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117TH CONGRESS
1ST SESSION

H. R. 3761

To amend the Federal Food, Drug, and Cosmetic Act to establish a time-limited provisional approval pathway, subject to specific obligations, for certain drugs and biological products, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

JUNE 8, 2021

Mr. GALLAGHER (for himself, Mr. QUIGLEY, Mr. WESTERMAN, and Mr. SWALWELL) introduced the following bill; which was referred to the Committee on Energy and Commerce

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A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish a time-limited provisional approval pathway, subject to specific obligations, for certain drugs and biological products, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Promising Pathway Act''.

SEC. 2. FINDINGS.

Congress finds as follows:

(1) The drugs and biological products intended to be reviewed under the pathway established under this Act are for the treatment and prevention of serious diseases or conditions, especially those for which there are no available on-label meaningful or disease-modifying treatments, where speed to access is critical.

(2) The approval pathway established under this Act is intended to allow drug and biological product applications to be more rapidly reviewed by the U.S. Food and Drug Administration (FDA), with the FDA reviewing various portions of new drug and biological product applications as they become available.

(3) The approval pathway established under this Act establishes a clear approval pathway that can be utilized by sponsors to receive rolling review of applications for drugs and biological products intended to treat serious diseases, including drugs and biological products intended to treat COVID–19 that reduce the risk of death, severe disease, and progression of symptoms in those exposed to the virus.

(4) The approval pathway established under this Act will enable sponsors to receive early, time-limited, and provisional approval for drugs and bio-

logical products that have demonstrated substantial evidence of safety and relevant early evidence that establishes that the drug provides a positive therapeutic outcome.

(5) The approval pathway established under this Act will allow for the use of real-world evidence and scientifically substantiated surrogates, other than those previously validated by the FDA, to predict the clinical benefits and ultimately support provisional approval.

(6) Drugs and biological products granted provisional approval under the pathway established under this Act are limited to a 2-year approval period, renewable every 2 years, for up to 6 years. Full approval can awarded at any time, for any drug or biological product provisionally approved under this pathway that establishes a 15 percent improvement in an important endpoint compared to standard therapies.

(7) The approval pathway established under this Act prohibits denial of coverage for any drug or biological product provisionally approved under this approval pathway on account of it being experimental.

(8) Informed consent is required for any patients using a drug or biological product approved under the provisional approval pathway established under this Act. Any patients using a drug or biological product reviewed under this approval pathway must participate in an observational registry until those drugs or biological products receive full approval, with approval contingent on registry participation.

(9) This Act requires that registries track aggregated, de-identified data that will be readily available to approved researchers for public health research purposes.

(10) This Act creates, within the Office of the Commissioner at the FDA, the position of the Patient Advocate General to provide assistance to patients and their families utilizing drugs and biological products.

SEC. 3. PROVISIONAL APPROVAL OF NEW HUMAN DRUGS.

(a) IN GENERAL.—Subchapter A of chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351 et seq.) is amended by adding at the end the following:

"SEC. 524B. PROVISIONAL APPROVAL OF NEW HUMAN DRUGS.

"(a) PRIORITY REVIEW AND EVALUATION OF APPLICATIONS.—

"(1) IN GENERAL.—The Secretary shall establish a priority review system to evaluate applications submitted under this pathway for provisional approval within 90 days of receipt of a completed application.

"(2) REVIEW OF APPLICATIONS DURING EPIDEMICS AND PANDEMICS.—In the case of an epidemic or pandemic, including with respect to COVID–19, the Secretary shall accept and review various portions of an application submitted under the pathway under this section for provisional approval on a rolling basis, and the review of any part of an application so submitted shall be completed not later than 3 weeks after submission.

"(3) OTHER DESIGNATIONS.—If a drug submitted for review under the pathway under this section is eligible for a special designation by the Secretary under this Act, including as a drug for a rare disease or condition under section 526, all benefits of such other designation shall be available for use under provisional approval, including any tax credits and waiving of fees under chapter VII.

''(b) ELIGIBILITY.—A drug may be eligible for provisional approval under this section if the Secretary determines that the drug is intended for the treatment, prevention, or medical diagnosis of—

''(1) a serious or life-threatening disease or condition for which there is a reasonable likelihood that premature death will occur without early medical intervention for an individual contracting or being diagnosed with such disease or condition; or

''(2) a disease or condition that poses a threat of epidemic or pandemic.

''(c) STANDARD OF REVIEW FOR APPROVAL.—

''(1) REQUIREMENTS.—An application for provisional approval under this section may be approved only if the Secretary determines that—

''(A) there is substantial evidence of safety for the drug, such that there is evidence consisting of adequate and well-controlled investigations, including clinical investigations, by experts qualified by scientific training and experience to evaluate the safety of the drug involved, on the basis of which it could fairly and responsibly be concluded that the drug will have the effect it purports or is represented to have under the conditions of use prescribed, rec-

ommended, or suggested in the labeling or proposed labeling; and

''(B) there is relevant early evidence based on adequate and well-controlled investigations, including early-stage clinical investigations, to establish that—

''(i) the drug provides a positive therapeutic outcome; and

''(ii) the outcome of the drug is consistent with or greater than currently marketed on-label therapies, with equal or fewer side effects, if there are currently marketed on-label therapies.

''(2) PROTOCOLS.—The Secretary shall promulgate rules that establish the appropriate protocols for a sponsor of an application for provisional approval under this section and the Commissioner to follow to enable rolling, real-time, mid-trial submission while preserving the integrity of the ongoing trial and without penalizing the sponsor for making use of this pathway.

''(3) REAL WORLD EVIDENCE.—The Secretary shall allow the use of real world evidence (as defined in section 505F(b)), including real world data used to generate real world evidence, to support an appli-

cation for provisional approval under this section, and to fulfill the follow-up requirements and support applications for full approval as described under section 505 or section 351 of the Public Health Service Act, as applicable.

"(4) USE OF SCIENTIFICALLY SUBSTANTIATED SURROGATES.—

"(A) IN GENERAL.—The sponsor of an application for provisional approval under this section may use scientifically substantiated surrogates to support such application.

"(B) DEFINITION.—In subparagraph (A), the term 'scientifically substantiated surrogates' means surrogate endpoints to predict clinical benefit other than such endpoints previously validated by the Secretary, based on—

"(i) epidemiologic, therapeutic, pathophysiologic, or other evidence; or

"(ii) an effect on a clinical endpoint other than survival or irreversible morbidity of interest.

"(d) TRANSPARENCY AND PATIENT MONITORING REQUIREMENTS.—

"(1) REGISTRIES.—

''(A) IN GENERAL.—The sponsor of a drug provisionally approved under this section shall require that all patients who use such drug participate in an observational registry and consent to the sponsor's collection, and submission to the registry, of data related to the patient's use of such drug until such drug receives full approval under section 505 or section 351 of the Public Health Service Act, or the provisional approval is rescinded.

''(B) REQUIREMENTS FOR REGISTRIES.—An observational registry described in subparagraph (A) may be run by a third party, such as a government, for profit, or non-profit organization, and shall track all patients who use the provisionally approved drug.

''(C) ACCESSIBILITY.—An observational registry described in subparagraph (A) shall be easily accessible for—

''(i) all patients who are participating in any registry related to a provisionally approved drug that allows for easy, unrestricted (or transparent) access for such patients to their patient data and related

information regarding their usage of the provisionally approved drug; and

''(ii) approved researchers and medical professionals who may access data maintained in the registry, which access shall be for public health research and only in a de-identified, aggregated manner.

''(2) FUNDING.—An observational registry under this subsection shall be maintained, as applicable—

''(A) by the sponsor of the drug provisionally approved under this section that is the subject of the registry;

''(B) by a third party, such as a government, for profit, or nonprofit organization; or

''(C) the Federal Government, in the case of any drug so approved that is intended to treat a disease or condition associated with an epidemic or pandemic.

''(3) SPONSOR REQUIREMENTS.—

''(A) IN GENERAL.—For any drug application provisionally approved under this section, the Secretary shall notify the sponsor of the exact data such sponsor is required to submit to an observational registry.

"(B) ANNUAL REVIEW OF THE REGISTRY; PENALTIES.—The Secretary shall conduct an annual review of observational registries established under this subsection. If, at such an annual review, less than 90 percent of patients are participating in an observational registry with respect to a drug approved under this section, the Secretary shall issue to the sponsor of such drug a civil monetary penalty of not more than $100,000. If a violation of this section is not corrected within the 30-day period following notification, the sponsor shall, in addition to any penalty under this subparagraph be subject to a civil monetary penalty of not more than $10,000 for each day of the violation after such period until the violation is corrected. If application patient participation in an observational registry is not at or above 90 percent within 6 months of issuance of such penalty, the provisional approval shall be withdrawn.

"(4) ANNUAL REPORT TO CONGRESS.—The Secretary shall submit an annual report to Congress on all drugs granted provisional approval under this section. Such report shall include—

"(A) the number of patients treated with each such drug, and the number of patients tracked in an observational registry with respect to each such drug;

"(B) a discussion of the minimum amount of data required in the registries, including patient treatments and uses, length of use, side effects encountered, relevant biomarkers or scientifically substantiated surrogates, scan results, cause of death and how long the patient lived, and adverse drug effects;

"(C) a list of all such drugs for which an application for full approval under section 505 of this Act or section 351 of the Public Health Service Act, or an application for an extension of provisional approval under this section, has been submitted; and

"(D) a list of all applications denied provisional approval under this section, together with an explanation for the decisions to deny each such application.

"(e) WITHDRAWAL OF PROVISIONAL APPROVAL.—

"(1) IN GENERAL.—The Secretary shall withdraw provisional approval under this section if there are a significant numbers of patients who experience

serious adverse effects, compared to the other currently marketed on-label therapies that are available for the applicable disease or condition.

''(2) EFFECT OF WITHDRAWAL.—If a provisional approval is withdrawn under this subsection, the sponsor may not make the drug available to any new patients, but may be allowed to continue to make such drug available to patients who started taking the drug prior to the date of withdrawal, for as long a period as dictated by patient need, as determined by the Secretary.

''(f) TRANSPARENCY.—Any scientific, medical, academic, or health care journal publishing an article explaining, releasing, conveying or announcing research findings which were funded by the Department of Health and Human Services shall be prohibited from publishing such research unless—

''(1) such article conveying research findings is made publicly available on the journal's internet website without a paywall or charge not later than 3 months after the date on which such article was first provided to subscribers of such journal (or first made available for purchase); and

''(2) the article's author or researcher or author's institution (or, in the case of multiple authors,

researchers, or institutions, all such authors, researchers, or institutions) received less than 30 percent of funding for such research from the Department of Health and Human Services throughout the period of time the research was conducted.

''(g) INFORMED CONSENT.—Prior to receiving a drug provisionally approved under this section, the sponsor of the drug shall receive from each patient, or the patient's representative, informed consent, through a signed informed consent form, acknowledging that such patient understands that the drug did not undergo the usual process for full approval of a drug by the Food and Drug Administration, and that such patient is willing to accept the risks involved in taking such drug.

''(h) POSTMARKET CONTROLS AND LABELING.—

''(1) FDA ANNUAL REVIEW OF REGISTRY DATA.—The Secretary shall annually review the data made available through the observational registries under subsection (d) and make a determination regarding whether the side effect profile of any drug approved under this pathway does not support the benefit provided, or the data shows the benefit is less than the benefits offered through other, fully approved drugs.

''(2) LABELING.—The sponsor of the provisionally approved drug shall ensure that all labeling and promotional materials for the drug bear the statement 'provisionally approved by the FDA pending a full demonstration of effectiveness under application number _____' (specifying the application number assigned by the Secretary in place of the blank). All promotional, educational and marketing materials for provisionally approved products shall be reviewed and approved by the Secretary before such materials are distributed.

''(3) RESCISSION OF PROVISIONAL APPROVAL.—If the Secretary determines that the side effect profile of any drug included in such observational registries does not support the benefit provided by such drug, or that the data shows that the benefit is less than the benefits offered through other, fully approved drugs, the Secretary shall rescind such provisional approval.

''(i) DURATION OF PROVISIONAL APPROVAL; REQUIREMENT TO BRING DRUG TO MARKET.—

''(1) DURATION; RENEWALS.—The period of provisional approval for a drug approved under this section is effective for a 2-year period. The sponsor may request renewal for provisional approval status

for up to 3 subsequent 2-year periods by the Secretary. Provisional approval status with respect to a drug shall not exceed a total of 6 years from the initial date the sponsor was awarded provisional approval status.

''(2) MARKETING REQUIREMENT.—If any drug that receives provisional approval status under this section is not brought to market within 180 days of the approval, such approval shall be rescinded.

''(j) LIMITATION ON LIABILITY.—With respect to any claim under State law alleging that a drug sold or otherwise made available pursuant to a grant of provisional approval under this section is unsafe or ineffective, no liability in a cause of action shall lie against a sponsor or manufacturer, unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable State law.

''(k) APPLYING FOR FULL APPROVAL.—

''(1) IN GENERAL.—Except as provided under paragraph (2), the sponsor of a drug granted provisional approval pursuant to this section may, at any point, submit an application for full approval of such drug under section 505 of this Act or section 351 of the Public Health Service Act, as applicable.

''(2) EFFECT OF RECESSION ON APPROVAL AND AUTOMATIC APPROVAL.—

''(A) IN GENERAL.—The sponsor of a drug granted provisional approval pursuant to this section that has been rescinded under subsection (h)(3), may submit an application for full approval of such drug under section 505 of this Act or section 351 of the Public Health Service Act at any time.

''(B) AUTOMATIC APPROVAL.—Such full approval may be awarded at any time for any drug granted provisional approval pursuant to this section if the sponsor of the drug establishes a 15 percent improvement in an important endpoint, including surrogate endpoints not validated by the Food and Drug Administration, compared to a standard drug.

''(3) REAL-TIME EPIDEMIC AND PANDEMIC VACCINE APPROVAL.—

''(A) IN GENERAL.—In the case of a vaccine developed in response to an epidemic or pandemic, including COVID–19, the Secretary shall share data information regarding the approval of the vaccine with the Advisory Committee on Immunization Practices of the Cen-

ters for Disease Control and Prevention as the review nears completion.

''(B) EVALUATION.—Any vaccine that has been approved by the Secretary for an epidemic or pandemic-related disease, including COVID–19, shall be evaluated by the Advisory Committee on Immunization Practices of the Centers for Disease Control and Prevention not later than 1 week after the date of submission to the Advisory Committee by the Secretary of the vaccine.

''(l) PATIENT ADVOCATE GENERAL.—Not later than 6 months after the date of enactment of the Promising Pathway Act, the Secretary shall establish within the Office of the Commissioner, the position of Patient Advocate General, who shall provide assistance to patients and their families who use drugs under evaluation in this pathway or drugs reviewed or approved under section 505 or section 351 of the Public Health Service Act. Such assistance shall include providing bi-informational communication about maintaining patient health, delivery of proper informed consent, participating in clinical investigations, completing required documentation in order to participate in the applicable programs, and providing other information.''.

(b) CONFORMING AMENDMENT.—Section 505(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(a)) is amended by inserting ", or there is in effect a provisional approval under section 524B with respect to such drug" before the period.

(c) REIMBURSEMENT.—

(1) PRIVATE HEALTH INSURERS.—Section 2719A of the Public Health Service Act (42 U.S.C. 300gg–19a) is amended by adding at the end the following:

"(e) TREATMENT OF CERTAIN DRUGS.—A group health plan or health insurance issuer of group or individual health insurance coverage shall not deny coverage of any drug provisionally approved under section 524B of the Federal Food, Drug, and Cosmetic Act on the basis of such drug being experimental. In determining coverage under the applicable plan or coverage, a group health plan or health insurance issuer shall treat a drug provisionally approved under such section in the same manner as such plan or coverage would treat a drug approved under section 505 of the Federal Food, Drug, and Cosmetic Act or section 351 of this Act. Nothing in this subsection shall be construed to require a group health plan or health insurance issuer to cover any specific drug provisionally approved under such section 524B.".

(2) FEDERAL HEALTH CARE PROGRAMS.—The requirement under subsection (e) of section 2719A of the Public Health Service Act (as added by paragraph (1)) shall apply with respect to coverage determinations under a Federal health care program (as defined in section 1128B(f) of the Social Security Act (42 U.S.C. 1320a–7b(f))) in the same manner such requirement applies under such subsection (e).

(3) CONFORMING AMENDMENT.—Section 1927(k)(2)(A)(i) of the Social Security Act (42 U.S.C. 1396r–8(k)(2)(A)(i)) is amended—

(A) by striking ''or which'' and inserting '', which''; and

(B) by inserting '', or which is provisionally approved under section 524B of such Act'' before the semicolon.

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