

118TH CONGRESS
1ST SESSION

H. R. 4408

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 30, 2023

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

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.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Promising Pathway
5 Act”.

1 **SEC. 2. PROVISIONAL APPROVAL OF NEW HUMAN DRUGS.**

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

5 **“SEC. 524C. PROVISIONAL APPROVAL OF NEW HUMAN**
6 **DRUGS.**

7 “(a) PRIORITY REVIEW AND EVALUATION OF APPLI-
8 CATIONS.—

9 “(1) IN GENERAL.—The Secretary shall estab-
10 lish a priority review system to evaluate applications
11 submitted under this section for provisional approval
12 within 90 days of receipt of a completed application.

13 “(2) OTHER DESIGNATIONS.—If a drug sub-
14 mitted for review under this section is eligible for a
15 special designation by the Secretary under this Act,
16 including as a drug for a rare disease or condition
17 under section 526, all benefits of such other designa-
18 tion shall be available for use under provisional ap-
19 proval, including any tax credits and waiving of fees
20 under chapter VII.

21 “(b) ELIGIBILITY.—A drug may be eligible for provi-
22 sional approval under this section if the Secretary deter-
23 mines that the drug is intended for the treatment, preven-
24 tion, or medical diagnosis of a serious or life-threatening
25 disease or condition for which there is a reasonable likeli-
26 hood that premature death will occur without early med-

1 ical intervention for an individual contracting or being di-
2 agnosed with such disease or condition.

3 “(c) STANDARD OF REVIEW FOR PROVISIONAL AP-
4 PROVAL.—

5 “(1) REQUIREMENTS.—An application for pro-
6 visional approval under this section may be approved
7 only if the Secretary determines that—

8 “(A) there is substantial evidence of safety
9 for the drug, such that there is evidence con-
10 sisting of adequate and well-controlled inves-
11 tigations, including clinical investigations, by
12 experts qualified by scientific training and expe-
13 rience to evaluate the safety of the drug in-
14 volved, on the basis of which it could fairly and
15 responsibly be concluded that the drug will have
16 the effect it purports or is represented to have
17 under the conditions of use prescribed, rec-
18 ommended, or suggested in the labeling or pro-
19 posed labeling; and

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

24 “(i) the drug provides a positive
25 therapeutic outcome; and

1 “(ii) the outcome of the drug is con-
2 sistent with or greater than currently mar-
3 keted on-label therapies, with equal or
4 fewer side effects, if there are currently
5 marketed on-label therapies.

6 “(2) PROTOCOLS.—The Secretary shall promul-
7 gate rules that establish the appropriate protocols to
8 enable rolling, real-time, mid-trial submission while
9 preserving the integrity of the ongoing trial and
10 without penalizing the sponsor for seeking provi-
11 sional approval under this section.

12 “(3) REAL WORLD EVIDENCE.—The Secretary
13 shall allow the use of real world evidence (as defined
14 in section 505F(b)), including real world data used
15 to generate real world evidence, to support an appli-
16 cation for provisional approval under this section,
17 and to fulfill the follow-up requirements and support
18 applications for approval under section 505 or sec-
19 tion 351 of the Public Health Service Act, as appli-
20 cable.

21 “(4) USE OF SCIENTIFICALLY SUBSTANTIATED
22 SURROGATES.—

23 “(A) IN GENERAL.—The sponsor of an ap-
24 plication for provisional approval under this sec-

1 tion may use scientifically substantiated surro-
2 gates to support such application.

3 “(B) DEFINITION.—In subparagraph (A),
4 the term ‘scientifically substantiated surrogates’
5 means surrogate endpoints to predict clinical
6 benefit other than such endpoints previously
7 validated by the Secretary, based on—

8 “(i) epidemiologic, therapeutic, patho-
9 physiologic, or other evidence; or

10 “(ii) an effect on a clinical endpoint
11 other than survival or irreversible mor-
12 bidity of interest.

13 “(d) TRANSPARENCY AND PATIENT MONITORING
14 REQUIREMENTS.—

15 “(1) REGISTRIES.—

16 “(A) IN GENERAL.—The sponsor of a drug
17 provisionally approved under this section shall
18 require that all patients who use such drug par-
19 ticipate in an observational registry and consent
20 to the sponsor’s collection, and submission to
21 the registry, of data related to the patient’s use
22 of such drug until such drug receives approval
23 under section 505 or section 351 of the Public
24 Health Service Act, or the provisional approval
25 is rescinded.

1 “(B) REQUIREMENTS FOR REGISTRIES.—
2 An observational registry described in subpara-
3 graph (A) may be run by a third party, such as
4 a government, for profit, or nonprofit organiza-
5 tion, and shall track all patients who use the
6 provisionally approved drug.

7 “(C) ACCESSIBILITY.—An observational
8 registry described in subparagraph (A) shall be
9 easily accessible for—

10 “(i) all patients who are participating
11 in any registry related to a provisionally
12 approved drug that allows for easy, unre-
13 stricted (or transparent) access for such
14 patients to their patient data and related
15 information regarding their usage of the
16 provisionally approved drug; and

17 “(ii) approved researchers and med-
18 ical professionals who may access data
19 maintained in the registry, which access
20 shall be for public health research and only
21 in a de-identified, aggregated manner.

22 “(2) FUNDING.—An observational registry
23 under this subsection shall be maintained, as appli-
24 cable—

1 “(A) by the sponsor of the drug provision-
2 ally approved under this section that is the sub-
3 ject of the registry; or

4 “(B) by a third party, such as a govern-
5 ment, for profit, or nonprofit organization.

6 “(3) SPONSOR REQUIREMENTS.—

7 “(A) IN GENERAL.—For any drug applica-
8 tion provisionally approved under this section,
9 the Secretary shall notify the sponsor of the
10 exact data such sponsor is required to submit
11 to an observational registry.

12 “(B) ANNUAL REVIEW OF THE REGISTRY;
13 PENALTIES.—The Secretary shall conduct an
14 annual review of observational registries estab-
15 lished under this subsection. If, at such an an-
16 nual review, fewer than 90 percent of patients
17 are participating in an observational registry
18 with respect to a drug approved under this sec-
19 tion, the Secretary shall issue to the sponsor of
20 such drug a civil monetary penalty of not more
21 than \$100,000. If a violation of this section is
22 not corrected within the 30-day period following
23 notification, the sponsor shall, in addition to
24 any penalty under this subparagraph be subject
25 to a civil monetary penalty of not more than

1 \$10,000 for each day of the violation after such
2 period until the violation is corrected. If appli-
3 cation patient participation in an observational
4 registry is not at or above 90 percent within 6
5 months of issuance of such penalty, the provi-
6 sional approval shall be withdrawn.

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

11 “(A) the number of patients treated with
12 each such drug, and the number of patients
13 tracked in an observational registry with re-
14 spect to each such drug;

15 “(B) a discussion of the minimum amount
16 of data required in the registries, including pa-
17 tient treatments and uses, length of use, side
18 effects encountered, relevant biomarkers or sci-
19 entifically substantiated surrogates, scan re-
20 sults, cause of death and how long the patient
21 lived, and adverse drug effects;

22 “(C) a list of all such drugs for which an
23 application for approval under section 505 of
24 this Act or section 351 of the Public Health
25 Service Act, or an application for an extension

1 of provisional approval under this section, has
2 been submitted; and

3 “(D) a list of all applications denied provi-
4 sional approval under this section, together with
5 an explanation for the decisions to deny each
6 such application.

7 “(e) WITHDRAWAL OF PROVISIONAL APPROVAL.—

8 “(1) IN GENERAL.—The Secretary shall with-
9 draw provisional approval under this section if there
10 are a significant numbers of patients who experience
11 serious adverse effects, compared to the other cur-
12 rently marketed on-label therapies that are available
13 for the applicable disease or condition.

14 “(2) EFFECT OF WITHDRAWAL.—If a provi-
15 sional approval is withdrawn under this subsection,
16 the sponsor may not make the drug available to any
17 new patients, but may be allowed to continue to
18 make such drug available to patients who started
19 taking the drug prior to the date of withdrawal, for
20 as long a period as dictated by patient need, as de-
21 termined by the Secretary.

22 “(f) TRANSPARENCY.—Any scientific, medical, aca-
23 demic, or health care journal publishing an article explain-
24 ing, releasing, conveying, or announcing research findings
25 which were funded by the Department of Health and

1 Human Services shall be prohibited from publishing such
2 research unless—

3 “(1) such article conveying research findings is
4 made publicly available on the journal’s internet
5 website without a paywall or charge not later than
6 3 months after the date on which such article was
7 first provided to subscribers of such journal (or first
8 made available for purchase); and

9 “(2) the article’s author or researcher or au-
10 thor’s institution (or, in the case of multiple authors,
11 researchers, or institutions, all such authors, re-
12 searchers, or institutions) received less than 30 per-
13 cent of funding for such research from the Depart-
14 ment of Health and Human Services throughout the
15 period of time the research was conducted.

16 “(g) INFORMED CONSENT.—Prior to receiving a drug
17 provisionally approved under this section, the sponsor of
18 the drug shall receive from each patient, or the patient’s
19 representative, informed consent, through a signed in-
20 formed consent form, acknowledging that such patient un-
21 derstands that the drug did not undergo the usual process
22 for approval of a drug by the Food and Drug Administra-
23 tion, and that such patient is willing to accept the risks
24 involved in taking such drug.

25 “(h) POSTMARKET CONTROLS AND LABELING.—

1 “(1) FDA ANNUAL REVIEW OF REGISTRY
2 DATA.—The Secretary shall annually review the data
3 made available through the observational registries
4 under subsection (d) and make a determination re-
5 garding whether the side effect profile of any drug
6 provisionally approved under this section does not
7 support the benefit provided, or the data shows the
8 benefit is less than the benefits offered through
9 other, fully approved drugs.

10 “(2) LABELING.—The sponsor of the provision-
11 ally approved drug shall ensure that all labeling and
12 promotional materials for the drug bear the state-
13 ment ‘provisionally approved by the FDA pending a
14 full demonstration of effectiveness under application
15 number _____’ (specifying the application
16 number assigned by the Secretary in place of the
17 blank). All promotional, educational and marketing
18 materials for provisionally approved products shall
19 be reviewed and approved by the Secretary before
20 such materials are distributed.

21 “(3) RESCISSION OF PROVISIONAL AP-
22 PROVAL.—If the Secretary determines that the side
23 effect profile of any drug included in such observa-
24 tional registries does not support the benefit pro-
25 vided by such drug, or that the data shows that the

1 benefit is less than the benefits offered through
2 other drugs approved under section 505 of this Act
3 or section 351 of the Public Health Service Act, the
4 Secretary shall rescind such provisional approval.

5 “(i) DURATION OF PROVISIONAL APPROVAL; RE-
6 QUIREMENT TO BRING DRUG TO MARKET.—

7 “(1) DURATION; RENEWALS.—The provisional
8 approval for a drug under this section is effective for
9 a 2-year period. The sponsor may request renewal
10 for provisional approval status for up to 3 subse-
11 quent 2-year periods. Provisional approval status
12 with respect to a drug shall not exceed a total of 8
13 years from the initial date the sponsor was awarded
14 provisional approval status.

15 “(2) MARKETING REQUIREMENT.—If any drug
16 that receives provisional approval under this section
17 is not brought to market within 180 days of the pro-
18 visional approval, such provisional approval shall be
19 rescinded.

20 “(j) LIMITATION ON LIABILITY.—With respect to any
21 claim under State law alleging that a drug sold or other-
22 wise made available pursuant to a grant of provisional ap-
23 proval under this section is unsafe or ineffective, no liabil-
24 ity in a cause of action shall lie against a sponsor or manu-
25 facturer, unless the relevant conduct constitutes reckless

1 or willful misconduct, gross negligence, or an intentional
2 tort under any applicable State law.

3 “(k) RIGHT TO PETITION AN ADVISORY COMMITTEE
4 FOR APPROVAL.—

5 “(1) IN GENERAL.—The sponsor of a drug
6 granted provisional approval pursuant to this section
7 may request, at any time after provisional approval
8 is granted under this section, a meeting with the ap-
9 propriate advisory committee (or advisory commit-
10 tees) to present safety and efficacy data for the pur-
11 poses of receiving a recommendation from such an
12 advisory committee for approval under section 505
13 of this Act or section 351 of the Public Health Serv-
14 ice Act of the provisionally approved drug. Such a
15 requested meeting shall be granted not later than 90
16 days after a request is made. Nothing in this para-
17 graph shall be construed to alter the processes and
18 timeframes for recommendation for approval by such
19 an advisory committee of the provisionally approved
20 drug or for approval of the provisionally approved
21 drug under section 505 of this Act or section 351
22 of the Public Health Service Act.

23 “(2) WAIVER OF ADEQUATE AND WELL-CON-
24 TROLLED STUDY REQUIREMENTS.—

1 “(A) IN GENERAL.—In considering wheth-
2 er to recommend a drug that was provisionally
3 approved under this section for approval under
4 section 505, the Director of the Center for
5 Drug Evaluation and Research shall consider
6 the option to waive requirements for adequate
7 and well-controlled studies in accordance with
8 the process described in section 314.126(c) of
9 title 21, Code of Federal Regulations (or suc-
10 cessor regulations).

11 “(B) BIOLOGICAL PRODUCTS.—In consid-
12 ering whether to recommend a biological prod-
13 uct that was provisionally approved under this
14 section for licensure under section 351 of the
15 Public Health Service Act, the Director of the
16 Center for Biologics Evaluation and Research
17 may, and shall consider the option to, waive re-
18 quirements, as applicable, for adequate and
19 well-controlled studies for such biological prod-
20 uct in accordance with the process described in
21 section 314.126(c) of title 21, Code of Federal
22 Regulations (or successor regulations).”.

23 (b) CONFORMING AMENDMENT.—Section 505(a) of
24 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
25 355(a)) is amended by inserting “, or there is in effect

1 a provisional approval under section 524C with respect to
2 such drug” before the period.

3 (c) REIMBURSEMENT.—

4 (1) PRIVATE HEALTH INSURERS.—Section
5 2719A of the Public Health Service Act (42 U.S.C.
6 300gg–19a) is amended by adding at the end the
7 following:

8 “(f) TREATMENT OF CERTAIN DRUGS.—A group
9 health plan or health insurance issuer of group or indi-
10 vidual health insurance coverage shall not deny coverage
11 of any drug provisionally approved under section 524C of
12 the Federal Food, Drug, and Cosmetic Act on the basis
13 of such drug being experimental. In determining coverage
14 under the applicable plan or coverage, a group health plan
15 or health insurance issuer shall treat a drug provisionally
16 approved under such section in the same manner as such
17 plan or coverage would treat a drug approved under sec-
18 tion 505 of the Federal Food, Drug, and Cosmetic Act
19 or section 351 of this Act. Nothing in this subsection shall
20 be construed to require a group health plan or health in-
21 surance issuer to cover any specific drug provisionally ap-
22 proved under such section 524C.”.

23 (2) FEDERAL HEALTH CARE PROGRAMS.—The
24 requirement under subsection (f) of section 2719A
25 of the Public Health Service Act (as added by para-

1 graph (1)) shall apply with respect to coverage de-
2 terminations under a Federal health care program
3 (as defined in section 1128B(f) of the Social Secu-
4 rity Act (42 U.S.C. 1320a–7b(f))) in the same man-
5 ner such requirement applies under such subsection
6 (f).

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

10 (A) by striking “or which” and inserting “,
11 which”; and

12 (B) by inserting “, or which is provision-
13 ally approved under section 524C of such Act”
14 before the semicolon.

○