Prader-Willi SYNDROME ASSOCIATION | USA
SAVING AND TRANSFORMING LIVES

D.C. Fly-In Hill Day: Issue Information

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Legislative Ask #1

Promising Pathway Act (PPA) 2.0

Introduced by Sen. Mike Braun (R-IN) and Sen. Kirsten Gillibrand (D-NY)

About Promising Pathway Act 2.0

For those living with life-threatening rare and progressive diseases, timely access to treatments can mean the difference between life and death. Unfortunately, U.S. Food and Drug Administration (FDA) drug approval pathways do not always deliver treatments to patients when there are no meaningful on-label treatments available.

Drugs going through 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. Many of the 10,000 known rare and progressive diseases also substantially reduce lifespan.

What does PPA do?

Access

PPA would allow the FDA to grant time-limited conditional approval for drugs intended only to treat rare, progressive, and congenital diseases that have demonstrated evidence of safety and early evidence of effectiveness. Patients would be able to access the conditionally approved drugs through their insurance. Sponsors must bring conditionally approved drugs to market within a reasonable time frame.

Guardrails

- The FDA has the authority to remove unsafe or ineffective drugs from the market.
- To retain conditional approval beyond 2 years, the sponsor must collect additional evidence of effectiveness.
- Drugs may only have conditional approval for 8 years max.
- Patients must participate in a registry during treatment.
- Patients must provide informed consent.
- Drug is automatically removed from the market if it cannot eventually attain full approval.