

LETTER TEXT

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

[Bipartisan House champions pending]

Re: Support for the BENEFIT Act of 2021

Dear Senators Wicker and Klobuchar and [House champions]:

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.

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. Thank you again for your leadership and we look forward to working with you to enact this legislation this Congress.

Sincerely,