

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.

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## 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.

1 *Be it enacted by the Senate and House of Representa-*  
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Rare Disease Fund  
5 Act of 2018” or the “RaD Fund Act of 2018”.

6 **SEC. 2. FINDINGS.**

7 The Congress finds the following:

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

1 breakthroughs require years of translational re-  
2 search at a cost of hundreds of millions of dollars  
3 per trial and have a substantial likelihood of failure.

4 (2) The drug development pipeline is laden with  
5 unfavorable probabilities. On average, for every  
6 5,000–10,000 compounds that enter the drug dis-  
7 covery pipeline, just 250 progress to preclinical de-  
8 velopment—and only one will become an approved  
9 drug.

10 (3) Biotech and life sciences traditional financ-  
11 ing vehicles of private and public equity are becom-  
12 ing less effective funding sources because the needs  
13 and expectations of limited partners and share-  
14 holders are not consistent with the increasing com-  
15 plexity, risk, and duration of biomedical innovation.

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

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

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

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

10              (7) This approach involves two components:

11                      (A) Creating large diversified portfolios,  
12                      called “megafunds”, consisting of biomedical  
13                      products at various stages of development.

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

17              (8) This innovation makes the investment op-  
18       portunity much more attractive to a large pool of in-  
19       stitutional investors that have historically not par-  
20       ticipated in financing for early-stage therapeutic de-  
21       velopment.

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

1           (10) A series of peer-reviewed simulations con-  
2           ducted by researchers at MIT suggested that a mod-  
3           est megafund model could be successfully imple-  
4           mented for rare diseases (e.g., rare genetic dis-  
5           orders, pediatric cancers, and orphan diseases) with  
6           as few as ten to twenty compounds and only \$400  
7           million in capital.

8           (11) A rare disease therapeutics fund could  
9           serve as a viable pilot project, while minimizing gov-  
10          ernmental exposure.

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

17          (13) The Food and Drug Administration  
18          (FDA) may grant the orphan designation for thera-  
19          pies being studied for a rare disease or condition af-  
20          fecting fewer than 200,000 people in the United  
21          States, which reduces costs and provides financial  
22          incentives to encourage development of such thera-  
23          pies for underserved patient populations.

24 **SEC. 3. RARE DISEASE THERAPEUTICS CORPORATION.**

25          (a) ESTABLISHMENT.—

1           (1) IN GENERAL.—The Secretary of the Treas-  
2           ury shall organize under the laws of a State a cor-  
3           poration to be known as the “Rare Disease Thera-  
4           peutics Corporation” (hereinafter in this Act re-  
5           ferred to as the “Corporation”).

6           (2) QUALIFIED PORTFOLIO MANAGER.—As soon  
7           as practicable after organization, the Corporation  
8           shall hire a qualified portfolio manager whose man-  
9           date will be to acquire and manage a portfolio of  
10          biomedical research assets on behalf of the Corpora-  
11          tion.

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

16          (c) PRIVATIZATION OF THE CORPORATION.—

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

22                 (2) TERMINATION OF GOVERNMENT OWNER-  
23                 SHIP.—Upon the issuance of the equity stock de-  
24                 scribed under paragraph (1), the Government shall

1 no longer hold any ownership interest in the Cor-  
2 poration.

3 (3) PROHIBITION ON DIVIDENDS.—The Cor-  
4 poration may not pay dividends on the equity stock  
5 of the Corporation while there are any outstanding  
6 guaranteed bonds of the Corporation issued pursu-  
7 ant to subsection (e)(1)(A).

8 (d) SALE OF OWNERSHIP INTERESTS.—

9 (1) IN GENERAL.—The Corporation—

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

12 (B) shall sell any rare disease therapy  
13 owned by the Corporation prior to the com-  
14 mencement of a phase 3 study (as such term is  
15 defined in section 312.21(b) of title 21, Code of  
16 Federal Regulations (or any successor regula-  
17 tions)).

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

25 (e) FUNDING THROUGH BOND ISSUANCES.—

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

5           (A) GUARANTEED BONDS.—The Corpora-  
6 tion shall issue a class of bonds, in an aggre-  
7 gate amount of not more than \$350,000,000,  
8 that is guaranteed by the United States.

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

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

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

23           (f) TREATMENT UNDER THE SECURITIES LAWS.—

24           (1) IN GENERAL.—For purposes only of the se-  
25 curities laws—

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

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

6           (2) ACCREDITED INVESTOR REQUIREMENT.—  
7           Securities issued under this Act may only be pur-  
8           chased by accredited investors.

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

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

19          (i) AUTHORIZATION OF APPROPRIATIONS.—

20                 (1) IN GENERAL.—There is authorized to be  
21                 appropriated to the Secretary \$3,000,000 to estab-  
22                 lish the Corporation and complete the privatization  
23                 of the Corporation.

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



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

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

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

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

13 **SEC. 4. DEFINITIONS.**

14 For purposes of this Act:

15 (1) ACCREDITED INVESTOR.—The term “ac-  
16 credited investor” has the meaning given such term  
17 under section 2(a) of the Securities Act of 1933 (15  
18 U.S.C. 77b(a)).

19 (2) CORPORATION.—The term “Corporation”  
20 means the Rare Disease Therapeutics Corporation  
21 established under section 3(a).

22 (3) RARE DISEASE THERAPEUTICS.—The term  
23 “rare disease therapeutics” means a compound, bio-  
24 logic, medical device, or companion diagnostic that  
25 has been designated as a therapy for a rare disease

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

3 (4) SECRETARY.—The term “Secretary” means  
4 the Secretary of the Treasury.

5 (5) SECURITIES LAWS.—The term “securities  
6 laws” has the meaning given that term under section  
7 3(a) of the Securities Exchange Act of 1934 (15  
8 U.S.C. 78c(a)).

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