Promising Pathway Act
(S. 4426)

Rationale

The Promising Pathway Act affords patients access to safe therapies that demonstrate promise for patients while the process of gathering additional evidence of efficacy proceeds. This bill preserves existing efficacy standards for final drug approval.

For individuals with rapidly progressive and 100% fatal diseases like ALS, timely access to promising therapies is essential and offers an opportunity for survival. Current U.S. Food and Drug Administration (FDA) approval pathways take far too long to help those currently living with ALS, as they usually pass within 2-5 years of diagnosis. Significantly, the standard FDA approval pathway takes well over 10 years. While an Accelerated Approval pathway exists, it is insufficient for diseases like ALS, which lack a recognized biomarker.

What the Bill does

The PPA calls for the FDA to use a priority review pathway to grant or deny conditional approval for drugs intended to treat rare, progressive, and terminal diseases that have demonstrated early evidence of safety and effectiveness.

Conditional approval would be subject to strong FDA guardrails and oversight:

- Granting of conditional approval requires proof of drug safety and effectiveness, which a drug sponsor may establish based on evidence from phase 1 and phase 2 clinical trials, respectively.
- Conditional approval is only for a two-year period, with three potential renewals based on additional evidence of efficacy.
- Patients must participate in an observational registry with data collection requirements approved by the FDA. The FDA must submit an annual report to Congress on all drugs granted conditional approval.
- The FDA can withdraw conditional approval at any time based on safety or efficacy and can deny renewal if the requirements for such renewal are not met. Conditional approval is automatically withdrawn if the sponsor does not seek renewal.

To achieve full marketing approval, drugs must still meet the existing FDA standards.

In sum, the PPA provides a pragmatic pathway for the FDA to conditionally approve drugs for individuals with rapidly progressive, terminal illnesses who will die of their diseases waiting for the usual FDA approval pathways to unfold. 100+ disease organizations have endorsed the bill.

Key Contacts

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