

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.

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## 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.

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 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 tigations, 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  
24 approved 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.

○