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115TH CONGRESS
2D SESSION

H. R. 6801

To establish a Rare Disease Therapeutics Corporation to encourage the development of high-risk, high-return therapies for rare diseases, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

SEPTEMBER 13, 2018

Mr. VARGAS (for himself, Mr. THOMAS J. ROONEY of Florida, and Mr. PETERS) introduced the following bill; which was referred to the Committee on Energy and Commerce

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A BILL

To establish a Rare Disease Therapeutics Corporation to encourage the development of high-risk, high-return therapies for rare diseases, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Rare Disease Fund Act of 2018'' or the ''RaD Fund Act of 2018''.

SEC. 2. FINDINGS.

The Congress finds the following:

(1) That biomedicine is far more advanced today than even a decade ago is indisputable, but

breakthroughs require years of translational research at a cost of hundreds of millions of dollars per trial and have a substantial likelihood of failure.

(2) The drug development pipeline is laden with unfavorable probabilities. On average, for every 5,000–10,000 compounds that enter the drug discovery pipeline, just 250 progress to preclinical development—and only one will become an approved drug.

(3) Biotech and life sciences traditional financing vehicles of private and public equity are becoming less effective funding sources because the needs and expectations of limited partners and shareholders are not consistent with the increasing complexity, risk, and duration of biomedical innovation.

(4) Industry professionals frequently refer to the ''Valley of Death''—a steadily widening funding and resource gap that currently exists between basic research and clinical development, effectively limiting the field of potential novel therapies, technologies, and treatments for patients.

(5) The life sciences industry needs novel approaches to early-stage drug development that better manage risk, lower capital cost, improve research ef-

fectiveness, create diverse portfolios, leverage risk-tolerant capital, and access new capital sources.

(6) One solution is to implement a financial structure in which a large number of biomedical programs are funded by a single entity to substantially diversify the portfolio and thereby reduce risk. The entity can use securitization to finance its activities by issuing debt, which opens up a much larger pool of capital for investment.

(7) This approach involves two components:

(A) Creating large diversified portfolios, called ''megafunds'', consisting of biomedical products at various stages of development.

(B) Structuring the financing for these portfolios as combinations of equity and securitized debt.

(8) This innovation makes the investment opportunity much more attractive to a large pool of institutional investors that have historically not participated in financing for early-stage therapeutic development.

(9) Diversification reduces risk, so that an entity can issue debt and equity, rather than the equity-only investments typically made by venture capital.

(10) A series of peer-reviewed simulations conducted by researchers at MIT suggested that a modest megafund model could be successfully implemented for rare diseases (e.g., rare genetic disorders, pediatric cancers, and orphan diseases) with as few as ten to twenty compounds and only $400 million in capital.

(11) A rare disease therapeutics fund could serve as a viable pilot project, while minimizing governmental exposure.

(12) In addition to appealing to traditional biotech VC investors, megafund investments may be attractive to pension funds, insurance companies, and other large institutional investors, while also potentially lowering drug prices for patients and the healthcare system.

(13) The Food and Drug Administration (FDA) may grant the orphan designation for therapies being studied for a rare disease or condition affecting fewer than 200,000 people in the United States, which reduces costs and provides financial incentives to encourage development of such therapies for underserved patient populations.

SEC. 3. RARE DISEASE THERAPEUTICS CORPORATION.

(a) ESTABLISHMENT.—

(1) IN GENERAL.—The Secretary of the Treasury shall organize under the laws of a State a corporation to be known as the ''Rare Disease Therapeutics Corporation'' (hereinafter in this Act referred to as the ''Corporation'').

(2) QUALIFIED PORTFOLIO MANAGER.—As soon as practicable after organization, the Corporation shall hire a qualified portfolio manager whose mandate will be to acquire and manage a portfolio of biomedical research assets on behalf of the Corporation.

(b) PURPOSE.—The purpose of the Corporation shall be to purchase rights to, fund the development of, and, once developed, sell ownership interests in rare disease therapeutics.

(c) PRIVATIZATION OF THE CORPORATION.—

(1) IN GENERAL.—As soon as practicable after the establishment of the Corporation, but in no case later than 2 years after the date of enactment of this Act, the Secretary shall issue equity stock in the Corporation to private investors.

(2) TERMINATION OF GOVERNMENT OWNERSHIP.—Upon the issuance of the equity stock described under paragraph (1), the Government shall

no longer hold any ownership interest in the Corporation.

(3) PROHIBITION ON DIVIDENDS.—The Corporation may not pay dividends on the equity stock of the Corporation while there are any outstanding guaranteed bonds of the Corporation issued pursuant to subsection (e)(1)(A).

(d) SALE OF OWNERSHIP INTERESTS.—

(1) IN GENERAL.—The Corporation—

(A) may sell a rare disease therapy owned by the Corporation at any time; and

(B) shall sell any rare disease therapy owned by the Corporation prior to the commencement of a phase 3 study (as such term is defined in section 312.21(b) of title 21, Code of Federal Regulations (or any successor regulations)).

(2) SALE REQUIREMENTS.—In any sale of a rare disease therapy, the Corporation shall make such sale through an open and transparent arms-length process and on commercially reasonable terms, which may include lump sum, upfront payments, milestone payments, royalty payments, or any combination thereof.

(e) FUNDING THROUGH BOND ISSUANCES.—

(1) IN GENERAL.—The Corporation shall issue one or more classes of bonds, with a maturity of no more than 12 years and carrying such interest as the Corporation determines appropriate:

(A) GUARANTEED BONDS.—The Corporation shall issue a class of bonds, in an aggregate amount of not more than $350,000,000, that is guaranteed by the United States.

(B) UNGUARANTEED BONDS.—The Corporation may issue one or more classes of bonds that are backed by the Corporation, but are not guaranteed by the United States.

(2) DEBT-TO-EQUITY RATIO OF GUARANTEED BONDS.—The Corporation may not issue any guaranteed bond pursuant to paragraph (1)(A) if the issuance of such bond would cause the Corporation to exceed a debt-to-equity ratio of 1 to 1.

(3) GUARANTEE FEE.—The Corporation shall pay the Secretary a guarantee fee, which shall be set by the Secretary in an amount equal to the expected cost of guaranteeing bonds of the Corporation under paragraph (1)(A).

(f) TREATMENT UNDER THE SECURITIES LAWS.—

(1) IN GENERAL.—For purposes only of the securities laws—

(A) securities of the Corporation shall be deemed to be securities that are not issued or guaranteed by the Government; and

(B) the Secretary shall be deemed to not be an instrumentality of the Government.

(2) ACCREDITED INVESTOR REQUIREMENT.—Securities issued under this Act may only be purchased by accredited investors.

(g) CORPORATION NOT GUARANTEED BY THE UNITED STATES.—Except as provided under subsection (e)(1)(A), the full faith and credit of the United States shall not be pledged to the Corporation or any security of the Corporation.

(h) DIVERSIFICATION REQUIREMENT.—The Corporation shall, during the 3-year period beginning on the date that the Corporation first purchases rights to a rare disease therapeutic, purchase the rights to at least 15 rare disease therapeutics.

(i) AUTHORIZATION OF APPROPRIATIONS.—

(1) IN GENERAL.—There is authorized to be appropriated to the Secretary $3,000,000 to establish the Corporation and complete the privatization of the Corporation.

(2) REPAYMENT OF APPROPRIATIONS.—Not later than the end of the 36-month period beginning

on the date the Corporation is privatized pursuant to subsection (c), the Corporation shall reimburse the Government for the amount of any appropriation made pursuant to paragraph (1), plus interest on such amount.

(j) SUNSET.—The Corporation shall terminate after the end of the 18-month period following the later of—

(1) the date on which the last bond issued under subsection (e) matures; and

(2) the date on which the Corporation receives the final payment for the sale of all rare disease therapeutics owned by the Corporation.

SEC. 4. DEFINITIONS.

For purposes of this Act:

(1) ACCREDITED INVESTOR.—The term ''accredited investor'' has the meaning given such term under section 2(a) of the Securities Act of 1933 (15 U.S.C. 77b(a)).

(2) CORPORATION.—The term ''Corporation'' means the Rare Disease Therapeutics Corporation established under section 3(a).

(3) RARE DISEASE THERAPEUTICS.—The term ''rare disease therapeutics'' means a compound, biologic, medical device, or companion diagnostic that has been designated as a therapy for a rare disease

or condition pursuant to section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb).

(4) SECRETARY.—The term ''Secretary'' means the Secretary of the Treasury.

(5) SECURITIES LAWS.—The term ''securities laws'' has the meaning given that term under section 3(a) of the Securities Exchange Act of 1934 (15 U.S.C. 78c(a)).

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