FIFTEENTH CONGRESS OF THE)
REPUBLIC OF THE PHILIPPINES)
First Regular Session)

SENATE

S.B. No. 2042

INTRODUCED BY HONORABLE EDGARDO J. ANGARA

EXPLANATORY NOTE

This Bill seeks to establish a system that will help ensure the early diagnosis and treatment of rare diseases in the Philippines through the establishment of an Office of Rare Disease in the Department of Health. The Office of Rare Disease will supervise the implementation of a research program on rare diseases, ensure the provision of early and sustainable care for patients suffering from rare diseases, and conduct public educational and information campaigns on rare diseases. The Bill also seeks to provide fiscal and regulatory incentives to developers, manufacturers, and importers of medicines, medical devices, diagnostic kits, and other pharmaceutical and nutritional products needed by patients suffering from rare diseases.

A "rare disease", otherwise called an "orphan disorder" is any health condition resulting from genetic defects that afflict no more than 1 of every 20,000 individuals in the country. Examples are Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phynelketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome, and the so-called *Lubag*, which is found only in the island of Panay. In the Philippines, it is estimated that less than 1000 individuals suffer from a rare disease. Many of these cases are due to genetic defects, with many patients exhibiting the symptoms during early childhood. Unfortunately, there is minimal interest among research institutions in learning more about these diseases because they affect only a small segment of the population. Moreover, drugs and healthcare products for these disorders have been called "orphan drugs" or "orphan products" because of minimal attention paid by pharmaceutical companies to them due to the high cost of production resulting from the absence of commercially significant demand. Thus, patients die during infancy or, if they manage to survive, suffer from chronic, debilitating illnesses that greatly diminish their quality of life, not to mention impose severe strain on their families.

This Bill will help provide patients with rare diseases better access to adequate medical care, health information, and healthcare products needed to treat their condition. It establishes a system to coordinate a sustainable research & development initiatives and resource generation efforts among relevant agencies of government and the private sector toward improving the quality of life of patients with rare diseases and their families.

Hence, we seek the early passage of this Bill.

EDGARDO J. ANGARA
Senator

FIFTEENTH CONGRESS OF THE)
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S. B. No. 2042

Introduced by SENATOR EDGARDO J. ANGARA

AN ACT TO HELP PERSONS AFFLICTED WITH RARE DISEASES BY CREATING AN OFFICE OF RARE DISEASES IN THE DEPARTMENT OF HEALTH, ENCOURAGING THE CONDUCT OF RESEARCH & DEVELOPMENT ACTIVITIES ON RARE DISEASES, AND PROVIDING FOR FISCAL AND REGULATORY INCENTIVES FOR THE MANUFACTURE OR IMPORTATION OF HEALTHCARE PRODUCTS FOR USE BY SUCH PERSONS.

Be it enacted by the Senate and the House of Representatives of the Philippines in Congress assembled:

ARTICLE 1
GENERAL PROVISIONS

Section 1. ***Short Title*** – This Act shall be known as the "Rare Diseases Act of the Philippines".

Section 2. ***Declaration of Policy***. - It is the policy of the State to protect and promote the right to health of the people, including the right of persons suffering from rare diseases, to have access to timely health information and adequate medical care. In pursuit of such policy, the State recognizes the leading role of the Department of Health in overseeing research & development activities on rare diseases and, while working with the private sector and nongovernmental organizations, in designing and implementing programs for the benefit of those afflicted with them. The State further recognizes that an effective public education program is vital in helping ensure the early diagnosis and treatment of rare diseases and in preventing those afflicted with them from being the subject of discrimination.

Section 3. ***Objectives*** - The objectives of this Act are as follows:

1) Ensure that every patient diagnosed to have a rare disease has access to timely health information and adequate medical care, including drugs and other healthcare products to treat or otherwise help them cope with their condition;

2) Establish an office within the Department of Health: (i) to oversee research & development activities on rare diseases; (ii) to design and maintain a rare disease registry which shall include data on rare diseases in the Philippines, patients afflicted with rare diseases, and orphan drugs and products; and (iii) to conduct public education programs to identify persons afflicted with rare diseases and help the public understand the special needs of such persons.
3) Provide regulatory and fiscal incentives to support research and development activities on rare diseases and the import or manufacture affordable orphan drugs or orphan products; and
4) Institutionalize a financial incentive system for agencies involved in clinical researches, patient care, medical information management, and other similar activities for the benefit of persons afflicted with a rare disease.

ARTICLE 2
DEFINITION OF TERMS

Section 4. ***Definitions.*** - Under this Act, the following terms shall have the meanings respectively given to them below:

1) *Healthcare practitioner* means any doctor of medicine, dentist, nurse, midwife, allied health professionals and other health care professionals duly licensed by the Professional Regulatory Commission.
2) *Healthcare institution* means hospitals, health infirmaries, health centers, lying-in centers or puericulture centers, whether public or private.
3) *Medical care* means any method used by a health care practitioner to prevent, diagnose, and remove the symptoms and cause of a disease.
4) *Orphan Drug* means any drug or medicine used to treat or alleviate the symptoms of persons afflicted with a rare disease and declared as such by the Department of Health upon recommendation of the National Institutes of Health.
5) *Orphan Product* means any healthcare or nutritional product, other than a drug or medicine, including, but not limited to, diagnostic kits, medical devices and biological products, used to prevent, diagnose, or treat rare diseases and declared as such by the Department of Health upon recommendation of the National Institutes of Health.
6) *Rare Disease* means Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phynelketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome, *Lubag,* and other diseases affecting no more than one (1) in twenty thousand (20,000) individuals in the country and recognized as such by the Department of Health upon recommendation of the National Institutes of Health. For the avoidance of doubt, it does not include catastrophic (*i.e.*, life

threatening, seriously debilitating, or serious and chronic) forms of more frequently occurring diseases.

7) *Rare Disease Registry* means the health information system maintained by the Department of Health, including the electronic database system, relating to data on rare diseases, persons afflicted with rare diseases, and orphan drugs and orphan products.

ARTICLE 3
DESIGNATION OF RARE DISEASE, ORPHAN DRUG, AND ORPHAN PRODUCT STATUS

Section 5. ***Designation of Rare Disease.*** - Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phynelketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome and *Lubag* are hereby designated as rare diseases. The Department of Health, upon recommendation of the National Institutes of Health, shall have the authority to designate any disease that afflicts no more than one (1) in twenty thousand (20,000) persons in the Philippines as a rare disease.

Section 6. ***Designation of Orphan Drug.*** – The Department of Health, *motu proprio* or upon application by any interested person, may designate any drug or medicine indicated for use by patients afflicted with any of the rare diseases as an orphan drug; *Provided,* that there is no existing drug or medicine in the Philippines that can provide the same or superior alternative therapy, as certified by the National Institutes of Health.

The drugs or medicines for the treatment or for the alleviation of symptoms of Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phynelketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome, *Lubag*, and other rare diseases designated as such by the Department of Health are hereby deemed to be orphan drugs. Within one hundred twenty (120) days from the effectivity of this Act, the Department of Health, in consultation with the National Institutes of Health, shall publish a list of orphan drugs for these rare diseases.

Section 7. ***Designation of Orphan Product.*** - The Department of Health, *motu proprio* or upon application by any interested person, may designate any healthcare or nutritional product, other than a drug or medicine, including, but not limited to, diagnostic kits, medical devices and biological products, used primarily to prevent, diagnose, or alleviate the symptoms of rare diseases as an orphan product; *Provided,* That there is no existing product in the Philippines that can provide the same or superior results, as certified by the National Institutes of Health.

Any healthcare or nutritional product, other than a drug or medicine, including, but not limited to, diagnostic kits, medical devices and biological products, used primarily to prevent,

diagnose, or alleviate the symptoms of Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phynelketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome, *Lubag*, and other rare diseases designated as such by the Department of Health, are hereby considered as orphan products. Within one hundred twenty days from the effectivity of this Act, the Department of Health, in consultation with the National Institutes of Health, shall publish a list of orphan products for these rare diseases.

Section 8. ***Permit for Restricted Use of an Orphan Drug/Orphan Product.*** – Any person may import any orphan drug or orphan product without need of obtaining a Certificate of Product Registration; *Provided,* that he first secures a Permit for Use of an Orphan Drug/Orphan Product from the Bureau of Food and Drugs. Within thirty (30) days from receipt of the following requirements, the Bureau of Food and Drugs shall issue a Permit for Use of an Orphan Drug/Orphan Product:

(1) Sworn application for the issuance of a Permit for Restricted Use of an Orphan Drug/Orphan Product, containing the name and address of the applicant and the estimated annual volume requirement of the drug or product;

(2) Certification from the Office of Rare Diseases that the drug or product qualifies as an orphan drug or orphan product;

(3) In the case of a drug or medicine, medical device and diagnostic kit: (i) the names and addresses of medical specialists qualified and authorized to use them; (ii) a written commitment on the part of all the authorized specialists to submit to the Bureau of Food and Drugs, with copies to the National Institutes of Health and the Office of Rare Disorders, no later than January 15 of each year, a Clinical Study Report for every patient administered the drug or product describing the quantity administered or used, the therapeutic or desired effect, and adverse reactions, if any;

(4) Certification that the drug or product is registered in the country of origin; and

(5) An Affidavit stating that the applicant shall be responsible for any death, injury or damage arising from the use of the orphan drug or orphan product and holding the Bureau of Food and Drugs and its officials and employees free and harmless therefrom.

The Permit for Use of an Orphan Drug/Orphan Product shall be effective for a period of three years, renewable for periods of three years thereafter.

Section 9. ***Rare Disease Registry.*** - The National Institutes of Health, with the assistance of the Office of Rare Diseases, shall maintain a national database of rare diseases, patients afflicted with rare diseases, orphan drugs and orphan products which, except for the names of the patients afflicted with rare diseases, shall be made available to any reputable research institution

working on rare diseases; *Provided,* That such access shall be subject to guidelines issued by the National Institutes of Health to protect the privacy of patients afflicted with rare diseases.

(b) All healthcare practitioners and health institutions shall be required to report to the National Institutes of Health newly-diagnosed cases of rare diseases and provide reports on the status of patients; *Provided,* That such reports shall be subject to guidelines issued by the National Institutes of Health to protect the privacy of patients afflicted with rare diseases.

(c) Health practitioners and health institutions shall inform patients afflicted with rare diseases of relevant orphan drugs and orphan products in the Rare Disease Registry.

ARTICLE 4
OFFICE OF RARE DISEASES

Section 10. ***Creation of Office of Rare Diseases.*** Within one hundred twenty (120) days from the effectivity of this Act, the Department of Health shall create an Office of Rare Diseases to: (i) to oversee research & development activities on rare diseases; (ii) to design and maintain a rare disease registry which shall include data on rare diseases in the Philippines, patients afflicted with rare diseases, and orphan drugs and products; and (iii) to conduct public education programs to identify persons afflicted with rare diseases and help the public understand the special needs of such persons.

In particular, it shall perform *inter alia* the following specific functions:

(1) Recommend agenda for the conduct and support of research and development initiatives on rare diseases, orphan drugs and orphan products to identify relevant research opportunities;

(2) Promote coordination and cooperation among institutions involved in researches on rare diseases, orphan drugs and orphan products;

(3) Promote sufficient allocation of resources for the conduct of research on rare disease, orphan drugs and orphan products;

(4) Enter into agreements with and award research grants for centers for researches doing studies on rare diseases, orphan drugs and products;

(5) Prepare annual reports describing the research and development activities supported by the Office, and identifying projects that should be conducted in the future on rare disease, orphan drugs and orphan products;

(6) Evaluate the activities of the National Institutes of Health in the management of information system and ensure that the Rare Diseases Registry is current and useful;

(7) Ensure appropriate coordination among interested agencies, manufacturers, and organizations representing patients afflicted with rare diseases;

(8) Inform health practitioners and the public regarding the availability of the orphan drugs and orphan products;

(9) Promote the development of updated treatment guidelines on the approach to patients suspected or diagnosed with rare diseases;

(10) Establish a referral system to facilitate the provision of appropriate medical care to patients with rare diseases

(11) Liaise with national and international patient, health and scientific organizations working on rare diseases, orphan drugs and orphan products to improve patient access.

The Office of Rare Diseases shall be comprised of an Assistant Secretary of the Department of Health, who shall act as Chairman, and one representative each from the Bureau of Food and Drugs, the National Institutes Health, a national patient support group, and a private healthcare company, as members.

ARTICLE 5
INCENTIVES FOR RARE DISEASE FUNDING AND RESOURCE GENERATON

Section 11. ***Fiscal Incentives.*** – The following shall be exempted from all taxes, whether national or local:

(1) Donations to the Office of Rare Diseases and the National Institutes of Health intended for researches on rare diseases, maintenance of the Rare Disease Registry, or for purchase of orphan drugs or orphan products for use solely by patients with rare diseases; and

(2) Procurement of orphan drugs and orphan products for use solely by patients with rare diseases, as certified by the National Institutes of Health;

In addition, orphan drugs and orphan products for donation solely to patients afflicted with rare diseases or institutions, as certified by the National Institutes of Health, shall be exempt from payments of all tariffs and duties.

ARTICLE 6
FINAL PROVISIONS

Section 12. ***Implementing Rules and Regulations.*** – Within one hundred twenty (120) days from effectivity of this Act, the Department of Health, in consultation with the National Institutes of Health, shall issue the implementing rules and regulations to this Act.

Section 13. ***Repealing Clause.*** - All general and special laws, decrees, executive orders, proclamations and administrative regulations, or any parts thereof, which are inconsistent with this Act are hereby repealed or modified accordingly.

Section 14. ***Separability Clause.*** - If, for any reason or reasons, any part of provisions of this Act shall be declared or held to be unconstitutional or invalid, other provision or provisions hereof which are not affected thereby shall continue to be in full force and effect.

Section 15. ***Effectivity.*** This Act shall take effect fifteen (15) days after its publication in at least two (2) newspapers of general circulation.

Approved,