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114TH CONGRESS
1ST SESSION

H. R. 3731

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

OCTOBER 9, 2015

Mr. VARGAS (for himself and Mr. ROONEY of Florida) 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'' or the ''RaD Fund Act''.

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 all stages of development; and

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

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

(8) A simulation 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 compounds and only $400 million in capital.

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

(10) In addition to appealing to traditional biotech VC investors, megafund investments may be attractive to pension funds, insurance companies, and other large institutional investors.

(11) 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.

SEC. 3. RARE DISEASE THERAPEUTICS CORPORATION.

(a) ESTABLISHMENT.—The Director of the National Institutes of Health shall organize under the laws of a State a corporation to be know as the ''Rare Disease Therapeutics Corporation'' (hereinafter in this Act referred to as 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, the Director shall sell equity stock in the Corporation to investors.

(2) GOVERNMENT STAKE.—

(A) IN GENERAL.—Notwithstanding paragraph (1), the board of directors of the Corporation and the Director may enter into an agreement under which the National Institutes of Health maintains an ownership interest in the Corporation in exchange for the National Institutes of Health providing the Corporation with intellectual property or other assistance, such as medicinal chemistry, toxicology, and high throughput screening services.

(B) LIMIT ON GOVERNMENT STAKE.—The amount of any ownership interest maintained by the National Institutes of Health pursuant to subparagraph (A) may not exceed 25 percent of the equity stock of the Corporation.

(3) PROHIBITION ON DIVIDENDS.—The Corporation may not pay dividends on the equity stock of the Corporation.

(4) BOARD OF DIRECTORS.—At all times, two of the members of the board of directors of the Corporation shall be chosen as follows:

(A) One member chosen by the Director.

(B) One member chosen by the Secretary of the Treasury.

(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 process and on commercially reasonable terms.

(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 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 Treasury a guarantee fee, which shall be set by the Secretary of the Treasury in an amount equal to the anticipated cost to the Treasury in guaranteeing bonds of the Corporation under paragraph (1)(A).

(f) TREATMENT UNDER THE SECURITIES LAWS.—For purposes only of the securities laws—

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

(2) the National Institutes of Health shall be deemed to not be an instrumentality of the Government.

(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) AUTHORIZATION OF APPROPRIATIONS.—There are authorized to be appropriated to the Director such sums as may be necessary to establish the Corporation and complete the privatization of the Corporation.

(i) 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 the rare disease therapeutics owned by the Corporation.

SEC. 4. RARE DISEASE THERAPEUTICS CORPORATION SCIENCE ADVISORY COUNCIL.

(a) ESTABLISHMENT.—There is established within the National Institutes of Health an advisory council to be known as the ''Rare Disease Therapeutics Corporation Science Advisory Council''.

(b) MEMBERS.—The members of the Advisory Council shall be selected by the Director.

(c) PURPOSE.—The purpose of the Advisory Council shall be to advise the Corporation on the purchase, sale, and development of rare disease therapeutics.

(d) DISCLOSURE OF CERTAIN EMPLOYMENT.—Each member of the Advisory Council shall disclose each company, partnership, or other entity with respect to which the member is an officer or director.

(e) SUNSET.—The Advisory Council shall terminate on the date that the Corporation terminates.

SEC. 5. DEFINITIONS.

For purposes of this Act:

(1) 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).

(2) ADVISORY COUNCIL.—The term ''Advisory Council'' means the Rare Disease Therapeutics Corporation Advisory Council established under section 4(a).

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

(4) DIRECTOR.—The term ''Director'' means the Director the National Institutes of Health.

(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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