support, the competent authority shall coordinate with relevant organizations (institutions) for assistance.

Article 12 (Deleted)

Article 13 Rare disease patients or their legal guardians may apply to the central competent authority by submitting an application form, together with certificates, care plans and relevant documents issued by a medical care or research institution specified in Article 10; after review and approval by the Review Committee, the central competent authority may provide subsidies for patients to travel overseas and participate in international medical cooperation projects.

When the medical cooperation project referred to in the preceding Paragraph involves laboratory testing, the application for subsidies may be made by a medical care or research institution specified in Article 10.

The application procedures, required documents, and other matters to be complied with for subsidies mentioned in the preceding two Paragraphs shall be stipulated by the central competent authority.

Article 14 Unless otherwise regulated in this Act, orphan drugs shall not be manufactured or imported without the registration and market approval, and drug permits issued, by the central competent authority.

Article 15 When drugs have primary indications for the prevention, diagnosis and treatment of rare diseases, applications may be submitted for registration and market approval as orphan drugs.

Required documents, review procedures and criteria to be used in processing applications for registration and market approval as stated in the preceding Paragraph shall be determined by the central competent authority.

Article 15-1 After the orphan drug has been approved or the request for its specific permission to import has been approved by the central competent authority, the Review Committee shall be consulted in the process of examining an application for listing an orphan drug in the National Health Insurance Pharmaceutical Benefits and Reimbursement Schedule.

Article 16 In processing applications for the registration and market approval of orphan drugs, the central competent authority may, where necessary, require the applicant to conduct domestic clinical trials. The contents of the clinical trial application process and results shall be adequately made public.

Article 17 Drug permits issued for orphan drugs that have received registration and market approval in accordance with this Act will have ten years validity period. During this period, the central competent authority shall not accept applications for registration and market approval of pharmaceuticals of the same kind.

If the manufacturing or importation of the orphan drugs referred to in the preceding Paragraph needs to be continued after the permit expires in ten years, application for extension must be made in advance to the central competent authority. Each extension period shall not exceed five years. During the extension period, applications for registration and market approval of pharmaceuticals of the same kind may be made to the central competent authority.

When orphan drugs have been issued permit licenses in accordance with this Act, and central competent authority proclaims that such drugs shall no longer be designated as orphan drugs, extensions for these licenses will

be subjected to the requirements stipulated in the Pharmaceutical Affairs Act.

The holders of a permit obtained under the provisions of Paragraph 1, unless otherwise due to force majeure circumstances, shall continuously offer orphan drugs within the valid period; if decide to terminate the manufacturing or importation of orphan drugs within the valid period, they shall notify the central competent authority in writing, six months prior to the termination

Article 18 Under any of the following conditions, the central competent authority may, irrespective of the restrictions stipulated in Paragraph 1 of the preceding Article, accept applications for registration and market approval of other pharmaceuticals of the same kind and issue permits:

1. A new applicant has obtained authorized consent from the holder of rights to an orphan drug that has received registration and market approval;
2. An orphan drug for which a new application is being submitted has the same indications and is of similar quality to an orphan drug that has already been approved, and is safer or more efficacious than the approved drug;
3. The holder of an orphan drug permit is unable to meet the demand for said drug;
4. The price of an orphan drug is determined by the central competent authority to be unreasonable.

When registration and market approval is received and permits are granted in accordance with Subparagraph 2 through Subparagraph 4 of the preceding Paragraph, the said permits shall be subjected to the provisions of the preceding Article.

Article 19 When orphan drugs have not yet received registration and market approval, or fall under the conditions specified in Subparagraph 3 or Subparagraph 4 of Paragraph 1 of the preceding Article, government organizations, medical care institutions, rare disease patients and their families, relevant foundations, societies and associations may apply to the central competent authority for a permit on an ad hoc basis. However, said permit may not be used for profit-making ventures.

The central competent authority may, where necessary, commission or designate relevant institutions or groups to process the ad hoc applications referred to in the preceding Paragraph.

Required documents, review procedures and other items to be complied

with in applying for the ad hoc application process referred to in the preceding two Paragraphs shall be determined by the central competent authority.

Article 20 When orphan drugs are determined to be hazardous to human health, or are suspected of being hazardous to human health, the central competent authority may order pharmaceutical firms or ad hoc applicants to recall said drugs within a specified deadline. Where necessary, the permits for said drugs may be revoked.

Article 21 The central competent authority shall compile annual reports for orphan drugs that have received market approval or for which permits have been issued through ad hoc application in accordance with this Act. Said reports shall detail the amounts used, number of users, adverse reactions, and any other relevant information.

Pharmaceutical firms and ad hoc applicants shall provide relevant information required in compiling the annual reports stipulated in the preceding Paragraph.

Article 22 When there are difficulties in manufacturing or importing non-orphan drugs in accordance with the provisions of the Pharmaceutical Affairs Act, and said drugs are reviewed by the Review Committee and determined to be beneficial for the treatment of specific diseases, provisions in this Act regarding registration, market approval and ad hoc application may be applied.

Article 23 The central competent authority shall make periodic announcement on the recognition, permission, revocation and annulment of rare diseases and orphan drugs.

Article 24 When applications are submitted for registration and market approval, clinical trials, issuing or extension of permits, or ad hoc application, fees shall be levied for review, registration or licensing; the fee schedules shall be determined by the central competent authority.

Article 25 The central competent authority may provide incentives to promote the supply, manufacturing or research and development of orphan drugs or special nutritional foods essential for the maintenance of life; measures regarding incentive targets, methods and items to be complied with by incentive recipients shall be determined by the central competent authority.

Article 26 Those who manufacture or import orphan drugs for which permits have not been issued, or who sell, supply, dispense, transport, store,

hold, brokering, transfer, or display for sale the orphan drugs with the knowledge that permits have not been issued for said drugs, shall be punished in accordance with the provisions of Article 82 and Article 83 of the Pharmaceutical Affairs Act.

Article 27 A fine of NT$ 30,000 to NT$ 150,000 shall be imposed for violation of Article 16; for major violations, pharmaceutical firms shall be prohibited from applying for registration and market approval for said pharmaceutical for two years, and the operations of medical care institutions may be suspended for a period of one month to one year.

Article 27-1 The central competent authority may impose a fine of no less than NT$ 100,000 and no more than NT$ 500,000 on those who terminates offering orphan drugs or fails to notify the central competent authority in writing six months prior to termination violating paragraph 4 of Article 17; if necessary, the drug permit license issued to the orphan drug may be revoked.

Article 28 When false documentation is submitted during application for orphan drug registration and market approval or permit extension, a fine of NT$ 20,000 to NT$ 100,000 shall be imposed, and the offender shall be prohibited from applying for registration and market approval for said pharmaceutical for two years; where a permit has already been obtained, said permit shall be revoked; cases where criminal liability is involved it shall be turned over to the judiciary for prosecution.

Article 29 When an orphan drug approved through ad hoc application is used for a profit-making venture in violation of Paragraph 1 of Article 19, a fine of NT$ 30,000 to NT$ 150,000 shall be imposed; any profits thus earned shall be confiscated, and the offender shall be prohibited from submitting an ad hoc application for an orphan drug for two years.

Article 30 When an orphan drug is not recalled within the deadline set by the central competent authority as stipulated in Article 20, a fine of NT$ 30,000 to NT$ 150,000 shall be imposed, with consecutive penalties to be levied until recall is made.

Article 31 When a pharmaceutical firm violates the provisions of Paragraph 2 of Article 21, a fine of NT$ 10,000 to NT$ 50,000 shall be imposed; when the offender is an ad hoc applicant, the central competent authority may deny further orphan drug permit applications made by said offender.

Article 32 The fines set forth in this Act shall be levied by the competent authorities.

The fines referred in the preceding Paragraph shall be paid within a specified time limit. Cases where fines are not paid within said time limit

shall be turned over to the courts for law enforcement.

Article 33 The central competent authority shall subsidize costs for prevention, screening and research on rare diseases; and allocate budgets to subsidize costs for orphan disease diagnosis, treatment, pharmaceuticals, supportive and palliative care and special nutritional foods essential for the maintenance of life not covered by the National Health Insurance Act. Measures regarding subsidization methods, details and other relevant items shall be determined by the central competent authority.

Subsidies specified in the preceding paragraph will be funded by the Tobacco Health and Welfare Surcharge, or donations from organizations and groups.

Article 34 Medical care institutions may apply for permission to import special nutritional foods essential for the maintenance of life for rare disease patients on ad hoc basis; required documents, application review procedures and other items to be complied with shall be determined by the central competent authority.

Article 34-1 The central competent authority shall assist each medical hospital and rare disease patients in emergent access to special nutritional foods essential for the maintenance of life and specific drugs for the treatment of rare diseases.

Article 35 The enforcement rules for this Act shall be determined by the central competent authority.

Article 36 This Act shall come into force six months from the date of announcement.

Amendments to this Act shall come into force from the date of announcement.

Article 37 (Deleted)