

111TH CONGRESS
2^D SESSION

H. R. 4732

To amend the Federal Food, Drug, and Cosmetic Act to create a new conditional approval system for drugs, biological products, and devices that is responsive to the needs of seriously ill patients, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

MARCH 2, 2010

Ms. WATSON introduced the following bill; which was referred to the Committee on Energy and Commerce, and in addition to the Committee on Ways and Means, for a period to be subsequently determined by the Speaker, in each case for consideration of such provisions as fall within the jurisdiction of the committee concerned

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to create a new conditional approval system for drugs, biological products, and devices that is responsive to the needs of seriously ill patients, 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 “Compassionate Access
5 Act of 2010”.

1 **SEC. 2. FINDINGS.**

2 Congress finds the following:

3 (1) As of 2009, the standards of the Food and
4 Drug Administration for approval of drugs, biologi-
5 cal products, and devices may deny the benefits of
6 medical progress to seriously ill patients who face
7 morbidity or death from their disease.

8 (2) Seriously ill patients have a right to take
9 actions to preserve their life by accessing available
10 investigational drugs, biological products, and de-
11 vices.

12 (3) The emphasis on statistical analysis of clin-
13 ical information needs to be balanced by a reliance
14 on clinical evaluation and patient-reported outcomes
15 and considered with an understanding of the risks to
16 patients from their disease, with the goal of pro-
17 viding additional treatment options for patients and
18 their physicians to consider.

19 (4) Food and Drug Administration advisory
20 committees should have greater representation of
21 medical clinicians and patient advocates who rep-
22 resent the interests of seriously ill patients in early
23 access to promising investigational therapies.

24 (5) The use of available investigational products
25 for treatment is the responsibility of the physician
26 and the seriously ill patient.

1 (6) The use of combinations of available inves-
2 tigational and approved products for treatment is
3 the responsibility of the physician and the seriously
4 ill patient.

5 (7) The Food and Drug Administration should
6 have the expertise and flexibility to address the
7 growing needs of seriously ill patients for individual-
8 ized or personalized therapies.

9 **SEC. 3. COMPASSIONATE INVESTIGATIONAL ACCESS AP-**
10 **PROVAL SYSTEM FOR DRUGS, BIOLOGICAL**
11 **PRODUCTS, AND DEVICES.**

12 (a) COMPASSIONATE INVESTIGATIONAL ACCESS.—
13 Section 561 of the Federal Food, Drug, and Cosmetic Act
14 (21 U.S.C. 360bbb) is amended—

15 (1) by redesignating subsections (d) and (e) as
16 subsections (e) and (f), respectively; and

17 (2) by inserting after subsection (c) the fol-
18 lowing:

19 “(d) COMPASSIONATE INVESTIGATIONAL ACCESS.—

20 “(1) PURPOSE.—The purpose of this subsection
21 is to facilitate the availability of promising new
22 drugs to seriously ill patients as early in the drug
23 development process as possible, before general mar-
24 keting begins.

1 “(2) ACCESS.—Notwithstanding any other pro-
2 vision of law, upon submission by a sponsor of an
3 application intended to provide widespread access to
4 an investigational drug, biological product, or device
5 for eligible patients (referred to in this subsection as
6 ‘Compassionate Investigational Access’), the Sec-
7 retary shall permit such investigational drug, biologi-
8 cal product, or device, to be made available for ex-
9 panded access under a treatment investigational new
10 drug application or treatment investigational device
11 exemption if the Secretary determines that the re-
12 quirements of this section are met with respect to
13 Compassionate Investigational Access.

14 “(3) COMPASSIONATE INVESTIGATIONAL AC-
15 CESS.—Notwithstanding any other provision of law,
16 an investigational drug, biological product, or device
17 that receives approval for Compassionate Investiga-
18 tional Access under this subsection shall be subject
19 to the provisions of section 505(i) or 520(g), as ap-
20 plicable, and regulations promulgated by the Sec-
21 retary pursuant to this Act. The Secretary and the
22 sponsor may inform national, State, and local med-
23 ical associations and societies, voluntary health asso-
24 ciations, and other appropriate persons about the
25 availability of an investigational drug or investiga-

1 tional device under Compassionate Investigational
2 Access as approved under this subsection. The infor-
3 mation submitted by the Secretary, in accordance
4 with the preceding sentence, shall be the same type
5 of information that is required by section 402(i)(3)
6 of the Public Health Service Act.

7 “(4) SUBMISSION OF APPLICATION.—

8 “(A) APPLICATION CONTENT.—A sponsor
9 of an investigational drug, biological product, or
10 device applying for Compassionate Investiga-
11 tional Access approval of the product shall sub-
12 mit to the Secretary a notice of claimed exemp-
13 tion under section 505(i) or 520(g), as applica-
14 ble, (referred to in this subsection as an ‘appli-
15 cation for Compassionate Investigational Ac-
16 cess’), which shall contain—

17 “(i) data and information from com-
18 pleted Phase I clinical investigations and
19 any other nonclinical or clinical investiga-
20 tions;

21 “(ii) preliminary evidence that the
22 product may be effective in humans
23 against a serious or life-threatening condi-
24 tion or disease, which evidence may be
25 based on uncontrolled data such as case

1 histories, information about the pharma-
2 cological mechanism of action, data from
3 animal and computer models, comparison
4 with historical data, or other preliminary
5 information, and may be based on a small
6 number of patients or a subset of the pa-
7 tient population;

8 “(iii) evidence that the product is safe
9 at the dose and duration proposed, consid-
10 ering whether the potential risk to a pa-
11 tient of the condition or disease outweighs
12 the potential risk to a patient of the pro-
13 posed dose and duration of treatment with
14 the product, consistent with the level of in-
15 formation needed to initiate a Phase II
16 clinical trial; and

17 “(iv) a statement that the sponsor is
18 actively pursuing marketing approval with
19 due diligence.

20 “(B) LIMITATION.—Compassionate Inves-
21 tigational Access approval shall be based upon
22 multiple considerations that shall include clin-
23 ical evaluation and unmet patient needs.

24 “(5) DETERMINATION BY SECRETARY.—

1 “(A) IN GENERAL.—Not later than 30
2 days after the receipt of an application for
3 Compassionate Investigational Access approval,
4 the Secretary shall either—

5 “(i) provide Compassionate Investiga-
6 tional Access approval of the application;
7 or

8 “(ii) refer the application to the Accel-
9 erated Approval Advisory Committee.

10 “(B) RECOMMENDATION.—Not later than
11 90 days after receipt of an application for Com-
12 passionate Investigational Access approval, the
13 Accelerated Approval Advisory Committee shall
14 issue a recommendation to the Secretary on
15 whether the Secretary shall provide Compas-
16 sionate Investigational Access approval of the
17 application.

18 “(C) FINAL DECISION.—Not later than 30
19 days after receipt of the recommendation from
20 the Accelerated Approval Advisory Committee,
21 the Secretary shall either provide Compas-
22 sionate Investigational Access approval of the
23 application or shall issue an order setting forth
24 a detailed explanation of the reasons why the
25 application was not so approved and the specific

1 data that the sponsor must provide so that the
2 application may be so approved.

3 “(6) APPEAL.—If the Secretary does not pro-
4 vide Compassionate Investigational Access approval
5 of an application, the sponsor of the application
6 shall have the right to appeal the decision to the
7 Secretary. The Secretary shall provide the sponsor
8 with a hearing not later than 30 days following the
9 nonapproval under this subsection of the application
10 and shall issue an order not later than 30 days fol-
11 lowing the hearing either concurring in the non-
12 approval or so approving the application. The Sec-
13 retary shall not delegate the responsibility described
14 in this paragraph to any other person.

15 “(7) CRITERIA.—In making a determination
16 under paragraph (5), the Secretary shall consider
17 whether the totality of the information available to
18 the Secretary regarding the safety and effectiveness
19 of an investigational drug, biological product, or de-
20 vice, as compared to the risk of morbidity or death
21 from a condition or disease, indicates that a patient
22 (who may be representative of a small patient sub-
23 population) may obtain more benefit than risk if
24 treated with the drug, biological product, or device.
25 If the potential risk to a patient of the condition or

1 disease outweighs the potential risk of the product,
2 and the product may possibly provide benefit to the
3 patient, the Secretary shall provide Compassionate
4 Investigational Access approval of the application.

5 “(8) PATIENT ELIGIBILITY FOR COMPAS-
6 SIONATE ACCESS.—In order for a patient to access
7 a product available through Compassionate Inves-
8 tigational Access, the physician must document in
9 writing that the patient—

10 “(A) is seriously ill;

11 “(B) has examined all treatment options
12 approved by the Secretary for the condition or
13 disease for which the patient is a reasonable
14 candidate; and

15 “(C) has unsuccessfully sought treatment
16 or obtained treatment that was not effective,
17 with an investigational drug, biological product,
18 or device for which such individual is a reason-
19 able candidate, which shall include consider-
20 ation of a patient’s ineligibility for participation
21 in clinical trials, the lack of source of supply
22 and geographic factors.

23 “(9) PRODUCT LABELING.—To receive Compas-
24 sionate Investigational Access approval under this
25 subsection, the sponsor of the product shall provide

1 labeling approved by the Secretary for the drug, bio-
2 logical product, or device that—

3 “(A) states that the product is intended
4 for use by a patient whose physician has docu-
5 mented in writing that the patient has—

6 “(i) examined all treatment options
7 approved by Secretary for the condition or
8 disease for which the patient is a reason-
9 able candidate; and

10 “(ii) unsuccessfully sought treatment,
11 or obtained treatment that was not effec-
12 tive with an investigational drug, biological
13 product, or device for which such indi-
14 vidual is a reasonable candidate, which
15 shall include a patient’s ineligibility for
16 participation in clinical trials, the lack of
17 source of supply and geographic factors;
18 and

19 “(B) states that every patient to whom the
20 product is administered shall, as a mandatory
21 condition of receiving the product, provide—

22 “(i) written informed consent, as de-
23 scribed under part 50 of title 21, Code of
24 Federal Regulations (or any successor reg-
25 ulations); and

1 “(ii) consent for the manufacturer of
2 the product to obtain data and information
3 about the patient and the patient’s use of
4 the product that may be used to support
5 an application for Accelerated Approval or
6 final approval.

7 “(10) CHARGING FOR COMPASSIONATE INVESTIGATIONAL ACCESS.—A sponsor or investigator
8 may charge for a Compassionate Investigational Access drug without notifying the Secretary or seeking
9 or obtaining prior approval of the amount charged,
10 provided the sponsor of the drug is actively pursuing
11 marketing approval with due diligence.

12 “(11) COMMENCEMENT OF REVIEW.—If the
13 Secretary determines, after preliminary evaluation of
14 the data and information submitted by the sponsor,
15 that the product may be effective, the Secretary
16 shall evaluate for filing, and may commence review
17 of portions of, an application under this subsection
18 before the sponsor submits a complete application.
19 The Secretary shall commence such review only if
20 the applicant provides a schedule for submission of
21 information necessary to make the application complete.
22 The Secretary shall commence such review only if
23 the applicant provides a schedule for submission of
24 information necessary to make the application complete.

25 “(12) IMMUNITY.—

1 “(A) IN GENERAL.—A manufacturer, dis-
2 tributor, administrator, sponsor, or physician
3 who manufactures, supplies, distributes or pre-
4 scribes a product approved under an application
5 for Compassionate Investigational Access shall
6 be immune from suit or liability caused by, aris-
7 ing out of, or relating to the design, develop-
8 ment, clinical testing and investigation, manu-
9 facture, labeling, distribution, sale, purchase,
10 donation, dispensing, prescribing, administra-
11 tion, efficacy, or use of a drug, biological prod-
12 uct, or device subject to an approved Compass-
13 ionate Investigational Access application.

14 “(B) CLAIMS.—No claim or cause of ac-
15 tion against a manufacturer, distributor, ad-
16 ministrator, sponsor, or physician who manu-
17 factures, supplies, distributes or prescribes a
18 product subject to an approved Compassionate
19 Investigational Access application shall exist in
20 any Federal or State court for claims of prop-
21 erty, personal injury, or death caused by, aris-
22 ing out of, or relating to the design, develop-
23 ment, clinical testing and investigation, manu-
24 facture, labeling, distribution, sale, purchase,
25 donation, dispensing, prescribing, administra-

1 tion, efficacy, or use of a drug, biological prod-
2 uct, or device subject to an approved Compass-
3 ionate Investigational Access application. Any
4 such claim or cause of action that is filed in
5 Federal or State court shall be immediately dis-
6 missed.

7 “(13) FINAL APPROVAL.—For purposes of this
8 Act, the term ‘final approval’ means—

9 “(A) with respect to a new drug or new bi-
10 ological product, approval of such drug or prod-
11 uct under section 505(b)(1) or 505(b)(2) or
12 section 351 of the Public Health Service Act, as
13 the case may be; and

14 “(B) with respect to a new device, clear-
15 ance of such device under section 510(k) or ap-
16 proval of such device under section 515(c)(1).”.

17 (b) ACCELERATED APPROVAL.—Chapter V of the
18 Federal, Food, Drug, and Cosmetic Act (21 U.S.C. 351
19 et seq.) is amended by inserting after section 561 the fol-
20 lowing:

21 **“SEC. 561A. ACCELERATED APPROVAL.**

22 “(a) IN GENERAL.—

23 “(1) IN GENERAL.—As soon as practicable
24 after the date of enactment of the Compassionate
25 Access Act of 2010, the Secretary shall promulgate

1 regulations to provide for the treatment of an inves-
2 tigational drug, biological product, or device that re-
3 ceives Accelerated Approval under this section. This
4 section shall be carried out in accordance with such
5 regulations (and any successor regulations).

6 “(2) APPLICATION.—A sponsor of an investiga-
7 tional drug, biological product, or device applying for
8 Accelerated Approval shall submit to the Secretary
9 an application as described under section 505(b)(1)
10 or 505(b)(2), or section 510(k) or 515(c)(1) of this
11 Act, or section 351(a) of the Public Health Service
12 Act, as applicable, which shall contain—

13 “(A) data and information that the drug,
14 biological product, or device has an effect on a
15 clinical endpoint or on a surrogate endpoint or
16 biomarker that is reasonably likely to predict
17 clinical benefit to a patient (who may be rep-
18 resentative of a small patient subpopulation)
19 suffering from a serious or life-threatening con-
20 dition or disease; and

21 “(B) a statement that the sponsor is ac-
22 tively pursuing marketing approval with due
23 diligence.

24 “(3) DETERMINATION BY SECRETARY.—

1 “(A) IN GENERAL.—Not later than 120
2 days after the receipt of an application for Ac-
3 celerated Approval, the Secretary shall either—

4 “(i) provide Accelerated Approval of
5 the application; or

6 “(ii) refer the application to the Accel-
7 erated Approval Advisory Committee.

8 “(B) RECOMMENDATION.—Not later than
9 90 days after receipt of an application for Ac-
10 celerated Approval, the Accelerated Approval
11 Advisory Committee shall issue a recommenda-
12 tion to the Secretary on whether the Secretary
13 should provide Accelerated Approval of the ap-
14 plication.

15 “(C) LIMITATION.—The Accelerated Ap-
16 proval Advisory Committee shall not consider
17 off-label uses of drugs, biological products, and
18 devices as existing or available therapies, to the
19 extent that the Accelerated Approval Advisory
20 Committee weighs existing or available thera-
21 pies in determination of whether an investiga-
22 tional drug provides an improvement over treat-
23 ments that are already available.

24 “(D) FINAL DECISION.—Not later than 30
25 days after receipt of the recommendation from

1 the Accelerated Approval Advisory Committee,
2 the Secretary shall either provide Accelerated
3 Approval of the application or issue an order
4 setting forth a detailed explanation of the rea-
5 sons why the application was not so approved
6 and the specific data that the sponsor must
7 provide so that the application may be so ap-
8 proved.

9 “(4) APPEAL.—If the Secretary does not pro-
10 vide Accelerated Approval of an application, the
11 sponsor of the application shall have the right to ap-
12 peal the decision to the Secretary. The Secretary
13 shall provide the sponsor with a hearing not later
14 than 30 days following the nonapproval under this
15 subsection of the application and shall issue an order
16 not later than 30 days following the hearing either
17 concurring in such nonapproval or so approving the
18 application. The Secretary shall not delegate the re-
19 sponsibility described in this paragraph to any other
20 person.

21 “(A) with respect to a new drug or new bi-
22 ological product, approval of such drug or prod-
23 uct under section 505(b)(1) or 505(b)(2) or
24 section 351 of the Public Health Service Act, as
25 the case may be; and

1 “(B) with respect to a new device, clear-
2 ance of such device under section 510(k) or ap-
3 proval of such device under section 515(c)(1).

4 “(b) ACCELERATED APPROVAL ADVISORY COM-
5 MITTEE.—

6 “(1) IN GENERAL.—In order to facilitate the
7 development and expedite the review of drugs, bio-
8 logical products, and devices intended to treat seri-
9 ous or life threatening conditions, the Secretary shall
10 establish the Accelerated Approval Advisory Com-
11 mittee (referred to in this subsection as the ‘Com-
12 mittee’).

13 “(2) DELEGATION.—The Secretary may dele-
14 gate decisionmaking authority for the Accelerated
15 Approval Advisory Committee to the Office of the
16 Commissioner of Food and Drugs. Such authority
17 shall not be further delegated.

18 “(3) COMPOSITION.—

19 “(A) IN GENERAL.—The Committee shall
20 be composed of 11 voting members, including 1
21 chairperson and 5 permanent members each of
22 whom shall serve a term of 3 years and may be
23 reappointed for a second 3-year term, and 5
24 nonpermanent members who shall be appointed
25 to the Committee for a specific meeting, or part

1 of a meeting, in order to provide adequate ex-
2 pertise in the subject being reviewed. The Com-
3 mittee shall include as voting members no less
4 than 2 representatives of patient interests, of
5 which 1 shall be a permanent member of the
6 Committee. The Committee shall include as
7 nonvoting members a representative of interests
8 of the drug, biological product, and device in-
9 dustry.

10 “(B) APPOINTMENTS.—The Secretary
11 shall appoint to the Committee persons who are
12 qualified by training and experience to evaluate
13 the safety and effectiveness of the types of
14 products to be referred to the Committee and
15 who, to the extent feasible, possess skill in the
16 use of, or experience in the development, manu-
17 facture, or utilization of, such products. The
18 Secretary shall make appointments to the Com-
19 mittee so that the Committee shall consist of
20 members with adequately diversified expertise
21 and practical experience in such fields as clin-
22 ical medicine, biological and physical sciences,
23 and other related professions. Scientific, indus-
24 try, and consumer organizations and members
25 of the public shall be afforded an opportunity to

1 nominate individuals for appointment to the
2 Committee. No individual who is in the regular
3 full-time employ of the United States and en-
4 gaged in the administration of this chapter may
5 be a member of the Committee.

6 “(4) COMPENSATION.—Committee members,
7 while attending meetings or conferences of the Com-
8 mittee or otherwise engaged in its business, shall be
9 entitled to receive compensation at rates to be fixed
10 by the Secretary, but not at rates exceeding the
11 daily equivalent of the rate in effect for grade GS-
12 18 of the General Schedule, for each day so en-
13 gaged, including traveltime, and while so serving
14 away from their homes or regular places of business
15 each member may be allowed travel expenses (in-
16 cluding per diem in lieu of subsistence) as author-
17 ized by section 5703 of title 5, for persons in the
18 Government service employed intermittently.

19 “(5) ASSISTANCE.—The Secretary shall furnish
20 the Committee with adequate clerical and other nec-
21 essary assistance.

22 “(6) ANNUAL TRAINING.—The Secretary shall
23 employ nongovernmental experts to provide annual
24 training to the Committee on the statutory and reg-
25 ulatory standards for product approval.

1 “(7) TIMELINE.—The Committee shall be
2 scheduled to meet at such times as may be appro-
3 priate for the Secretary to meet applicable statutory
4 deadlines.

5 “(8) MEETINGS.—

6 “(A) OPPORTUNITIES FOR INTERESTED
7 PERSONS.—Any person whose product is spe-
8 cifically the subject of review by the Committee
9 shall have—

10 “(i) the same access to data and in-
11 formation submitted to the Committee as
12 the Secretary;

13 “(ii) the opportunity to submit, for re-
14 view by the Committee, data or informa-
15 tion, which shall be submitted to the Sec-
16 retary for prompt transmittal to the Com-
17 mittee;

18 “(iii) the same opportunity as the
19 Secretary to participate in meetings of the
20 Committee; and

21 “(iv) consent for the manufacturer of
22 the product to obtain data about adverse
23 events relating to the patient’s use of the
24 product.

1 “(B) ADEQUATE TIME; FREE AND OPEN
2 PARTICIPATION.—Any meetings of the Com-
3 mittee shall provide adequate time for initial
4 presentations and for response to any differing
5 views by persons whose products are specifically
6 the subject of the Committee review.

7 “(C) SUMMARIES.—At all meetings of the
8 Committee, the Secretary shall provide a sum-
9 mary to the Committee of all applications sub-
10 mitted under this subsection and section 561(d)
11 that the Committee did not consider that were
12 approved by the Secretary since the last meet-
13 ing of the Committee.

14 “(c) FINAL APPROVAL.—For purposes of this Act,
15 the term ‘final approval’ means—

16 “(1) with respect to a new drug or new biologi-
17 cal product, approval of such drug or product under
18 section 505(b)(1) or 505(b)(2) or section 351 of the
19 Public Health Service Act, as the case may be; and

20 “(2) with respect to a new device, clearance of
21 such device under section 510(k) or approval of such
22 device under section 515(c)(1).”.

23 “(c) REGULATIONS.—The Secretary of Health and
24 Human Services shall promulgate regulations that define
25 the terms “seriously ill” and “serious or life-threatening”

1 for purposes of the amendments made by this Act, consid-
2 ering either—

3 (1) the medical prognosis for an individual’s life
4 expectancy from a disease or condition; or

5 (2) the prospect of irreversible disability from a
6 disease or condition.

7 (d) CONFORMING AMENDMENT.—Subsection (a)(2)
8 of section 351 of the Public Health Service Act is amended
9 adding at the end the following subparagraphs:

10 “(E) COMPASSIONATE INVESTIGATIONAL
11 ACCESS.—A person that submits an application
12 for a license under this paragraph and seeks
13 approval of the license for the purpose of Com-
14 passionate Investigational Access under section
15 561 of the Federal Food, Drug ,and Cosmetic
16 Act shall submit to the Secretary as part of the
17 application any information required under
18 such section.

19 “(F) ACCELERATED APPROVAL.—A person
20 that submits an application for a license under
21 this paragraph and seeks accelerated approval
22 of the license under section 561A of the Federal
23 Food, Drug, and Cosmetic Act shall submit to
24 the Secretary as part of the application any in-
25 formation required under such section.”.

1 **SEC. 4. EXPANDED ACCESS TO INVESTIGATIONAL DRUGS**
2 **AND DEVICES.**

3 Chapter V of the Federal Food, Drug, and Cosmetic
4 Act (21 U.S.C. 351 et seq.) is amended by inserting after
5 section 561A, as added by section 3, the following:

6 **“SEC. 561B. EXPANDED ACCESS TO INVESTIGATIONAL**
7 **DRUGS AND DEVICES.**

8 “(a) EXPANDED ACCESS PROGRAM.—The Secretary
9 shall establish a new program to expand access to inves-
10 tigational treatments for individuals with serious or life
11 threatening conditions and diseases. In carrying out this
12 expanded access program, the Secretary shall publish and
13 broadly disseminate written guidance that—

14 “(1) describes such expanded access programs
15 for investigational drugs, biological products, and de-
16 vices intended to treat serious or life-threatening
17 conditions or diseases;

18 “(2) encourages and facilitates submission of
19 applications and approvals under sections 561(d)
20 and 561A; and

21 “(3) facilitates the provision of investigational
22 drugs, biological products, and devices to seriously ill
23 individuals without unreasonable delay by recog-
24 nizing that the use of available investigational prod-
25 ucts for treatment is the responsibility of the physi-
26 cian and the patient, and also by recognizing the

1 goal of providing additional treatment options for
2 patients and their physicians to consider.

3 “(b) IMPLEMENTATION OF EXPANDED ACCESS PRO-
4 GRAMS.—

5 “(1) TRAINING OF PERSONNEL.—Not later
6 than 90 days after the date of enactment of this sec-
7 tion, the Secretary shall implement training pro-
8 grams at the Food and Drug Administration with
9 respect to the expanded access programs established
10 under this section.

11 “(2) POLICIES, REGULATIONS, AND GUID-
12 ANCE.—The Secretary shall establish policies, regu-
13 lations, and guidance designed to most directly ben-
14 efit seriously ill patients.

15 “(c) DEVELOPMENT OF SURROGATE ENDPOINTS
16 AND BIOMARKERS.—

17 “(1) IN GENERAL.—The Secretary shall—

18 “(A) establish a program or expand upon
19 an existing program to encourage the develop-
20 ment of surrogate endpoints and biomarkers
21 that are reasonably likely to predict clinical
22 benefit for serious or life-threatening conditions
23 for which there exist significant unmet patient
24 needs;

1 “(B) request the Institute of Medicine to
2 undertake a study to identify validated surro-
3 gate endpoints and biomarkers, and recommend
4 research to validate surrogate endpoints and
5 biomarkers, that may support approvals for
6 products intended for the treatment of serious
7 or life-threatening conditions or diseases; and

8 “(C) make widely available to the public a
9 list of drugs, biological products, and devices
10 that are being investigated for serious or life-
11 threatening conditions or diseases and that
12 have not yet received approval under section
13 section 561(d) or 561A for marketing.

14 “(2) STUDY CONTENT.—The study under para-
15 graph (1)(B) shall include endpoints and biomarkers
16 that address the unmet medical needs of subpopula-
17 tions of patients and that facilitate the development
18 of individualized treatment approaches for patients
19 with serious or life-threatening conditions or dis-
20 eases.”.

1 **SEC. 5. DEMONSTRATION PROJECT FOR COVERAGE OF**
2 **CERTAIN DRUGS, BIOLOGICAL PRODUCTS,**
3 **AND DEVICES UNDER THE MEDICARE PRO-**
4 **GRAM.**

5 (a) IN GENERAL.—The Secretary of Health and
6 Human Services (in this section referred to as the “Sec-
7 retary”) shall establish a demonstration project under the
8 Medicare program under title XVIII of the Social Security
9 Act (42 U.S.C. 1395 et seq.) under which payment is
10 made for drugs, biological products, and devices approved
11 for Compassionate Investigational Access under section
12 section 561(d) of the Federal Food, Drug, and Cosmetic
13 Act in the case where the drug, biological product, or de-
14 vice is not otherwise covered under the Medicare program
15 or by any other organization or entity (including a public
16 assistance program or the sponsor of the application for
17 such drug, biological product, or device).

18 (b) DURATION.—The demonstration project under
19 this section shall be conducted for a 5-year period.

20 (c) FUNDING.—

21 (1) IN GENERAL.—The Secretary shall provide
22 for the transfer from the Federal Hospital Insurance
23 Trust Fund under section 1817 of the Social Secu-
24 rity Act (42 U.S.C. 1395i) and the Federal Supple-
25 mentary Medical Insurance Trust Fund under sec-
26 tion 1841 of such Act (42 U.S.C. 1395t), in such

1 proportion as the Secretary determines to be appro-
2 priate, of such sums as are necessary for the costs
3 of carrying out the demonstration project under this
4 section.

5 (2) BUDGET NEUTRALITY.—In conducting the
6 demonstration project under this section, the Sec-
7 retary shall ensure that the aggregate payments
8 made by the Secretary do not exceed the amount
9 which the Secretary estimates would have been paid
10 if the demonstration project under this section was
11 not implemented.

12 (d) WAIVER AUTHORITY.—The Secretary may waive
13 such requirements of title XVIII of the Social Security Act
14 (42 U.S.C. 1395 et seq.) as may be necessary for the pur-
15 pose of carrying out the demonstration project under this
16 section.

17 (e) REPORT.—Not later than 90 days after the last
18 day of the 5-year period of the demonstration project
19 under this section, the Secretary shall submit to Congress
20 a report describing the rates of utilization by Medicare
21 beneficiaries of drugs, biological products, and devices ap-
22 proved for Compassionate Investigational Access and the
23 total cost of payments made under the Medicare program
24 resulting from the demonstration project. The report shall

1 describe recommendations for legislation or administrative
2 action as the Secretary deems appropriate.

3 (f) TERMINATION.—The Secretary shall terminate
4 payments under this section on the day after the last day
5 of the 5-year period of the demonstration project under
6 this section.

7 **SEC. 6. MODERNIZATION OF THE FOOD AND DRUG ADMIN-**
8 **ISTRATION.**

9 (a) IN GENERAL.—Subchapter E of chapter V of the
10 Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb
11 et seq.) is amended by adding at the end the following:

12 **“SEC. 568. POLICIES RELATED TO STUDY EVALUATION IN-**
13 **FORMATION.**

14 “(a) IN GENERAL.—

15 “(1) NONSTATISTICAL MEASURES.—The Sec-
16 retary shall give consideration to clinical judgment
17 and risks to the patient from the disease or condi-
18 tion involved in the evaluation of the safety and ef-
19 fectiveness of drugs, biological products, and devices
20 that treat serious or life-threatening diseases or con-
21 ditions. This policy shall apply—

22 “(A) in evaluating clinical study designs
23 and endpoints; and

1 “(B) in making decisions with respect to
2 product applications for approval under section
3 section 561(d) or 561A.

4 “(2) TYPES OF NONSTATISTICAL MEASURES.—
5 The policy established under paragraph (1), for the
6 purposes described in such paragraph—

7 “(A) shall include such nonstatistical infor-
8 mation as—

9 “(i) clinical evaluation information,
10 such as case history reports;

11 “(ii) scientific and clinical studies de-
12 signed to measure or define mechanisms of
13 action or molecular targeting;

14 “(iii) data from animal and computer
15 models; and

16 “(iv) comparison with historical data;
17 and

18 “(B) shall incorporate the use of—

19 “(i) evaluations of the adverse effect
20 of delaying the availability of an investiga-
21 tional drug to even a small subpopulation
22 of seriously ill patients; and

23 “(ii) scientific, observational, or clin-
24 ical studies designed and conducted to col-
25 lect well-documented information.

1 “(b) MEETINGS.—A meeting to address any pending
2 scientific, medical, regulatory, or other issue relating to
3 the development, investigation, review, or other aspect of
4 a drug, biological product, or device shall ordinarily be
5 held not later than 15 days of the receipt of a written
6 request for the meeting by the sponsor of the product,
7 which may be extended to 30 days for good cause. Such
8 meetings shall ordinarily be conducted in person, but may
9 be conducted by telephone or other form of communication
10 if both parties agree. In order to reduce the burden of
11 meetings, only those Food and Drug Administration em-
12 ployees who are intended to actively participate in the dis-
13 cussion shall attend a meeting. Minutes of a meeting shall
14 be promptly prepared and exchanged by both parties im-
15 mediately following the meeting and shall accurately sum-
16 marize what occurred at the meeting.

17 “(c) RULE OF CONSTRUCTION.—The provisions of
18 this chapter and section 351 of the Public Health Service
19 Act shall be construed to incorporate the policy established
20 in this section.”.

21 (b) CONFORMING AMENDMENT.—Subsection (j) of
22 section 351 of the Public Health Service Act is amended
23 by—

- 24 (1) striking “and” before “505-1”; and
25 (2) inserting “, and 568” after “505-1”.

1 **SEC. 7. MEMBERSHIP OF ONCOLOGY DRUGS ADVISORY**
2 **COMMITTEE AND THE CELLULAR, TISSUE,**
3 **AND GENE THERAPY ADVISORY COMMITTEE.**

4 Notwithstanding any other provision of law, member-
5 ship of the Oncology Drugs Advisory Committee, the Cel-
6 lular, Tissue, and Gene Therapy Advisory Committee of
7 the Food and Drug Administration, and any other com-
8 mittee created by such Administration to evaluate or ad-
9 vise with respect to applications submitted under section
10 section 561(d) or 561A of the Federal Food, Drug, and
11 Cosmetic Act (as added by this Act), shall consist of no
12 less than 2 patient representatives who are voting mem-
13 bers of the committee.

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