



Ensure Patient Perspectives Are Included in FDA Benefit-Risk Assessments: Cosponsor the S. 373/H.R. 4472, the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act

Overview

Congress and the Food and Drug Administration (FDA) have made considerable progress in driving forward policies and procedures to ensure the patient perspective is considered by FDA reviewers evaluating candidate drugs and other medical products. As a result of numerous provisions of both the Prescription Drug User Fee Act (PDUFA) of 2012, (FDASIA) and the 21st Century Cures Act in 2016, the FDA now has programs and policies in place to evaluate the benefits and risks of potential therapies and to gather and assess patient perspectives.

But while much progress has been made, some significant gaps remain. One such gap is the lack of any requirement in law today that the FDA include patient experience or patient-focused drug development (PFDD) data as a part of its risk-benefit framework. This means that the agency's signature tool for evaluating risk-benefit does not have to data from the patient perspective that could be critical to informing the agency's evaluation and, ultimately, decision on whether or not to approve a product.

The BENEFIT Act

To address this gap, Senators Roger Wicker (R-MS) and Amy Klobuchar (D-MN) and Representatives Doris Matsui (D-CA) and Brad Wenstrup (R-OH) have introduced S.373/H.R. 4472, the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act. This legislation will amend the Food, Drug and Cosmetic Act (FDCA) to ensure that patient experience, PFDD and related data – including information developed by a product sponsor or a third party such as a patient advocacy organization or academic institution – be considered as part of the risk-benefit assessment. This action will send an important signal to all stakeholders that patient experience and PFDD data will be fully incorporated into the agency's review process and will encourage such entities to develop scientifically rigorous and meaningful tools and data.

The BENEFIT Act will also enhance an important transparency and accountability provision included in the 21st Century Cures Act by requiring the FDA to share how such patient experience and PFDD data was considered within the risk-benefit assessment for any approved therapies. This will provide additional learnings to all stakeholders, particularly patients, and help further refine and develop such tools going forward.

Conclusion

The nascent field of patient engagement in drug development continues to flourish thanks to a continued interest and focus by Congress. The BENEFIT Act will continue this evolution by filling a sizeable gap by ensuring such data is fully considered as part of the FDA's risk-benefit assessment for any new products. **Advance patient engagement by cosponsoring the BENEFIT Act today.**

Senate: Support S. 373 the BENEFIT Act (Wicker-Klobuchar)

Contact [Ruth McDonald@klobuchar.senate.gov](mailto:Ruth.McDonald@klobuchar.senate.gov) or [Kirby Miller@wicker.senate.gov](mailto:Kirby.Miller@wicker.senate.gov)

House: Support H.R. 4472 the BENEFIT Act (Matsui-Wenstrup)

Contact: Christina.McCauley@mail.house.gov (Matsui) or Casey.Quinn@mail.house.gov (Wenstrup)

February 18, 2022

The Honorable Roger Wicker
U.S. Senate
555 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Amy Klobuchar
U.S. Senate
425 Dirksen Senate Building
Washington, DC 20510

The Honorable Doris Matsui
House of Representatives
2311 Rayburn House Office Building
Washington, DC 20515

The Honorable Brad Wenstrup
House of Representatives
2419 Rayburn House Office Building
Washington, DC 20515

RE: Support for the BENEFIT Act of 2021 (S. 373 and H.R. 4472)

Dear Senators Wicker and Klobuchar and Representatives Matsui and Wenstrup:

Thank you for your tireless efforts to encourage development of and expand access to treatments and cures for patients, including those with rare diseases. On behalf of the undersigned patient advocacy organizations, we write in strong support of your legislation, the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act of 2021, S. 373 and H.R. 4472.

As you know, the 21st Century Cures Act (P.L. 114-255) includes sections 3001 and 3002, the Patient-Focused Impact Assessment (PFIA), which has accelerated the field of patient-focused drug development (PFDD). FDA now has a number of programs and policies in place to gather and assess patient perspectives within the regulatory review process, and patient advocacy organizations have been deeply engaged with the FDA over the past several years to develop PFDD tools that produce scientifically valid patient experience information. Tremendous progress has been made over the past decade since the fifth Prescription Drug User Fee Act (PDUFA) was authorized, including with PFIA and other provisions of 21st Century Cures. Now is the time to take the next step in moving patient perspectives and experience forward by enacting the BENEFIT Act.

The BENEFIT Act would require FDA to include in the benefit-risk assessment framework of a new drug application how patient experience data was considered in the review process. Currently, FDA includes patient experience data in reviews, but does not indicate how such data impacted the drug approval. Providing this information to the public, and patient communities making significant investments in developing PFDD, builds on transparency from PFIA and will accelerate PFDD strategies more broadly.

The field of patient engagement in drug development continues to flourish thanks to the continued interest and focus by Congress. The BENEFIT Act will build upon this foundation and fill a gap by appropriately disclosing how this data is considered as part of FDA review of new therapies. The BENEFIT Act initially passed the Senate in 2017 but further action was deferred as the 21st Century Cures was being implemented.

Now is the time to take this critical step in building the PFDD environment by passing the BENEFIT Act. The Cures 2.0 Act recognizes this as well by including a parallel provision to the BENEFIT Act. Thank you again for your leadership and we look forward to working with you to enact this legislation this Congress.

Sincerely,

Alport Syndrome Foundation
ALS Association
Alstrom Syndrome International
Ara Parseghian Medical Research Foundation
Barth Syndrome Foundation
Best Day Ever Foundation
Beyond Celiac
Casimir LLC
Coalition Duchenne
CSNK2A1 Foundation
Cure CMD
Cure HHT
Cure Sanfilippo Foundation
Cure SMA
CureDuchenne
Dravet Syndrome Foundation
Dup15q Alliance
Emily's Entourage
EveryLife Foundation for Rare Diseases
FND Hope
FORCE: Facing Our Risk of Cancer Empowered
Foundation for Prader-Willi Research
Foundation to Eradicate Duchenne
Genetic Alliance
Global Liver Institute
Hannah's Hope Fund
Hemophilia Federation of America
Hope For Marian
Immune Deficiency Foundation
International Pemphigus and Pemphigoid Foundation
Jett Foundation
Kindness Over Muscular Dystrophy
Little Hercules Foundation
Little Miss Hannah Foundation
Lupus Foundation of America
M-CM Network
MLD Foundation
National Ataxia Foundation
National Multiple Sclerosis Society
National Niemann Pick Disease Foundation
NBIA Disorders Association
Organic Acidemia Association
Parent Project Muscular Dystrophy
Phelan-McDermid Syndrome Foundation
PXE International
RASopathies Network
Rivkin Center for Ovarian Cancer
RUNX1 Research Program

Ryan's Quest
Samantha Search for the Cure
Siegel Rare Neuroimmune Association
SYNGAP1 Foundation
The Firefly Fund
The Life Raft group
The Sudden Arrhythmia Death Syndromes (SADS) Foundation
Team Joseph
Tuberous Sclerosis Alliance
Usher 1F Collaborative
Wiskott-Aldrich Foundation
Zack Heger Foundation

117TH CONGRESS
1ST SESSION

S. 373

To strengthen the use of patient-experience data within the benefit-risk framework for approval of new drugs.

IN THE SENATE OF THE UNITED STATES

FEBRUARY 23, 2021

Mr. WICKER (for himself and Ms. KLOBUCHAR) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To strengthen the use of patient-experience data within the benefit-risk framework for approval of new drugs.

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 “Better Empowerment
5 Now to Enhance Framework and Improve Treatments Act
6 of 2021” or the “BENEFIT Act of 2021”.

1 **SEC. 2. STRENGTHENING THE USE OF PATIENT-EXPERI-**
2 **ENCE DATA WITHIN BENEFIT-RISK FRAME-**
3 **WORK.**

4 Section 569C of the Federal Food, Drug, and Cos-
5 metic Act (21 U.S.C. 360bbb–8c) is amended—

6 (1) in subsection (a)(1)—

7 (A) in subparagraph (A), by striking “;
8 and” and inserting a semicolon;

9 (B) in subparagraph (B), by striking the
10 period and inserting “; and”; and

11 (C) by adding at the end the following:

12 “(C) as part of the risk-benefit assessment
13 framework in the new drug approval process de-
14 scribed in section 505(d), considering relevant
15 patient-focused drug development data, such as
16 data from patient preference studies (benefit-
17 risk), patient reported outcome data, or patient
18 experience data, developed by the sponsor of an
19 application or another party.”; and

20 (2) in subsection (b)(1), by inserting “, includ-
21 ing a description of how such data and information
22 were considered in the risk benefit assessment de-
23 scribed in section 505(d)” before the period.

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Ensure Patient Perspectives Are Included in FDA Benefit-Risk Assessments

Dear Colleague:

We invite you to cosponsor [H.R. 4472](#), the **Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act**, legislation that would elevate the use of patient-focused drug development (PFDD) data within the Food and Drug Administration's (FDA) benefit-risk framework for drug approval.

Congress and the FDA have made considerable progress in advancing policies to ensure the patient perspective is considered by FDA when evaluating candidate drugs and other medical products. As a result of the Prescription Drug User Fee Act (PDUFA) updates from 2012, known as FDASIA and several provisions in the 21st Century Cures Act passed into law in 2016, the FDA has several programs in place to gather and assess patient experience data.

While we celebrate the progress that has been made, we also recognize that significant gaps remain, including the lack of any requirement in law today that the FDA must include any patient experience or patient-focused drug development data as part of its risk-benefit framework.

The BENEFIT Act will address this gap by amending the Food, Drug and Cosmetic Act (FDCA) to ensure that patient experience, PFDD, and related data – including information developed by a product sponsor or a third party such as a patient advocacy organization or academic institution – is considered as part of the risk-benefit assessment. This action will send an important signal to all stakeholders that patient experience and PFDD data will be fully incorporated into the agency's review process and will encourage such entities to develop scientifically rigorous and meaningful tools and data.

The BENEFIT Act will also enhance an important transparency and accountability provision included in the 21st Century Cures Act by requiring the FDA to explain how such patient experience and PFDD data were considered within the risk-benefit assessment for any approved therapies. This will provide additional learnings to all stakeholders, particularly patients, and help further refine and develop such tools going forward. Accordingly, Section 204 of the Cures 2.0 Act contains a provision that parallels this legislation.

For more information on the legislation or if you would like a cosponsor H.R. 4472, please contact Christina McCauley with Congresswoman Matsui at Christina.McCauley@mail.house.gov or Casey Quinn with Congressman Wenstrup at Casey.Quinn@mail.house.gov. Thank you for your consideration.

Sincerely,

DORIS MATSUI
Member of Congress

BRAD R. WENSTRUP, D.P.M
Member of Congress

117TH CONGRESS
1ST SESSION

H. R. 4472

To strengthen the use of patient-experience data within the benefit-risk framework for approval of new drugs.

IN THE HOUSE OF REPRESENTATIVES

JULY 16, 2021

Ms. MATSUI (for herself and Mr. WENSTRUP) introduced the following bill;
which was referred to the Committee on Energy and Commerce

A BILL

To strengthen the use of patient-experience data within the benefit-risk framework for approval of new drugs.

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 “Better Empowerment
5 Now to Enhance Framework and Improve Treatments Act
6 of 2021” or the “BENEFIT Act of 2021”.

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