



Ensure Patient Perspectives Are Included in FDA Benefit-Risk Assessments: Cosponsor the S. 526/H.R. 1092 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) authorization of 2012 (known as FDASIA) and the 21st Century Cures Act passed into law 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 the patient perspectives.

But while much progress has been made, some significant gaps remain. One such gap is the lack of a requirement in law that the FDA include patient experience or patient-focused drug development (PFDD) data as part of its risk-benefit framework. Examples of patient experience data include:

- *Patient reported outcomes* (how a drug impacts activities of daily living ie: whether they can feed themselves, be independent etc.)
- *Patient testimonials* (qualitative data/patient stories of “living with”)
- *Patient preference data* (how much risk patients are willing to take)
- *Natural History Data* (the natural progression of the disease without intervention)

The agency’s signature tool for evaluating risk-benefit of a drug does not currently explicitly include 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. 526/H.R. 1092** the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act.

Currently, FDA indicates whether it receives submitted patient experience data – including information developed by a product sponsor or a third party such as a patient advocacy organization or academic institution – but not whether or how it was used in the review process. **This legislation will amend the Food, Drug and Cosmetic Act (FDCA) to require that FDA include in the risk-benefit framework a description of how submitted patient experience data and information were considered.** This action will enhance transparency and accountability, sending an important signal to all stakeholders that patient experience data will be incorporated into the agency’s review process, encouraging such entities to continue developing and refining scientifically rigorous and meaningful tools and data.

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. **Advance patient engagement by cosponsoring the BENEFIT Act today.**

Senate: Support S. 526 the BENEFIT Act (Wicker-Klobuchar)

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House: Support H.R. 1092 the BENEFIT Act (Matsui-Wenstrup)

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