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(Original Signature of Member)

117TH CONGRESS
1ST SESSION

H. R.

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

Mr. GALLAGHER introduced the following bill; which was referred to the
Committee on _____

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”.

6 **SEC. 2. FINDINGS.**

7 Congress finds as follows:

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

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

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

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

1 logical products that have demonstrated substantial
2 evidence of safety and relevant early evidence that
3 establishes that the drug provides a positive thera-
4 peutic outcome.

5 (5) The approval pathway established under
6 this Act will allow for the use of real-world evidence
7 and scientifically substantiated surrogates, other
8 than those previously validated by the FDA, to pre-
9 dict the clinical benefits and ultimately support pro-
10 visional approval.

11 (6) Drugs and biological products granted pro-
12 visional approval under the pathway established
13 under this Act are limited to a 2-year approval pe-
14 riod, renewable every 2 years, for up to 6 years. Full
15 approval can awarded at any time, for any drug or
16 biological product provisionally approved under this
17 pathway that establishes a 15 percent improvement
18 in an important endpoint compared to standard
19 therapies.

20 (7) The approval pathway established under
21 this Act prohibits denial of coverage for any drug or
22 biological product provisionally approved under this
23 approval pathway on account of it being experi-
24 mental.

1 (8) Informed consent is required for any pa-
2 tients using a drug or biological product approved
3 under the provisional approval pathway established
4 under this Act. Any patients using a drug or biologi-
5 cal product reviewed under this approval pathway
6 must participate in an observational registry until
7 those drugs or biological products receive full ap-
8 proval, with approval contingent on registry partici-
9 pation.

10 (9) This Act requires that registries track ag-
11 gregated, de-identified data that will be readily avail-
12 able to approved researchers for public health re-
13 search purposes.

14 (10) This Act creates, within the Office of the
15 Commissioner at the FDA, the position of the Pa-
16 tient Advocate General to provide assistance to pa-
17 tients and their families utilizing drugs and biologi-
18 cal products.

19 **SEC. 3. PROVISIONAL APPROVAL OF NEW HUMAN DRUGS.**

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

1 **“SEC. 524B. PROVISIONAL APPROVAL OF NEW HUMAN**
2 **DRUGS.**

3 “(a) PRIORITY REVIEW AND EVALUATION OF APPLI-
4 CATIONS.—

5 “(1) IN GENERAL.—The Secretary shall estab-
6 lish a priority review system to evaluate applications
7 submitted under this pathway for provisional ap-
8 proval within 90 days of receipt of a completed ap-
9 plication.

10 “(2) REVIEW OF APPLICATIONS DURING
11 EPIDEMICS AND PANDEMICS.—In the case of an epi-
12 demic or pandemic, including with respect to
13 COVID–19, the Secretary shall accept and review
14 various portions of an application submitted under
15 the pathway under this section for provisional ap-
16 proval on a rolling basis, and the review of any part
17 of an application so submitted shall be completed
18 not later than 3 weeks after submission.

19 “(3) OTHER DESIGNATIONS.—If a drug sub-
20 mitted for review under the pathway under this sec-
21 tion is eligible for a special designation by the Sec-
22 retary under this Act, including as a drug for a rare
23 disease or condition under section 526, all benefits
24 of such other designation shall be available for use
25 under provisional approval, including any tax credits
26 and waiving of fees under chapter VII.

1 “(b) ELIGIBILITY.—A drug may be eligible for provi-
2 sional approval under this section if the Secretary deter-
3 mines that the drug is intended for the treatment, preven-
4 tion, or medical diagnosis of—

5 “(1) a serious or life-threatening disease or con-
6 dition for which there is a reasonable likelihood that
7 premature death will occur without early medical
8 intervention for an individual contracting or being
9 diagnosed with such disease or condition; or

10 “(2) a disease or condition that poses a threat
11 of epidemic or pandemic.

12 “(c) STANDARD OF REVIEW FOR APPROVAL.—

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

16 “(A) there is substantial evidence of safety
17 for the drug, such that there is evidence con-
18 sisting of adequate and well-controlled inves-
19 tigation, including clinical investigations, by
20 experts qualified by scientific training and expe-
21 rience to evaluate the safety of the drug in-
22 volved, on the basis of which it could fairly and
23 responsibly be concluded that the drug will have
24 the effect it purports or is represented to have
25 under the conditions of use prescribed, rec-

1 ommended, or suggested in the labeling or pro-
2 posed labeling; and

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

7 “(i) the drug provides a positive
8 therapeutic outcome; and

9 “(ii) the outcome of the drug is con-
10 sistent with or greater than currently mar-
11 keted on-label therapies, with equal or
12 fewer side effects, if there are currently
13 marketed on-label therapies.

14 “(2) PROTOCOLS.—The Secretary shall promul-
15 gate rules that establish the appropriate protocols
16 for a sponsor of an application for provisional ap-
17 proval under this section and the Commissioner to
18 follow to enable rolling, real-time, mid-trial submis-
19 sion while preserving the integrity of the ongoing
20 trial and without penalizing the sponsor for making
21 use of this pathway.

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

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

6 “(4) USE OF SCIENTIFICALLY-SUBSTANTIATED
7 SURROGATES.—

8 “(A) IN GENERAL.—The sponsor of an ap-
9 plication for provisional approval under this sec-
10 tion may use scientifically-substantiated surro-
11 gates to support such application.

12 “(B) DEFINITION.—In subparagraph (A),
13 the term ‘scientifically-substantiated surrogates’
14 means surrogate endpoints to predict clinical
15 benefit other than such endpoints previously
16 validated by the Secretary, based on—

17 “(i) epidemiologic, therapeutic, patho-
18 physiologic, or other evidence; or

19 “(ii) an effect on a clinical endpoint
20 other than survival or irreversible mor-
21 bidity of interest.

22 “(d) TRANSPARENCY AND PATIENT MONITORING
23 REQUIREMENTS.—

24 “(1) REGISTRIES.—

1 “(A) IN GENERAL.—The sponsor of a drug
2 provisionally approved under this section shall
3 require that all patients who use such drug par-
4 ticipate in an observational registry and consent
5 to the sponsor’s collection, and submission to
6 the registry, of data related to the patient’s use
7 of such drug until such drug receives full ap-
8 proval under section 505 or section 351 of the
9 Public Health Service Act, or the provisional
10 approval is rescinded.

11 “(B) REQUIREMENTS FOR REGISTRIES.—
12 An observational registry described in subpara-
13 graph (A) may be run by a third party, such as
14 a government, for profit, or non-profit organiza-
15 tion, and shall track all patients who use the
16 provisionally approved drug.

17 “(C) ACCESSIBILITY.—An observational
18 registry described in subparagraph (A) shall be
19 easily accessible for—

20 “(i) all patients who are participating
21 in any registry related to a provisionally
22 approved drug that allows for easy, unre-
23 stricted (or transparent) access for such
24 patients to their patient data and related

1 information regarding their usage of the
2 provisionally approved drug; and

3 “(ii) approved researchers and med-
4 ical professionals who may access data
5 maintained in the registry, which access
6 shall be for public health research and only
7 in a de-identified, aggregated manner.

8 “(2) FUNDING.—An observational registry
9 under this subsection shall be maintained, as appli-
10 cable—

11 “(A) by the sponsor of the drug provision-
12 ally approved under this section that is the sub-
13 ject of the registry;

14 “(B) by a third party, such as a govern-
15 ment, for profit, or nonprofit organization; or

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

20 “(3) SPONSOR REQUIREMENTS.—

21 “(A) IN GENERAL.—For any drug applica-
22 tion provisionally approved under this section,
23 the Secretary shall notify the sponsor of the
24 exact data such sponsor is required to submit
25 to an observational registry.

1 “(B) ANNUAL REVIEW OF THE REGISTRY;
2 PENALTIES.—The Secretary shall conduct an
3 annual review of observational registries estab-
4 lished under this subsection. If, at such an an-
5 nual review, less than 90 percent of patients are
6 participating in an observational registry with
7 respect to a drug approved under this section,
8 the Secretary shall issue to the sponsor of such
9 drug a civil monetary penalty of not more than
10 \$100,000. If a violation of this section is not
11 corrected within the 30-day period following no-
12 tification, the sponsor shall, in addition to any
13 penalty under this subparagraph be subject to
14 a civil monetary penalty of not more than
15 \$10,000 for each day of the violation after such
16 period until the violation is corrected. If appli-
17 cation patient participation in an observational
18 registry is not at or above 90 percent within 6
19 months of issuance of such penalty, the provi-
20 sional approval shall be withdrawn.

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

1 “(A) the number of patients treated with
2 each such drug, and the number of patients
3 tracked in an observational registry with re-
4 spect to each such drug;

5 “(B) a discussion of the minimum amount
6 of data required in the registries, including pa-
7 tient treatments and uses, length of use, side
8 effects encountered, relevant biomarkers or sci-
9 entifically substantiated surrogates, scan re-
10 sults, cause of death and how long the patient
11 lived, and adverse drug effects;

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

18 “(D) a list of all applications denied provi-
19 sional approval under this section, together with
20 an explanation for the decisions to deny each
21 such application.

22 “(e) WITHDRAWAL OF PROVISIONAL APPROVAL.—

23 “(1) IN GENERAL.—The Secretary shall with-
24 draw provisional approval under this section if there
25 are a significant numbers of patients who experience

1 serious adverse effects, compared to the other cur-
2 rently marketed on-label therapies that are available
3 for the applicable disease or condition.

4 “(2) EFFECT OF WITHDRAWAL.—If a provi-
5 sional approval is withdrawn under this subsection,
6 the sponsor may not make the drug available to any
7 new patients, but may be allowed to continue to
8 make such drug available to patients who started
9 taking the drug prior to the date of withdrawal, for
10 as long a period as dictated by patient need, as de-
11 termined by the Secretary.

12 “(f) TRANSPARENCY.—Any scientific, medical, aca-
13 demic, or health care journal publishing an article explain-
14 ing, releasing, conveying or announcing research findings
15 which were funded by the Department of Health and
16 Human Services shall be prohibited from publishing such
17 research unless—

18 “(1) such article conveying research findings is
19 made publicly available on the journal’s internet
20 website without a paywall or charge not later than
21 3 months after the date on which such article was
22 first provided to subscribers of such journal (or first
23 made available for purchase); and

24 “(2) the article’s author or researcher or au-
25 thor’s institution (or, in the case of multiple authors,

1 researchers, or institutions, all such authors, re-
2 searchers, or institutions) received less than 30 per-
3 cent of funding for such research from the Depart-
4 ment of Health and Human Services throughout the
5 period of time the research was conducted.

6 “(g) INFORMED CONSENT.—Prior to receiving a drug
7 provisionally approved under this section, the sponsor of
8 the drug shall receive from each patient, or the patient’s
9 representative, informed consent, through a signed in-
10 formed consent form, acknowledging that such patient un-
11 derstands that the drug did not undergo the usual process
12 for full approval of a drug by the Food and Drug Adminis-
13 tration, and that such patient is willing to accept the risks
14 involved in taking such drug.

15 “(h) POSTMARKET CONTROLS AND LABELING.—

16 “(1) FDA ANNUAL REVIEW OF REGISTRY
17 DATA.—The Secretary shall annually review the data
18 made available through the observational registries
19 under subsection (d) and make a determination re-
20 garding whether the side effect profile of any drug
21 approved under this pathway does not support the
22 benefit provided, or the data shows the benefit is
23 less than the benefits offered through other, fully-ap-
24 proved drugs.

1 “(2) LABELING.—The sponsor of the provision-
2 ally approved drug shall ensure that all labeling and
3 promotional materials for the drug bear the state-
4 ment ‘provisionally approved by the FDA pending a
5 full demonstration of effectiveness under application
6 number _____’ (specifying the application
7 number assigned by the Secretary in place of the
8 blank). All promotional, educational and marketing
9 materials for provisionally approved products shall
10 be reviewed and approved by the Secretary before
11 such materials are distributed.

12 “(3) RESCISSION OF PROVISIONAL AP-
13 PROVAL.—If the Secretary determines that the side
14 effect profile of any drug included in such observa-
15 tional registries does not support the benefit pro-
16 vided by such drug, or that the data shows that the
17 benefit is less than the benefits offered through
18 other, fully-approved drugs, the Secretary shall re-
19 scind such provisional approval.

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

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

1 for up to 3 subsequent 2-year periods by the Sec-
2 retary. Provisional approval status with respect to a
3 drug shall not exceed a total of 6 years from the ini-
4 tial date the sponsor was awarded provisional ap-
5 proval status.

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

10 “(j) **LIMITATION ON LIABILITY.**—With respect to any
11 claim under State law alleging that a drug sold or other-
12 wise made available pursuant to a grant of provisional ap-
13 proval under this section is unsafe or ineffective, no liabil-
14 ity in a cause of action shall lie against a sponsor or manu-
15 facturer, unless the relevant conduct constitutes reckless
16 or willful misconduct, gross negligence, or an intentional
17 tort under any applicable State law.

18 “(k) **APPLYING FOR FULL APPROVAL.**—

19 “(1) **IN GENERAL.**—Except as provided under
20 paragraph (2), the sponsor of a drug granted provi-
21 sional approval pursuant to this section may, at any
22 point, submit an application for full approval of such
23 drug under section 505 of this Act or section 351
24 of the Public Health Service Act, as applicable.

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

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

10 “(B) AUTOMATIC APPROVAL.—Such full
11 approval may be awarded at any time for any
12 drug granted provisional approval pursuant to
13 this section if the sponsor of the drug estab-
14 lishes a 15 percent improvement in an impor-
15 tant endpoint, including surrogate endpoints
16 not validated by the Food and Drug Adminis-
17 tration, compared to a standard drug.

18 “(3) REAL-TIME EPIDEMIC AND PANDEMIC VAC-
19 CINE APPROVAL.—

20 “(A) IN GENERAL.—In the case of a vac-
21 cine developed in response to an epidemic or
22 pandemic, including COVID–19, the Secretary
23 shall share data information regarding the ap-
24 proval of the vaccine with the Advisory Com-
25 mittee on Immunization Practices of the Cen-

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

3 “(B) EVALUATION.—Any vaccine that has
4 been approved by the Secretary for an epidemic
5 or pandemic-related disease, including COVID–
6 19, shall be evaluated by the Advisory Com-
7 mittee on Immunization Practices of the Cen-
8 ters for Disease Control and Prevention not
9 later than 1 week after the date of submission
10 to the Advisory Committee by the Secretary of
11 the vaccine.

12 “(I) PATIENT ADVOCATE GENERAL.—Not later than
13 6 months after the date of enactment of the Promising
14 Pathway Act, the Secretary shall establish within the Of-
15 fice of the Commissioner, the position of Patient Advocate
16 General, who shall provide assistance to patients and their
17 families who use drugs under evaluation in this pathway
18 or drugs reviewed or approved under section 505 or sec-
19 tion 351 of the Public Health Service Act. Such assistance
20 shall include providing bi-informational communication
21 about maintaining patient health, delivery of proper in-
22 formed consent, participating in clinical investigations,
23 completing required documentation in order to participate
24 in the applicable programs, and providing other informa-
25 tion.”.

1 (b) CONFORMING AMENDMENT.—Section 505(a) of
2 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
3 355(a)) is amended by inserting “, or there is in effect
4 a provisional approval under section 524B with respect to
5 such drug” before the period.

6 (c) REIMBURSEMENT.—

7 (1) PRIVATE HEALTH INSURERS.—Section
8 2719A of the Public Health Service Act (42 U.S.C.
9 300gg–19a) is amended by adding at the end the
10 following:

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

1 (2) FEDERAL HEALTH CARE PROGRAMS.—The
2 requirement under subsection (e) of section 2719A
3 of the Public Health Service Act (as added by para-
4 graph (1)) shall apply with respect to coverage de-
5 terminations under a Federal health care program
6 (as defined in section 1128B(f) of the Social Secu-
7 rity Act (42 U.S.C. 1320a–7b(f))) in the same man-
8 ner such requirement applies under such subsection
9 (e).

10 (3) CONFORMING AMENDMENT.—Section
11 1927(k)(2)(A)(i) of the Social Security Act (42
12 U.S.C. 1396r–8(k)(2)(A)(i)) is amended—

13 (A) by striking “or which” and inserting “,
14 which”; and

15 (B) by inserting “, or which is provision-
16 ally approved under section 524B of such Act”
17 before the semicolon.