

110TH CONGRESS
2D SESSION

H. R. 6270

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

JUNE 12, 2008

Ms. WATSON (for herself, Mr. BURTON of Indiana, Mr. LATHAM, Ms. EDDIE BERNICE JOHNSON of Texas, Ms. SOLIS, Ms. JACKSON-LEE of Texas, Mr. CUMMINGS, and Mr. CONYERS) 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,*

1 **SECTION 1. SHORT TITLE.**

2 This Act may be cited as the “Access, Compassion,
3 Care, and Ethics for Seriously Ill Patients Act” or the
4 “ACCESS Act”.

5 **SEC. 2. FINDINGS.**

6 Congress finds the following:

7 (1) As of 2008, the standards of the Food and
8 Drug Administration for approval of drugs, biologi-
9 cal products, and devices may deny the benefits of
10 medical progress to seriously ill patients who face
11 morbidity or death from their disease.

12 (2) Seriously ill patients have a right to take
13 actions to preserve their life by accessing available
14 investigational drugs, biological products, and de-
15 vices.

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

23 (4) Food and Drug Administration advisory
24 committees should have greater representation of
25 medical clinicians and patient advocates who rep-

1 resent the interests of seriously ill patients in early
2 access to promising investigational therapies.

3 (5) The use of available investigational products
4 for treatment is the responsibility of the physician
5 and the seriously-ill patient.

6 (6) The use of combinations of available inves-
7 tigational and approved products for treatment is
8 the responsibility of the physician and the seriously-
9 ill patient.

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

14 **SEC. 3. COMPASSIONATE INVESTIGATIONAL ACCESS AP-**
15 **PROVAL SYSTEM FOR DRUGS, BIOLOGICAL**
16 **PRODUCTS, AND DEVICES.**

17 (a) COMPASSIONATE INVESTIGATIONAL ACCESS.—
18 Section 561 of the Federal Food, Drug, and Cosmetic Act
19 (21 U.S.C. 360bbb) is amended—

20 (1) by redesignating subsections (d) and (e) as
21 subsections (e) and (f), respectively; and

22 (2) by inserting after subsection (c) the fol-
23 lowing:

24 “(d) COMPASSIONATE INVESTIGATIONAL ACCESS.—

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

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

19 “(3) COMPASSIONATE INVESTIGATIONAL AC-
20 CESS.—Notwithstanding any other provision of law,
21 an investigational drug, biological product, or device
22 that receives approval for Compassionate Investiga-
23 tional Access under this subsection shall be subject
24 to the provisions of section 505(i) or 520(g), as ap-
25 plicable, and regulations promulgated by the Sec-

1 retary pursuant to this Act. The Secretary and the
2 sponsor may inform national, State, and local med-
3 ical associations and societies, voluntary health asso-
4 ciations, and other appropriate persons about the
5 availability of an investigational drug or investiga-
6 tional device under Compassionate Investigational
7 Access as approved under this subsection. The infor-
8 mation submitted by the Secretary, in accordance
9 with the preceding sentence, shall be the same type
10 of information that is required by section 402(i)(3)
11 of the Public Health Service Act.

12 “(4) SUBMISSION OF APPLICATION.—

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

22 “(i) data and information from com-
23 pleted Phase I clinical investigations and
24 any other nonclinical or clinical investiga-
25 tions;

1 “(ii) preliminary evidence that the
2 product may be effective in humans
3 against a serious or life-threatening condi-
4 tion or disease, which evidence may be
5 based on uncontrolled data such as case
6 histories, information about the pharma-
7 cological mechanism of action, data from
8 animal and computer models, comparison
9 with historical data, or other preliminary
10 information, and may be based on a small
11 number of patients or a subset of the pa-
12 tient population;

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

22 “(iv) a statement that the sponsor is
23 actively pursuing marketing approval with
24 due diligence.

1 “(B) LIMITATION.—Compassionate Inves-
2 tigational Access approval shall be based upon
3 multiple considerations that shall include clin-
4 ical evaluation and unmet patient needs.

5 “(5) DETERMINATION BY SECRETARY.—

6 “(A) IN GENERAL.—Not later than 30
7 days after the receipt of an application for
8 Compassionate Investigational Access approval,
9 the Secretary shall either—

10 “(i) provide Compassionate Investiga-
11 tional Access approval of the application;
12 or

13 “(ii) refer the application to the Accel-
14 erated Approval Advisory Committee.

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

23 “(C) FINAL DECISION.—Not later than 30
24 days after receipt of the recommendation from
25 the Accelerated Approval Advisory Committee,

1 the Secretary shall either provide Compas-
2 sionate Investigational Access approval of the
3 application or shall issue an order setting forth
4 a detailed explanation of the reasons why the
5 application was not so approved and the specific
6 data that the sponsor must provide so that the
7 application may be so approved.

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

20 “(7) CRITERIA.—In making a determination
21 under paragraph (5), the Secretary shall consider
22 whether the totality of the information available to
23 the Secretary regarding the safety and effectiveness
24 of an investigational drug, biological product, or de-
25 vice, as compared to the risk of morbidity or death

1 from a condition or disease, indicates that a patient
2 (who may be representative of a small patient sub-
3 population) may obtain more benefit than risk if
4 treated with the drug, biological product, or device.
5 If the potential risk to a patient of the condition or
6 disease outweighs the potential risk of the product,
7 and the product may possibly provide benefit to the
8 patient, the Secretary shall provide Compassionate
9 Investigational Access approval of the application.

10 “(8) PATIENT ELIGIBILITY FOR COMPAS-
11 SIONATE ACCESS.—In order for a patient to access
12 a product available through Compassionate Inves-
13 tigational Access, the physician must document in
14 writing that the patient—

15 “(A) is seriously ill;

16 “(B) has exhausted all treatment options
17 approved by the Secretary for the condition or
18 disease for which the patient is a reasonable
19 candidate; and

20 “(C) has unsuccessfully sought treatment
21 or obtained treatment that was not effective,
22 with an investigational drug, biological product,
23 or device for which such individual is a reason-
24 able candidate, which shall include consider-
25 ation of a patient’s ineligibility for participation

1 in clinical trials, the lack of source of supply
2 and geographic factors.

3 “(9) PRODUCT LABELING.—To receive Compas-
4 sionate Investigational Access approval under this
5 subsection, the sponsor of the product shall provide
6 labeling approved by the Secretary for the drug, bio-
7 logical product, or device that—

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

11 “(i) exhausted all treatment options
12 approved by Secretary for the condition or
13 disease for which the patient is a reason-
14 able candidate; and

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

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

4 “(i) written informed consent, as de-
5 scribed under part 50 of title 21, Code of
6 Federal Regulations (or any successor reg-
7 ulations); and

8 “(ii) consent for the manufacturer of
9 the product to obtain data and information
10 about the patient and the patient’s use of
11 the product that may be used to support
12 an application for Accelerated Approval or
13 final approval.

14 “(10) CHARGING FOR COMPASSIONATE INVES-
15 TIGATIONAL ACCESS.—A sponsor or investigator
16 may charge for a Compassionate Investigational Ac-
17 cess drug without notifying the Secretary or seeking
18 or obtaining prior approval of the amount charged,
19 provided the sponsor of the drug is actively pursuing
20 marketing approval with due diligence.

21 “(11) COMMENCEMENT OF REVIEW.—If the
22 Secretary determines, after preliminary evaluation of
23 the data and information submitted by the sponsor,
24 that the product may be effective, the Secretary
25 shall evaluate for filing, and may commence review

1 of portions of, an application under this subsection
2 before the sponsor submits a complete application.
3 The Secretary shall commence such review only if
4 the applicant provides a schedule for submission of
5 information necessary to make the application com-
6 plete.

7 “(12) IMMUNITY.—

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

21 “(B) CLAIMS.—No claim or cause of ac-
22 tion against a manufacturer, distributor, ad-
23 ministrator, sponsor, or physician who manu-
24 factures, supplies, distributes or prescribes a
25 product subject to an approved Compassionate

1 Investigational Access application shall exist in
2 any Federal or State court for claims of prop-
3 erty, personal injury, or death caused by, aris-
4 ing out of, or relating to the design, develop-
5 ment, clinical testing and investigation, manu-
6 facture, labeling, distribution, sale, purchase,
7 donation, dispensing, prescribing, administra-
8 tion, efficacy, or use of a drug, biological prod-
9 uct, or device subject to an approved Compas-
10 sionate Investigational Access application. Any
11 such claim or cause of action that is filed in
12 Federal or State court shall be immediately dis-
13 missed.

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

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

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

24 (b) ACCELERATED APPROVAL.—Chapter V of the
25 Federal, Food, Drug, and Cosmetic Act (21 U.S.C. 351

1 et seq.) is amended by inserting after section 561 the fol-
2 lowing:

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

4 “(a) IN GENERAL.—

5 “(1) IN GENERAL.—As soon as practicable
6 after the date of enactment of the Access, Compassion,
7 Care, and Ethics for Seriously Ill Patients Act,
8 the Secretary shall promulgate regulations to pro-
9 vide for the treatment of an investigational drug, bi-
10 ological product, or device that receives Accelerated
11 Approval under this section. This section shall be
12 carried out in accordance with such regulations (and
13 any successor regulations).

14 “(2) APPLICATION.—A sponsor of an investiga-
15 tional drug, biological product, or device applying for
16 Accelerated Approval shall submit to the Secretary
17 an application as described under section 505(b)(1)
18 or 505(b)(2), or section 510(k) or 515(c)(1) of this
19 Act, or section 351(a) of the Public Health Service
20 Act, as applicable, which shall contain—

21 “(A) data and information that the drug,
22 biological product, or device has an effect on a
23 clinical endpoint or on a surrogate endpoint or
24 biomarker that is reasonably likely to predict
25 clinical benefit to a patient (who may be rep-

1 representative of a small patient subpopulation)
2 suffering from a serious or life-threatening con-
3 dition or disease; and

4 “(B) a statement that the sponsor is ac-
5 tively pursuing marketing approval with due
6 diligence.

7 “(3) DETERMINATION BY SECRETARY.—

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

11 “(i) provide Accelerated Approval of
12 the application; or

13 “(ii) refer the application to the Accel-
14 erated Approval Advisory Committee.

15 “(B) RECOMMENDATION.—Not later than
16 90 days after receipt of an application for Ac-
17 celerated Approval, the Accelerated Approval
18 Advisory Committee shall issue a recommenda-
19 tion to the Secretary on whether the Secretary
20 should provide Accelerated Approval of the ap-
21 plication.

22 “(C) LIMITATION.—The Accelerated Ap-
23 proval Advisory Committee shall not consider
24 off-label uses of drugs, biological products, and
25 devices as existing or available therapies, to the

1 extent that the Accelerated Approval Advisory
2 Committee weighs existing or available thera-
3 pies in determination of whether an investiga-
4 tional drug provides an improvement over treat-
5 ments that are already available.

6 “(D) FINAL DECISION.—Not later than 30
7 days after receipt of the recommendation from
8 the Accelerated Approval Advisory Committee,
9 the Secretary shall either provide Accelerated
10 Approval of the application or issue an order
11 setting forth a detailed explanation of the rea-
12 sons why the application was not so approved
13 and the specific data that the sponsor must
14 provide so that the application may be so ap-
15 proved.

16 “(4) APPEAL.—If the Secretary does not pro-
17 vide Accelerated Approval of an application, the
18 sponsor of the application shall have the right to ap-
19 peal the decision to the Secretary. The Secretary
20 shall provide the sponsor with a hearing not later
21 than 30 days following the nonapproval under this
22 subsection of the application and shall issue an order
23 not later than 30 days following the hearing either
24 concurring in such nonapproval or so approving the
25 application. The Secretary shall not delegate the re-

1 sponsibility described in this paragraph to any other
2 person.

3 “(A) with respect to a new drug or new bi-
4 ological product, approval of such drug or prod-
5 uct under section 505(b)(1) or 505(b)(2) or
6 section 351 of the Public Health Service Act, as
7 the case may be; and

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

11 “(b) ACCELERATED APPROVAL ADVISORY COM-
12 MITTEE.—

13 “(1) IN GENERAL.—In order to facilitate the
14 development and expedite the review of drugs, bio-
15 logical products, and devices intended to treat seri-
16 ous or life threatening conditions, the Secretary shall
17 establish the Accelerated Approval Advisory Com-
18 mittee (referred to in this subsection as the ‘Com-
19 mittee’).

20 “(2) DELEGATION.—The Secretary may dele-
21 gate decisionmaking authority for the Accelerated
22 Approval Advisory Committee to the Office of the
23 Commissioner of Food and Drugs. Such authority
24 shall not be further delegated.

25 “(3) COMPOSITION.—

1 “(A) IN GENERAL.—The Committee shall
2 be composed of 11 voting members, including 1
3 chairperson and 5 permanent members each of
4 whom shall serve a term of 3 years and may be
5 reappointed for a second 3-year term, and 5
6 nonpermanent members who shall be appointed
7 to the Committee for a specific meeting, or part
8 of a meeting, in order to provide adequate ex-
9 pertise in the subject being reviewed. The Com-
10 mittee shall include as voting members no less
11 than 2 representatives of patient interests, of
12 which 1 shall be a permanent member of the
13 Committee. The Committee shall include as
14 nonvoting members a representative of interests
15 of the drug, biological product, and device in-
16 dustry.

17 “(B) APPOINTMENTS.—The Secretary
18 shall appoint to the Committee persons who are
19 qualified by training and experience to evaluate
20 the safety and effectiveness of the types of
21 products to be referred to the Committee and
22 who, to the extent feasible, possess skill in the
23 use of, or experience in the development, manu-
24 facture, or utilization of, such products. The
25 Secretary shall make appointments to the Com-

1 mittee so that the Committee shall consist of
2 members with adequately diversified expertise
3 and practical experience in such fields as clin-
4 ical medicine, biological and physical sciences,
5 and other related professions. Scientific, indus-
6 try, and consumer organizations and members
7 of the public shall be afforded an opportunity to
8 nominate individuals for appointment to the
9 Committee. No individual who is in the regular
10 full-time employ of the United States and en-
11 gaged in the administration of this chapter may
12 be a member of the Committee.

13 “(4) COMPENSATION.—Committee members,
14 while attending meetings or conferences of the Com-
15 mittee or otherwise engaged in its business, shall be
16 entitled to receive compensation at rates to be fixed
17 by the Secretary, but not at rates exceeding the
18 daily equivalent of the rate in effect for grade GS-
19 18 of the General Schedule, for each day so en-
20 gaged, including traveltime, and while so serving
21 away from their homes or regular places of business
22 each member may be allowed travel expenses (in-
23 cluding per diem in lieu of subsistence) as author-
24 ized by section 5703 of title 5, for persons in the
25 Government service employed intermittently.

1 “(5) ASSISTANCE.—The Secretary shall furnish
2 the Committee with adequate clerical and other nec-
3 essary assistance.

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

8 “(7) TIMELINE.—The Committee shall be
9 scheduled to meet at such times as may be appro-
10 priate for the Secretary to meet applicable statutory
11 deadlines.

12 “(8) MEETINGS.—

13 “(A) OPPORTUNITIES FOR INTERESTED
14 PERSONS.—Any person whose product is spe-
15 cifically the subject of review by the Committee
16 shall have—

17 “(i) the same access to data and in-
18 formation submitted to the Committee as
19 the Secretary;

20 “(ii) the opportunity to submit, for re-
21 view by the Committee, data or informa-
22 tion, which shall be submitted to the Sec-
23 retary for prompt transmittal to the Com-
24 mittee;

1 “(iii) the same opportunity as the
2 Secretary to participate in meetings of the
3 Committee; and

4 “(iv) consent for the manufacturer of
5 the product to obtain data about adverse
6 events relating to the patient’s use of the
7 product.

8 “(B) ADEQUATE TIME; FREE AND OPEN
9 PARTICIPATION.—Any meetings of the Com-
10 mittee shall provide adequate time for initial
11 presentations and for response to any differing
12 views by persons whose products are specifically
13 the subject of the Committee review.

14 “(C) SUMMARIES.—At all meetings of the
15 Committee, the Secretary shall provide a sum-
16 mary to the Committee of all applications sub-
17 mitted under this subsection and section 561(d)
18 that the Committee did not consider that were
19 approved by the Secretary since the last meet-
20 ing of the Committee.

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

23 “(1) with respect to a new drug or new biologi-
24 cal product, approval of such drug or product under

1 section 505(b)(1) or 505(b)(2) or section 351 of the
2 Public Health Service Act, as the case may be; and

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

6 (c) REGULATIONS.—The Secretary of Health and
7 Human Services shall promulgate regulations that define
8 the terms “seriously ill” and “serious or life-threatening”
9 for purposes of the amendments made by this Act, consid-
10 ering either—

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

13 (2) the prospect of irreversible disability from a
14 disease or condition.

15 **SEC. 4. EXPANDED ACCESS TO INVESTIGATIONAL DRUGS**
16 **AND DEVICES.**

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

20 **“SEC. 561B. EXPANDED ACCESS TO INVESTIGATIONAL**
21 **DRUGS AND DEVICES.**

22 “(a) EXPANDED ACCESS PROGRAM.—The Secretary
23 shall establish a new program to expand access to inves-
24 tigational treatments for individuals with serious or life
25 threatening conditions and diseases. In carrying out this

1 expanded access program, the Secretary shall publish and
2 broadly disseminate written guidance that—

3 “(1) describes such expanded access programs
4 for investigational drugs, biological products, and de-
5 vices intended to treat serious or life-threatening
6 conditions or diseases;

7 “(2) encourages and facilitates submission of
8 applications and approvals under section 561(d) and
9 561A; and

10 “(3) facilitates the provision of investigational
11 drugs, biological products, and devices to seriously ill
12 individuals without unreasonable delay by recog-
13 nizing that the use of available investigational prod-
14 ucts for treatment is the responsibility of the physi-
15 cian and the patient, and also by recognizing the
16 goal of providing additional treatment options for
17 patients and their physicians to consider.

18 “(b) IMPLEMENTATION OF EXPANDED ACCESS PRO-
19 GRAMS.—

20 “(1) TRAINING OF PERSONNEL.—Not later
21 than 90 days after the date of enactment of this sec-
22 tion, the Secretary shall implement training pro-
23 grams at the Food and Drug Administration with
24 respect to the expanded access programs established
25 under this section.

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

5 “(c) DEVELOPMENT OF SURROGATE ENDPOINTS
6 AND BIOMARKERS.—

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

8 “(A) establish a program or expand upon
9 an existing program to encourage the develop-
10 ment of surrogate endpoints and biomarkers
11 that are reasonably likely to predict clinical
12 benefit for serious or life-threatening conditions
13 for which there exist significant unmet patient
14 needs;

15 “(B) request the Institute of Medicine to
16 undertake a study to identify validated surro-
17 gate endpoints and biomarkers, and recommend
18 research to validate surrogate endpoints and
19 biomarkers, that may support approvals for
20 products intended for the treatment of serious
21 or life-threatening conditions or diseases; and

22 “(C) make widely available to the public a
23 list of drugs, biological products, and devices
24 that are being investigated for serious or life-
25 threatening conditions or diseases and that

1 have not yet received approval under section
2 561(d) or 561A for marketing.

3 “(2) STUDY CONTENT.—The study under para-
4 graph (1)(B) shall include endpoints and biomarkers
5 that address the unmet medical needs of subpopula-
6 tions of patients and that facilitate the development
7 of individualized treatment approaches for patients
8 with serious or life-threatening conditions or dis-
9 eases.”.

10 **SEC. 5. DEMONSTRATION PROJECT FOR COVERAGE OF**
11 **CERTAIN DRUGS, BIOLOGICAL PRODUCTS,**
12 **AND DEVICES UNDER THE MEDICARE PRO-**
13 **GRAM.**

14 (a) IN GENERAL.—The Secretary of Health and
15 Human Services (in this section referred to as the “Sec-
16 retary”) shall establish a demonstration project under the
17 Medicare program under title XVIII of the Social Security
18 Act (42 U.S.C. 1395 et seq.) under which payment is
19 made for drugs, biological products, and devices approved
20 for Compassionate Investigational Access under section
21 561(d) of the Federal Food, Drug, and Cosmetic Act in
22 the case where the drug, biological product, or device is
23 not otherwise covered under the Medicare program or by
24 any other organization or entity (including a public assist-

1 ance program or the sponsor of the application for such
2 drug, biological product, or device).

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

5 (c) FUNDING.—

6 (1) IN GENERAL.—The Secretary shall provide
7 for the transfer from the Federal Hospital Insurance
8 Trust Fund under section 1817 of the Social Secu-
9 rity Act (42 U.S.C. 1395i) and the Federal Supple-
10 mentary Medical Insurance Trust Fund under sec-
11 tion 1841 of such Act (42 U.S.C. 1395t), in such
12 proportion as the Secretary determines to be appro-
13 priate, of such sums as are necessary for the costs
14 of carrying out the demonstration project under this
15 section.

16 (2) BUDGET NEUTRALITY.—In conducting the
17 demonstration project under this section, the Sec-
18 retary shall ensure that the aggregate payments
19 made by the Secretary do not exceed the amount
20 which the Secretary estimates would have been paid
21 if the demonstration project under this section was
22 not implemented.

23 (d) WAIVER AUTHORITY.—The Secretary may waive
24 such requirements of title XVIII of the Social Security Act
25 (42 U.S.C. 1395 et seq.) as may be necessary for the pur-

1 pose of carrying out the demonstration project under this
2 section.

3 (e) REPORT.—Not later than 90 days after the last
4 day of the 5-year period of the demonstration project
5 under this section, the Secretary shall submit to Congress
6 a report describing the rates of utilization by Medicare
7 beneficiaries of drugs, biological products, and devices ap-
8 proved for Compassionate Investigational Access and the
9 total cost of payments made under the Medicare program
10 resulting from the demonstration project. The report shall
11 describe recommendations for legislation or administrative
12 action as the Secretary deems appropriate.

13 (f) TERMINATION.—The Secretary shall terminate
14 payments under this section on the day after the last day
15 of the 5-year period of the demonstration project under
16 this section.

17 **SEC. 6. USE OF PART B DEFINITION OF MEDICALLY AC-**
18 **CEPTED INDICATION FOR PART D DRUGS.**

19 (a) IN GENERAL.—Section 1860D–2(e) of the Social
20 Security Act (42 U.S.C. 1395w–102(e)) is amended—

21 (1) in paragraph (1), in the matter following
22 subparagraph (B), by striking “(as defined in sec-
23 tion 1927(k)(6))” and inserting “(as defined in
24 paragraph (4))”; and

1 (2) by adding at the end the following new
2 paragraph:

3 “(4) MEDICALLY ACCEPTED INDICATION DE-
4 FINED.—

5 “(A) IN GENERAL.—Subject to subpara-
6 graph (B), for purposes of paragraph (1), the
7 term ‘medically accepted indication’ has the
8 meaning given that term—

9 “(i) in the case of a covered part D
10 drug used in an anticancer
11 chemotherapeutic regimen, in section
12 1861(t)(2)(B), except that in applying
13 such section, ‘PDP sponsor or MA–PD
14 sponsor’ shall be substituted for ‘carrier’
15 each place it appears and the compendia
16 identified in section 1927(g)(1)(B)(i)(III)
17 shall be deemed to be included in the com-
18 pendia described in section
19 1861(t)(2)(B)(ii)(I); and

20 “(ii) in the case of any other covered
21 part D drug, in section 1927(k)(6).

22 “(B) CONSIDERATION OF OTHER CRITERIA
23 ON A CASE-BY-CASE BASIS.—Nothing in this
24 subsection shall preclude a PDP sponsor offer-
25 ing a prescription drug plan or an MA organi-

1 zation offering an MA–PD plan from, after a
2 request by an individual enrolled in the plan for
3 a coverage determination under section 1860D–
4 4(g)(1), providing coverage of a covered part D
5 drug for the individual in the case where the
6 sponsor or organization determines, based on
7 guidance provided by the Secretary for deter-
8 mining whether the use of a covered part D
9 drug is for a medically accepted indication and
10 supportive clinical evidence in peer reviewed
11 medical literature, that the use of the covered
12 part D drug is for a medically accepted indica-
13 tion.”.

14 (b) **EFFECTIVE DATE.**—The amendments made by
15 this section shall apply to plan years beginning on or after
16 January 1, 2010.

17 **SEC. 7. MODERNIZATION OF THE FOOD AND DRUG ADMIN-**
18 **ISTRATION.**

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

22 **“SEC. 568. POLICIES RELATED TO STUDY EVALUATION IN-**
23 **FORMATION.**

24 **“(a) IN GENERAL.—**

1 “(1) NONSTATISTICAL MEASURES.—The Sec-
2 retary shall give consideration to clinical judgment
3 and risks to the patient from the disease or condi-
4 tion involved in the evaluation of the safety and ef-
5 fectiveness of drugs, biological products, and devices
6 that treat serious or life-threatening diseases or con-
7 ditions. This policy shall apply—

8 “(A) in evaluating clinical study designs
9 and endpoints; and

10 “(B) in making decisions with respect to
11 product applications for approval under section
12 561(d) or 561A.

13 “(2) TYPES OF NONSTATISTICAL MEASURES.—
14 The policy established under paragraph (1), for the
15 purposes described in such paragraph—

16 “(A) shall include such nonstatistical infor-
17 mation as—

18 “(i) clinical evaluation information,
19 such as case history reports;

20 “(ii) scientific and clinical studies de-
21 signed to measure or define mechanisms of
22 action or molecular targeting;

23 “(iii) data from animal and computer
24 models; and

1 “(iv) comparison with historical data;

2 and

3 “(B) shall incorporate the use of—

4 “(i) evaluations of the adverse effect
5 of delaying the availability of an investiga-
6 tional drug to even a small subpopulation
7 of seriously ill patients; and

8 “(ii) scientific, observational, or clin-
9 ical studies designed and conducted to col-
10 lect well-documented information.

11 “(b) MEETINGS.—A meeting to address any pending
12 scientific, medical, regulatory, or other issue relating to
13 the development, investigation, review, or other aspect of
14 a drug, biological product, or device shall ordinarily be
15 held not later than 15 days of the receipt of a written
16 request for the meeting by the sponsor of the product,
17 which may be extended to 30 days for good cause. Such
18 meetings shall ordinarily be conducted in person, but may
19 be conducted by telephone or other form of communication
20 if both parties agree. In order to reduce the burden of
21 meetings, only those Food and Drug Administration em-
22 ployees who are intended to actively participate in the dis-
23 cussion shall attend a meeting. Minutes of a meeting shall
24 be promptly prepared and exchanged by both parties im-

1 mediate following the meeting and shall accurately sum-
2 marize what occurred at the meeting.

3 “(c) **RULE OF CONSTRUCTION.**—The provisions of
4 this chapter and section 351 of the Public Health Service
5 Act shall be construed to incorporate the policy established
6 in this section.”.

7 **SEC. 8. MEMBERSHIP OF ONCOLOGY DRUGS ADVISORY**
8 **COMMITTEE AND THE CELLULAR, TISSUE,**
9 **AND GENE THERAPY ADVISORY COMMITTEE.**

10 Notwithstanding any other provision of law, member-
11 ship of the Oncology Drugs Advisory Committee, the Cel-
12 lular, Tissue, and Gene Therapy Advisory Committee of
13 the Food and Drug Administration, and any other com-
14 mittee created by such Administration to evaluate or ad-
15 vise with respect to applications submitted under section
16 561(d) or 561A of the Federal Food, Drug, and Cosmetic
17 Act (as added by this Act), shall consist of no less than
18 2 patient representatives who are voting members of the
19 committee.

○