

118TH CONGRESS
2D SESSION

S. 4426

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

IN THE SENATE OF THE UNITED STATES

MAY 23, 2024

Mr. BRAUN (for himself, Mrs. GILLIBRAND, Mr. CRAMER, Mr. MANCHIN, Mr. SCHMITT, Mr. PADILLA, Mr. VANCE, Mr. BOOKER, Mr. HAWLEY, Mr. WELCH, and Ms. MURKOWSKI) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish a time-limited conditional 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 2.0”.

1 **SEC. 2. CONDITIONAL APPROVAL OF NEW HUMAN DRUGS**
2 **FOR INDIVIDUALS WITH RARE, PROGRES-**
3 **SIVE, AND SERIOUS DISEASES.**

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

7 **“SEC. 524C. CONDITIONAL APPROVAL OF HUMAN DRUGS**
8 **FOR INDIVIDUALS WITH RARE, PROGRES-**
9 **SIVE, AND SERIOUS DISEASES.**

10 “(a) **CONDITIONAL APPROVAL; PRIORITY REVIEW;**
11 **OTHER DESIGNATIONS.—**

12 “(1) IN GENERAL.—The sponsor of a drug may
13 file with the Secretary an application for conditional
14 approval of an eligible drug described in subsection
15 (b). The Secretary shall approve or deny such appli-
16 cation in accordance with subsection (c).

17 “(2) PRIORITY REVIEW.—The Secretary shall
18 give priority review to an application for conditional
19 approval of an eligible drug described in subsection
20 (b).

21 “(3) OTHER DESIGNATIONS.—If a drug that is
22 granted conditional approval under this section is el-
23 igible for a special designation by the Secretary
24 under this Act, including as a drug for a rare dis-
25 ease or condition under section 526, all applicable
26 benefits of such other designation shall be available

1 for use under such conditional approval, including
2 any tax credits and waiving of fees under chapter
3 VII.

4 “(4) OTHER PROGRAMS.—A sponsor of a drug
5 seeking conditional approval of such drug under this
6 section may also seek designation, exclusivity, or ap-
7 proval, as applicable, of such drug under other appli-
8 cable provisions of this Act or the Public Health
9 Service Act, subject to the requirements of such pro-
10 visions.

11 “(b) ELIGIBILITY.—

12 “(1) IN GENERAL.—A drug may be eligible for
13 conditional approval under this section if such drug
14 is intended to treat a disease or condition that is—

15 “(A) rapidly progressive, terminal, and has
16 substantial unmet medical need, as determined
17 by the Secretary; or

18 “(B) a rare disease or condition (as de-
19 fined in section 526(a)(2)) that results in a
20 substantially shortened lifespan, substantial re-
21 duction in quality of life, or other substantial
22 adverse health effects, as determined by the
23 Secretary.

24 “(2) EXCLUSION FROM ELIGIBILITY.—A drug
25 that is intended to treat or respond to a material

1 threat identified by the Secretary of Homeland Secu-
2 rity under section 319F-2(c)(2)(A)(ii) shall not be
3 eligible for conditional approval under this section.

4 “(c) STANDARD OF REVIEW FOR CONDITIONAL AP-
5 PROVAL.—

6 “(1) REQUIREMENTS.—The Secretary shall
7 only approve an application for conditional approval
8 of a drug under this section if—

9 “(A) the Secretary determines that—

10 “(i)(I) evidence of safety for the drug
11 has been established by—

12 “(aa) the completion of a phase 1
13 clinical investigation of the drug (as
14 described in section 312.21 of title 21,
15 Code of Federal Regulations (or suc-
16 cessor regulations)); or

17 “(bb) another demonstration of
18 safety, as determined appropriate by
19 the Secretary; and

20 “(II) evidence of effectiveness in
21 treating a given indication (which indica-
22 tion is congruent with the eligibility re-
23 quirements of subsection (b)), as estab-
24 lished by an ongoing or completed phase 2
25 clinical investigation of the drug (as de-

1 scribed in section 312.21 of title 21, Code
2 of Federal Regulations (or successor regu-
3 lations)); or

4 “(ii) in the case of a drug that is in-
5 tended to treat a terminal pediatric rare
6 disease or condition (as defined in section
7 526(a)(2)) that does not predominately af-
8 fect adults—

9 “(I) evidence of safety for the
10 drug has been established in accord-
11 ance with clause (i)(I); and

12 “(II) the drug shows preliminary
13 evidence of clinical effectiveness based
14 upon studies in animal models; and

15 “(B) the sponsor has provided a written
16 affirmation of the sponsor’s intent to pursue
17 under section 505 of this Act or section 351 of
18 the Public Health Service Act approval of the
19 drug, which affirmation shall include a justifica-
20 tion and a plan for pursuing such approval.

21 “(2) ROLLING, REAL-TIME REVIEW.—

22 “(A) IN GENERAL.—If the Secretary deter-
23 mines, after preliminary evaluation of data sub-
24 mitted by the sponsor, that a drug may meet
25 the standard for conditional approval, the spon-

1 sor may submit portions of an application for
2 conditional approval of a drug under this sec-
3 tion for evaluation by the Secretary before the
4 sponsor submits a complete application, which
5 submission shall include—

6 “(i) a schedule for submission of in-
7 formation necessary to make the applica-
8 tion complete; and

9 “(ii) a payment of any fee that may
10 be required under section 736.

11 “(B) REVIEW.—The Secretary—

12 “(i) shall evaluate each application
13 submitted under subparagraph (A) to as-
14 sess whether such application is complete
15 or ready to be filed; and

16 “(ii) may commence review of portions
17 of such application for approval.

18 “(3) USE OF REAL-WORLD EVIDENCE.—

19 “(A) IN GENERAL.—The Secretary shall
20 allow the use of real world evidence (as defined
21 in section 505F(b)), including real world data
22 used to generate real world evidence, and of ex-
23 ternal sources of data, including prospective or
24 retrospective natural history data, to support an

1 application for conditional approval under this
2 section.

3 “(B) DATA INTEGRITY REQUIREMENTS.—
4 In using evidence described in subparagraph
5 (A) to support an application for conditional
6 approval under this section, the sponsor shall
7 consider the guidance of the Food and Drug
8 Administration entitled ‘Data Standards for
9 Drug and Biological Product Submissions Con-
10 taining Real-World Data’ and dated December
11 2023 (or successor guidance).

12 “(d) FDA AUTHORITY TO WITHDRAW CONDITIONAL
13 APPROVAL.—

14 “(1) IN GENERAL.—The Secretary may with-
15 draw the conditional approval of a drug under this
16 section if—

17 “(A) after adequate review of appropriate
18 safety data, including data from an observa-
19 tional registry established under subsection (g),
20 the Secretary determines that such data no
21 longer supports conditional approval;

22 “(B) the Secretary determines that the ap-
23 plication for conditional approval submitted
24 under subsection (a)(1) contained an untrue
25 statement of material fact; or

1 “(C) the Secretary determines that the
2 drug is no longer eligible under subsection (b).

3 “(2) FDA EXAMINATION AUTHORITY.—

4 “(A) IN GENERAL.—For purposes deter-
5 mining whether to withdraw the conditional ap-
6 proval of a drug under paragraph (1), the Sec-
7 retary may—

8 “(i) review any available clinical data
9 made available through clinical trials or an
10 observational registry under subsection (g),
11 applicable to such drug; and

12 “(ii) determine whether the sponsor of
13 such drug is in violation of a requirement
14 established under paragraph (3) or (4) of
15 section 505(o) or section 505–1 with re-
16 spect to the drug.

17 “(B) TRANSPARENCY.—

18 “(i) IN GENERAL.—The Secretary
19 may require drug sponsors and observa-
20 tional registries under subsection (g) to
21 submit the data described in subparagraph
22 (A) for the purposes of the review under
23 that subparagraph.

24 “(ii) FINES.—The Secretary may levy
25 fines on sponsors and observational reg-

1 istries that do not comply with a request
2 for data under clause (i) within such rea-
3 sonable timeframe as is established by the
4 Secretary.

5 “(3) EFFECT OF WITHDRAWAL.—

6 “(A) AVAILABILITY TO NEW PATIENTS.—

7 “(i) IN GENERAL.—If a conditional
8 approval is withdrawn under this sub-
9 section, the sponsor may not make the
10 drug available to any new patients, but
11 may continue to make such drug available
12 to patients who started taking the drug
13 prior to the date of withdrawal.

14 “(ii) EFFECT.—Nothing in this sub-
15 paragraph shall be construed to require—

16 “(I) a patient to continue taking
17 a conditionally approved drug if such
18 patient decides to stop taking such
19 drug; or

20 “(II) the sponsor to ensure such
21 drug continues to be manufactured
22 after the date of withdrawal.

23 “(B) CIVIL MONETARY PENALTY.—Any
24 sponsor who makes available to new patients a
25 drug for which conditional approval has been

1 withdrawn under this subsection shall be sub-
2 ject to such civil monetary penalty as is deter-
3 mined by the Secretary.

4 “(4) WITHDRAWAL NOTICE.—Upon deter-
5 mining to withdraw the conditional approval of a
6 drug under paragraph (1), the Secretary shall sub-
7 mit written notice to the sponsor of such drug and
8 such withdrawal shall be effective on the date that
9 is 14 days after the date of such submission of no-
10 tice.

11 “(5) APPEALS.—Not later than 180 days after
12 the date of enactment of the Promising Pathway Act
13 2.0, the Secretary, by rule, shall establish a process
14 by which a sponsor of a drug for which conditional
15 approval was withdrawn under paragraph (1) may
16 appeal such withdrawal.

17 “(6) AUTOMATIC WITHDRAWAL.—

18 “(A) IN GENERAL.—If the sponsor of a
19 drug that receives conditional approval under
20 this section does not submit an application for
21 renewal of such conditional approval under sub-
22 section (f)(2) by the deadline under that sub-
23 section, such conditional approval shall auto-
24 matically be withdrawn in accordance with

1 paragraph (3) on the date on which such condi-
2 tional approval expires.

3 “(B) MARKETING REQUIREMENT.—If any
4 drug that receives conditional approval under
5 this section is not brought to market within 1
6 year of the date on which the conditional ap-
7 proval is granted, such conditional approval,
8 along with any benefits described in subsection
9 (a)(3), shall automatically be withdrawn in ac-
10 cordance with paragraph (3) on such date.

11 “(C) NO RIGHT TO APPEAL; EFFECT OF
12 AUTOMATIC WITHDRAWAL.—

13 “(i) IN GENERAL.—A sponsor shall
14 not have the right to appeal an automatic
15 withdrawal under this paragraph.

16 “(ii) EFFECT.—The Secretary shall
17 have no means or power to prevent an
18 automatic withdrawal under this para-
19 graph from occurring.

20 “(e) LABELING; REVIEW OF MATERIALS.—

21 “(1) IN GENERAL.—Sponsors may not make
22 available to patients a drug conditionally approved
23 under this section, unless—

24 “(A) all labeling and advertising of such
25 drug contains the statement ‘conditionally ap-

1 proved for a limited population' in a prominent
2 manner and adjacent to, and not more promi-
3 nent than—

4 “(i) the proprietary name of such
5 drug, if any; or

6 “(ii) if there is no proprietary name,
7 the established name of such drug, if any,
8 as defined in section 502(e)(3), or, in the
9 case of a drug that is a biological product,
10 the proper name, as defined by regulation;
11 and

12 “(B) the prescribing information for the
13 drug required by section 201.57 of title 21,
14 Code of Federal Regulations (or any successor
15 regulation) includes the following statement:
16 ‘This drug is conditionally approved for use in
17 a limited and specific population. This drug has
18 not received full approval by the Food and
19 Drug Administration. Conditional approval of
20 this drug may be withdrawn at short notice.’

21 “(2) SUBMISSION.—Not later than 45 days be-
22 fore such materials are distributed, all promotional,
23 educational, and marketing materials for such drug
24 shall be submitted to the Secretary for review.

1 “(3) PUBLIC LIST.—The Secretary shall main-
2 tain a list of all drugs conditionally approved under
3 this section on a publicly accessible website. Such
4 website shall briefly describe what each conditionally
5 approved drugs is and list the 1 or more diseases or
6 conditions for which the drug is indicated.

7 “(f) RENEWAL OF CONDITIONAL APPROVAL; RE-
8 QUIREMENT TO BRING DRUG TO MARKET.—

9 “(1) DURATION; RENEWALS.—The conditional
10 approval for a drug under this section is effective for
11 a 2-year period. The sponsor may request renewal of
12 such conditional approval for up to 3 subsequent 2-
13 year periods. Conditional approval with respect to a
14 drug shall not exceed a total of 8 years from the ini-
15 tial date the drug was granted conditional approval.

16 “(2) APPLICATIONS FOR RENEWAL OF CONDI-
17 TIONAL APPROVAL.—

18 “(A) IN GENERAL.—Except as provided in
19 subparagraph (C), the sponsor of a drug seek-
20 ing a renewal of conditional approval for such
21 drug under this subsection shall submit to the
22 Secretary, not later than 180 days before the
23 date on which such conditional approval expires,
24 an application that contains the applicable in-
25 formation described in paragraph (3) in a

1 standardized format determined by the Sec-
2 retary.

3 “(B) PROCESS FOR GRANTING RENEW-
4 ALS.—Not later than 180 days after the date of
5 enactment of the Promising Pathway Act 2.0,
6 the Secretary, by rule, shall establish the proc-
7 ess for granting a renewal under this sub-
8 section.

9 “(C) EXEMPTION FOR SMALL POPULATION
10 DISEASES.—

11 “(i) IN GENERAL.—The Secretary
12 shall exempt from the requirements of sub-
13 paragraph (A) and paragraph (3) an appli-
14 cation for a renewal of conditional approval
15 for a drug under this subsection if the Sec-
16 retary determines that the population af-
17 fected by the disease or condition that the
18 drug is intended to treat does not support
19 additional preliminary evidence of effective-
20 ness (as defined in paragraph (3)(D)).

21 “(ii) APPLICATION FOR EXEMP-
22 TION.—Sponsors may submit an applica-
23 tion for exemption under this subpara-
24 graph not later than 180 days before the

1 date on which the conditional approval ex-
2 pires.

3 “(iii) APPLICATION PROCESS.—Not
4 later than 180 days after the date of en-
5 actment of the Promising Pathway Act
6 2.0, the Secretary shall establish a stand-
7 ardized application process for purposes of
8 this subparagraph.

9 “(iv) DEADLINE.—The Secretary shall
10 approve or deny an application under this
11 subparagraph before the date on which the
12 conditional approval expires.

13 “(v) APPEALS.—Not later than 180
14 days after the date of enactment of the
15 Promising Pathway Act 2.0, the Secretary
16 shall establish a process under which a
17 sponsor may appeal a denial of an applica-
18 tion under this subparagraph.

19 “(3) ADDITIONAL PRELIMINARY EVIDENCE OF
20 EFFECTIVENESS.—The information described in this
21 paragraph is the following:

22 “(A) FOR THE FIRST APPROVAL RE-
23 NEWAL.—With respect to an application under
24 paragraph (2) for the first renewal of condi-
25 tional approval for a drug under this sub-

1 section, additional preliminary evidence of effec-
2 tiveness of the drug, as compared to the evi-
3 dence provided in the initial application for con-
4 ditional approval for the drug under subsection
5 (c).

6 “(B) FOR THE SECOND APPROVAL RE-
7 NEWAL.—With respect to an application under
8 paragraph (2) for the second renewal of condi-
9 tional approval for a drug under this sub-
10 section, additional preliminary evidence of effec-
11 tiveness of the drug, as compared to the evi-
12 dence provided in the renewal application de-
13 scribed in subparagraph (A).

14 “(C) FOR THE FINAL APPROVAL RE-
15 NEWAL.—With respect to an application under
16 paragraph (2) for the third renewal of condi-
17 tional approval for a drug under this sub-
18 section, a written affirmation from the head of
19 the drug’s review division of the Office of New
20 Drugs or the Office of Therapeutic Products
21 asserting that a third renewal is necessary—

22 “(i) for patients who have benefitted
23 from such drug to retain access to such
24 drug; and

1 “(ii) to generate additional prelimi-
2 nary evidence of effectiveness for the pur-
3 poses of attaining approval under section
4 505 of this Act or section 351 of the Pub-
5 lic Health Service Act.

6 “(D) DEFINITION.—In this paragraph, the
7 term ‘preliminary evidence of effectiveness’
8 means—

9 “(i) clinical evidence generated by an
10 ongoing or completed clinical trial con-
11 ducted in accordance with section 11.22 of
12 title 42, Code of Federal Regulations (or
13 successor regulations);

14 “(ii) real-world evidence (as defined in
15 section 505F(b)); or

16 “(iii) evidence from an observational
17 registry under subsection (g).

18 “(4) DENIAL OF RENEWAL ON THE BASIS OF
19 DATA FRAUD.—The Secretary may deny the applica-
20 tion for renewal of conditional approval for a drug
21 under this subsection if the Secretary, in conducting
22 a review under subsection (d)(2), finds that the evi-
23 dence provided in such application under subpara-
24 graph (A) or (B) of paragraph (3) was fraudulently
25 manipulated by the applicable observational registry

1 and that such application substantially relies on
2 such data.

3 “(g) OBSERVATIONAL REGISTRIES.—

4 “(1) ESTABLISHMENT.—

5 “(A) IN GENERAL.—Subject to subpara-
6 graph (C), the sponsor of a drug conditionally
7 approved under this section shall establish an
8 observational registry, for patients who are or
9 will be treated with such drug, that pertains to
10 the disease or condition that the drug is in-
11 tended to treat.

12 “(B) REGISTRIES.—In establishing an ob-
13 servational registry for a drug under subpara-
14 graph (A), the sponsor may—

15 “(i) establish a new observational reg-
16 istry;

17 “(ii) use an existing observational reg-
18 istry that pertains to the disease or condi-
19 tion such drug is intended to treat;

20 “(iii) combine 1 or more existing ob-
21 servational registries that pertain to the
22 disease or condition such drug is intended
23 to treat with a new observational registry;
24 or

1 “(iv) combine 2 or more existing ob-
2 servational registries that pertain to the
3 disease or condition such drug is intended
4 to treat.

5 “(C) APPROVAL OF REGISTRY AND RIGHT
6 TO APPEAL.—Not later than 180 days after the
7 date of enactment of the Promising Pathway
8 Act 2.0, the Secretary shall establish—

9 “(i) a process to approve or deny the
10 establishment of an observational registry
11 under subparagraph (A); and

12 “(ii) a process for sponsors that re-
13 ceived such a denial to appeal the denial.

14 “(2) REQUIREMENT FOR PATIENTS TO ENROLL
15 IN OBSERVATIONAL REGISTRY.—

16 “(A) IN GENERAL.—A drug conditionally
17 approved under this section shall not be made
18 available to a patient unless such patient is en-
19 rolled in the applicable observational registry
20 described in paragraph (1).

21 “(B) INFORMED CONSENT.—

22 “(i) IN GENERAL.—Prior to enrolling
23 in an observational registry under subpara-
24 graph (A), a patient shall provide informed
25 consent in accordance with clause (ii).

1 “(ii) APPLICATION OF CERTAIN RE-
2 QUIREMENTS.—The requirements for in-
3 formed consent under part 50 of sub-
4 chapter A of chapter I of title 21, Code of
5 Federal Regulations (or successor regula-
6 tions), shall apply to enrollment an obser-
7 vational registry under this paragraph.

8 “(3) SUBMISSION OF PATIENT DATA.—

9 “(A) IN GENERAL.—The sponsor of a drug
10 conditionally approved under this section shall
11 be responsible for obtaining and submitting pa-
12 tient data to the applicable observational reg-
13 istry described in paragraph (1).

14 “(B) SUBMISSION STANDARDS.—Not later
15 than 180 days after date of enactment of the
16 Promising Pathway Act 2.0, the Secretary shall
17 establish data submission standards for spon-
18 sors to comply with for purposes of subpara-
19 graph (A) to ensure that registry data is con-
20 sistent and clinically informed.

21 “(4) REQUIREMENTS FOR REGISTRIES.—An ob-
22 servational registry described in paragraph (1) for a
23 drug conditionally approved under this section may
24 be operated by the sponsor of such drug or, at the

1 sponsor’s discretion, a third party, for-profit organi-
2 zation, or nonprofit organization.

3 “(5) RISK AND BENEFIT DATA.—

4 “(A) IN GENERAL.—The sponsor of a drug
5 conditionally approved under this section shall
6 submit relevant risk and benefit data to the ap-
7 plicable observational registry described in
8 paragraph (1).

9 “(B) ONLINE PORTAL.—The Secretary
10 shall operate an online portal on an existing
11 website of the Secretary for sponsors to submit
12 data described in subparagraph (A).

13 “(6) ACCESSIBILITY.—

14 “(A) IN GENERAL.—An observational reg-
15 istry described in paragraph (1) shall—

16 “(i) not later than 30 days after re-
17 ceipt of a request, provide patients (or
18 their designated representatives) with ac-
19 cess to such patient’s personal registry in-
20 formation; and

21 “(ii) provide approved researchers and
22 medical professionals access to de-identi-
23 fied and aggregated data from the registry
24 for the purposes of indication- and disease-
25 specific and translational research into

1 conditions and diseases relating to the dis-
2 ease or condition that the drug tracked by
3 the observational registry is intended to
4 treat.

5 “(B) APPROVED RESEARCHERS AND MED-
6 ICAL PROFESSIONALS.—Not later than 180
7 days after the date of enactment of the Prom-
8 ising Pathway Act 2.0, the Secretary, by rule,
9 shall establish a process for approving research-
10 ers and medical professionals for purposes of
11 subparagraph (A)(ii).

12 “(7) EFFECT.—Nothing in this section shall be
13 construed to modify or limit the Secretary’s author-
14 ity to require for a drug conditionally approved
15 under this section any type of postapproval study
16 under any other provision of law, including sections
17 505(o)(3), 505B, and 506.

18 “(h) PURSUIT OF A DIFFERENT INDICATION.—

19 “(1) IN GENERAL.—In the case of a drug con-
20 ditionally approved under this section for which such
21 approval was withdrawn under subsection (d), ex-
22 pired under subsection (f)(1), or was denied for re-
23 newal under subsection (f)(4), not later than 2 years
24 after the date of withdrawal, expiration, or denial, as
25 applicable, the sponsor of such drug shall have the

1 opportunity to petition the Secretary to receive con-
2 ditional approval of such drug, in accordance with
3 this section, for a different indication.

4 “(2) PROCESS.—Not later than 180 days after
5 the date of enactment of the Promising Pathway Act
6 2.0, the Secretary shall establish a process for peti-
7 tions under paragraph (1).

8 “(i) TRANSITION TO OTHER FORMS OF APPROVAL.—

9 “(1) IN GENERAL.—A drug that receives condi-
10 tional approval under this section may be granted
11 approval under section 505 of this Act or section
12 351 of the Public Health Service Act during the pe-
13 riod in which such conditional approval is in effect.
14 Effective on the date on which approval for such
15 drug is granted under section 505 of this Act or sec-
16 tion 351 of the Public Health Service Act, such con-
17 ditional approval shall be automatically withdrawn in
18 accordance with subsection (d)(3).

19 “(2) CONSIDERATION OF CERTAIN EVI-
20 DENCE.—In determining whether to approve under
21 section 505 of this Act or section 351 of the Public
22 Health Service Act a drug that has received condi-
23 tional approval under this section, the Secretary may
24 consider evidence from the observational registry for
25 the drug under subsection (g).

1 “(j) INFORMED CONSENT.—

2 “(1) IN GENERAL.—Prior to being prescribed a
3 drug conditionally approved under this section, a pa-
4 tient shall provide informed consent in accordance
5 with paragraph (2).

6 “(2) APPLICATION OF CERTAIN REQUIRE-
7 MENTS.—The requirements for informed consent
8 under part 50 of subchapter A of chapter I of title
9 21, Code of Federal Regulations (or successor regu-
10 lations), shall apply to drugs conditionally approved
11 under this section.

12 “(3) OBSERVATIONAL REGISTRIES.—An obser-
13 vational registry established for a drug in accord-
14 ance with subsection (g) may obtain, and maintain
15 records of, informed consent of a patient on behalf
16 of the drug sponsor, in accordance with paragraph
17 (2).

18 “(4) COMMON RULE.—Drugs conditionally ap-
19 proved under this section shall comply with subpart
20 A of part 46 of title 45, Code of Federal Regulations
21 (commonly known as the ‘Common Rule’) (or suc-
22 cessor regulations), if applicable.

23 “(k) LIMITATION ON LIABILITY.—With respect to
24 any claim under State law relating to a drug made avail-
25 able pursuant to a grant of conditional approval under this

1 section, no liability shall lie against a sponsor or manufac-
2 turer of the drug, or any health care provider who pre-
3 scribes or administers the drug, absent intentional wrong-
4 doing.

5 “(1) REPORT TO CONGRESS.—

6 “(1) IN GENERAL.—Not later than 2 years
7 after the date of enactment of the Promising Path-
8 way Act 2.0, and once every 2 years thereafter, the
9 Secretary, in collaboration with drug sponsors, shall
10 submit a report to Congress on all drugs granted
11 conditional approval under this section. Such report
12 shall include—

13 “(A) an estimated number of patients
14 treated with each such drug, and the number of
15 patients tracked in an observational registry
16 under subsection (g) with respect to each such
17 drug, if applicable;

18 “(B) a discussion, at an aggregate level ,
19 of the types and amounts of data obtained
20 through observational registries under sub-
21 section (g), such as patient treatments and
22 uses, length of use, side effects encountered,
23 relevant biomarkers, scan results, cause of
24 death and how long the patient lived, and ad-
25 verse drug effects;

1 “(C) a list of all such drugs for which an
2 application for approval under this section, or
3 an application for an extension of conditional
4 approval under this section, has been sub-
5 mitted; and

6 “(D) the number of all applications grant-
7 ed and denied conditional approval under this
8 section.

9 “(2) SPONSOR PARTICIPATION.—Not later than
10 180 days before the date on which the Secretary
11 submits a report under paragraph (1), the sponsor
12 of a drug conditionally approved under this section
13 shall provide to the Secretary the information de-
14 scribed in subparagraphs (A) and (B) of paragraph
15 (1), as applicable.

16 “(3) NOTICE AUTHORITY.—The Secretary may
17 notify sponsors of drugs conditionally approved
18 under this section and observational registries under
19 subsection (g) as necessary to complete a report
20 under paragraph (1).”.

21 (b) CONFORMING AMENDMENT.—Section 505(a) of
22 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
23 355(a)) is amended by inserting “, or there is in effect
24 a conditional approval under section 524C with respect to
25 such drug” before the period.

1 (c) REIMBURSEMENT.—

2 (1) PRIVATE HEALTH INSURERS.—Section
3 2719A of the Public Health Service Act (42 U.S.C.
4 300gg–19a) is amended by adding at the end the
5 following:

6 “(f) COVERAGE OF CERTAIN DRUGS.—A group
7 health plan or health insurance issuer offering group or
8 individual health insurance coverage shall provide coverage
9 for, and shall not impose any cost sharing requirements
10 for, drugs conditionally approved under section 524C of
11 the Federal Food, Drug, and Cosmetic Act for patients
12 who have the disease or condition the drug is intended
13 to treat.”.

14 (2) FEDERAL HEALTH CARE PROGRAMS.—The
15 requirement under subsection (f) of section 2719A
16 of the Public Health Service Act (as added by para-
17 graph (1)) shall apply with respect to coverage de-
18 terminations under a Federal health care program
19 (as defined in section 1128B(f) of the Social Secu-
20 rity Act (42 U.S.C. 1320a–7b(f))) in the same man-
21 ner such requirement applies under such subsection
22 (f).

23 (3) CONFORMING AMENDMENT.—Section
24 1927(k)(2)(A)(i) of the Social Security Act (42
25 U.S.C. 1396r–8(k)(2)(A)(i)) is amended—

1 (A) by striking “or which” and inserting “,
2 which”; and

3 (B) by inserting “, or which is condi-
4 tionally approved under section 524C of such
5 Act” before the semicolon.

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