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

Senate
Office of the Secretary

'14 JUN 10 P3 :46

SENATE

S.B. No. 2279

Introduced by SENATOR CYNTHIA A. VILLAR

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

EXPLANATORY NOTE

Section 15, Article II of the 1987 Philippine Constitution states that:

> "The State shall protect and promote the right to health of the people and instill health consciousness among them."

Additionally, the Republic of the Philippines, being a party to the Convention on the Rights of the Child (CRC), committed itself to "recognize the right of the child to the enjoyment of the highest attainable standard of health" (Article 24 [1], CRC) and to take appropriate measures to "ensure the provision of necessary medical assistance and health care to all children" (Article 24 [2b], CRC).

In furtherance of the foregoing, this Bill seeks to provide for the creation of a comprehensive and sustainable health system for rare diseases integrated into existing public health care system. The rationale for establishing a national health care system for rare disorders as part of the country's healthcare delivery system is further expressed in Section 11, Article XIII of the 1987 Philippines Constitution, *to wit*:

> "The 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."

Briefly, a "rare disease", otherwise called an "orphan disorder", is any health condition resulting from genetic defects that rarely affect the general population, which are often chronic, progressive, degenerative, and life-threatening. Currently, there are about 6,000-8,000 identified rare diseases, 75% of which affect children. Of this percentage, 30% of them die before they reach the age of five (5) years.

In the Philippines alone, these rare diseases affect 1 in every 20,000 Filipino children. Although these diseases inflict a small number of individuals, treatment is usually life term and costly, making it beyond the reach of most Filipino patients. Furthermore, the quality of life of patients is often compromised by the lack or loss of autonomy, high level of pain and suffering for the patient and their family.[1]

The passage of this Bill will help provide patients with rare diseases, and their families, better access to adequate medical care, health information, and healthcare products needed to treat their condition. This will ensure the provision of early and sustainable care for patients suffering from rare diseases, relevant researches on rare diseases, and integration of the health care activities for informational program on rare diseases for the general public and health care practitioners.

Hence, in recognition of our constitutional and international commitment to improve the health of the people, the early passage of this Bill is recommended.

CYNTHIA A. VILLAR

[1] "Rare Diseases", Philippine Society for Orphan Disorders, http://www.psod.org.ph/rare-diseases/.

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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 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 addressing 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 and 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 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:

    i) 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.

    ii) 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.

iii) 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.

iv) Facilitate the regular collaborative activities among stakeholders regarding the realization of the objectives of this Bill.

2. 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;

3. 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) *DOH* means the Department of Health.

2) *DOLE* means the Department of Labor and Employment.

3) *DSWD* means the Department of Social and Welfare Development.

4) *FDA* means the Food and Drug Administration.

5) *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.

6) *Healthcare institutions* means hospitals, health infirmaries, health centers, lying-in centers or puericulture centers, whether public or private.

7) *Medical care* means any method used by a health care practitioner to prevent, diagnose, and remove the symptoms and cause of a disease.

8) *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.

9) *Newborn Screening Follow-up Clinics (NSFC)* are regional medical centers recognized by the DOH for having the expertise and capability for follow-up care of newborn's screened with metabolic, genetic and rare disorders.

10) *NIH* means the National Institutes of Health.

11) *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.

12) *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.

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

14) *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, Prader-Willi 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.

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

16) *Telegenetics* Referral System is an established system utilizing electronic communications (i.e. video conferencing, emails, among others) which aims to make genetics services accessible to all patients with genetic conditions.

ARTICLE 3
RARE DISORDERS

Section 5. *Obligation of healthcare practitioners* - Any 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.

2) To ensure that afflicted person is referred to a Regional Newborn Screening Follow-Up Clinic Centers identified by the DOH as referral centers for treating rare diseases.

3) To report the case for entry into the Rare Disease Registry.

Section 6. *Referral of patients with rare disease*– Patients suspected or diagnosed with rare disease shall be referred to a Regional Newborn Screening Follow-Up Clinic Centers identified by the DOH as referral centers for treatment of rare diseases.

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 geneticist or genetic counselor for genetic counseling.

2) In the absence of a specialist in the area, the referral center must coordinate with NIH through the DOH Centers for Health Development for co-management of the patient with a specialist.

3) Establish a system in collaboration with local 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 7. ***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 and Welfare Development shall provide assistance to person with rare disease to ensure that their social welfare and benefits as mandated in the Magna Carta for disabled persons.

ARTICLE 4
IDENTIFICATION, REFERRAL AND MANAGEMENT OF PERSON WITH RARE DISORDERS

Section 8. ***Continuing Education, and Training of Health personnel*** –The Department of Health and the National Institutes of Health together 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.

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

Section 9. ***Public Information about rare disorders*** **-** The Department of Health with the assistance of the National Institutes of Health 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 10. ***Referral of person afflicted with rare disease*** - All patients suspected or diagnosed to have a rare disease should be referred to the DOH designated newborn screening follow-up clinics for follow up and management. All persons afflicted with a rare disorder must be seen and managed by a specialist regularly. In the absence of a specialist in the designated newborn screening follow-up clinics, designated persons should coordinate with NIH through the CHD for co-management with a specialist.

Section 11. ***Availability of specialist for the management of afflicted person with rare disorders*** - The Department of Health with the assistance of NIH should ensure the availability of a specialist for the management of persons afflicted with rare diseases

ARTICLE 5
DESIGNATION OF RARE DISEASE, ORPHAN DRUG, AND ORPHAN PRODUCT STATUS

Section 12. ***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; and

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

Section 13. ***Designation of Rare Disease.*** **-** The Department of Health, upon recommendation of the National Institutes of Health and RDTWG, shall have the authority to designate any disease that is recognized to rarely afflict the population of the country.

Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phenylketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome andPrader-Willi Syndrome are hereby designated as rare diseases.

Additional diseases should be approved by the DOH as recommended by the NIH and RDTWG.

Section 14. ***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.

The drugs or medicines for the treatment or for the alleviation of symptoms of Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phenylketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome and Prader-Willi Syndromeare hereby deemed to be orphan drugs.

Within one hundred twenty (120) days from the effectivity of this Act, the Department of Health shall publish a list of orphan drugs for these rare diseases.

Section15. ***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 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 Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phenylketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome, Prader-Willi Syndrome are hereby considered as orphan products.

Within one hundred twenty (120) days from the effectivity of this Act, the Department of Health shall publish a list of orphan products for these rare diseases.

Section 16. ***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 within thirty days from receipt of the following requirements, shall issue a Permit for Use of an Orphan Drug/Orphan Product:

1) A 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 Department of Health 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 Food and Drug Administration with copies to the Department of Health 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 Food and Drug Administration 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 17. ***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; *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.

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 18. ***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 management of persons afflicted with rare disorders and their families;

7) Allot budget for the implementation of the law.

Section 19. ***Other implementing agencies*** - The Food and Drug administration, NIH, Department of Social and Welfare Development and Department of Labor and Employment shall each perform the mandated task in this Bill.

1) The Food and Drug Administration shall ensure that orphan drugs and products are permitted in the country for the purposes of treating rare diseases.

2) The NIH shall serve provide the technical assistance to the DOH in implementing this Bill.

3) The 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.

ARTICLE 7
RESOURCE GENERATON AND INCENTIVES FOR RARE DISEASES FUNDING

Section 20. ***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 as part of its Catastrophic Illness Resource Fund.

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

Section 21. ***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 22. ***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 23. ***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 24. ***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 25. ***Effectivity*** - This Act shall take effect fifteen (15) days after its publication in at least two (2) newspapers of general circulation.

Approved,