

110TH CONGRESS
1ST SESSION

H. R. 2589

To amend the Federal Food, Drug, and Cosmetic Act and the Public Health Service Act to reauthorize and amend the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act.

IN THE HOUSE OF REPRESENTATIVES

JUNE 6, 2007

Ms. ESHOO introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To amend the Federal Food, Drug, and Cosmetic Act and the Public Health Service Act to reauthorize and amend the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act.

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; TABLE OF CONTENTS.**

4 (a) SHORT TITLE.—This Act may be cited as the
5 “Improving Pharmaceuticals for Children Act of 2007”.

6 (b) TABLE OF CONTENTS.—The table of contents for
7 this Act is as follows:

Sec. 1. Short title; table of contents.

Sec. 2. Reauthorization of Best Pharmaceuticals for Children Act.

Sec. 3. Reauthorization of Pediatric Research Equity Act.

Sec. 4. Government Accountability Office report.

1 **SEC. 2. REAUTHORIZATION OF BEST PHARMACEUTICALS**
2 **FOR CHILDREN ACT.**

3 (a) PEDIATRIC STUDIES OF DRUGS.—

4 (1) IN GENERAL.—Section 505A of the Federal
5 Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is
6 amended to read as follows:

7 **“SEC. 505A. PEDIATRIC STUDIES OF DRUGS.**

8 “(a) DEFINITIONS.—As used in this section, the term
9 ‘pediatric studies’ or ‘studies’ means at least one clinical
10 investigation (that, at the Secretary’s discretion, may in-
11 clude pharmacokinetic studies) in pediatric age groups (in-
12 cluding neonates in appropriate cases) in which a drug
13 is anticipated to be used, and at the discretion of the Sec-
14 retary, may include preclinical studies.

15 “(b) MARKET EXCLUSIVITY FOR NEW DRUGS.—

16 “(1) IN GENERAL.—Except as provided in para-
17 graph (2), if, prior to approval of an application that
18 is submitted under section 505(b)(1), the Secretary
19 determines that information relating to the use of a
20 new drug in the pediatric population may produce
21 health benefits in that population, the Secretary
22 makes a written request for pediatric studies (which
23 shall include a timeframe for completing such stud-
24 ies), the applicant agrees to the request, such stud-

1 ies are completed using appropriate formulations for
2 each age group for which the study is requested
3 within any such timeframe, and the reports thereof
4 are submitted and accepted in accordance with sub-
5 section (d)(3), and if the Secretary determines that
6 labeling changes are appropriate, such changes are
7 made within the timeframe requested by the Sec-
8 retary—

9 “(A)(i)(I) the period referred to in sub-
10 section (c)(3)(E)(ii) of section 505, and in sub-
11 section (j)(5)(F)(ii) of such section, is deemed
12 to be five years and six months rather than five
13 years, and the references in subsections
14 (c)(3)(E)(ii) and (j)(5)(F)(ii) of such section to
15 four years, to forty-eight months, and to seven
16 and one-half years are deemed to be four and
17 one-half years, fifty-four months, and eight
18 years, respectively; or

19 “(II) the period referred to in clauses (iii)
20 and (iv) of subsection (c)(3)(E) of such section,
21 and in clauses (iii) and (iv) of subsection
22 (j)(5)(F) of such section, is deemed to be three
23 years and six months rather than three years;
24 and

1 “(ii) if the drug is designated under sec-
2 tion 526 for a rare disease or condition, the pe-
3 riod referred to in section 527(a) is deemed to
4 be seven years and six months rather than
5 seven years; and

6 “(B)(i) if the drug is the subject of—

7 “(I) a listed patent for which a certifi-
8 cation has been submitted under sub-
9 section (b)(2)(A)(ii) or (j)(2)(A)(vii)(II) of
10 section 505 and for which pediatric studies
11 were submitted prior to the expiration of
12 the patent (including any patent exten-
13 sions); or

14 “(II) a listed patent for which a cer-
15 tification has been submitted under sub-
16 sections (b)(2)(A)(iii) or (j)(2)(A)(vii)(III)
17 of section 505,

18 the period during which an application may not
19 be approved under section 505(c)(3) or section
20 505(j)(5)(B) shall be extended by a period of
21 six months after the date the patent expires (in-
22 cluding any patent extensions); or

23 “(ii) if the drug is the subject of a listed
24 patent for which a certification has been sub-
25 mitted under subsection (b)(2)(A)(iv) or

1 (j)(2)(A)(vii)(IV) of section 505, and in the pat-
2 ent infringement litigation resulting from the
3 certification the court determines that the pat-
4 ent is valid and would be infringed, the period
5 during which an application may not be ap-
6 proved under section 505(c)(3) or section
7 505(j)(5)(B) shall be extended by a period of
8 six months after the date the patent expires (in-
9 cluding any patent extensions).

10 “(2) EXCEPTION.—The Secretary shall not ex-
11 tend the period referred to in paragraph (1)(A) or
12 (1)(B) if the determination is made later than one
13 year prior to the expiration of such period.

14 “(c) MARKET EXCLUSIVITY FOR ALREADY-MAR-
15 KETED DRUGS.—

16 “(1) IN GENERAL.—Except as provided in para-
17 graph (2), if the Secretary determines that informa-
18 tion relating to the use of an approved drug in the
19 pediatric population may produce health benefits in
20 that population and makes a written request to the
21 holder of an approved application under section
22 505(b)(1) for pediatric studies (which shall include
23 a timeframe for completing such studies), the holder
24 agrees to the request, such studies are completed
25 using appropriate formulations for each age group

1 for which the study is requested within any such
2 timeframe and the reports thereof are submitted and
3 accepted in accordance with subsection (d)(3), and if
4 the Secretary determines that labeling changes are
5 appropriate and such changes are made within the
6 timeframe requested by the Secretary—

7 “(A)(i)(I) the period referred to in sub-
8 section (c)(3)(E)(ii) of section 505, and in sub-
9 section (j)(5)(F)(ii) of such section, is deemed
10 to be five years and six months rather than five
11 years, and the references in subsections
12 (c)(3)(E)(ii) and (j)(5)(F)(ii) of such section to
13 four years, to forty-eight months, and to seven
14 and one-half years are deemed to be four and
15 one-half years, fifty-four months, and eight
16 years, respectively; or

17 “(II) the period referred to in clauses (iii)
18 and (iv) of subsection (c)(3)(D) of such section,
19 and in clauses (iii) and (iv) of subsection
20 (j)(5)(F) of such section, is deemed to be three
21 years and six months rather than three years;
22 and

23 “(ii) if the drug is designated under sec-
24 tion 526 for a rare disease or condition, the pe-
25 riod referred to in section 527(a) is deemed to

1 be seven years and six months rather than
2 seven years; and

3 “(B)(i) if the drug is the subject of—

4 “(I) a listed patent for which a certifi-
5 cation has been submitted under sub-
6 section (b)(2)(A)(ii) or (j)(2)(A)(vii)(II) of
7 section 505 and for which pediatric studies
8 were submitted prior to the expiration of
9 the patent (including any patent exten-
10 sions); or

11 “(II) a listed patent for which a cer-
12 tification has been submitted under sub-
13 section (b)(2)(A)(iii) or (j)(2)(A)(vii)(III)
14 of section 505,

15 the period during which an application may not
16 be approved under section 505(c)(3) or section
17 505(j)(5)(B)(ii) shall be extended by a period of
18 six months after the date the patent expires (in-
19 cluding any patent extensions); or

20 “(ii) if the drug is the subject of a listed
21 patent for which a certification has been sub-
22 mitted under subsection (b)(2)(A)(iv) or
23 (j)(2)(A)(vii)(IV) of section 505, and in the pat-
24 ent infringement litigation resulting from the
25 certification the court determines that the pat-

1 ent is valid and would be infringed, the period
2 during which an application may not be ap-
3 proved under section 505(c)(3) or section
4 505(j)(5)(B) shall be extended by a period of
5 six months after the date the patent expires (in-
6 cluding any patent extensions)

7 “(2) EXCEPTION.—The Secretary shall not ex-
8 tend the period referred to in paragraph (1)(A) or
9 (1)(B) if the determination is made later than one
10 year prior to the expiration of such period.

11 “(d) CONDUCT OF PEDIATRIC STUDIES.—

12 “(1) REQUEST FOR STUDIES.—

13 “(A) IN GENERAL.—The Secretary may,
14 after consultation with the sponsor of an appli-
15 cation for an investigational new drug under
16 section 505(I), the sponsor of an application for
17 a new drug under section 505(b)(1), or the
18 holder of an approved application for a drug
19 under section 505(b)(1) issue to the sponsor or
20 holder a written request for the conduct of pedi-
21 atric studies for such drug. In issuing such re-
22 quest, the Secretary shall take into account
23 adequate representation of children of ethnic
24 and racial minorities. Such request to conduct
25 pediatric studies shall be in writing and shall

1 include a timeframe for such studies and a re-
2 quest to the sponsor or holder to propose pedi-
3 atric labeling resulting from such studies.

4 “(B) SINGLE WRITTEN REQUEST.—A sin-
5 gle written request—

6 “(i) may related to more than one use
7 of a drug; and

8 “(ii) may include uses that are both
9 approved and unapproved.

10 “(2) WRITTEN REQUEST FOR PEDIATRIC STUD-
11 IES.—

12 “(A) REQUEST AND RESPONSE.—

13 “(i) IN GENERAL.—If the Secretary
14 makes a written request for pediatric stud-
15 ies (including neonates, as appropriate)
16 under subsection (b) or (c), the applicant
17 or holder, not later than 180 days after re-
18 ceiving the written request, shall respond
19 to the Secretary as to the intention of the
20 applicant or holder to act on the request
21 by—

22 “(I) indicating when the pediatric
23 studies will be initiated, if the appli-
24 cant or holder agrees to the request;
25 or

1 “(II) indicating that the appli-
2 cant or holder does not agree to the
3 request and stating the reasons for
4 declining the request.

5 “(ii) DISAGREE WITH REQUEST.—If,
6 on or after the date of the enactment of
7 the Improving Pharmaceuticals for Chil-
8 dren Act of 2007, the applicant or holder
9 does not agree to the request on the
10 grounds that it is not possible to develop
11 the appropriate pediatric formulation, the
12 applicant or holder shall submit to the Sec-
13 retary the reasons such pediatric formula-
14 tion cannot be developed.

15 “(B) ADVERSE EVENT REPORTS.—An ap-
16 plicant or holder that, on or after the date of
17 the enactment of the Improving Pharma-
18 ceuticals for Children Act of 2007, agrees to
19 the request for such studies shall provide the
20 Secretary, at the same time as the submission
21 of the reports of such studies, with all
22 postmarket adverse event reports regarding the
23 drug that is the subject of such studies and are
24 available prior to submission of such reports.

1 “(3) MEETING THE STUDIES REQUIREMENT.—
2 Not later than 180 days after the submission of the
3 reports of the studies, the Secretary shall accept or
4 reject such reports and so notify the sponsor or
5 holder. The Secretary’s only responsibility in accept-
6 ing or rejecting the reports shall be to determine,
7 within the 180-day period, whether the studies fairly
8 respond to the written request, have been conducted
9 in accordance with commonly accepted scientific
10 principles and protocols, and have been reported in
11 accordance with the requirements of the Secretary
12 for filing.

13 “(4) EFFECT OF SUBSECTION.—Nothing in this
14 subsection alters or amends section 301(j) of this
15 Act or section 552 of title 5 or section 1905 of title
16 18, United States Code.

17 “(e) NOTICE OF DETERMINATIONS ON STUDIES RE-
18 QUIREMENT.—

19 “(1) IN GENERAL.—The Secretary shall publish
20 a notice of any determination, made on or after the
21 date of the enactment of the Improving Pharma-
22 ceuticals for Children Act of 2007, that the require-
23 ments of subsection (d) have been met and that sub-
24 missions and approvals under subsection (b)(2) or
25 (j) of section 505 for a drug will be subject to the

1 provisions of this section. Such notice shall be pub-
2 lished not later than 30 days after the date of the
3 Secretary's determination regarding market exclu-
4 sivity and shall include a copy of the written request
5 made under subsection (b) or (c).

6 “(2) IDENTIFICATION OF CERTAIN DRUGS.—
7 The Secretary shall publish a notice identifying any
8 drug for which, on or after the date of the enact-
9 ment of the Improving Pharmaceuticals for Children
10 Act of 2007, a pediatric formulation was developed,
11 studied, and found to be safe and effective in the pe-
12 diatric population (or specified subpopulation) if the
13 pediatric formulation for such drug is not introduced
14 onto the market within one year after the date that
15 the Secretary publishes the notice described in para-
16 graph (1). Such notice identifying such drug shall be
17 published not later than 30 days after the date of
18 the expiration of such one year period.

19 “(f) INTERNAL REVIEW OF WRITTEN REQUESTS
20 AND PEDIATRIC STUDIES.—

21 “(1) INTERNAL REVIEW.—

22 “(A) IN GENERAL.—The Secretary shall
23 establish an internal review committee to review
24 all written requests issued on or after the date
25 of the enactment of the Improving Pharma-

1 ceuticals for Children Act of 2007, in accord-
2 ance with paragraph (2).

3 “(B) MEMBERS.—The committee estab-
4 lished under subparagraph (A) shall include in-
5 dividuals with expertise in pediatrics, biophar-
6 macology, statistics, drugs and drug formula-
7 tions, legal issues, pediatric ethics, the appro-
8 priate expertise, such as expertise in child and
9 adolescent psychiatry, pertaining to the pedi-
10 atric product under review, one or more experts
11 from the Office of Pediatric Therapeutics, and
12 other individuals designated by the Secretary.

13 “(2) REVIEW OF WRITTEN REQUESTS.—The
14 committee established under paragraph (1) shall re-
15 view all written requests issued pursuant to this sec-
16 tion prior to being issued.

17 “(3) TRACKING PEDIATRIC STUDIES AND LA-
18 BELING CHANGES.—The Secretary shall track and
19 make available to the public, in an easily accessible
20 manner, including through posting on the website of
21 the Food and Drug Administration—

22 “(A) the number of studies conducted
23 under this section and under section 409I of
24 the Public Health Service Act (42 U.S.C.
25 284m);

1 “(B) the specific drugs and biological prod-
2 ucts and their uses, including labeled and off-
3 labeled indications, studied under such sections;

4 “(C) the types of studies conducted under
5 such sections, including trial design, the num-
6 ber of pediatric patients studied, and the num-
7 ber of centers and countries involved;

8 “(D) the number of pediatric formulations
9 developed and the number of pediatric formula-
10 tions not developed and the reasons such for-
11 mulations were not developed;

12 “(E) the labeling changes made as a result
13 of studies conducted under such sections;

14 “(F) an annual summary of labeling
15 changes made as a result of studies conducted
16 under such sections for distribution pursuant to
17 subsection (k)(2); and

18 “(G) information regarding reports sub-
19 mitted on or after the date of the enactment of
20 the Improving Pharmaceuticals for Children
21 Act of 2007.

22 “(4) COMMITTEE.—The Committee established
23 under paragraph (1) is the committee established in
24 section 505B(f)(1).”

1 “(g) LIMITATIONS.—Notwithstanding subsection
2 (c)(2), a drug to which the six-month period under sub-
3 section (b) or (c) has already been applied—

4 “(1) may receive an additional six-month period
5 under subsection (c)(1)(A)(i)(II) for a supplemental
6 application if all other requirements under this sec-
7 tion are satisfied; and

8 “(2) may not receive any additional such period
9 under subsection (c)(1)(A)(ii).

10 “(h) RELATIONSHIP TO PEDIATRIC RESEARCH RE-
11 QUIREMENTS.—Notwithstanding any other provision of
12 law, if any pediatric study is required by a provision of
13 law (including a regulation) other than this section and
14 such study meets the completeness, timeliness, and other
15 requirements of this section, such study shall be deemed
16 to satisfy the requirement for market exclusivity pursuant
17 to this section.

18 “(i) LABELING CHANGES.—

19 “(1) PRIORITY STATUS FOR PEDIATRIC APPLI-
20 CATIONS AND SUPPLEMENTS.—Any application or
21 supplement to an application under section 505 pro-
22 posing a labeling change as a result of any pediatric
23 study conducted pursuant to this section—

24 “(A) shall be considered to be a priority
25 application or supplement; and

1 “(B) shall be subject to the performance
2 goals established by the Commissioner for pri-
3 ority drugs.

4 “(2) DISPUTE RESOLUTION.—

5 “(A) REQUEST FOR LABELING CHANGE
6 AND FAILURE TO AGREE.—If, on or after the
7 date of the enactment of the Improving Phar-
8 maceuticals for Children Act of 2007, the Com-
9 missioner determines that the sponsor and the
10 Commissioner have been unable to reach agree-
11 ment on appropriate changes to the labeling for
12 the drug that is the subject of the application,
13 not later than 180 days after the date of sub-
14 mission of the application—

15 “(i) the Commissioner shall request
16 that the sponsor of the application make
17 any labeling change that the Commissioner
18 determines to be appropriate; and

19 “(ii) if the sponsor of the application
20 does not agree within 30 days after the
21 Commissioner’s request to make a labeling
22 change requested by the Commissioner, the
23 Commissioner shall refer the matter to the
24 Pediatric Advisory Committee.

1 “(B) ACTION BY THE PEDIATRIC ADVISORY
2 COMMITTEE.—Not later than 90 days after re-
3 ceiving a referral under subparagraph (A)(ii),
4 the Pediatric Advisory Committee shall—

5 “(i) review the pediatric study reports;

6 and

7 “(ii) make a recommendation to the
8 Commissioner concerning appropriate la-
9 beling changes, if any.

10 “(C) CONSIDERATION OF RECOMMENDA-
11 TIONS.—The Commissioner shall consider the
12 recommendations of the Pediatric Advisory
13 Committee and, if appropriate, not later than
14 30 days after receiving the recommendation,
15 make a request to the sponsor of the applica-
16 tion to make any labeling change that the Com-
17 missioner determines to be appropriate.

18 “(D) MISBRANDING.—If the sponsor of the
19 application, within 30 days after receiving a re-
20 quest under subparagraph (c), does not agree to
21 make a labeling change requested by the Com-
22 missioner, the Commissioner may deem the
23 drug that is the subject of the application to be
24 misbranded.

1 “(E) NO EFFECT ON AUTHORITY.—Noth-
2 ing in this subsection limits the authority of the
3 United States to bring an enforcement action
4 under this Act when a drug lacks appropriate
5 pediatric labeling. Neither course of action (the
6 Pediatric Advisory Committee process or an en-
7 forcement action referred to in the preceding
8 sentence) shall preclude, delay, or serve as the
9 basis to stay the other course of action.

10 “(j) OTHER LABELING CHANGES.—If, on or after the
11 date of the enactment of the Improving Pharmaceuticals
12 for Children Act of 2007, the Secretary determines that
13 a pediatric study conducted under this section does or does
14 not demonstrate that the drug that is the subject of the
15 study is safe and effective in pediatric populations or sub-
16 populations, including whether such study results are in-
17 conclusive, the Secretary shall order the labeling of such
18 product to include information about the results of the
19 study and a statement of the Secretary’s determination.

20 “(k) DISSEMINATION OF PEDIATRIC INFORMA-
21 TION.—

22 “(1) IN GENERAL.—Not later than 180 days
23 after the date of submission of a report on a pedi-
24 atric study under this section, the Secretary shall
25 make available to the public the medical, statistical,

1 and clinical pharmacology reviews of pediatric stud-
2 ies conducted under subsection (b) or (c).

3 “(2) DISSEMINATION OF INFORMATION RE-
4 GARDING LABELING CHANGES.—Beginning on the
5 date of the enactment of the Improving Pharma-
6 ceuticals for Children Act of 2007, the Secretary
7 shall require that the sponsors of the studies that re-
8 sult in labeling changes that are reflected in the an-
9 nual summary developed pursuant to subsection
10 (f)(3)(F) distribute, at least annually (or more fre-
11 quently if the Secretary determines that it would be
12 beneficial to the public health), such information to
13 physicians and other health care providers.

14 “(3) EFFECT OF SUBSECTION.—Nothing in this
15 subsection alters or amends section 301(j) of this
16 Act or section 552 of title 5 or section 1905 of title
17 18, United States Code.

18 “(1) ADVERSE EVENT REPORTING.—

19 “(1) REPORTING IN YEAR ONE.—Beginning on
20 the date of the enactment of the Improving Pharma-
21 ceuticals for Children Act of 2007, during the one-
22 year period beginning on the date a labeling change
23 is made pursuant to subsection (I), the Secretary
24 shall ensure that all adverse event reports that have
25 been received for such drug (regardless of when such

1 report was received) are referred to the Office of Pe-
2 diatric Therapeutics established under section 6 of
3 the Best Pharmaceuticals for Children Act (Public
4 Law 107–109). In considering the reports, the Di-
5 rector of such Office shall provide for the review of
6 the reports by the Pediatric Advisory Committee, in-
7 cluding obtaining any recommendations of such
8 Committee regarding whether the Secretary should
9 take action under this Act in response to such re-
10 ports.

11 “(2) REPORTING IN SUBSEQUENT YEARS.—Fol-
12 lowing the one-year period described in paragraph
13 (1), the Secretary shall, as appropriate, refer to the
14 Office of Pediatric Therapeutics all pediatric adverse
15 event reports for a drug for which a pediatric study
16 was conducted under this section. In considering
17 such reports, the Director of such Office may pro-
18 vide for the review of such reports by the Pediatric
19 Advisory Committee, including obtaining any rec-
20 ommendation of such Committee regarding whether
21 the Secretary should take action in response to such
22 reports.

23 “(3) EFFECT.—The requirements of this sub-
24 section shall supplement, not supplant, other review
25 of such adverse event reports by the Secretary.

1 “(m) CLARIFICATION OF INTERACTION OF MARKET
2 EXCLUSIVITY UNDER THIS SECTION AND MARKET EX-
3 CLUSIVITY AWARDED TO AN APPLICANT FOR APPROVAL
4 OF A DRUG UNDER SECTION 505(j).—If a 180-day period
5 under section 505(j)(5)(B)(iv) overlaps with a 6-month ex-
6 clusivity period under this section, so that the applicant
7 for approval of a drug under section 505(j) entitled to the
8 180-day period under that section loses a portion of the
9 180-day period to which the applicant is entitled for the
10 drug, the 180-day period shall be extended from—

11 “(1) the date on which the 180-day period
12 would have expired by the number of days of the
13 overlap, if the 180-day period would, but for the ap-
14 plication of this subsection, expire after the 6-month
15 exclusivity period; or

16 “(2) the date on which the 6-month exclusivity
17 period expires, by the number of days of the overlap
18 if the 180-day period would, but for the application
19 of this subsection, expire during the six-month exclu-
20 sivity period.

21 “(n) REFERRAL IF PEDIATRIC STUDIES NOT COM-
22 PLETED.—

23 “(1) IN GENERAL.—Beginning on the date of
24 the enactment of the Improving Pharmaceuticals for
25 Children Act of 2007, if pediatric studies have not

1 been completed under subsection (d) and if the Sec-
2 retary, through the committee established under
3 subsection (f), determines that there is a continuing
4 need for information relating to the use of the drug
5 in the pediatric population (including neonates, as
6 appropriate), the Secretary shall—

7 “(A) for a drug for which listed patents
8 have not expired, make a determination regard-
9 ing whether an assessment shall be required to
10 be submitted under section 505B; or

11 “(B) for a drug that has no listed patents
12 or has 1 or more listed patents that have ex-
13 pired, determine whether there are funds avail-
14 able under section 736 to award a grant to con-
15 duct the requested studies pursuant to para-
16 graph (2).

17 “(2) FUNDING OF STUDIES.—If, pursuant to
18 paragraph (1), the Secretary determines that there
19 are funds available under section 736 to award a
20 grant to conduct the requested pediatric studies,
21 then the Secretary shall issue a proposal to award
22 a grant to conduct the requested studies. If the Sec-
23 retary determines that funds are not available under
24 section 736, the Secretary shall refer the drug for
25 inclusion on the list established under section 409I

1 of the Public Health Services Act or the conduct of
2 studies.

3 “(3) PUBLIC NOTICE.—The Secretary shall give
4 the public notice of—

5 “(A) a decision under paragraph (1)(A)
6 not to require an assessment under section
7 505B and the basis for such decision;

8 “(B) the name of any drug, its manufac-
9 turer, and the indications to be studied pursu-
10 ant to a grant made under paragraph (2); and

11 “(C) any decision under paragraph (2) to
12 include a drug on the list established under sec-
13 tion 409I of the Public Health Services Act.

14 “(4) EFFECT OF SUBSECTION.—Nothing in this
15 subsection alters or amends section 301(j) of this
16 Act or section 552 of title 5 or section 1905 of Title
17 18, United States Code

18 “(o) PROMPT APPROVAL OF DRUGS UNDER SECTION
19 505(j) WHEN PEDIATRIC INFORMATION IS ADDED TO LA-
20 BELING.—

21 “(1) GENERAL RULE.—A drug for which an ap-
22 plication has been submitted or approved under sec-
23 tion 505(j) shall not be considered ineligible for ap-
24 proval under that section or misbranded under sec-
25 tion 502 on the basis that the labeling of the drug

1 omits a pediatric indication or any other aspect of
2 labeling pertaining to pediatric use when the omitted
3 indication or other aspect is protected by patent or
4 by exclusivity under clause (iii) or (iv) of section
5 505(j)(5)(F).

6 “(2) LABELING.—Notwithstanding clauses (iii)
7 and (iv) of section 505(j)(5)(F), the Secretary may
8 require that the labeling of a drug approved under
9 section 505(j) that omits a pediatric indication or
10 other aspect of labeling as described in paragraph
11 (1) include—

12 “(A) a statement that, because of mar-
13 keting exclusivity for a manufacturer—

14 “(i) the drug is not labeled for pedi-
15 atric use; or

16 “(ii) in the case of a drug for which
17 there is an additional pediatric use not re-
18 ferred to in paragraph (1), the drug is not
19 labeled for the pediatric use under para-
20 graph (1); and

21 “(B) a statement of any appropriate pedi-
22 atric contraindications, warnings, or pre-
23 cautions that the Secretary considers necessary.

1 “(3) PRESERVATION OF PEDIATRIC EXCLU-
2 SIVITY AND OTHER PROVISIONS.—This subsection
3 does not affect—

4 “(A) the availability or scope of exclusivity
5 under this section;

6 “(B) the availability or scope of exclusivity
7 under section 505 for pediatric formulations;

8 “(C) the question of the eligibility for ap-
9 proval of any application under section 505(j)
10 that omits any other conditions of approval en-
11 titled to exclusivity under clause (iii) or (iv) of
12 section 505(j)(5)(F); or

13 “(D) except as expressly provided in para-
14 graphs (1) and (2), the operation of section
15 505.

16 “(p) INSTITUTE OF MEDICINE STUDY.—Not later
17 than 3 years after the date of the enactment of the Im-
18 proving Pharmaceuticals for Children Act of 2007, the
19 Secretary shall enter into a contract with the Institute of
20 Medicine to conduct a study and report to Congress re-
21 garding the written requests made and the studies con-
22 ducted pursuant to this section. The Institute of Medicine
23 may devise an appropriate mechanism to review a rep-
24 resentative sample of requests made and studies conducted

1 pursuant to this section in order to conduct such study.

2 Such study shall—

3 “(1) review such representative written requests
4 issued by the Secretary since 1997 under sub-
5 sections (b) and (c);

6 “(2) review and assess such representative pedi-
7 atric studies conducted under subsections (b) and (c)
8 since 1997 and labeling changes made as a result of
9 such studies;

10 “(3) review the use of extrapolation for pedi-
11 atric subpopulations, the use of alternative endpoints
12 for pediatric populations, neonatal assessment tools,
13 and ethical issues in pediatric clinical trials; and

14 “(4) make recommendations regarding appro-
15 priate incentives for encouraging pediatric studies of
16 biologics.

17 “(q) SUNSET.—A drug may not receive any 6-month
18 period under subsection (b) or (c) unless—

19 “(1) on or before October 1, 2012, the Sec-
20 retary makes a written request for pediatric studies
21 of the drug;

22 “(2) on or before October 1, 2012, an applica-
23 tion for the drug is accepted for filing under section
24 505(b); and

25 “(3) all requirements of this section are met.”.

1 (2) EFFECTIVE DATE.—The amendment made
2 by this subsection shall apply to written requests
3 under section 505A of the Federal Food, Drug, and
4 Cosmetic Act (21 U.S.C. 355a) made after the date
5 of the enactment of this Act.

6 (b) PROGRAM FOR PEDIATRIC STUDIES OF DRUGS.—
7 Section 409I of the Public Health Service Act (42 U.S.C.
8 284m) is amended to read as follows:

9 **“SEC. 409I. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS.**

10 “(a) LIST OF PRIORITY ISSUES IN PEDIATRIC
11 THERAPEUTICS.—

12 “(1) IN GENERAL.—Not later than one year
13 after the date of the enactment of the Improving
14 Pharmaceuticals for Children Act of 2007, the Sec-
15 retary, acting through the Director of the National
16 Institutes of Health and in consultation with the
17 Commissioner of Food and Drugs and experts in pe-
18 diatric research, shall develop and publish a priority
19 list of needs in pediatric therapeutics, including
20 drugs or indications that require study. The list
21 shall be revised every three years.

22 “(2) CONSIDERATION OF AVAILABLE INFORMA-
23 TION.—In developing and prioritizing the list under
24 paragraph (1), the Secretary shall consider—

1 “(A) therapeutic gaps in pediatrics that
2 may include developmental pharmacology,
3 pharmacogenetic determinants of drug re-
4 sponse, metabolism of drugs and biologics in
5 children, and pediatric clinical trials;

6 “(B) particular pediatric diseases, dis-
7 orders or conditions where more complete
8 knowledge and testing of therapeutics, including
9 drugs and biologics, may be beneficial in pedi-
10 atric populations; and

11 “(C) the adequacy of necessary infrastruc-
12 ture to conduct pediatric pharmacological re-
13 search, including research networks and trained
14 pediatric investigators.

15 “(b) PEDIATRIC STUDIES AND RESEARCH.—The
16 Secretary, acting through the National Institutes of
17 Health, shall award funds to entities that have the exper-
18 tise to conduct pediatric clinical trials or other research
19 (including qualified universities, hospitals, laboratories,
20 contract research organizations, practice groups, federally
21 funded programs such as pediatric pharmacology research
22 units, other public or private institutions, or individuals)
23 to enable the entities to conduct the drug studies or other
24 research on the issues described in subsection (a). The

1 Secretary may use contracts, grants or other appropriate
2 funding mechanisms to award funds under this subsection.

3 “(c) PROCESS FOR PROPOSED PEDIATRIC STUDY
4 REQUESTS AND LABELING CHANGES.—

5 “(1) SUBMISSION OF PROPOSED PEDIATRIC
6 STUDY REQUEST.—The Director of the National In-
7 stitutes of Health shall, as appropriate, submit pro-
8 posed pediatric study requests for consideration by
9 the Commissioner of the Food and Drugs for pedi-
10 atric studies of a specific pediatric indication identi-
11 fied under subsection (a). Such a proposed pediatric
12 study request shall be made in a manner equivalent
13 to a written request made under subsection (b) or
14 (c) of Section 505A of the Federal Food, Drug, and
15 Cosmetic Act (21 U.S.C. 355a), including with re-
16 spect to the information provided on the pediatric
17 studies to be conducted pursuant to the request. The
18 Director of the National Institutes of Health may
19 submit a proposed pediatric study request for a drug
20 for which—

21 “(A)(i) there is an approved application
22 under section 505(j) of the Federal Food,
23 Drug, and Cosmetic Act (21 U.S.C. 355(j)); or

1 “(ii) there is a submitted application that
2 could be approved under the criteria of such
3 section; and

4 “(B) there is no patent protection or mar-
5 ket exclusivity protection for at least one form
6 of the drug under the Federal Food, Drug, and
7 Cosmetic Act; and

8 “(C) additional studies are needed to as-
9 sess the safety and effectiveness of the use of
10 the drug in the pediatric population.

11 “(2) WRITTEN REQUEST TO HOLDERS OF AP-
12 PROVED APPLICATIONS FOR DRUGS LACKING EXCLU-
13 SIVITY.—The Commissioner of the Food and Drugs,
14 in consultation with the Director of the National In-
15 stitutes of Health, may issue a written request based
16 on the proposed pediatric study request for the indi-
17 cation or indications submitted pursuant to para-
18 graph (1) (which shall include a timeframe for nego-
19 tiations for an agreement) for pediatric studies con-
20 cerning a drug identified under subsection (a) to all
21 holders of an approved application for the drug
22 under section 505 of the Federal Food, Drug, and
23 Cosmetic Act (21 U.S.C. 355). Such a written re-
24 quest shall be made in a manner equivalent to the
25 manner in which a written request is made under

1 subsection (a) or (b) of section 505A of such Act
2 (21 U.S.C. 355a), including with respect to informa-
3 tion provided on the pediatric studies to be con-
4 ducted pursuant to the request and using appro-
5 priate formulations for each age group for which the
6 study is requested.

7 “(3) REQUESTS FOR PROPOSALS.—If the Com-
8 missioner of the Food and Drugs does not receive a
9 response to a written request issued under para-
10 graph (2) not later than 30 days after the date on
11 which a request was issued, the Secretary, acting
12 through the Director of the National Institutes of
13 Health and in consultation with the Commissioner of
14 the Food and Drugs, shall publish a request for pro-
15 posals to conduct the pediatric studies described in
16 the written request in accordance with subsection
17 (b).

18 “(4) DISQUALIFICATION.—A holder that re-
19 ceives a first right of refusal shall not be entitled to
20 respond to a request for proposals under paragraph
21 (3).

22 “(5) CONTRACTS, GRANTS, OR OTHER FUNDING
23 MECHANISMS.—A contract, grant or other funding
24 may be awarded under this section only if a proposal
25 is submitted to the Secretary in such form and man-

1 ner, and containing such agreements, assurances,
2 and information as the Secretary determines to be
3 necessary to carry out this section.

4 “(6) REPORTING OF STUDIES.—

5 “(A) IN GENERAL.—On completion of a
6 pediatric study in accordance with an award
7 under this section, a report concerning the
8 study shall be submitted to the Director of the
9 National Institutes of Health and the Commis-
10 sioner of Food and Drugs. The report shall in-
11 clude all data generated in connection with the
12 study, including a written request if issued.

13 “(B) AVAILABILITY OF REPORTS.—Each
14 report submitted under subparagraph (A) shall
15 be considered to be in the public domain (sub-
16 ject to section 505A(d)(4)(D) of the Federal
17 Food, Drug, and Cosmetic Act (21 U.S.C.
18 355a(d)(4)(D)) and shall be assigned a docket
19 number by the Commissioner of Food and
20 Drugs. An interested person may submit writ-
21 ten comments concerning such pediatric studies
22 to the Commissioner of Food and Drugs, and
23 the written comments shall become part of the
24 docket file with respect to each of the drugs.

1 “(C) ACTION BY COMMISSIONER.—The
2 Commissioner of Food and Drugs shall take ap-
3 propriate action in response to the reports sub-
4 mitted under subparagraph (A) in accordance
5 with paragraph (7).

6 “(7) REQUESTS FOR LABELING CHANGE.—Dur-
7 ing the 180-day period after the date on which a re-
8 port is submitted under paragraph (6)(A), the Com-
9 missioner of Food and Drugs shall—

10 “(A) review the report and such other data
11 as are available concerning the safe and effec-
12 tive use in the pediatric population of the drug
13 studied;

14 “(B) negotiate with the holders of ap-
15 proved applications for the drug studied for any
16 labeling changes that the Commissioner of Food
17 and Drugs determines to be appropriate and re-
18 quests the holders to make; and

19 “(C)(i) place in the public docket file a
20 copy of the report and of any requested labeling
21 changes; and

22 “(ii) publish in the Federal Register and
23 through a posting on the website of the Food
24 and Drug Administration a summary of the re-

1 port and a copy of any requested labeling
2 changes.

3 “(8) DISPUTE RESOLUTION.—

4 “(A) REFERRAL TO PEDIATRIC ADVISORY
5 COMMITTEE.—If, not later than the end of the
6 180-day period specified in paragraph (7), the
7 holder of an approved application for the drug
8 involved does not agree to any labeling change
9 requested by the Commissioner of Food and
10 Drugs under that paragraph, the Commissioner
11 of Food and Drugs shall refer the request to
12 the Pediatric Advisory Committee.

13 “(B) ACTION BY THE PEDIATRIC ADVISORY
14 COMMITTEE.—Not later than 90 days after re-
15 ceiving a referral under subparagraph (A), the
16 Pediatric Advisory Committee shall—

17 “(i) review the available information
18 on the safe and effective use of the drug
19 in the pediatric population, including study
20 reports submitted under this section; and

21 “(ii) make a recommendation to the
22 Commissioner of Food and Drugs as to ap-
23 propriate labeling changes, if any.

24 “(9) FDA DETERMINATION.—Not later than 30
25 days after receiving a recommendation from the Pe-

1 pediatric Advisory Committee under paragraph
2 (8)(B)(ii) with respect to a drug, the Commissioner
3 of Food and Drugs shall consider the recommenda-
4 tion and, if appropriate, make a request to the hold-
5 ers of approved applications for the drug to make
6 any labeling change that the Commissioner of Food
7 and Drugs determines to be appropriate.

8 “(10) FAILURE TO AGREE.—If a holder of an
9 approved application for a drug, within 30 days
10 after receiving a request to make a labeling change
11 under paragraph (9), does not agree to make a re-
12 quested labeling change, the Commissioner of Food
13 and Drugs may deem the drug to be misbranded
14 under the Federal Food, Drug, and Cosmetic Act
15 (21 U.S.C. 301 et seq.).

16 “(11) NO EFFECT ON AUTHORITY.—Nothing in
17 this subsection limits the authority of the United
18 States to bring an enforcement action under the
19 Federal Food, Drug, and Cosmetic Act when a drug
20 lacks appropriate pediatric labeling. Neither course
21 of action (the Pediatric Advisory Committee process
22 or an enforcement action referred to in the pre-
23 ceding sentence) shall preclude, delay, or serve as
24 the basis to stay the other course of action.

1 “(d) DISSEMINATION OF PEDIATRIC INFORMA-
2 TION.—Not later than one year after the date of the enact-
3 ment of the Improving Pharmaceuticals for Children Act
4 of 2007, the Secretary, acting through the Director of the
5 National Institutes of Health, shall study the feasibility
6 of establishing a compilation of information on pediatric
7 drug use and report the findings to Congress.

8 “(e) AUTHORIZATION OF APPROPRIATIONS.—

9 “(1) IN GENERAL.—There are authorized to be
10 appropriated to carry out this section—

11 “(A) \$200,000,000 for fiscal year 2008;

12 and

13 “(B) such sums as are necessary for each
14 of the four succeeding fiscal years.

15 “(2) AVAILABILITY.—Any amount appropriated
16 under paragraph (1) shall remain available to carry
17 out this section until expended.”.

18 “(c) FEES RELATING TO DRUGS.—Section 735(6) of
19 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
20 379(6)) is amended by adding at the end the following
21 new subparagraph:

22 “(G) Activities relating to the support of
23 studies of drugs on pediatric populations under
24 section 505A(n)(1).”.

25 “(d) TRAINING OF PEDIATRIC PHARMACOLOGISTS.—

1 (1) INVESTMENT IN TOMORROW'S PEDIATRIC
2 RESEARCHERS.—Section 452G(2) of the Public
3 Health Service Act (42 U.S.C. 285g–10(2)) is
4 amended by inserting before the period at the end
5 the following: “, including pediatric pharmacological
6 research”.

7 (2) PEDIATRIC RESEARCH LOAN REPAYMENT
8 PROGRAM.—Section 487F(a)(1) of the Public Health
9 Service Act (42 U.S.C. 288–6(a)(1)) is amended by
10 inserting “including pediatric pharmacological re-
11 search,” after “pediatric research,”.

12 (e) FOUNDATION FOR THE NATIONAL INSTITUTES
13 OF HEALTH.—Section 499(c)(1)(c) of the Public Health
14 Service Act (42 U.S.C. 290b(c)(1)(c)) is amended by
15 striking “and studies listed by the Secretary pursuant to
16 section 409I(a)(1)(A) of this Act and referred under sec-
17 tion 505A(d)(4)(c) of the Federal Food, Drug, and Cos-
18 metic Act (21 U.S.C. 355(a)(d)(4)(c))”.

19 (f) CONTINUATION OF OPERATION OF COM-
20 MITTEE.—Section 14 of the Best Pharmaceuticals for
21 Children Act (42 U.S.C. 284m note) is amended by adding
22 at the end the following new subsection:

23 “(d) CONTINUATION OF OPERATION OF COM-
24 MITTEE.—Notwithstanding section 14 of the Federal Ad-
25 visory Committee Act (5 U.S.C. App.), the advisory com-

1 mittee shall continue to operate during the five-year period
2 beginning on the date of the enactment of the Improving
3 Pharmaceuticals for Children Act of 2007.”.

4 (g) PEDIATRIC SUBCOMMITTEE OF THE ONCOLOGIC
5 DRUGS ADVISORY COMMITTEE.—Section 15 of the Best
6 Pharmaceuticals for Children Act (42 U.S.C. 284m note)
7 is amended—

8 (1) in subsection (a)—

9 (A) in paragraph (1)—

10 (i) in subparagraph (B), by striking
11 “and” after the semicolon;

12 (ii) in subparagraph (c), by striking
13 the period at the end and inserting “;
14 and”; and

15 (iii) by adding at the end the fol-
16 lowing new subparagraph:

17 “(D) provide recommendations to the in-
18 ternal review committee created under section
19 505A(f) of the Federal Food, Drug, and Cos-
20 metic Act (21 U.S.C. 355a(f)) regarding the
21 implementation of amendments to sections
22 505A and 505B of the Federal Food, Drug,
23 and Cosmetic Act (21 U.S.C. 355a and 355c)
24 with respect to the treatment of pediatric can-
25 cers.”; and

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

3 “(3) CONTINUATION OF OPERATION OF SUB-
4 COMMITTEE.—Notwithstanding section 14 of the
5 Federal Advisory Committee Act (5 U.S.C. App.),
6 the Subcommittee shall continue to operate during
7 the five-year period beginning on the date of the en-
8 actment of the Improving Pharmaceuticals for Chil-
9 dren Act of 2007.”; and

10 (2) in subsection (d), by striking “2003” and
11 inserting “2009”.

12 (h) EFFECTIVE DATE AND LIMITATION FOR RULE
13 RELATING TO TOLL-FREE NUMBER FOR ADVERSE
14 EVENTS ON LABELING FOR HUMAN DRUG PRODUCTS.—

15 (1) IN GENERAL.—Notwithstanding subchapter
16 II of chapter 5, and chapter 7, of title 5, United
17 States Code (commonly known as the “Administra-
18 tive Procedure Act”) and any other provision of law,
19 the proposed rule issued by the Commissioner of
20 Food and Drugs entitled “Toll-Free Number for Re-
21 porting Adverse Events on Labeling for Human
22 Drug Products,” 69 Fed. Reg. 21778, (April 22,
23 2004) shall take effect on January 1, 2008, unless
24 such Commissioner issues the final rule before such
25 date.

1 (2) LIMITATION.—The proposed rule that takes
2 effect under subsection (a), or the final rule de-
3 scribed under subsection (a), shall, notwithstanding
4 section 17(a) of the Best Pharmaceuticals for Chil-
5 dren Act (21 U.S.C. 355b(a)), not apply to a drug—

6 (A) for which an application is approved
7 under section 505 of the Federal Food, Drug,
8 and Cosmetic Act (21 U.S.C. 355);

9 (B) that is not described under section
10 503(b)(1) of such Act (21 U.S.C. 353(b)(1));

11 and

12 (C) the packaging of which includes a toll-
13 free number through which consumers can re-
14 port complaints to the manufacturer or dis-
15 tributor of the drug.

16 **SEC. 3. REAUTHORIZATION OF PEDIATRIC RESEARCH EQ-**
17 **UITY ACT.**

18 Section 505B of the Federal Food, Drug, and Cos-
19 metic Act (21 U.S.C. 355c) is amended to read as follows:

20 **“SEC. 505B. RESEARCH INTO PEDIATRIC USES FOR DRUGS**
21 **AND BIOLOGICAL PRODUCTS.**

22 **“(a) NEW DRUGS AND BIOLOGICAL PRODUCTS.—**

23 **“(1) IN GENERAL.—**A person that submits an
24 application (or supplement to an application)—

1 “(A) under section 505 for a new active in-
2 ingredient, new indication, new dosage form, new
3 dosing regimen, or new route of administration;
4 or

5 “(B) under section 351 of the Public
6 Health Service Act (42 U.S.C. 262) for a new
7 active ingredient, new indication, new dosage
8 form, new dosing regimen, or new route of ad-
9 ministration; shall submit with the application
10 the assessments described in paragraph (2).

11 “(2) ASSESSMENTS.—

12 “(A) IN GENERAL.—The assessments re-
13 ferred to in paragraph (1) shall contain data,
14 gathered using appropriate formulations for
15 each age group for which the assessment is re-
16 quired, that are adequate—

17 “(i) to assess the safety and effective-
18 ness of the drug or the biological product
19 for the claimed indications in all relevant
20 pediatric subpopulations; and

21 “(ii) to support dosing and adminis-
22 tration for each pediatric subpopulation for
23 which the drug or the biological product is
24 safe and effective.

1 “(B) SIMILAR COURSE OF DISEASE OR
2 SIMILAR EFFECT OF DRUG OR BIOLOGICAL
3 PRODUCT.—

4 “(i) IN GENERAL.—If the course of
5 the disease and the effects of the drug are
6 sufficiently similar in adults and pediatric
7 patients, the Secretary may conclude that
8 pediatric effectiveness can be extrapolated
9 from adequate and well-controlled studies
10 in adults, usually supplemented with other
11 information obtained in pediatric patients,
12 such as pharmacokinetic studies.

13 “(ii) EXTRAPOLATION BETWEEN AGE
14 GROUPS.—A study may not be needed in
15 each pediatric age group if data from one
16 age group can be extrapolated to another
17 age group.

18 “(iii) INFORMATION ON EXTRAPO-
19 LATION.—A brief documentation of the sci-
20 entific data supporting the conclusion
21 under clauses (i) and (ii) shall be included
22 in the medical review that is collected as
23 part of the application under section 505
24 of this Act or section 351 of the Public
25 Health Service Act (42 U.S.C. 262).

1 “(3) DEFERRAL.—

2 “(A) IN GENERAL.—On the initiative of
3 the Secretary or at the request of the applicant,
4 the Secretary may defer submission of some or
5 all assessments required under paragraph (1)
6 until a specified date after approval of the drug
7 or issuance of the license for a biological prod-
8 uct if—

9 “(i) the Secretary finds that—

10 “(I) the drug or biological prod-
11 uct is ready for approval for use in
12 adults before pediatric studies are
13 complete;

14 “(II) pediatric studies should be
15 delayed until additional safety or ef-
16 fectiveness data have been collected;
17 or

18 “(III) there is another appro-
19 priate reason for deferral; and

20 “(ii) the applicant submits to the Sec-
21 retary—

22 “(I) certification of the grounds
23 for deferring the assessments;

24 “(II) a description of the planned
25 or ongoing studies;

1 “(III) evidence that the studies
2 are being conducted or will be con-
3 ducted with due diligence and at the
4 earliest possible time; and

5 “(IV) a timeline for the comple-
6 tion of such studies.

7 “(B) ANNUAL REVIEW.—

8 “(i) IN GENERAL.—On an annual
9 basis following the approval of a deferral
10 under subparagraph (A), the applicant
11 shall submit to the Secretary the following
12 information:

13 “(I) Information detailing the
14 progress made in conducting pediatric
15 studies.

16 “(II) If no progress has been
17 made in conducting such studies, evi-
18 dence and documentation that such
19 studies will be conducted with due
20 diligence and at the earliest possible
21 time.

22 “(ii) PUBLIC AVAILABILITY.—The in-
23 formation submitted through the annual
24 review under clause (I) shall promptly be
25 made available to the public in an easily

1 accessible manner, including through the
2 website of the Food and Drug Administra-
3 tion.

4 “(4) WAIVERS.—

5 “(A) FULL WAIVER.—On the initiative of
6 the Secretary or at the request of an applicant,
7 the Secretary shall grant a full waiver, as ap-
8 propriate, of the requirement to submit assess-
9 ments for a drug or biological product under
10 this subsection if the applicant certifies and the
11 Secretary finds that—

12 “(i) necessary studies are impossible
13 or highly impracticable (because, for exam-
14 ple, the number of patients is so small or
15 the patients are geographically dispersed);

16 “(ii) there is evidence strongly sug-
17 gesting that the drug or biological product
18 would be ineffective or unsafe in all pedi-
19 atric age groups; or

20 “(iii) The drug or biological product—

21 “(I) does not represent a mean-
22 ingful therapeutic benefit over existing
23 therapies for pediatric patients; and

1 “(II) is not likely to be used in a
2 substantial number of pediatric pa-
3 tients.

4 “(B) PARTIAL WAIVER.—On the initiative
5 of the Secretary or at the request of an appli-
6 cant, the Secretary shall grant a partial waiver,
7 as appropriate, of the requirement to submit as-
8 sessments for a drug or biological product
9 under this subsection with respect to a specific
10 pediatric age group if the applicant certifies
11 and the secretary finds that—

12 “(i) necessary studies are impossible
13 or highly impracticable (because, for exam-
14 ple, the number of patients in that age
15 group is so small or patients in that age
16 group are geographically dispersed);

17 “(ii) there is evidence strongly sug-
18 gesting that the drug or biological product
19 would be ineffective or unsafe in that age
20 group;

21 “(iii) the drug or biological product—

22 “(I) does not represent a mean-
23 ingful therapeutic benefit over existing
24 therapies for pediatric patients in that
25 age group; and

1 “(II) is not likely to be used by
2 a substantial number of pediatric pa-
3 tients in that age group; or

4 “(iv) the applicant can demonstrate
5 that reasonable attempts to produce a pe-
6 diatric formulation necessary for that age
7 group have failed.

8 “(C) PEDIATRIC FORMULATION NOT POS-
9 SIBLE.—If a waiver is granted on the ground
10 that it is not possible to develop a pediatric for-
11 mulation, the waiver shall cover only the pedi-
12 atric groups requiring that formulation. An ap-
13 plicant seeking either a full or partial waiver
14 shall submit to the Secretary documentation de-
15 tailing why a pediatric formulation cannot be
16 developed and, if the waiver is granted, the ap-
17 plicant’s submission shall promptly be made
18 available to the public in an easily accessible
19 manner, including through posting on the
20 website of the Food and Drug Administration.

21 “(D) LABELING REQUIREMENT.—If the
22 Secretary grants a full or partial waiver because
23 there is evidence that a drug or biological prod-
24 uct would be ineffective or unsafe in pediatric
25 populations, the information shall be included

1 in the labeling for the drug or biological prod-
2 uct.

3 “(b) MARKETED DRUGS AND BIOLOGICAL PROD-
4 UCTS.—

5 “(1) IN GENERAL.—After providing notice in
6 the form of a letter and an opportunity for written
7 response and a meeting, which may include an advi-
8 sory committee meeting, the Secretary may (by
9 order in the form of a letter) require the sponsor or
10 holder of an approved application for a drug under
11 section 505 or the holder of a license for a biological
12 product under section 351 of the Public Health
13 Service Act (42 U.S.C. 262) to submit by a specified
14 date the assessments described in subsection (a)(2)
15 and the written request, as appropriate, if the Sec-
16 retary finds that—

17 “(A)(i) the drug or biological product is
18 used for a substantial number of pediatric pa-
19 tients for the labeled indications; and

20 “(ii) adequate pediatric labeling could con-
21 fer a benefit on pediatric patients;

22 “(B) there is reason to believe that the
23 drug or biological product would represent a
24 meaningful therapeutic benefit over existing

1 therapies for pediatric patients for 1 or more of
2 the claimed indications; or

3 “(C) the absence of adequate pediatric la-
4 beling could pose a risk to pediatric patients.

5 “(2) WAIVERS.—

6 “(A) FULL WAIVER.—At the request of an
7 applicant, the Secretary shall grant a full waiv-
8 er, as appropriate, of the requirement to submit
9 assessments under this subsection if the appli-
10 cant certifies and the Secretary finds that—

11 “(i) necessary studies are impossible
12 or highly impracticable (because, for exam-
13 ple, the number of patients in that age
14 group is so small or patients in that age
15 group are geographically dispersed); or

16 “(ii) there is evidence strongly sug-
17 gesting that the drug or biological product
18 would be ineffective or unsafe in all pedi-
19 atric age groups.

20 “(B) PARTIAL WAIVER.—At the request of
21 an applicant, the Secretary shall grant a partial
22 waiver, as appropriate, of the requirement to
23 submit assessments under this subsection with
24 respect to a specific pediatric age group if the

1 applicant certifies and the Secretary finds
2 that—

3 “(i) necessary studies are impossible
4 or highly impracticable (because, for exam-
5 ple, the number of patients in that age
6 group is so small or patients in that age
7 group are geographically dispersed);

8 “(ii) there is evidence strongly sug-
9 gesting that the drug or biological product
10 would be ineffective or unsafe in that age
11 group;

12 “(iii)(I) the drug or biological prod-
13 uct—

14 “(aa) does not represent a mean-
15 ingful therapeutic benefit over existing
16 therapies for pediatric patients in that
17 age group; and

18 “(bb) is not likely to be used in
19 a substantial number of pediatric pa-
20 tients in that age group; and

21 “(II) the absence of adequate labeling
22 could not pose significant risks to pediatric
23 patients; or

24 “(iv) the applicant can demonstrate
25 that reasonable attempts to produce a pe-

1 diatric formulation necessary for that age
2 group have failed.

3 “(C) PEDIATRIC FORMULATION NOT POS-
4 SIBLE.—If a waiver is granted on the ground
5 that it is not possible to develop a pediatric for-
6 mulation, the waiver shall cover only the pedi-
7 atric groups requiring that formulation. An ap-
8 plicant seeking either a full or partial waiver
9 shall submit to the Secretary documentation de-
10 tailing why a pediatric formulation cannot be
11 developed and, if the waiver is granted, the ap-
12 plicant’s submission shall promptly be made
13 available to the public in an easily accessible
14 manner, including through posting on the
15 website of the Food and Drug Administration.

16 “(D) LABELING REQUIREMENT.—If the
17 Secretary grants a full or partial waiver because
18 there is evidence that a drug or biological prod-
19 uct would be ineffective or unsafe in pediatric
20 populations, the information shall be included
21 in the labeling for the drug or biological prod-
22 uct.

23 “(c) MEANINGFUL THERAPEUTIC BENEFIT.—For
24 the purposes of paragraph (4)(A)(iii)(I) and (4)(B)(iii)(I)
25 of subsection (a) and paragraphs (1)(B)(I) and

1 (2)(B)(iii)(I)(aa) of subsection (b), a drug or biological
2 product shall be considered to represent a meaningful
3 therapeutic benefit over existing therapies if the Secretary
4 determines that—

5 “(1) if approved, the drug or biological product
6 could represent an improvement in the treatment,
7 diagnosis, or prevention of a disease, compared with
8 marketed products adequately labeled for that use in
9 the relevant pediatric population; or

10 “(2) the drug or biological product is in a class
11 of products or for an indication for which there is
12 a need for additional options.

13 “(d) SUBMISSION OF ASSESSMENTS.—If a person
14 fails to submit an assessment described in subsection
15 (a)(2), or a request for approval of a pediatric formulation
16 described in subsection (a) or (b), in accordance with ap-
17 plicable provisions of subsections (a) and (b)—

18 “(1) the drug or biological product that is the
19 subject of the assessment or request may be consid-
20 ered misbranded solely because of that failure and
21 subject to relevant enforcement action (except that
22 the drug or biological product shall not be subject to
23 action under section 303); but

24 “(2) the failure to submit the assessment or re-
25 quest shall not be the basis for a proceeding—

1 “(A) to withdraw approval for a drug
2 under section 505(e); or

3 “(B) to revoke the license for a biological
4 product under section 351 of the Public Health
5 Service Act (42 U.S.C. 262).

6 “(e) MEETINGS.—Before and during the investiga-
7 tional process for a new drug or biological product, the
8 Secretary shall meet at appropriate times with the sponsor
9 of the new drug or biological product to discuss—

10 “(1) information that the sponsor submits on
11 plans and timelines for pediatric studies; or

12 “(2) any planned request by the sponsor for
13 waiver or deferral of pediatric studies.

14 “(f) REVIEW OF PEDIATRIC ASSESSMENTS, DEFER-
15 RALS, AND WAIVERS.—

16 “(1) REVIEW.—The Secretary shall create an
17 internal committee to review all pediatric assess-
18 ments issued under this section and all deferral and
19 waiver requests made pursuant to this section. Such
20 internal committee shall include individuals, each of
21 whom is an employee of the Food and Drug Admin-
22 istration, with expertise in pediatrics, biopharma-
23 cology, statistics, drugs and drug formulations, legal
24 issues, pediatric ethics, the appropriate expertise,
25 such as expertise in child and adolescent psychiatry,

1 pertaining to the pediatric product under review, one
2 or more experts from the Office of Pediatric Thera-
3 peutics, and other individuals designated by the Sec-
4 retary.

5 “(2) ACTION BY COMMITTEE.—The committee
6 established under paragraph (1) may perform a
7 function under this section using appropriate mem-
8 bers of the committee described in paragraph (1)
9 and need not convene all members of the committee
10 described in paragraph (1) in order to perform a
11 function under this section.

12 “(3) DOCUMENTATION OF COMMITTEE AC-
13 TION.—For each drug or biological product, the
14 committee established under this paragraph shall
15 document for each function described in paragraph
16 (4) which members of the committee participated in
17 such function.

18 “(4) REVIEW OF REQUESTS FOR PEDIATRIC AS-
19 SESSMENTS, DEFERRALS AND WAIVERS.—All re-
20 quests for a pediatric assessment issued pursuant to
21 this section and all requests for deferrals and waiv-
22 ers from the requirement to conduct a pediatric as-
23 sessment under this section shall be reviewed by the
24 committee established under paragraph (1).

1 “(5) TRACKING OF ASSESSMENTS AND LABEL-
2 ING CHANGES.—The Secretary shall track and make
3 available to the public in an easily accessible man-
4 ner, including through post on the website of the
5 Food and Drug Administration—

6 “(A) the number of assessments conducted
7 under this section;

8 “(B) the specific drugs and biological prod-
9 ucts and their uses assessed under this section;

10 “(C) the types of assessments conducted
11 under this section, including trial design, the
12 number of pediatric patients studied, and the
13 number of centers and countries involved;

14 “(D) the total number of deferrals re-
15 quested and granted under this section and, if
16 granted, the reasons for such deferrals, the
17 timeline for completion, and the number com-
18 pleted and pending by the specified date, as
19 outlined in subsection (a)(3);

20 “(E) the number of waivers requested and
21 granted under this section and, if granted, the
22 reasons for the waivers;

23 “(F) the number of pediatric formulations
24 developed and the number of pediatric formula-

1 tions not developed and the reasons any such
2 formulation were not developed;

3 “(G) the labeling changes made as a result
4 of assessments conducted under this section;

5 “(H) an annual summary of labeling
6 changes made as a result of assessments con-
7 ducted under this section for distribution pursu-
8 ant to subsection (h)(2); and

9 “(I) an annual summary of information
10 submitted pursuant to subsection (a)(3)(B).

11 “(6) COMMITTEE.—The committee established
12 under paragraph (1) is the committee established
13 under section 505A(f)(1).

14 “(g) LABELING CHANGES.—

15 “(1) PRIORITY STATUS FOR PEDIATRIC APPLI-
16 CATIONS.—Any supplement to an application under
17 section 505 and section 351 of the Public Health
18 Service Act proposing a labeling change as a result
19 of any pediatric assessments conducted pursuant to
20 this section—

21 “(A) shall be considered a priority applica-
22 tion or supplement; and

23 “(B) shall be subject to the performance
24 goals established by the Commissioner for pri-
25 ority drugs.

1 “(2) DISPUTE RESOLUTION.—

2 “(A) REQUEST FOR LABELING CHANGE
3 AND FAILURE TO AGREE.—If the Commissioner
4 determines that a sponsor and the Commis-
5 sioner have been unable to reach agreement on
6 appropriate changes to the labeling for the drug
7 that is the subject of the application or supple-
8 ment, not later than 180 days after the date of
9 the submission of the application or supple-
10 ment—

11 “(i) the Commissioner shall request
12 that the sponsor of the application make
13 any labeling change that the Commissioner
14 determines to be appropriate; and

15 “(ii) if the sponsor does not agree
16 within 30 days after the Commissioner’s
17 request to make a labeling change re-
18 quested by the Commissioner, the Commis-
19 sioner shall refer the matter to the Pedi-
20 atric Advisory Committee.

21 “(B) ACTION BY THE PEDIATRIC ADVISORY
22 COMMITTEE.—Not later than 90 days after re-
23 ceiving a referral under subparagraph (A)(ii),
24 the Pediatric Advisory Committee shall—

1 “(i) review the pediatric study reports;

2 and

3 “(ii) make a recommendation to the
4 Commissioner concerning appropriate la-
5 beling changes, if any.

6 “(C) CONSIDERATION OF RECOMMENDA-
7 TIONS.—The Commissioner shall consider the
8 recommendations of the Pediatric Advisory
9 Committee and, if appropriate, not later than
10 30 days after receiving the recommendation,
11 make a request to the sponsor of the applica-
12 tion to make any labeling changes that the
13 Commissioner determines to be appropriate.

14 “(D) MISBRANDING.—If the sponsor of the
15 application, within 30 days after receiving a re-
16 quest under subparagraph (c), does not agree to
17 make a labeling change requested by the Com-
18 missioner, the Commissioner may deem the
19 drug that is the subject of the application to be
20 misbranded.

21 “(E) NO EFFECT ON AUTHORITY.—Noth-
22 ing in this subsection limits the authority of the
23 United States to bring an enforcement action
24 under this Act when a drug lacks appropriate
25 pediatric labeling. Neither course of action (the

1 Pediatric Advisory Committee process or an en-
2 forcement action referred to in the preceding
3 sentence) shall preclude, delay, or serve as the
4 basis to stay the other course of action.

5 “(3) OTHER LABELING CHANGES.—If the Sec-
6 retary makes a determination that a pediatric as-
7 sessment conducted under this section does or does
8 not demonstrate that the drug that is the subject of
9 such assessment is safe and effective in pediatric
10 populations or subpopulations, including whether
11 such assessment results are inconclusive, the Sec-
12 retary shall order the label of such product to in-
13 clude information about the results of the assess-
14 ment and a statement of the Secretary’s determina-
15 tion.

16 “(h) DISSEMINATION OF PEDIATRIC INFORMA-
17 TION.—

18 “(1) IN GENERAL.—Not later than 180 days
19 after the date of submission of a pediatric assess-
20 ment under this section, the Secretary shall make
21 available to the public in an easily accessible manner
22 the medical, statistical, and clinical pharmacology re-
23 views of such pediatric assessments, and shall post
24 such assessments on the website of the Food and
25 Drug Administration.

1 “(2) DISSEMINATION OF INFORMATION RE-
2 GARDING LABELING CHANGES.—The Secretary shall
3 require that the sponsors of the assessments that re-
4 sult in labeling changes that are reflected in the an-
5 nual summary developed pursuant to subsection
6 (f)(5)(H) distribute such information to physicians
7 and other health care providers.

8 “(3) EFFECT OF SUBSECTION.—Nothing in this
9 subsection shall alter or amend Section 301(j) of
10 this Act or section 552 of title 5 or section 1905 of
11 title 18, United States Code.

12 “(i) ADVERSE EVENT REPORTING.—

13 “(1) REPORTING IN YEAR ONE.—During the
14 one-year period beginning on the date a labeling
15 change is made pursuant to subsection (g), the Sec-
16 retary shall ensure that all adverse event reports
17 that have been received for such drug (regardless of
18 when such report was received) are referred to the
19 Office of Pediatric Therapeutics. In considering the
20 report, the Director of such Office shall provide for
21 the review of the report by the Pediatric Advisory
22 Committee, including obtaining any recommenda-
23 tions of such committee regarding whether the Sec-
24 retary should take action under this Act in response
25 to such report.

1 “(2) REPORTING IN SUBSEQUENT YEARS.—Fol-
2 lowing the one-year period described in paragraph
3 (1), the Secretary shall, as appropriate, refer to the
4 Office of Pediatric Therapeutics all pediatric adverse
5 event reports for a drug for which a pediatric study
6 was conducted under this section. In considering the
7 report, the Director of such Office may provide for
8 the review of the report by the Pediatric Advisory
9 Committee, including obtaining any recommendation
10 of such Committee regarding whether the Secretary
11 should take action in response to such report.

12 “(3) EFFECT.—The requirements of this sub-
13 section shall supplement, not supplant, other review
14 of such adverse event reports by the Secretary.

15 “(j) SCOPE OF AUTHORITY.—Nothing in this section
16 provides to the Secretary any authority to require a pedi-
17 atric assessment of any drug or biological product, or any
18 assessment regarding other populations or uses of a drug
19 or biological product, other than the pediatric assessments
20 described in this section.

21 “(k) ORPHAN DRUGS.—Unless the Secretary re-
22 quires otherwise by regulation, this section does not apply
23 to any drug for an indication for which orphan designation
24 has been granted under section 526.

25 “(l) INSTITUTE OF MEDICINE STUDY.—

1 “(1) IN GENERAL.—Not later than three years
2 after the date of the enactment of the Improving
3 Pharmaceuticals for Children Act of 2007, the Sec-
4 retary shall contract with the Institute of Medicine
5 to conduct a study and report to Congress regarding
6 the pediatric studies conducted pursuant to this sec-
7 tion since 1997.

8 “(2) CONTENT OF STUDY.—The study under
9 paragraph (1) shall review and assess—

10 “(A) pediatric studies conducted pursuant
11 to this section since 1997 and labeling changes
12 made as a result of such studies; and

13 “(B) the use of extrapolation for pediatric
14 subpopulations, the use of alternative endpoints
15 for pediatric populations, neonatal assessment
16 tools, the number and type of pediatric adverse
17 events, and ethical issues in pediatric clinical
18 trials.

19 “(3) REPRESENTATIVE SAMPLE.—The Institute
20 of Medicine may devise an appropriate mechanism to
21 review a representative sample of studies conducted
22 pursuant to this section from each review division
23 within the Center for Drug Evaluation and Research
24 in order to make the requested assessment.”.

1 **SEC. 4. GOVERNMENT ACCOUNTABILITY OFFICE REPORT.**

2 Not later than September 1, 2011, the Comptroller
3 General of the United States, in consultation with the Sec-
4 retary of Health and Human Services, shall submit to
5 Congress a report that addresses the effectiveness of sec-
6 tions 505A and 505B of the Federal Food, Drug, and Cos-
7 metic Act (21 U.S.C. 355a) and section 409I of the Public
8 Health Service Act (42 U.S.C. 284m) in ensuring that
9 medicines used by children are tested and properly labeled.
10 Such report shall include—

11 (1) the number and importance of drugs and
12 biological products for children that are being tested
13 as a result of the amendments made by this Act and
14 the importance for children, health care providers,
15 parents, and others of labeling changes made as a
16 result of such testing;

17 (2) the number and importance of drugs and
18 biological products for children that are not being
19 tested for their use notwithstanding the provisions of
20 this Act and possible reasons for the lack of testing,
21 including whether the number of written requests
22 declined by sponsors or holders of drugs subject to
23 section 505A(g)(2) of the Federal Food, Drug, and
24 Cosmetic Act (21 U.S.C. 355a(g)(2)) has increased
25 or decreased as a result of the amendments made by
26 this Act;

1 (3) the number of drugs and biological products
2 for which testing is being done and labeling changes
3 required, including the date labeling changes are
4 made and which labeling changes required the use of
5 the dispute resolution process established pursuant
6 to the amendments made by this Act, together with
7 a description of the outcomes of such process, in-
8 cluding a description of the disputes and the rec-
9 ommendations of the Pediatric Advisory Committee;

10 (4) any recommendations for modifications to
11 the programs established under sections 505A and
12 505B of the Federal Food, Drug, and Cosmetic Act
13 (21 U.S.C. 355a) and section 409I of the Public
14 Health Service Act (42 U.S.C. 284m) that the Sec-
15 retary determines to be appropriate, including a de-
16 tailed rationale for each recommendation; and

17 (5)(A) the efforts made by the Secretary to in-
18 crease the number of studies conducted in the
19 neonate population; and

20 (B) the results of those efforts, including efforts
21 made to encourage the conduct of appropriate stud-
22 ies in neonates by companies with products that
23 have sufficient safety and other information to make
24 the conduct of the studies ethical and safe.

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