

110<sup>TH</sup> CONGRESS  
2<sup>D</sup> SESSION

# S. 3046

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.

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## IN THE SENATE OF THE UNITED STATES

MAY 21, 2008

Mr. BROWNBACK (for himself, Mr. CASEY, Mr. COLEMAN, Mr. SPECTER, and Mr. INHOFE) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

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## 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 “Access, Compassion,  
5       Care, and Ethics for Seriously Ill Patients Act” or the  
6       “ACCESS Act”.

1 **SEC. 2. FINDINGS.**

2 Congress finds the following:

3 (1) As of 2008, 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 (e) 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 exhausted 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) exhausted 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                  complete.

23                  “(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 Access, Compass-  
 25                   sion, Care, and Ethics for Seriously Ill Patients Act,

1 the Secretary shall promulgate regulations to pro-  
2 vide for the treatment of an investigational drug, bi-  
3 ological product, or device that receives Accelerated  
4 Approval under this section. This section shall be  
5 carried out in accordance with such regulations (and  
6 any successor regulations).

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

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

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

25 “(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 **SEC. 4. EXPANDED ACCESS TO INVESTIGATIONAL DRUGS**  
8 **AND DEVICES.**

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

12 **“SEC. 561B. EXPANDED ACCESS TO INVESTIGATIONAL**  
13 **DRUGS AND DEVICES.**

14 “(a) **EXPANDED ACCESS PROGRAM.**—The Secretary  
15 shall establish a new program to expand access to inves-  
16 tigational treatments for individuals with serious or life  
17 threatening conditions and diseases. In carrying out this  
18 expanded access program, the Secretary shall publish and  
19 broadly disseminate written guidance that—

20 “(1) describes such expanded access programs  
21 for investigational drugs, biological products, and de-  
22 vices intended to treat serious or life-threatening  
23 conditions or diseases;

1           “(2) encourages and facilitates submission of  
2 applications and approvals under section 561(d) and  
3 561A; and

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

12           “(b) IMPLEMENTATION OF EXPANDED ACCESS PRO-  
13 GRAMS.—

14           “(1) TRAINING OF PERSONNEL.—Not later  
15 than 90 days after the date of enactment of this sec-  
16 tion, the Secretary shall implement training pro-  
17 grams at the Food and Drug Administration with  
18 respect to the expanded access programs established  
19 under this section.

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

24           “(c) DEVELOPMENT OF SURROGATE ENDPOINTS  
25 AND BIOMARKERS.—

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

2 “(A) establish a program or expand upon  
3 an existing program to encourage the develop-  
4 ment of surrogate endpoints and biomarkers  
5 that are reasonably likely to predict clinical  
6 benefit for serious or life-threatening conditions  
7 for which there exist significant unmet patient  
8 needs;

9 “(B) request the Institute of Medicine to  
10 undertake a study to identify validated surro-  
11 gate endpoints and biomarkers, and recommend  
12 research to validate surrogate endpoints and  
13 biomarkers, that may support approvals for  
14 products intended for the treatment of serious  
15 or life-threatening conditions or diseases; and

16 “(C) make widely available to the public a  
17 list of drugs, biological products, and devices  
18 that are being investigated for serious or life-  
19 threatening conditions or diseases and that  
20 have not yet received approval under section  
21 561(d) or 561A for marketing.

22 “(2) STUDY CONTENT.—The study under para-  
23 graph (1)(B) shall include endpoints and biomarkers  
24 that address the unmet medical needs of subpopula-  
25 tions of patients and that facilitate the development

1 of individualized treatment approaches for patients  
2 with serious or life-threatening conditions or dis-  
3 eases.”.

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

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

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

23 (c) FUNDING.—

24 (1) IN GENERAL.—The Secretary shall provide  
25 for the transfer from the Federal Hospital Insurance

1 Trust Fund under section 1817 of the Social Secu-  
2 rity Act (42 U.S.C. 1395i) and the Federal Supple-  
3 mentary Medical Insurance Trust Fund under sec-  
4 tion 1841 of such Act (42 U.S.C. 1395t), in such  
5 proportion as the Secretary determines to be appro-  
6 priate, of such sums as are necessary for the costs  
7 of carrying out the demonstration project under this  
8 section.

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

16 (d) WAIVER AUTHORITY.—The Secretary may waive  
17 such requirements of title XVIII of the Social Security Act  
18 (42 U.S.C. 1395 et seq.) as may be necessary for the pur-  
19 pose of carrying out the demonstration project under this  
20 section.

21 (e) REPORT.—Not later than 90 days after the last  
22 day of the 5-year period of the demonstration project  
23 under this section, the Secretary shall submit to Congress  
24 a report describing the rates of utilization by Medicare  
25 beneficiaries of drugs, biological products, and devices ap-

1 proved for Compassionate Investigational Access and the  
 2 total cost of payments made under the Medicare program  
 3 resulting from the demonstration project. The report shall  
 4 describe recommendations for legislation or administrative  
 5 action as the Secretary deems appropriate.

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

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

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

14 (1) in paragraph (1), in the matter following  
 15 subparagraph (B), by striking “(as defined in sec-  
 16 tion 1927(k)(6))” and inserting “(as defined in  
 17 paragraph (4))”; and

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

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

22 “(A) IN GENERAL.—Subject to subpara-  
 23 graph (B), for purposes of paragraph (1), the  
 24 term ‘medically accepted indication’ has the  
 25 meaning given that term—

1           “(i) in the case of a covered part D  
2           drug used in an anticancer  
3           chemotherapeutic regimen, in section  
4           1861(t)(2)(B), except that in applying  
5           such section, ‘PDP sponsor or MA–PD  
6           sponsor’ shall be substituted for ‘carrier’  
7           each place it appears and the compendia  
8           identified in section 1927(g)(1)(B)(i)(III)  
9           shall be deemed to be included in the com-  
10          pendia described in section  
11          1861(t)(2)(B)(ii)(I); and

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

14          “(B) CONSIDERATION OF OTHER CRITERIA  
15          ON A CASE-BY-CASE BASIS.—Nothing in this  
16          subsection shall preclude a PDP sponsor offer-  
17          ing a prescription drug plan or an MA organi-  
18          zation offering an MA–PD plan from, after a  
19          request by an individual enrolled in the plan for  
20          a coverage determination under section 1860D–  
21          4(g)(1), providing coverage of a covered part D  
22          drug for the individual in the case where the  
23          sponsor or organization determines, based on  
24          guidance provided by the Secretary for deter-  
25          mining whether the use of a covered part D

1 drug is for a medically accepted indication and  
2 supportive clinical evidence in peer reviewed  
3 medical literature, that the use of the covered  
4 part D drug is for a medically accepted indica-  
5 tion.”.

6 (b) EFFECTIVE DATE.—The amendments made by  
7 this section shall apply to plan years beginning on or after  
8 January 1, 2010.

9 **SEC. 7. MODERNIZATION OF THE FOOD AND DRUG ADMIN-**  
10 **ISTRATION.**

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

14 **“SEC. 568. POLICIES RELATED TO STUDY EVALUATION IN-**  
15 **FORMATION.**

16 “(a) IN GENERAL.—

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

24 “(A) in evaluating clinical study designs  
25 and endpoints; and

1           “(B) in making decisions with respect to  
2           product applications for approval under section  
3           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 **SEC. 8. MEMBERSHIP OF ONCOLOGY DRUGS ADVISORY**  
22 **COMMITTEE AND THE CELLULAR, TISSUE,**  
23 **AND GENE THERAPY ADVISORY COMMITTEE.**

24           Notwithstanding any other provision of law, member-  
25 ship of the Oncology Drugs Advisory Committee, the Cel-

1 lular, Tissue, and Gene Therapy Advisory Committee of  
2 the Food and Drug Administration, and any other com-  
3 mittee created by such Administration to evaluate or ad-  
4 vise with respect to applications submitted under section  
5 561(d) or 561A of the Federal Food, Drug, and Cosmetic  
6 Act (as added by this Act), shall consist of no less than  
7 2 patient representatives who are voting members of the  
8 committee.

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