



The Promising Pathway Act (S. 1906)
Introduced by Sen. Braun (R-IN) and Sen. Gillibrand (D-NY)

Background: For individuals with life-threatening, serious diseases, timely access to treatments can mean the difference between life and death. The U.S. Food and Drug Administration (FDA) drug approval pathways are unfortunately *not* designed to deliver treatments to patients with life-threatening, serious diseases when there are no meaningful on-label treatments available *in a safe and timely manner*. Currently, drugs that receive Fast Track designation for the FDA's Accelerated Approval pathway—the FDA's fastest drug approval pathway—take an average of six years before they are approved—and even longer before patients can access them. Very few of those diagnosed with ALS, for instance, live beyond five years. Similarly, those diagnosed with diffuse midline glioma (DIPG) rarely survive beyond 1 year past diagnosis.

What the Bill Does: The *Promising Pathway Act* would grant time-limited provisional approval for drugs intended to treat life-threatening, serious diseases that have demonstrated substantial evidence of safety and robust early evidence that establishes the benefit of the drug is likely to outweigh the risk of side effects. This new pathway would allow drug sponsors to use real-world evidence and scientifically substantiated surrogates—surrogate endpoints other than those previously validated by the FDA—to predict the clinical benefits of the drug and support an application for provisional approval. Provisional approval is limited to four (4) two-year periods, each requiring separate renewal from FDA. Drug sponsors may submit a request for full approval at any time while the drug has provisional approval status. A drug with provisional approval status that receives full drug approval must be brought to the market within 60 days of receiving full approval. The FDA is authorized to withdraw provisional approval status for a drug that presents a significant number of serious adverse effects compared to other treatments used for that disease.

Endorsements (as of July 2023):

- I AM ALS
- Americans for Prosperity
- The Foundation for Government Accountability
- The Cure Starts Now
- Musella Foundation for Brain Tumor Research & Information
- Stealth Biopharmaceuticals
- PharmEssentia
- Soleno Therapeutics
- Team Gleason
- Best Day Ever Foundation
- Aidan's Avengers
- Whitley's Wishes, Inc.
- Robert Connor Dawes Foundation
- Reflections of Grace
- Yuvaan Tiwari Foundation

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