Cosponsor the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act (H.R. 4472 and S.373)

Background
Congress and the 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 numbers provisions of both PDUFA and the 21st Century Cures Act that passed into law, 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.

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 seek an amendment and originally introduced in 2021.

Bill To Be Introduced on or before Rare Disease Week of February 27, 2023
Senators Roger Wicker (-MS) and Amy Klobuchar (D-MN) and Representatives Doris Matsui (D-CA), and Brad Wenstrup (R-OH) will be introducing the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act.

When Introduced, Key Bill Provisions will address Gaps
- Lack of a law requiring that the FDA include patient experience or patient-focuses 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 i.e.; 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)

Legislation Amendment to the Food, Drug, and Cosmetic Act (FDCA)
- 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
- Inform all stakeholders that patient experience data will be incorporated into the agency’s review process
- Encourage stakeholders to develop and refine scientific rigorous and meaningful reporting 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 DAS’s risk-benefit assessment. Advance Patient engagement cosponsoring the BENEFIT Act today.