16th CONGRESS OF THE REPUBLIC)
OF THE PHILIPPINES)
Second Regular Session)

SENATE

S. B. NO. 2383

Introduced by Senator Sonny M. Angara

AN ACT PROMULGATING A COMPREHENSIVE POLICY IN ADDRESSING THE NEEDS OF PERSONS AFFLICTED BY RARE DISORDERS

Explanatory Note

A "rare disease" is any health condition resulting from genetic defects that rarely affect the general population. There are 6,000-8,000 rare diseases, also known as "orphan disorders" majority of which are genetic in origin and manifest at birth or early in childhood.[1][2] Rare diseases are often chronic, progressive, degenerative, and life-threatening.

With many rare diseases, a patient's quality of life is often compromised by the lack or loss of autonomy and high level pain, inflicting emotional strife as well on both the patient and their families.

These diseases afflict a small number of people, as the Global Genes Project estimated that only 4 percent of the world population has a rare disease. With such a small population, pharmaceutical companies and other medical companies find it difficult to achieve the economies of scale that justify any targeted research or mass production of "orphan treatments or medicines." Such a situation makes treatment, often life-long, costly and far beyond the reach of most Filipino patients.

However, every Filipino should be provided access to proper healthcare, as enshrined in Section 15 Article 2 of the 1987 Constitution which emphasizes that "[t]he State shall protect and promote the right to health of the people and instill health consciousness among them". Section 11 Article 13 further provides that "[t]he State shall adopt an integrated and comprehensive approach to health development which shall endeavor to make essential goods, health and other services available to all people at affordable cost."

Furthermore, the United Nations Convention on the Rights of the Child, which the Philippines ratified on July 26, 1990, requires States Parties to "recognize the right of the child to the enjoyment of the highest attainable standard of health" (Art. 24[1]) and to "ensure the provision of necessary medical assistance and health care to all children" (Art. 24[2b]).

Simply, it should be national policy that the state endeavors toward universal healthcare, even for rare diseases.

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[1] EURORDIS-RARE DISEASES EUROPE : http//www.eurordis org/about rare diseases

[2] National Organization for Rare Disorders (US National Institutes of Health): https://www.rarediseases.org/rare-disease-information/rare-diseases

Lately, facets of a true universal healthcare system were put in place—including mandatory health insurance coverage, assured long-term health financing through higher excise taxes on alcohol and tobacco products, and a nationwide health research system.

The foregoing measure aims to build on these foundations, seeking to create a comprehensive and sustainable health system for rare diseases integrated into the existing public health care system. This will ensure the provision of early and sustainable care for patients suffering from rare diseases; relevant and well-coordinated research and development initiatives across the appropriate government agencies and private sector proponents; and integration of the health care activities for informational program on rare diseases for the general public and health care practitioners. As a result, patients with rare diseases and their families will be afforded better access to adequate medical care, health information, and healthcare products needed to treat their condition.

In recognition of our constitutional and international obligations to improve the health conditions of the people, the immediate enactment of this bill is earnestly sought.

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

ARTICLE 1

GENERAL PROVISIONS

SECTION 1. 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 survival and full and healthy development as individuals through access to timely health information and adequate medical care. In pursuit of such policy, the State shall institutionalize a system that is comprehensive, integrative and sustainable and will facilitate collaboration among government and non-government agencies and organizations at the national and local levels, private sector, professional health organizations, academic institutions, communities and families towards the provision of early and sustainable care of every person afflicted with rare disorders. The state recognizes the crucial role of research in defining health programs and activities to address the needs of patients with rare disorders. The State also 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 ridicule and stigmatization. The State further recognizes the leading role of the Department of Health in implementing the Rare Disease Program, overseeing the provision of care, and working with the other government agencies, the private sector and non-governmental organizations, in designing and implementing programs, including research & development activities *on rare diseases, for the benefit of those afflicted with them.*

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

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

2) Establish a comprehensive and sustainable health care system integrated within the public health care delivery system that will ensure the provision of early and sustainable care for patients suffering from rare diseases;

3) Design and maintain the Rare Disease Registry which shall include data on rare diseases in the Philippines, patients afflicted with rare diseases, and orphan drugs and products. This data shall be utilized in formulating policies, identifying program interventions and designing researches that will eventually address the needs of patients with rare disease;

4) Integrate public educational and informational campaigns in the current programs of the DOH to identify persons afflicted with rare disease and help the public understand the special needs of such persons;

5) Facilitate the regular collaborative activities among stakeholders regarding the realization of the objectives of this Bill;

6) Provide regulatory and fiscal incentives to support research and development activities on rare diseases and the manufacturing of affordable orphan drugs or orphan products or the importation of these; and

7) 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 practitioners 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 institutions 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) *Medical Food* are food products specifically formulated for the dietary management of specific inherited metabolic disorders. These are prescribed by physicians with special training in the care of patients with rare disorders to provide the necessary nutrition and treatment that is essential for the survival of these patients.

5)National Comprehensive Newborn Screening System, as established by R.A. 9288,is the existing network of medical specialists, nurses, laboratories and hospitals screening and treating these genetic diseases, many of which are also rare disorders.

6)Newborn Screening Continuity Clinic refers to an ambulatory clinic based in a tertiary hospital identified by the DOH to be part of the National Comprehensive Newborn Screening System Treatment Network. It is equipped to facilitate continuity of care of patients confirmed with conditions included in the expanded newborn screening in its area of coverage.

7) NIH means the National Institutes of Health.

8) 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.

9) *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.

10) Rare Disease means disorders such as Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phenylketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome and other diseases with similar rare occurrence as recognized 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.

11) Rare Disease Registry means the secure health information system, including the electronic database system, relating to data on rare diseases, persons afflicted with rare diseases, and orphan drugs and orphan product.

12) *RDTWG* means Rare Diseases Technical Working Group, a DOH designated pool of experts on rare diseases tasked with identifying rare diseases, drugs and products.

13) Telegenetics Referral System refers to a computer network system that provides remote genetic clinical consultations to physicians in the provinces for their patients

ARTICLE 3

RARE DISORDERS

Section5. Obligation of healthcare practitioners - Health care practitioners who attend to a person with rare disorders are obligated to the following:

1) To give the patient and their family substantial information about the significance of diagnosis and management and the rights of the affected individual to benefits of persons with disability;

2) To ensure that the afflicted person is referred to Newborn Screening Continuity

Clinics identified by the Department of Health (DOH) as referral centers for treating rare diseases; and

Section 6.Designation of persons with rare diseases as persons with disabilities – Individuals with rare disease shall be included among those with disabilities and enjoy the same rights under the Magna Carta for disabled persons as mandated in Republic Act 9442.

1) The Department of Labor and Employment shall ensure that abled persons with rare disease are given opportunity for work and employment to become productive members of the society.

2) The Department of Social Welfare and Development shall provide assistance to person with rare disease to ensure that their social welfare and benefits are provided as mandated in the Magna Carta for disabled persons

ARTICLE 4

IDENTIFICATION, REFERRAL AND MANAGEMENT OF PERSON WITH RARE DISORDERS

Section 7. Continuing Education and Training of Health personnel –The Department of Health and the National Institutes of Health in collaboration with health professional societies and academic health institutions shall:

1) Conduct continuing education, information and training programs for health personnel on the identification and referral of persons with rare disorders for medical management; and

2) Educate health personnel on the importance of reporting of any cases of orphan disorders for the Rare Disease Registry

Section 8. Public Information about rare disorders -The Department of Health with the assistance of the NIH and other government agencies, professional societies and non-government organizations shall conduct culturally sensitive public educational and information campaigns on the nature of rare diseases, identify persons afflicted with rare disease and help the general public understand the special needs of the afflicted persons and be spared for being the subject of ridicule and discrimination.

Section 9. Referral of patients with rare disease– Patients highly suspected of or diagnosed with rare disease shall be referred to a Newborn Screening Continuity Clinic identified by the DOH as referral centers for treatment of rare diseases under the National Comprehensive Newborn Screening System.

1) Timely referral ensures that the afflicted person receives the adequate care of his/her condition and referral of the person afflicted and her/his families to a metabolic specialist, geneticist or genetic counselor for genetic counseling.

2) In the absence of a medical specialist in the area, the Newborn Screening Continuity Clinic shall coordinate with the National Institutes of Health (NIH) for co-management of the patient with a specialist.

3) Establish a system in collaboration with local government unit and agencies that will ensure that the afflicted person receives sustainable medical management of the disease.

4) Reports the case to NIH for its inclusion to the Rare Disease Registry.

Section 10. Availability of specialist for the management of afflicted person with rare disorders - The DOH with the assistance of NIH shall ensure the availability of a medical specialist for the management of persons afflicted with rare diseases

ARTICLE 5

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

Section 11. The Rare Disease Technical Working Group - The Department of Health shall convene the Rare Diseases Technical Working Group (RDTWG) which shall have the following roles and responsibilities:

1) Designate diseases that are 'rare diseases'

2) Designate orphan drugs and products corresponding to the rare diseases

3) Formulate policies that shall regulate the approval and certification of orphan drugs and products

4) Establish a system to ensure the regular updating of information, diagnosis and treatment of rare disorders in order to provide for the comprehensive health care of these patients.

Section 12. Designation of Rare Disease - The Department of Health, upon recommendation of the NIH and RDTWG, shall have the authority to designate any disease that is recognized to rarely afflict the population of the country.

Section 13. Designation of Orphan Drug – The Department of Health, *motuproprio* 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. Within one hundred twenty days from the effectivity of this Act, the DOH shall publish a list of orphan drugs for these rare diseases.

Section14. Designation of Orphan Product - The Department of Health, *motuproprio* 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 Food and Drug Administration. 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 are hereby considered as orphan products. Within one hundred twenty days from the effectivity of this Act,

the Department of Health shall publish a list of orphan products for these rare diseases.

Section 15. 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 Food and Drug Administration (FDA) within thirty days from receipt of requirements issued by the FDA.

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 16. The Rare Disease Registry – All patients diagnosed with a rare disease shall be included in this national database for rare disease case registries.

1) All healthcare practitioners and health institutions shall be required to report to the Rare Disease Registry of the National Institutes of Health diagnosed cases of rare diseases and provide reports on status of patients; p*rovided,* 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.

2) 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 6

IMPLEMENTATION

Section 17. Lead Agency - The Department of Health shall be the lead agency in the implementation of this Act. For the purposes of achieving the objectives of this act, the DOH shall:

1) Establish the Technical Working group for the Rare Diseases;

2) Develop the implementing rules and regulation for the implementation of this Bill within one hundred eighty (180) days from the enactment of the Law;

3) Coordinate with the National Institutes of Health for the technical assistance in the implementation of the Act;

4) Coordinate with all government and non-government agencies that will be involved in the implementation of the Act;

5) Designate referral centers in strategic location in the country for the timely and sustainable medical management of persons afflicted with rare disorders;

6) Organize a pool of medical specialists who will be responsible in the diagnosis and management of persons afflicted with rare disorders and their families; and

7) Allot budget for the implementation of the law.

Section 18. Other implementing agencies - The Food and Drug Administration, NIH, Department of Interior and Local Government (DILG), Department of Education (DepEd), Department of Social and Welfare Development (DSWD), Department of Labor and Employment (DOLE) and the Department of Science and Technology (DOST) shall each perform the mandated task in this Bill.

1) Food and Drug Administration shall ensure that medical foods, orphan drugs and products are permitted in the country for the purposes of treating rare diseases;

2) NIH shall provide the technical assistance to the DOH in the implementation of this Act.

3) DILG, DepEd, DSWD and DOLE shall ensure that persons with rare diseases are given the opportunity to be productive members of the society and that they are given the same rights and benefits as persons with disability.

4) DOST shall provide mechanisms to further research for a better understanding of rare diseases in the country. DOST shall develop low cost medical foods and products for the patients.

ARTICLE 7

RESOURCE GENERATON AND INCENTIVES FOR RARE DISEASES FUNDING

Section 19. Source of funds for maintaining medical management of persons afflicted with rare diseases - The Department of Health shall ensure the establishment of a system that will facilitate the qualification of afflicted person as one of the beneficiaries of the services for sustainable compliance to the medical management of the rare disease:

1) The Philippine Health Insurance Corporation shall include the cost of treatment of rare disease in the benefit package.

2) Provisions from the Sin Taxes collection shall be directed to cover the cost of care for patients with rare diseases

Section 20. Fiscal Incentives – The following shall be exempted from all taxes, whether national or local:

1) Donations to the 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) Orphan Drugs and Orphan Products for use solely by patients with rare diseases, as certified by the Food and Drug Administration.

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 8

FINAL PROVISIONS

Section 21. Implementing Rules and Regulations – Within one hundred twenty 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 22. 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 23. Separability - 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 24. Effectivity - This Act shall take effect fifteen (15) days after its publication in at least two (2) newspapers of general circulation.

Approved,