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

Senate
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SENATE

S.B. NO 2098

RECEIVED BY:

INTRODUCED BY SENATOR PIA S. CAYETANO

AN ACT PROMULGATING A COMPREHENSIVE POLICY IN ADDRESSING THE NEEDS OF PERSONS WITH RARE DISEASE

EXPLANATORY NOTE

A "rare disease", otherwise called an "orphan disorder", is any health condition resulting from genetic defects that rarely affect the general population. There are 6,000 to 8,000 rare diseases, majority are genetic in origin and manifest at birth or early in childhood. Rare diseases are often chronic, progressive, degenerative, and life-threatening. 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. 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.

The right of the person to be provided proper health care finds anchor in the 1987 Constitution. In particular, Section 15 of Article 2, states that "The State shall protect and promote the right to health of the people and instill health consciousness among them". Furthermore, the United Nations Convention on the Rights of the Child, which the Philippines ratified on July 26, 1990, requires State 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]).

This bill provides for the creation of a comprehensive and sustainable health system for rare diseases integrated into existing public health care system. This will ensure the provision of early and sustainable care for patients suffering from rare disease, 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. 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. It establishes a system to coordinate 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.

The rationale for establishing a national health care system for rare disorders as part of the country's healthcare delivery system finds further justification and expression in Section 11 of Article 13 of the Constitution: "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."

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

PIA S. CAYETANO
Senator

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ARTICLE 1
GENERAL PROVISIONS

SECTION 1. ***Short Title.***– This Act shall be known as the "Rare Disease Act of 2014."

SEC. 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 disease, 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 disease. The State recognizes the crucial role of research in defining health programs and activities addressing the needs of patients with rare disease. 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 (DOH) 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.

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

1. Endeavor 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 endeavor to provide early and sustainable care for patients suffering from rare disease;

    ii) Design and maintain the Rare Disease Registry which shall include data on rare disease in the Philippines, patients afflicted with rare disease, 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; and

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

2. Provide regulatory and fiscal incentives to support research and development activities on rare disease and the import or manufacture of affordable orphan drugs or orphan products; and

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 rare disease.

ARTICLE 2
DEFINITION OF TERMS

SEC. 4. *Definitions.* – For the purpose of this Act, the following terms shall be defined as follows:

1) *Commercial use* means the selling of orphan drugs, imported from sources abroad, at a profit;

2) *Healthcare practitioner* means any doctor of medicine, dentist, nurse, midwife, allied health professionals or other health care professionals duly licensed by the Professional Regulatory Commission;

3) *Healthcare institution* means hospitals, health infirmaries, health centers, lying-in centers or puericulture centers, whether public or private;

4) *Medical care* means any method used by a health care practitioner to inform, brief, prevent, diagnose, manage, or remove the symptoms and cause of a disease;

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 disease;

6) Newborn Screening Center (NSC) means a facility equipped with a newborn screening laboratory that complies with the standards established by the National Institutes of Health (NIH) and provides all required laboratory tests and recall/follow-up programs for newborns with metabolic, genetic, or rare disease;

7) *Orphan Drug* means any drug or medicine used to treat or alleviate the symptoms of persons afflicted with a rare disease and designated as such by the Food and Drug Administration (FDA) upon recommendation of the National Institutes of Health (NIH);

8) *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 designated as such by the FDA upon recommendation of the NIH;

9) *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 DOH upon recommendation of the NIH but excluding catastrophic (i.e. life threatening, seriously debilitating, or serious and chronic) forms of more frequently occurring diseases;

10) *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;

11) *Rare Disease Technical Working Group (RDTWG)* means a DOH-designated pool of experts on rare disease tasked with identifying rare disease, orphan drugs, and orphan products;

12) *Telegenetics Referral System* is an established system utilizing electronic communications, including but not limited to video conferencing and emails, which aims to make genetics services accessible to all patients with genetic conditions;

ARTICLE 3
IDENTIFICATION, REFERRAL, AND MANAGEMENT OF PERSONS WITH RARE DISEASE

SEC. 5. *Identification of Persons with Rare Disease.* – The DOH, in coordination with the NIH, shall create a Rare Disease Registry. It shall endeavor to comply with set global standards, if applicable. All patients diagnosed with rare disease shall be included in this registry.

All healthcare practitioners and health institutions shall be required to report to the Rare Disease Registry of the DOH and NIH diagnosed cases of rare disease and provide reports on the status of patients; *Provided,* that such reports shall be subject to guidelines issued by the NIH to protect the privacy of patients afflicted with rare disease.

SEC. 6. *Referral of Patients with Rare Disease.* – Patients suspected or diagnosed with rare disease shall be referred to Regional Newborn Screening Centers *(NSC)* identified by the DOH as referral centers for medical care of rare disease. This ensures that the afflicted person receives the adequate care for his/her condition and is referred to a healthcare practitioner specializing on rare disease. In the absence of a specialist in the area, the referral center must coordinate with NIH through the DOH Centers for Health Development (CHD) for co-management of the patient with a specialist.

There shall also be a system established in collaboration with local units and agencies that will endeavor to provide the afflicted person with sustainable medical care of the disease and have his/her case reported to NIH for its inclusion to the Rare Disease Registry.

SEC. 7. *Management of Persons with Rare Disease.* -The DOH, with the assistance of NIH, should ensure the proper management of persons with rare disease

through the creation of a Rare Disease Management Program under the National Center for Disease Prevention and Control of the DOH.

ARTICLE 4
PERSONS WITH RARE DISEASE AS PERSONS WITH DISABILITIES (PWDs)

SEC. 8. ***Designation of Persons with Rare Disease as Persons with Disabilities (PWDs).*** – Individuals with rare disease shall be considered as persons with disabilities (PWDs), in accordance with Republic Act No. 7277, as amended, or the Magna Carta for Disabled Persons.

SEC. 9. ***Rights and Privileges of Persons with Rare Disease.*** – The appropriate national government agency shall ensure that they accorded the same rights and privileges as PWDs, to wit:

1) The Department of Social and Welfare Development (DSWD) shall provide assistance to persons with rare disease to ensure that their social welfare and benefits as mandated under Republic Act No. 7277, as amended, or the Magna Carta for Disabled Persons, are granted.

2) The Department of Labor and Employment (DOLE) shall ensure that able-persons with rare disease are given the opportunity for work and employment to become productive members of the society.

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

SEC. 10. ***The Rare Disease Technical Working Group (RDTWG).*** - The DOH shall convene the RDTWG which shall have the following roles and responsibilities:

1) Designate rare disease and update the list periodically;

2) Designate and periodically update orphan drugs and products corresponding to the rare disease; and

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

SEC. 11. ***Designation of Rare Disease.*** - The DOH, 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. Gaucher Disease, Maple Syrup Urine Disease, Pompe Disease, Galactosemia, Phenylketonuria, Methylmalonic Acidemia, Urea Cycle Defects, Hurler Syndrome, Hunter Syndrome and Prader-Willi Syndrome are hereby designated as rare disease. Additional diseases should be approved by the DOH, as recommended by the NIH and RDTWG.

SEC. 12. ***Designation of Orphan Drug.*** – The FDA, *motu proprio* or upon application by any interested person, may designate any drug or medicine indicated for use by patients afflicted with a rare disease as an orphan drug; *Provided,* that no existing drug or medicine in the Philippines that can yield the same or superior results shall be designated as an orphan drug. Within one hundred twenty (120) days from the effectivity of this Act, the FDA shall publish a list of orphan drugs for rare disease and shall periodically update the said list.

SEC. 13. ***Designation of Orphan Product.*** - The FDA, *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 disease as an orphan product; *Provided,* that no existing product in the Philippines that can yield the same or superior results shall be designated as an orphan product. Within one hundred twenty (120) days from the effectivity of this Act, the FDA shall publish a list of orphan products for rare disease and shall periodically update the said list.

SEC. 14. *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 the said importation shall not be for commercial use; *Provided, further,* that a Permit for Use of an Orphan Drug/Orphan Product shall be secured from the FDA. The Permit for Restricted Use of an Orphan Drug/Orphan Product shall be issued by the FDA if the applicant meets the following requirements:

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 FDA that the drug or product qualifies as an orphan drug or orphan product;

3) In the case of a drug or medicine, medical device, or 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 FDA with copies to the DOH no later than January 15 of each year, a Clinical Study Report for every patient administered with 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 hold the FDA and its officials and employees free and harmless from any death, injury, or damage arising from the use of the orphan drug or orphan product.

Within thirty (30) days from receipt of the following requirements, the FDA shall issue a Permit for Restricted Use of an Orphan Drug/Orphan Product which shall be effective for a period of three (3) years, renewable for periods of three (3) years thereafter.

ARTICLE 6
IMPLEMENTATION

SEC. 15. *Lead Agency.* - The DOH 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 RDTWG;

2) Develop the implementing rules and regulation for the implementation of this Act within one hundred twenty (120) days from the enactment of the Law;

3) Coordinate with the NIH for the technical assistance in the implementation of this Act;

4) Coordinate with all government and non-government agencies or organizations involved in the implementation of this Act;

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

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

SEC. 16. *Other Implementing Agencies.* - The FDA, NIH, DSWD, DOLE, and other relevant government agencies shall perform the following tasks:

1) The FDA shall ensure that orphan drugs and products are permitted in the country for the purpose of treating rare disease;

2) The NIH shall serve provide the technical assistance to the DOH in implementing this Act;

3) The DSWD and DOLE shall ensure that persons with rare disease are given the opportunity to be productive members of the society and that they are given the same rights and benefits as PWDs; and

4) All other relevant government agencies shall assist in the full implementation of this Act.

SEC. 17. *Obligation of Healthcare Practitioners.* - Any health care practitioner who attends to a person with rare disease is obligated to the following:

1) To give the patient and his family substantial information about the significance of diagnosis and management or refer them to a healthcare practitioner specializing on rare disease;

2) To ensure that the afflicted person is referred to a Regional NSC identified by the DOH as referral centers for treating rare disease;

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

4) To inform the patient afflicted with rare disease of relevant orphan drugs and orphan products.

SEC. 18. *Continuing Education and Training of Health Personnel.* –The DOH and the NIH, 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 disease for medical management; and

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

ARTICLE 7
RESOURCE GENERATON AND FISCAL INCENTIVES

SEC. 19. *Source of Funds for Maintaining Medical Management of Persons with Rare Disease.* - The DOH shall ensure the establishment of a system that will

facilitate the qualification of an afflicted person as one of the beneficiaries of the services for sustainable compliance to the medical management of the rare disease:

1. To the extent actuarially possible, the treatment of rare disease shall be included in the benefit package, to be provided in the guidelines set by the Philippine Health Insurance Corporation (PHIC).

2. Incremental Revenues from the Excise Tax on Alcohol and Tobacco Products as provided in Republic Act No. 10351 shall include medical assistance to patients with rare disease, to be provided in the guidelines set by DOH.

SEC. 20. *Fiscal Incentives.* – The following shall be exempted from all taxes and customs duties, as applicable, whether national or local:

1) Donations intended for researches on rare disease, maintenance of the Rare Disease Registry, or for purchase of orphan drugs or orphan products for use solely by patients with rare disease; and

2) Orphan Drugs and Orphan Products for use solely by patients with rare disease, as certified by the FDA.

ARTICLE 8
FINAL PROVISIONS

SEC. 21. *Implementing Rules and Regulations.* – Within one hundred twenty days (120) from effectivity of this Act, the DOH, in consultation with the NIH, shall issue the implementing rules and regulations to this Act.

SEC. 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.

SEC. 23. *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.

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

Approved,