

# Promising Pathway Act: S.1644 + H.R.3761

*A bill to Create a Provisional Approval Pathway at the FDA*

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## Summary

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The Promising Pathway Act (PPA) calls for the U.S. Food and Drug Administration (FDA) to establish a rolling, real-time, priority review pathway to grant or deny provisional approval status for drugs intended to treat, prevent, or diagnose serious or life-threatening diseases or conditions—including those posing a threat of epidemic or pandemic (e.g., COVID-19). The granting of provision approval depends on many factors, including the demonstration of substantial evidence of safety and relevant early evidence of positive therapeutic outcome(s). The granting of provisional approval status requires CMS insurances to cover the drugs and prohibits private insurance from denying coverage due to a drug's unapproved status.

## Rationale

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For individuals with life-threatening, serious diseases, timely access to treatment is essential to their life. Without these treatments, they could die. To prevent their death, the drug development process should include efficient access to promising therapies for patients with progressive diseases that, if left untreated, may significantly affect their daily lives or lead to premature death. The coronavirus pandemic has shown the need for accelerated pathways when a treatment's safety is demonstrated, some efficacy has been evidenced, and there are no other options to save or extend lives. This is the reality lived by those diagnosed with ALS and a host of other terminal diseases.

We support efforts to bring safe, promising therapies to more people living with terminal diagnoses, while clinical research continues to reach full FDA approval. The provisions in S. 3872 aim to provide such a pathway.

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## Contact to Support

Sen. Mike Braun (R-IN) – [Audrey Arbogast](#)

Rep. Bruce Westerman (R-AK) - [Patrick Dumas](#)

## Legislative Components

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- + Drugs qualifying to apply for provisional approval must be intended for the treatment, prevention, or medical diagnosis of serious or life-threatening diseases or conditions in which there is a reasonable likelihood that premature death or disability will occur without early medical intervention, a disease or condition that poses a threat of epidemic or pandemic, or a disease or condition that is associated with morbidity that substantially impacts day-to-day functioning.
- + To receive provisional approval status, a drug must demonstrate substantial evidence of safety and relevant early evidence of positive therapeutic outcome(s).
- + This pathway will allow for the use of real-world evidence and scientifically substantiated surrogates to predict the clinical benefits and ultimately support provisional approval.
- + PPA requires the FDA to issue guidance that establishes clear protocols for enabling sponsors to submit rolling, real-time, mid-trial provisional approval applications. This provision preserves the integrity of ongoing clinical trial design, development, and enrollment, as well as prohibits sponsors from being penalized for utilizing this pathway mid-trial.
- + Drugs and biological products granted provisional approval are limited to a 2-year approval period, renewable every 2 years, for up to 6 years.
- + The sponsor of a provisionally approved drug must ensure that all patients who use the drug participate in an observational registry and consent to the collection of, and submission of, data related to the patient's use of the drug until the drug receives full approval. Importantly, the registries must be readily accessible to patients—as well as allow approved researchers and medical professionals to access the aggregated and de-identified data for public health research.
- + The registries can be run by third party governmental, for-profit, or nonprofit entities—but must track the effect of provisionally approved drugs on patients, including patient treatments, uses, length of use, side effects, scan results, and adverse drug effects.
- + Drug sponsors (often biopharmaceutical companies) may apply for full approval for a drug at any time under the pathway. The FDA may also withdraw provisional approval of a drug with a significant number of reported adverse effects.
- + PPA establishes, within 6 months, the position of Patient Advocate General within the Office of the Commissioner at the FDA to increase transparency and provide assistance to patients.
- + PPA prohibits any group health plans, health insurance coverage providers, and federal healthcare programs (e.g., Medicare) from denying coverage of a provisionally approved drug on the basis of it being experimental and mandates that provisionally approved drugs be treated in the same manner as drugs fully approved by the FDA under other review pathways.