

112TH CONGRESS
2D SESSION

S. _____

To amend the Federal Food, Drug, and Cosmetic Act with respect to certain reauthorizations.

IN THE SENATE OF THE UNITED STATES

_____ introduced the following bill; which was read twice and referred to the Committee on _____

A BILL

To amend the Federal Food, Drug, and Cosmetic Act with respect to certain reauthorizations.

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 “_____ Act
5 of _____”.**]**

6 **SEC. 2. ENHANCEMENT OF ACCELERATED PATIENT AC-**
7 **CESS TO NEW MEDICAL TREATMENTS.**

8 (a) FINDINGS; SENSE OF CONGRESS.—

9 (1) FINDINGS.—Congress finds as follows:

1 (A) The Food and Drug Administration
2 (referred to in this section as the “FDA”)
3 serves a critical role in helping to assure that
4 new medicines are safe and effective. Regu-
5 latory innovation is 1 element of the Nation’s
6 strategy to address serious and life-threatening
7 diseases or conditions by promoting investment
8 in and development of innovative treatments for
9 unmet medical needs.

10 (B) During the 2 decades following the es-
11 tablishment of the accelerated approval mecha-
12 nism, advances in medical sciences, including
13 genomics, molecular biology, and bioinformatics,
14 have provided an unprecedented understanding
15 of the underlying biological mechanism and
16 pathogenesis of disease. A new generation of
17 modern, targeted medicines is under develop-
18 ment to treat serious and life-threatening dis-
19 eases, some applying drug development strate-
20 gies based on biomarkers or pharmacogenomics,
21 predictive toxicology, clinical trial enrichment
22 techniques, and novel clinical trial designs, such
23 as adaptive clinical trials.

24 (C) As a result of these remarkable sci-
25 entific and medical advances, the FDA should

1 be encouraged to implement more broadly effective
2 processes for the expedited development
3 and review of innovative new medicines intended
4 to address unmet medical needs for serious
5 or life-threatening diseases or conditions,
6 including those for rare diseases or conditions,
7 using a broad range of surrogate or clinical
8 endpoints and modern scientific tools earlier in
9 the drug development cycle when appropriate.
10 This may result in fewer, smaller, or shorter
11 clinical trials for the intended patient population
12 or targeted subpopulation without compromising
13 or altering the high standards of the
14 FDA for the approval of drugs.

15 (D) Patients benefit from expedited access
16 to safe and effective innovative therapies to
17 treat unmet medical needs for serious or life-
18 threatening diseases or conditions.

19 (E) For these reasons, the statutory authority
20 in effect on the day before the date of
21 enactment of this Act governing expedited approval
22 of drugs for serious or life-threatening
23 diseases or conditions should be amended in
24 order to enhance the authority of the FDA to
25 consider appropriate scientific data, methods,

1 and tools, and to expedite development and ac-
2 cess to novel treatments for patients with a
3 broad range of serious or life-threatening dis-
4 eases or conditions.

5 (2) SENSE OF CONGRESS.—It is the sense of
6 Congress that the Food and Drug Administration
7 should apply the accelerated approval and fast track
8 provisions set forth in section 506 of the Federal
9 Food, Drug, and Cosmetic Act (21 U.S.C. 356), as
10 amended by this section, to help expedite the devel-
11 opment and availability to patients of treatments for
12 serious or life-threatening diseases or conditions
13 while maintaining safety and effectiveness standards
14 for such treatments.

15 (b) EXPEDITED APPROVAL OF DRUGS FOR SERIOUS
16 OR LIFE-THREATENING DISEASES OR CONDITIONS.—Sec-
17 tion 506 of the Federal Food, Drug, and Cosmetic Act
18 (21 U.S.C. 356) is amended to read as follows:

19 **“SEC. 506. EXPEDITED APPROVAL OF DRUGS FOR SERIOUS**
20 **OR LIFE-THREATENING DISEASES OR CONDI-**
21 **TIONS.**

22 “(a) DESIGNATION OF DRUG AS FAST TRACK PROD-
23 UCT.—

24 “(1) IN GENERAL.—The Secretary shall, at the
25 request of the sponsor of a new drug, facilitate the

1 development and expedite the review of such drug if
2 it is intended, whether alone or in combination with
3 one or more other drugs, for the treatment of a seri-
4 ous or life-threatening disease or condition, and it
5 demonstrates the potential to address unmet medical
6 needs for such a disease or condition. (In this sec-
7 tion, such a drug is referred to as a ‘fast track prod-
8 uct’.)

9 “(2) REQUEST FOR DESIGNATION.—The spon-
10 sor of a new drug may request the Secretary to des-
11 ignate the drug as a fast track product. A request
12 for the designation may be made concurrently with,
13 or at any time after, submission of an application
14 for the investigation of the drug under section 505(i)
15 or section 351(a)(3) of the Public Health Service
16 Act.

17 “(3) DESIGNATION.—Within 60 calendar days
18 after the receipt of a request under paragraph (2),
19 the Secretary shall determine whether the drug that
20 is the subject of the request meets the criteria de-
21 scribed in paragraph (1). If the Secretary finds that
22 the drug meets the criteria, the Secretary shall des-
23 ignate the drug as a fast track product and shall
24 take such actions as are appropriate to expedite the

1 development and review of the application for ap-
2 proval of such product.

3 “(b) ACCELERATED APPROVAL OF A DRUG FOR A
4 SERIOUS OR LIFE-THREATENING DISEASE OR CONDI-
5 TION, INCLUDING A FAST TRACK PRODUCT.—

6 “(1) IN GENERAL.—

7 “(A) ACCELERATED APPROVAL.—The Sec-
8 retary may approve an application for approval
9 of a product for a serious or life-threatening
10 disease or condition, including a fast track
11 product, under section 505(c) or section 351(a)
12 of the Public Health Service Act upon a deter-
13 mination that the product has an effect on a
14 surrogate endpoint that is reasonably likely to
15 predict clinical benefit, or on a clinical endpoint
16 that can be measured earlier than irreversible
17 morbidity or mortality, that is reasonably likely
18 to predict an effect on irreversible morbidity or
19 mortality or other clinical benefit, taking into
20 account the severity or rarity of the condition
21 and the availability of alternative treatments.
22 The approval described in the preceding sen-
23 tence is referred to in this section as ‘acceler-
24 ated approval’.

1 “(B) EVIDENCE.—The evidence to support
2 that an endpoint is reasonably likely to predict
3 clinical benefit under subparagraph (A) may in-
4 clude epidemiological, pathophysiological, thera-
5 peutic or other evidence developed using bio-
6 markers, for example, or other scientific meth-
7 ods or tools.

8 “(2) LIMITATION.—Approval of a product
9 under this subsection may be subject to 1 or both
10 of the following requirements:

11 “(A) That the sponsor conduct appropriate
12 post-approval studies to verify and describe the
13 predicted effect on irreversible morbidity or
14 mortality or other clinical benefit.

15 “(B) That the sponsor submit copies of all
16 promotional materials related to the product
17 during the preapproval review period and, fol-
18 lowing approval and for such period thereafter
19 as the Secretary determines to be appropriate,
20 at least 30 days prior to dissemination of the
21 materials.

22 “(3) EXPEDITED WITHDRAWAL OF AP-
23 PROVAL.—The Secretary may withdraw approval of
24 a product approved under accelerated approval using
25 expedited procedures (as prescribed by the Secretary

1 in regulations which shall include an opportunity for
2 an informal hearing) if—

3 “(A) the sponsor fails to conduct any re-
4 quired post-approval study of the drug with due
5 diligence;

6 “(B) a study required to verify and de-
7 scribe the predicted effect on irreversible mor-
8 bidity or mortality or other clinical benefit of
9 the product fails to verify and describe such ef-
10 fect or benefit;

11 “(C) other evidence demonstrates that the
12 product is not safe or effective under the condi-
13 tions of use; or

14 “(D) the sponsor disseminates false or
15 misleading promotional materials with respect
16 to the product.

17 “(c) REVIEW OF INCOMPLETE APPLICATIONS FOR
18 APPROVAL OF A FAST TRACK PRODUCT.—

19 “(1) IN GENERAL.—If the Secretary deter-
20 mines, after preliminary evaluation of clinical data
21 submitted by the sponsor, that a fast track product
22 may be effective, the Secretary shall evaluate for fil-
23 ing, and may commence review of portions of, an ap-
24 plication for the approval of the product before the
25 sponsor submits a complete application. The Sec-

1 retary shall commence such review only if the appli-
2 cant—

3 “(A) provides a schedule for submission of
4 information necessary to make the application
5 complete; and

6 “(B) pays any fee that may be required
7 under section 736.

8 “(2) EXCEPTION.—Any time period for review
9 of human drug applications that has been agreed to
10 by the Secretary and that has been set forth in goals
11 identified in letters of the Secretary (relating to the
12 use of fees collected under section 736 to expedite
13 the drug development process and the review of
14 human drug applications) shall not apply to an ap-
15 plication submitted under paragraph (1) until the
16 date on which the application is complete.

17 “(d) AWARENESS EFFORTS.—The Secretary shall—

18 “(1) develop and disseminate to physicians, pa-
19 tient organizations, pharmaceutical and bio-
20 technology companies, and other appropriate persons
21 a description of the provisions of this section appli-
22 cable to accelerated approval and fast track prod-
23 ucts; and

24 “(2) establish a program to encourage the de-
25 velopment of surrogate and clinical endpoints, in-

1 including biomarkers, and other scientific methods and
2 tools that can assist the Secretary in determining
3 whether the evidence submitted in an application is
4 reasonably likely to predict clinical benefit for seri-
5 ous or life-threatening conditions for which signifi-
6 cant unmet medical needs exist.

7 “(e) CONSTRUCTION.—

8 “(1) PURPOSE.—The amendments made by the
9 **[insert short title]** to this section are intended to
10 encourage the Secretary to utilize innovative and
11 flexible approaches to the assessment of products
12 under accelerated approval for treatments for pa-
13 tients with serious or life-threatening diseases or
14 conditions and unmet medical needs.

15 “(2) CONSTRUCTION.—Nothing in this section
16 shall be construed to alter the standards of evidence
17 under subsection (c) or (d) of section 505 (including
18 the substantial evidence standard in section 505(d))
19 of this Act or under section 351(a) of the Public
20 Health Service Act. Such sections and standards of
21 evidence apply to the review and approval of prod-
22 ucts under this section, including whether a product
23 is safe and effective. Nothing in this section alters
24 the ability of the Secretary to rely on evidence that
25 does not come from adequate and well-controlled in-

1 investigations for the purpose of determining whether
2 an endpoint is reasonably likely to predict clinical
3 benefit as described in subsection (b)(1)(B).”.

4 (c) GUIDANCE; AMENDED REGULATIONS.—

5 (1) DRAFT GUIDANCE.—Not later than 1 year
6 after the date of enactment of this Act, the Sec-
7 retary of Health and Human Services (referred to in
8 this section as the “Secretary”) shall issue draft
9 guidance to implement the amendments made by
10 this section. In developing such guidance, the Sec-
11 retary shall specifically consider issues arising under
12 the accelerated approval and fast track processes
13 under section 506 of the Federal Food, Drug, and
14 Cosmetic Act, as amended by subsection (b), for
15 drugs designated for a rare disease or condition
16 under section 526 of such Act (21 U.S.C. 360bb).

17 (2) FINAL GUIDANCE.—Not later than 1 year
18 after the issuance of draft guidance under para-
19 graph (1), and after an opportunity for public com-
20 ment, the Secretary shall issue final guidance.

21 (3) CONFORMING CHANGES.—The Secretary
22 shall issue, as necessary, conforming amendments to
23 the applicable regulations under title 21, Code of
24 Federal Regulations, governing accelerated approval.

1 (4) NO EFFECT OF INACTION ON REQUESTS.—

2 If the Secretary fails to issue final guidance or
3 amended regulations as required by this subsection,
4 such failure shall not preclude the review of, or ac-
5 tion on, a request for designation or an application
6 for approval submitted pursuant to section 506 of
7 the Federal Food, Drug, and Cosmetic Act, as
8 amended by subsection (b).

9 (d) INDEPENDENT REVIEW.—The Secretary may, in
10 conjunction with other planned reviews, contract with an
11 independent entity with expertise in assessing the quality
12 and efficiency of biopharmaceutical development and regu-
13 latory review programs to evaluate the Food and Drug Ad-
14 ministration's application of the processes described in
15 section 506 of the Federal Food, Drug, and Cosmetic Act,
16 as amended by subsection (b), and the impact of such
17 processes on the development and timely availability of in-
18 novative treatments for patients suffering from serious or
19 life-threatening conditions. Any such evaluation shall in-
20 clude consultation with regulated industries, patient advo-
21 cacy and disease research foundations, and relevant aca-
22 demic medical centers.

1 **SEC. 3. BREAKTHROUGH THERAPIES.**

2 (a) IN GENERAL.—Section 506 of the Federal Food,
3 Drug, and Cosmetic Act (21 U.S.C. 356), as amended by
4 section 2, is further amended—

5 (1) by redesignating subsections (a) through (c)
6 as subsections (b) through (d), respectively;

7 (2) by redesignating subsection (d) as sub-
8 section (f);

9 (3) by inserting before subsection (b), as so re-
10 designated, the following:

11 “(a) DESIGNATION OF A DRUG AS A BREAKTHROUGH
12 THERAPY.—

13 “(1) IN GENERAL.—The Secretary shall, at the
14 request of the sponsor of a drug, expedite the devel-
15 opment and review of such drug if the drug is in-
16 tended, alone or in combination with 1 or more other
17 drugs, to treat a serious or life-threatening disease
18 or condition and preliminary clinical evidence indi-
19 cates that the drug may demonstrate substantial im-
20 provement over existing therapies on 1 or more clini-
21 cally significant endpoints, such as substantial treat-
22 ment effects observed early in clinical development.
23 (In this section, such a drug is referred to as a
24 ‘breakthrough therapy’.)

25 “(2) REQUEST FOR DESIGNATION.—The spon-
26 sor of a drug may request the Secretary to designate

1 the drug as a breakthrough therapy. A request for
2 the designation may be made concurrently with, or
3 at any time after, the submission of an application
4 for the investigation of the drug under section 505(i)
5 or section 351(a)(3) of the Public Health Service
6 Act.

7 “(3) DESIGNATION.—

8 “(A) IN GENERAL.—Not later than 60 cal-
9 endar days after the receipt of a request under
10 paragraph (2), the Secretary shall determine
11 whether the drug that is the subject of the re-
12 quest meets the criteria described in paragraph
13 (1). If the Secretary finds that the drug meets
14 the criteria, the Secretary shall designate the
15 drug as a breakthrough therapy and shall take
16 such actions as are appropriate to expedite the
17 development and review of the application for
18 approval of such drug.

19 “(B) ACTIONS.—The actions to expedite
20 the development and review of an application
21 under subparagraph (A) may include, as appro-
22 priate—

23 “(i) holding meetings with the sponsor
24 and the review team throughout the devel-
25 opment of the drug;

1 “(ii) providing timely advice to, and
2 interactive communication with, the spon-
3 sor regarding the development of the drug
4 to ensure that the development program to
5 gather the non-clinical and clinical data
6 necessary for approval is as efficient as
7 practicable;

8 “(iii) involving senior managers and
9 experienced review staff, as appropriate, in
10 a collaborative, cross-disciplinary review;

11 “(iv) assigning a cross-disciplinary
12 project lead for the Food and Drug Ad-
13 ministration review team to facilitate an
14 efficient review of the development pro-
15 gram and to serve as a scientific liaison be-
16 tween the review team and the sponsor;
17 and

18 “(v) taking steps to ensure that the
19 design of the clinical trials is as efficient as
20 practicable, when scientifically appropriate,
21 such as by minimizing the number of pa-
22 tients enrolled in the trials and the dura-
23 tion of the trials.”;

24 (4) in subsection (f)(1), as so redesignated, by
25 striking “applicable to accelerated approval” and in-

1 serting “applicable to breakthrough therapies, accel-
2 erated approval, and”; and

3 (5) by adding at the end the following:

4 “(g) REPORT.—Beginning in fiscal year 2013, the
5 Secretary shall annually prepare and submit to the Com-
6 mittee on Health, Education, Labor, and Pensions of the
7 Senate and the Committee on Energy and Commerce of
8 the House of Representatives, and make publicly available,
9 with respect to this section for the previous fiscal year—

10 “(1) the number of drugs for which a sponsor
11 requested designation as a breakthrough therapy;

12 “(2) the number of products designated as a
13 breakthrough therapy; and

14 “(3) for each breakthrough therapy approved in
15 the fiscal year—

16 “(A) the point in the drug development
17 and review process at which such breakthrough
18 designation occurred;

19 “(B) the total time from designation as a
20 breakthrough therapy to a final decision on the
21 approvability of the drug; and

22 “(C) the number of breakthrough therapies
23 approved, including the number approved on
24 the first review out of the total number of such
25 therapies so approved.”.

1 (b) GUIDANCE; AMENDED REGULATIONS.—

2 (1) IN GENERAL.—

3 (A) GUIDANCE.—Not later than 18
4 months after the date of enactment of this Act,
5 the Secretary of Health and Human Services
6 (referred to in this section as the “Secretary”)
7 shall issue draft guidance on implementing the
8 requirements with respect to breakthrough
9 therapies, as set forth in section 506(a) of the
10 Federal Food, Drug, and Cosmetic Act (21
11 U.S.C. 356(a)), as amended by this section.
12 The Secretary shall issue final guidance not
13 later than 1 year after the close of the comment
14 period for the draft guidance.

15 (B) AMENDED REGULATIONS.—If the Sec-
16 retary determines that it is necessary to amend
17 the regulations under title 21, Code of Federal
18 Regulations in order to implement the amend-
19 ments made by this section to section 506(a) of
20 the Federal Food, Drug, and Cosmetic Act, the
21 Secretary shall amend such regulations not
22 later than 2 years after the date of enactment
23 of this Act.

24 (2) REQUIREMENTS.—Guidance promulgated
25 under this section shall—

1 (A) specify the process and criteria by
2 which the Secretary makes a designation under
3 section 506(a)(3) of the Federal Food, Drug,
4 and Cosmetic Act; and

5 (B) specify the actions the Secretary shall
6 take to expedite the development and review of
7 a breakthrough therapy pursuant to such des-
8 ignation under such section 506(a)(3), includ-
9 ing updating good review management practices
10 to reflect breakthrough therapies.

11 (c) INDEPENDENT REVIEW.—Not later than 3 years
12 after the date of enactment of this Act, the Comptroller
13 General of the United States, in consultation with appro-
14 priate experts, shall assess the manner by which the Food
15 and Drug Administration has applied the processes de-
16 scribed in section 506(a) of the Federal Food, Drug, and
17 Cosmetic Act, as amended by this section, and the impact
18 of such processes on the development and timely avail-
19 ability of innovative treatments for patients affected by se-
20 rious or life-threatening conditions. Such assessment shall
21 be made publicly available upon completion.

22 (d) CONFORMING AMENDMENTS.—Section 506B(e)
23 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
24 356b) is amended by striking “section 506(b)(2)(A)” each

1 place such term appears and inserting “section
2 506(e)(2)(A)”.

3 **SEC. 4. GUIDANCE DOCUMENT REGARDING PRODUCT PRO-**
4 **MOTION USING THE INTERNET.**

5 Not later than 2 years after the date of enactment
6 this Act, the Secretary of Health and Human Services
7 shall issue a guidance document that describes the policy
8 of the Food and Drug Administration regarding the pro-
9 motion, using the Internet (including social media), of
10 medical products that are regulated by such Administra-
11 tion.

12 **SEC. 5. REAUTHORIZATION OF PROVISION RELATING TO**
13 **EXCLUSIVITY OF CERTAIN DRUGS CON-**
14 **TAINING SINGLE ENANTIOMERS.**

15 Section 505(u)(4) of the Federal Food, Drug, and
16 Cosmetic Act (21 U.S.C. 355(u)(4)) is amended by strik-
17 ing “2012” and inserting “2017”.

18 **SEC. 6. REAUTHORIZATION OF THE CRITICAL PATH PUB-**
19 **LIC-PRIVATE PARTNERSHIPS.**

20 Section 566(f) of the Federal Food, Drug, and Cos-
21 metic Act (21 U.S.C. 360bbb–5(f)) is amended by striking
22 “2012” and inserting “2017”.

1 **SEC. 7. CONSULTATION WITH EXTERNAL EXPERTS ON**
2 **RARE DISEASES, TARGETED THERAPIES, AND**
3 **GENETIC TARGETING OF TREATMENTS.**

4 Subchapter E of chapter V of the Federal Food,
5 Drug, and Cosmetic Act (21 U.S.C. 360bbb et seq.) is
6 amended by adding at the end the following:

7 **“SEC. 568. CONSULTATION WITH EXTERNAL EXPERTS ON**
8 **RARE DISEASES, TARGETED THERAPIES, AND**
9 **GENETIC TARGETING OF TREATMENTS.**

10 “(a) IN GENERAL.—

11 “(1) OPPORTUNITIES FOR CONSULTATION.—

12 The Secretary shall ensure that opportunities exist,
13 at a time the Secretary determines appropriate, for
14 consultation with external experts on the topics de-
15 scribed in subsection (c), for the purpose of pro-
16 moting the efficiency of and informing the review by
17 the Food and Drug Administration of drugs and bio-
18 logic products for rare diseases and drugs and bio-
19 logic products that are genetically targeted.

20 “(2) CONSULTATION.—The Center for Drug
21 Evaluation and Research and the Center for Bio-
22 logics Evaluation and Research shall, when appro-
23 priate, seek the opinion of external experts on any
24 topic, including the topics described in subsection
25 (c), by initiating contact with such experts. External
26 experts may also request the opportunity to meet

1 with a review division regarding any topic described
2 in subsection (c).

3 “(b) EXTERNAL EXPERTS.—The external experts
4 under subsection (a) may include—

5 “(1) representatives of patient, consumer, re-
6 search, and health professional organizations with
7 expertise relevant to the review of rare disease prod-
8 ucts;

9 “(2) experts on rare diseases, rare subtypes of
10 rare and other diseases, and genetic targeting of
11 treatments, including experts from academia; and

12 “(3) experts in innovative clinical trial designs
13 for small target populations.

14 “(c) TOPICS FOR CONSULTATION.—Topics for con-
15 sultation may include—

16 “(1) rare diseases;

17 “(2) the severity of rare diseases;

18 “(3) the unmet medical need associated with
19 rare diseases;

20 “(4) the willingness and ability of individuals
21 with a rare disease to participate in clinical trials;

22 “(5) an assessment of the benefits and risks,
23 including side effects, of current and investigational
24 therapies;

