Section 1. Short Title.

The bill may be referred to as the "Promising Pathway Act 2.0."

Section 2. Provisional Approval of New Human Drugs.

Section 3 of the bill amends subchapter A of chapter 5 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351) to add the following at the end:

Section 524C. Provisional Approval of New Human Drugs.

Subsection a—Priority Review and Evaluation of Applications for Conditional Approval

Sponsors may apply for time limited conditional approval of an eligible drug. FDA must review all eligible conditional approval applications through its priority review.

Subsection b—Eligibility

Eligibility for this pathway is exclusive to drugs intended to treat those with rare (as defined by the orphan drug act) OR rapidly progressive and terminal diseases.

Subsection c—Standard of Review for Conditional Approval

This section

- requires eligible drugs to have completed Phase 1 (or equivalent) and demonstrate evidence of effectiveness from Phase 2 to gain initial 2 year conditional approval,
- plugs in existing rolling review language from statute instead of creating new standards,
- requires sponsors to make a written affirmation of their intent to pursue full approval,
- allows sponsors to use RWE on the condition that such RWE complies with the new FDA guidance on RWE, and

Subsection d—FDA Authority to Withdraw Conditional Approval

This section

- provides explicit authorities for FDA to pull a conditionally approved drug for safety, for material misrepresentations in applications, if eligibility criteria are no longer met, and if relevant data no longer support conditional approval.
- gives FDA examination authorities to conduct limited investigations of registries and other data to determine if a conditional approval should be withdrawn,
- allows FDA to levy penalties on companies that continue to make available drugs that have had conditional approval withdrawn,
- requires FDA to provide 2 weeks written notice of intent to withdraw conditional approval,
- directs FDA to create a process for sponsors to appeal withdrawals, and
- requires conditional approval to be automatically removed from market at the end of the conditional approval period, forcing the sponsor to pursue full approval or for the drug to be removed entirely.

Subsection e—Labeling; Review of Materials

This section provides labelling requirements modeled closely after language from the Limited Population Pathway for Antibacterial and Antifungal Drugs. It also requires any promotional materials to be reviewed by FDA within 30 days of dissemination and directs FDA to maintain a list of conditionally approved drugs on their website for the public to view.

Subsection f—Renewal of Conditional Approval; Requirement to Bring Drug to Market

This section requires sponsors to apply to FDA for a renewal of conditional approval every two years or automatically lose conditional approval. The first renewal application must include additional evidence of efficacy as compared to initial conditional approval application, the second renewal must include additional evidence of efficacy compared to first renewal application, and the third renewal must include written affirmation from drug review division head that an additional 2 years of conditional approval is needed for patient benefit & drug development. The additional evidence must consist of one of, or a combination of, the following:

- clinical evidence generated by an ongoing or completed clinical trial
- real world evidence generated in accordance with 505F(b)
- evidence from an observational registry

There are exemptions for rare populations that cannot feasibly produce such data.

This section also

- requires FDA to approve or deny applications for renewal,
- allows FDA to deny renewals on the basis that a registry which has provided data to support renewal has engaged in data manipulation/fraud, and
- requires sponsors to bring a conditionally approved drug to market within 180 days.

Subsection g—Observational Registries

This section requires sponsors to establish an observational registry for their conditionally approved drug. Patients are required to participate in a registry to gain access to a conditionally approved drug. Sponsors may create a new registry, utilize an existing one, or combine existing registries for the purposes of this subsection. Patients must provide informed consent to participate in registry. Sponsors are responsible for obtaining and submitting patient data to registry, while FDA must promulgate data submission standards. Secretary must maintain an online portal for registry data submissions.

This section also gives FDA authority to approve or deny registries that could be used for approval renewals later on. FDA must create processes for approvals/denials and appeals of denials for registries. This section also

- requires registry to provide participants with their own personal data upon request, and
- grants approved researchers access to de-identified and aggregated data for the purposes of translational research, as well as requires FDA to establish a process for approving researchers for these purposes.

Subsection h—Pursuit of a Different Indication

This section allows sponsors to apply for conditional approval for a different indication for drugs that have had conditional approval withdrawn. FDA may approve or deny these applications.

Subsection i—Transition to Other Forms of Approval

This section explains that sponsors may pursue traditional or accelerated approval at any time the drug is under conditional approval. It also allows sponsors to submit evidence generated in connection with PPA in support of NDA for traditional approval

Subsection j—Informed Consent

This sections requires patients to provide informed consent to sponsor (or registry) to receive a conditionally approved drug. PPA 2.0 employs existing informed consent regulations. Registries may obtain and maintain informed consent from patients. This section also establishes that the HHS common rule is maintained for drugs that conditionally approved drugs that qualify.

Subsection k—Limitation on Liability

This section establishes that sponsors and health care providers may not be sued because of these drugs unless they've committed intentional wrongdoing.

Subsection l—Report to Congress

A report on conditionally approved drugs must be submitted to congress every 2 years by FDA. It requires FDA to submit report to congress on certain information pertaining to conditionally approved drugs. It grants FDA authority to notice and demand information from sponsors as needed. Sponsors must provide the needed information to FDA.

Subsection m—Insurance Coverage

This section requires private and public payers to cover conditionally approved drugs for the period of their conditional approval.