FREQUENTLY ASKED QUESTIONS:

Promising Pathway Act (H.R. 3761/S. 1644)

Why is the Promising Pathway Act needed?

The current Food and Drug Administration (FDA) approval process is failing many patients with terminal illnesses that want timely access to provisionally approved, and potentially life-saving treatments. Mired in red tape and bureaucracy, the approval process takes between 10 to 12 years on average before treatments are available to patients (if they succeed at all). Patients with diseases like ALS or muscular dystrophy often don't have that long to wait. That's where the Promising Pathway Act (PPA) fits in. This bipartisan bill would establish a rolling, real-time priority review pathway for promising new drugs and biologics that could extend patients' life expectancy under the care of their doctors.

How does the PPA build upon the "Right to Try Act" signed into law in 2017?

The PPA builds upon Right to Try (RTT), which introduced new alternatives to FDA that support patient access to investigational therapies. Right to Try (RTT) applied to experimental drugs, whereas PPA addresses provisionally approved drugs.

PPA includes FDA in the decision making process. The PPA allows FDA to review patient data while taking provisional drugs. This means that drug manufacturers will be able to build upon ongoing clinical trials and collect more real-world evidence to support traditional FDA market approval.

Under RTT, drug manufacturers can choose to stop producing the drug at any time or deny RTT. The PPA, however, incenticizes manufacturers to continue clinical trials, innovate, and invest in additional therapies.

How does the PPA compare to expanded access (also called compassionate use)?

Expanded access requires drug manufacturers to create a separate protocol for granting patients access to an investigational drug outside of the clinical trial. The PPA would allow more individuals with rare and life-threatening diseases to access promising therapies under the same provisional approval, and does not create additional requirements for the manufacturer.

FDA allows drug manufacturers to charge patients or their insurers the direct costs of the expanded access program, including manufacturing and shipping costs. Patients that are granted expanded access often cannot afford the associated costs. The PPA allows patients to access provisional drugs under the supervision of their doctors. This allows patients to remain in their homes and continue their jobs, eliminating the need for patients to uproot their lives to find new clinical trial sites or cover the costs of shipping and manufacturing the drug.

FDA does not allow drug manufacturers to use clinical data from patient expanded access to the investigational drug to go towards supporting FDA approval. The PPA allows for the drug manufacturer to submit patient and real-world data as evidence demonstrating safety and efficacy, allowing for greater utilization of data.

Expanded access can deter enrollment in clinical trials because, through expanded access, the patient is guaranteed to get the investigational drug—whereas patients enrolled in clinical trial have a chance of receiving a placebo. The PPA would grant patients access to provisionally approved drugs, those that are safe and have shown early evidence of efficacy. The PPA would also ensure patients don't lose access to

clinical trials and provides a six year window for them to participate. If treatments are working, under the PPA, patients would be able to continue receiving those treatments. Patients would not receive placebos through the PPA.

Is the PPA safe for patients?

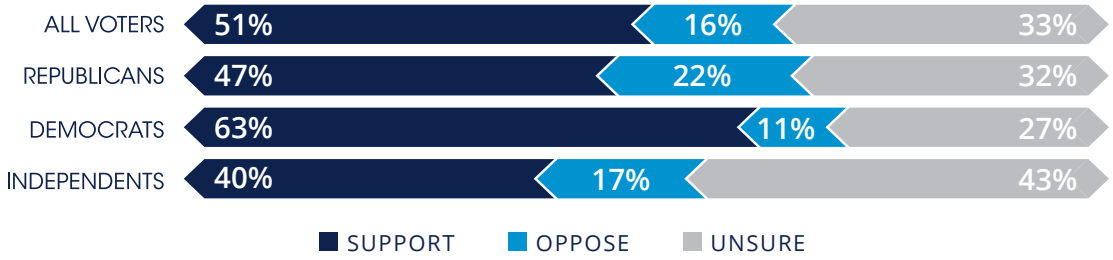
Yes. Under the current system, once a treatment is approved, there exists no centralized, third-party tracking system on patient responses or side effects. The PPA provides the framework to protect patient safety, while also providing patients with the opportunity to access potentially life-saving treatments the current system prevents them from accessing. Through the PPA, third-party registries are required both for communication to the patient population and researchers, but also to eventually assist in the full drug approval determination.

Is the PPA bipartisan?

Yes. In the House, H.R. 3761 is supported by Rep. Mike Gallagher (R-WI), and a list of nine bipartisan co-sponsors, including an equal number of Democrats and Republicans. In the Senate, S. 1644 has been introduced by Senator Mike Braun (R-IN) and has six co-sponsors.

Does the public support the PPA?

Yes. According to new polling results released by the Center for Excellence in Polling, 51 percent of likely voters support the early approval of medications that have been proven to be safe and effective in treating serious of life-threatening conditions, including majorities of Democrats and Independents.



Which organizations support the PPA?

The PPA has been endorsed by a diverse list of stakeholders, including in a recent letter led by the Foundation for Government Accountability (FGA) and signed by 10 patient advocacy and taxpayer groups to congressional leadership, which notes:

> *First drafted in 2019, the PPA has received extensive input from stakeholders, including patient groups, pharmaceutical companies, and taxpayer groups, and has been thoughtfully revised to strike an appropriate balance between access to innovative treatments and patient safety. The legislation has gained bipartisan support because it prioritizes patients that are suffering while maintaining necessary safeguards that many, including members of Congress, value. For these reasons, we strongly support S. 1644/H.R. 3761, the Promising Pathway Act, and urge you to enact it as soon as possible.*

In addition to FGA, the letter was signed by Americans for Prosperity, Best Day Ever Foundation, DIPG Advocacy Group—Jack's Angels, FreedomWorks, Heritage Action for America, LiveLikeLou Foundation, Project ALS, Taxpayers Protection Alliance, the DIPG/DMG Collaborative, and the Pediatric Brain Tumor Consortium Foundation.

Read letter at: thefga.org/press/fga-leads-diverse-coalition-letter-supporting-promising-pathway-act