

Calendar No. 184107TH CONGRESS
1ST SESSION**S. 838****[Report No. 107-79]**

To amend the Federal Food, Drug, and Cosmetic Act to improve the safety and efficacy of pharmaceuticals for children.

IN THE SENATE OF THE UNITED STATES

MAY 7, 2001

Mr. DODD (for himself, Mr. DEWINE, Ms. MIKULSKI, Mr. FRIST, Mr. JEFFORDS, Mr. BOND, Ms. COLLINS, Ms. LANDRIEU, Mrs. FEINSTEIN, Mr. CORZINE, Mr. CARPER, Mrs. MURRAY, Mr. REED, Mr. BINGAMAN, and Mr. ROBERTS) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

OCTOBER 4, 2001

Reported by Mr. KENNEDY, with an amendment

[Strike out all after the enacting clause and insert the part printed in *italic*]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to improve the safety and efficacy of pharmaceuticals for children.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

1 **SECTION 1. SHORT TITLE.**

2 This Act may be cited as the “Best Pharmaceuticals
3 for Children Act”.

4 **SEC. 2. PEDIATRIC STUDIES OF ALREADY-MARKETED**
5 **DRUGS.**

6 Section 505A of the Federal Food, Drug, and Cos-
7 metic Act (21 U.S.C. 355a) is amended—

8 (1) by striking subsection (b); and

9 (2) in subsection (c)—

10 (A) by inserting after “the Secretary” the
11 following: “determines that information relating
12 to the use of an approved drug in the pediatric
13 population may produce health benefits in that
14 population and”; and

15 (B) by striking “concerning a drug identi-
16 fied in the list described in subsection (b)”.

17 **SEC. 3. RESEARCH FUND FOR THE STUDY OF OFF-PATENT**
18 **DRUGS.**

19 Part B of title IV of the Public Health Service Act
20 (42 U.S.C. 284 et seq.) is amended—

21 (1) by redesignating the second section 409C,
22 relating to clinical research (42 U.S.C. 284k), as
23 section 409G;

24 (2) by redesignating the second section 409D,
25 relating to enhancement awards (42 U.S.C. 284l), as
26 section 409H; and

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

2 **“SEC. 409I. PROGRAM FOR PEDIATRIC STUDIES OF OFF-**
 3 **PATENT DRUGS.**

4 “(a) LIST OF OFF-PATENT DRUGS FOR WHICH PE-
 5 DIATRIC STUDIES ARE NEEDED.—

6 “(1) IN GENERAL.—Not later than 1 year after
 7 the date of enactment of this section, the Secretary,
 8 acting through the Director of the National Insti-
 9 tutes of Health and in consultation with the Com-
 10 missioner of Food and Drugs and experts in pedi-
 11 atric research (including United States Pharma-
 12 ceopocia), shall develop, prioritize, and publish a list
 13 of approved drugs for which—

14 “(A) there is no patent or market exclu-
 15 sivity protection; and

16 “(B) additional studies are needed to as-
 17 sess the safety and effectiveness of the use of
 18 the drug in the pediatric population.

19 “(2) CONSIDERATION OF AVAILABLE INFORMA-
 20 TION.—In developing the list under paragraph (1),
 21 the Secretary shall consider, for each drug on the
 22 list—

23 “(A) the availability of information con-
 24 cerning the safe and effective use of the drug
 25 in the pediatric population;

1 “(B) whether additional information is
2 needed; and

3 “(C) whether new pediatric studies con-
4 cerning the drug may produce health benefits in
5 the pediatric population.

6 “(b) CONTRACTS FOR PEDIATRIC STUDIES.—The
7 Secretary shall award contracts to entities that have the
8 expertise to conduct pediatric clinical trials (including
9 qualified universities, hospitals, laboratories, contract re-
10 search organizations, federally funded programs such as
11 pediatric pharmacology research units, other public or pri-
12 vate institutions, or individuals) to enable the entities to
13 conduct pediatric studies concerning one or more drugs
14 identified in the list described in subsection (a).

15 “(c) PROCESS FOR CONTRACTS AND LABELING
16 CHANGES.—

17 “(1) WRITTEN REQUEST TO HOLDERS OF AP-
18 PROVED APPLICATIONS FOR OFF-PATENT DRUGS.—

19 “(A) IN GENERAL.—The Commissioner of
20 Food and Drugs, in consultation with the Di-
21 rector of National Institutes of Health, may
22 issue a written request for pediatric studies
23 concerning a drug identified in the list de-
24 scribed in subsection (a) to all holders of an ap-
25 proved application for the drug under section

1 505 of the Federal Food, Drug, and Cosmetic
2 Act. Such a request shall be made in accord-
3 ance with section 505A of the Federal Food,
4 Drug, and Cosmetic Act.

5 “(B) PUBLICATION OF REQUEST.—If the
6 Commissioner of Food and Drugs does not re-
7 ceive a response to a written request issued
8 under subparagraph (A) within 30 days of the
9 date on which a request was issued, the Sec-
10 retary, acting through the Director of National
11 Institutes of Health, shall publish a request for
12 contract proposals to conduct the pediatric
13 studies described in the written request.

14 “(2) CONTRACTS.—A contract under this sec-
15 tion may be awarded only if a proposal for the con-
16 tract is submitted to the Secretary in such form and
17 manner, and containing such agreements, assur-
18 ances, and information as the Secretary determines
19 to be necessary to carry out this section.

20 “(3) REPORTING OF STUDIES.—

21 “(A) Upon completion of a pediatric study
22 in accordance with a contract awarded under
23 this section, a report concerning the study shall
24 be submitted to the Director of National Insti-
25 tutes of Health and the Commissioner of Food

1 and Drugs. The report shall include all data
2 generated in connection with the study.

3 “(B) AVAILABILITY OF REPORTS.—Each
4 report submitted under subparagraph (A) shall
5 be considered to be in the public domain, and
6 shall be assigned a docket number by the Com-
7 missioner of Food and Drugs. An interested
8 person may submit written comments con-
9 cerning such pediatric studies to the Commis-
10 sioner of Food and Drugs, and the written com-
11 ments shall become part of the docket file with
12 respect to each the drug.

13 “(C) ACTION BY COMMISSIONER.—The
14 Commissioner of Food and Drugs shall take ap-
15 propriate action in response to the reports sub-
16 mitted under subparagraph (A) in accordance
17 with paragraph (4).

18 “(4) REQUEST FOR LABELING CHANGES.—Dur-
19 ing the 180-day period after the date on which a re-
20 port is submitted under paragraph (3)(A), the Com-
21 missioner of Food and Drugs shall—

22 “(A) review the report and such other data
23 as are available concerning the safe and effec-
24 tive use in the pediatric population of the drug
25 studied; and

1 “(B) negotiate with the holders of ap-
2 proved applications for the drug studied for any
3 labeling changes that the Commissioner of Food
4 and Drugs determines to be appropriate and re-
5 quests the holders to make; and

6 “(C)(i) place in the public docket file a
7 copy of the report and of any requested labeling
8 changes; and

9 “(ii) publish in the Federal Register a
10 summary of the report and a copy of any re-
11 quested labeling changes.

12 “(5) DISPUTE RESOLUTION.—If, not later than
13 the end of the 180-day period specified in paragraph
14 (4), the holder of an approved application for the
15 drug involved does not agree to any labeling change
16 requested by the Commissioner of Food and Drugs
17 under that paragraph—

18 “(A) the Commissioner of Food and Drugs
19 shall immediately refer the request to the Pedi-
20 atric Advisory Subcommittee of the Anti-Infec-
21 tive Drugs Advisory Committee; and

22 “(B) not later than 60 days after receiving
23 the referral, the Subcommittee shall—

24 “(i) review the available information
25 on the safe and effective use of the drug

1 in the pediatric population, including study
2 reports submitted under this section; and

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

6 “(6) FDA DETERMINATION.—Not later than 30
7 days after receiving a recommendation from the
8 Subcommittee under paragraph (5)B(ii) with respect
9 to a drug, the Commissioner of Food and Drugs
10 shall consider the recommendation and, if appro-
11 priate, make a request to the holders of approved
12 applications for the drug to make any labeling
13 change that the Commissioner of Food and Drugs
14 determines to be appropriate.

15 “(7) FAILURE TO AGREE.—If a holder of an
16 approved application for a drug, within 30 days
17 after receiving a request to make a labeling change
18 under paragraph (6), does not agree to make a re-
19 quested labeling change, the Commissioner may
20 deem the drug to be misbranded under the Federal
21 Food, Drug, and Cosmetic Act.

22 “(d) AUTHORIZATION OF APPROPRIATIONS.—

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

1 “(A) \$200,000,000 for fiscal year 2002;
2 and

3 “(B) such sums as are necessary for each
4 of the 5 succeeding fiscal years.

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

8 **SEC. 4. TIMELY LABELING CHANGES FOR DRUGS GRANTED**
9 **EXCLUSIVITY; DRUG FEES.**

10 (a) **ELIMINATION OF USER FEE WAIVER FOR PEDI-**
11 **ATRIC SUPPLEMENTS.**—Section 736(a)(1) of the Federal
12 Food, Drug, and Cosmetic Act (21 U.S.C. 379h(A)(1))
13 is amended—

14 (1) by striking subparagraph (F); and

15 (2) by redesignating subparagraph (G) as sub-
16 paragraph (F).

17 (b) **LABELING CHANGES.**—Section 505A of the Fed-
18 eral Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is
19 amended by adding at the end the following:

20 “(1) **LABELING SUPPLEMENTS.**—

21 “(1) **PRIORITY STATUS FOR PEDIATRIC SUP-**
22 **PLEMENTS.**—Any supplement to a human drug ap-
23 plication submitted under this section—

24 “(A) shall be considered to be a priority
25 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.—If the Commis-
5 sioner determines that a supplemental application
6 submitted under this section is approvable and that
7 the only open issue for final action on the supple-
8 ment is the reaching of an agreement between the
9 sponsor of the application and the Commissioner on
10 appropriate changes to the labeling for the drug that
11 is the subject of the application—

12 “(A) not later than 180 days after the date
13 of submission of the supplemental application—

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

18 “(ii) if the sponsor of the application
19 does not agree to make a labeling change
20 requested by the Commissioner by that
21 date, the Commissioner shall immediately
22 refer the matter to the Pediatric Advisory
23 Subcommittee of the Anti-Infective Drugs
24 Advisory Committee;

1 “(B) not later than 60 days after receiving
2 the referral, the Pediatric Advisory Sub-
3 committee of the Anti-Infective Drugs Advisory
4 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) the Commissioner shall consider the
11 recommendations of the Pediatric Advisory
12 Subcommittee of the Anti-Infective Drugs Advi-
13 sory Committee and, if appropriate, not later
14 than 30 days after receiving the recommenda-
15 tion, make a request to the sponsor of the ap-
16 plication to make any labeling change that the
17 Commissioner determines to be appropriate;
18 and

19 “(D) if the sponsor of the application,
20 within 30 days after receiving a request under
21 subparagraph (D), does not agree to make a la-
22 beling change requested by the Commissioner,
23 the Commissioner may deem the drug that is
24 the subject of the application to be mis-
25 branded.”.

1 **SEC. 5. OFFICE OF PEDIATRIC THERAPEUTICS.**

2 (a) **ESTABLISHMENT.**—The Secretary of Health and
3 Human Services shall establish an Office of Pediatric
4 Therapeutics within the Office of the Commissioner of
5 Food and Drugs.

6 (b) **DUTIES.**—The Office of Pediatric Therapeutics
7 shall be responsible for oversight and coordination of all
8 activities of the Food and Drug Administration that may
9 have any effect on a pediatric population or the practice
10 of pediatrics or may in any other way involve pediatric
11 issues.

12 (c) **STAFF.**—The staff of the Office of Pediatric
13 Therapeutics shall include—

14 (1) 1 or more individuals with expertise con-
15 cerning ethical issues presented by the conduct of
16 clinical research in the pediatric population; and

17 (2) 1 or more individuals with expertise in pedi-
18 atries who shall consult with all components of the
19 Food and Drug Administration concerning activities
20 described in subsection (b).

21 **SEC. 6. NEONATES.**

22 Section 505A(g) of the Federal Food, Drug, and Cos-
23 metic Act (21 U.S.C. 355a(g)) is amended by inserting
24 “(including neonates in appropriate cases)” after “pedi-
25 atrie age groups”.

1 **SEC. 7. SUNSET.**

2 Section 505A of the Federal Food, Drug, and Cos-
 3 metic Act (21 U.S.C. 355a) is amended by striking sub-
 4 section (j) and inserting the following:

5 “(j) SUNSET.—A drug may not receive any 6-month
 6 period under subsection (a) or (c) unless—

7 “(1) on or before October 1, 2007, the Sec-
 8 retary makes a written request for pediatric studies
 9 of the drug;

10 “(2) on or before October 1, 2007, an applica-
 11 tion for the drug is submitted under section
 12 505(b)(1); and

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

14 **SEC. 8. DISSEMINATION OF PEDIATRIC INFORMATION.**

15 Section 505A of the Federal Food, Drug, and Cos-
 16 metic Act (21 U.S.C. 355a) (as amended by section 4(b))
 17 is amended by adding at the end the following:

18 “(m) DISSEMINATION OF PEDIATRIC INFORMA-
 19 TION.—

20 “(1) IN GENERAL.—Not later than 180 days
 21 after the date of submission of a supplemental appli-
 22 cation under this section, the Commissioner shall
 23 make available to the public a summary of the med-
 24 ical and clinical pharmacology reviews of pediatric
 25 studies conducted for the supplement, including by
 26 publication in the Federal Register.

1 “~~(2)~~ EFFECT OF SUBSECTION.—Nothing in this
2 subsection alters or amends in any way section 552
3 of title 5 or section 1905 of title 18, United States
4 Code.”.

5 **SEC. 9. TECHNICAL AND CONFORMING AMENDMENTS.**

6 Section 505A of the Federal Food, Drug, and Cos-
7 metic Act (21 U.S.C. 355a) (as amended by sections 2(1),
8 4(b), 7, and 8) is amended—

9 (1) by redesignating subsections (a), (g), (h),
10 (i), (j), (l), and (m) as subsections (b), (a), (g), (h),
11 (l), (i), and (j), respectively;

12 (2) by moving the subsections so as to appear
13 in alphabetical order; and

14 (3) in paragraphs (1), (2), and (3) of sub-
15 section (d) and subsections (e), (g) (as redesignated
16 by paragraph (1)), and (l) (as redesignated by para-
17 graph (1)), by striking “subsection (a) or (e)” and
18 inserting “subsection (b) or (e)”.

19 **SECTION 1. SHORT TITLE.**

20 *This Act may be cited as the “Best Pharmaceuticals*
21 *for Children Act”.*

22 **SEC. 2. PEDIATRIC STUDIES OF ALREADY-MARKETED**
23 **DRUGS.**

24 Section 505A of the Federal Food, Drug, and Cosmetic
25 Act (21 U.S.C. 355a) is amended—

1 (1) *by striking subsection (b); and*

2 (2) *in subsection (c)—*

3 (A) *by inserting after “the Secretary” the*
 4 *following: “determines that information relating*
 5 *to the use of an approved drug in the pediatric*
 6 *population may produce health benefits in that*
 7 *population and”;* and

8 (B) *by striking “concerning a drug identi-*
 9 *fied in the list described in subsection (b)”.*

10 **SEC. 3. RESEARCH FUND FOR THE STUDY OF DRUGS LACK-**
 11 **ING EXCLUSIVITY.**

12 *Part B of title IV of the Public Health Service Act*
 13 *(42 U.S.C. 284 et seq.) is amended—*

14 (1) *by redesignating the second section 409C, re-*
 15 *lating to clinical research (42 U.S.C. 284k), as section*
 16 *409G;*

17 (2) *by redesignating the second section 409D, re-*
 18 *lating to enhancement awards (42 U.S.C. 284l), as*
 19 *section 409H; and*

20 (3) *by adding at the end the following:*

21 **“SEC. 409I. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS**
 22 **LACKING EXCLUSIVITY.**

23 “(a) *LIST OF DRUGS LACKING EXCLUSIVITY FOR*
 24 *WHICH PEDIATRIC STUDIES ARE NEEDED.—*

1 “(1) *IN GENERAL.*—Not later than 1 year after
2 the date of enactment of this section, the Secretary,
3 acting through the Director of the National Institutes
4 of Health and in consultation with the Commissioner
5 of Food and Drugs and experts in pediatric research,
6 shall develop, prioritize, and publish an annual list
7 of approved drugs for which—

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

11 “(ii) *there is a submitted application that*
12 *could be approved under the criteria of section*
13 *505(j) of the Federal Food, Drug, and Cosmetic*
14 *Act (21 U.S.C. 355(j)); or*

15 “(iii) *there is no patent protection or mar-*
16 *ket exclusivity protection under the Federal*
17 *Food, Drug, and Cosmetic Act (21 U.S.C. 301 et*
18 *seq.); and*

19 “(B) *additional studies are needed to assess*
20 *the safety and effectiveness of the use of the drug*
21 *in the pediatric population.*

22 “(2) *CONSIDERATION OF AVAILABLE INFORMA-*
23 *TION.*—In developing the list under paragraph (1),
24 the Secretary shall consider, for each drug on the
25 list—

1 “(A) *the availability of information con-*
2 *cerning the safe and effective use of the drug in*
3 *the pediatric population;*

4 “(B) *whether additional information is*
5 *needed;*

6 “(C) *whether new pediatric studies con-*
7 *cerning the drug may produce health benefits in*
8 *the pediatric population; and*

9 “(D) *whether reformulation of the drug is*
10 *necessary;*

11 “(b) *CONTRACTS FOR PEDIATRIC STUDIES.—The Sec-*
12 *retary shall award contracts to entities that have the exper-*
13 *tise to conduct pediatric clinical trials (including qualified*
14 *universities, hospitals, laboratories, contract research orga-*
15 *nizations, federally funded programs such as pediatric*
16 *pharmacology research units, other public or private insti-*
17 *tutions, or individuals) to enable the entities to conduct pe-*
18 *diatric studies concerning one or more drugs identified in*
19 *the list described in subsection (a).*

20 “(c) *PROCESS FOR CONTRACTS AND LABELING*
21 *CHANGES.—*

22 “(1) *WRITTEN REQUEST TO HOLDERS OF AP-*
23 *PROVED APPLICATIONS FOR DRUGS LACKING EXCLU-*
24 *SIVITY.—*

1 “(A) *IN GENERAL.*—*The Commissioner of*
2 *Food and Drugs, in consultation with the Direc-*
3 *tor of National Institutes of Health, may issue a*
4 *written request (which shall include a timeframe*
5 *for negotiations for an agreement) for pediatric*
6 *studies concerning a drug identified in the list*
7 *described in subsection (a) to all holders of an*
8 *approved application for the drug under section*
9 *505 of the Federal Food, Drug, and Cosmetic*
10 *Act. Such a request shall be made in accordance*
11 *with section 505A of the Federal Food, Drug,*
12 *and Cosmetic Act.*

13 “(B) *PUBLICATION OF REQUEST.*—*If the*
14 *Commissioner of Food and Drugs does not re-*
15 *ceive a response to a written request issued*
16 *under subparagraph (A) within 30 days of the*
17 *date on which a request was issued, the Sec-*
18 *retary, acting through the Director of National*
19 *Institutes of Health and in consultation with the*
20 *Commissioner of Food and Drugs, shall publish*
21 *a request for contract proposals to conduct the*
22 *pediatric studies described in the written request.*

23 “(C) *DISQUALIFICATION.*—*A holder that re-*
24 *ceives a first right of refusal shall not be entitled*

1 to respond to a request for contract proposals
2 under subparagraph (B).

3 “(D) *GUIDANCE.*—Not later than 270 days
4 after the date of enactment of this section, the
5 Commissioner of Food and Drugs shall promul-
6 gate guidance to establish the process for the sub-
7 mission of responses to written requests under
8 subparagraph (A).

9 “(2) *CONTRACTS.*—A contract under this section
10 may be awarded only if a proposal for the contract
11 is submitted to the Secretary in such form and man-
12 ner, and containing such agreements, assurances, and
13 information as the Secretary determines to be nec-
14 essary to carry out this section.

15 “(3) *REPORTING OF STUDIES.*—

16 “(A) Upon completion of a pediatric study
17 in accordance with a contract awarded under
18 this section, a report concerning the study shall
19 be submitted to the Director of National Insti-
20 tutes of Health and the Commissioner of Food
21 and Drugs. The report shall include all data gen-
22 erated in connection with the study.

23 “(B) *AVAILABILITY OF REPORTS.*—Each re-
24 port submitted under subparagraph (A) shall be
25 considered to be in the public domain, and shall

1 *be assigned a docket number by the Commis-*
2 *sioner of Food and Drugs. An interested person*
3 *may submit written comments concerning such*
4 *pediatric studies to the Commissioner of Food*
5 *and Drugs, and the written comments shall be-*
6 *come part of the docket file with respect to each*
7 *of the drugs.*

8 “(C) *ACTION BY COMMISSIONER.*—*The Com-*
9 *missioner of Food and Drugs shall take appro-*
10 *priate action in response to the reports submitted*
11 *under subparagraph (A) in accordance with*
12 *paragraph (4).*

13 “(4) *REQUEST FOR LABELING CHANGES.*—*Dur-*
14 *ing the 180-day period after the date on which a re-*
15 *port is submitted under paragraph (3)(A), the Com-*
16 *missioner of Food and Drugs shall—*

17 “(A) *review the report and such other data*
18 *as are available concerning the safe and effective*
19 *use in the pediatric population of the drug stud-*
20 *ied; and*

21 “(B) *negotiate with the holders of approved*
22 *applications for the drug studied for any label-*
23 *ing changes that the Commissioner of Food and*
24 *Drugs determines to be appropriate and requests*
25 *the holders to make; and*

1 “(C)(i) place in the public docket file a copy
2 of the report and of any requested labeling
3 changes; and

4 “(ii) publish in the Federal Register a sum-
5 mary of the report and a copy of any requested
6 labeling changes.

7 “(5) DISPUTE RESOLUTION.—If, not later than
8 the end of the 180-day period specified in paragraph
9 (4), the holder of an approved application for the
10 drug involved does not agree to any labeling change
11 requested by the Commissioner of Food and Drugs
12 under that paragraph—

13 “(A) the Commissioner of Food and Drugs
14 shall immediately refer the request to the Pedi-
15 atric Advisory Subcommittee of the Anti-Infec-
16 tive Drugs Advisory Committee; and

17 “(B) not later than 90 days after receiving
18 the referral, the Subcommittee shall—

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

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

1 “(6) *FDA DETERMINATION.*—Not later than 30
2 days after receiving a recommendation from the Sub-
3 committee under paragraph (5)(B)(ii) with respect to
4 a drug, the Commissioner of Food and Drugs shall
5 consider the recommendation and, if appropriate,
6 make a request to the holders of approved applica-
7 tions for the drug to make any labeling change that
8 the Commissioner of Food and Drugs determines to be
9 appropriate.

10 “(7) *FAILURE TO AGREE.*—If a holder of an ap-
11 proved application for a drug, within 30 days after
12 receiving a request to make a labeling change under
13 paragraph (6), does not agree to make a requested la-
14 beling change, the Commissioner may deem the drug
15 to be misbranded under the Federal Food, Drug, and
16 Cosmetic Act.

17 “(8) *RECOMMENDATION FOR FORMULATION*
18 *CHANGES.*—If a pediatric study completed under pub-
19 lic contract indicates that a formulation change is
20 necessary and the Secretary agrees, the Secretary
21 shall send a nonbinding letter of recommendation re-
22 garding that change to each holder of an approved
23 application.

24 “(d) *AUTHORIZATION OF APPROPRIATIONS.*—

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

3 “(A) \$200,000,000 for fiscal year 2002; and

4 “(B) *such sums as are necessary for each of*
5 *the 5 succeeding fiscal years.*

6 “(2) *AVAILABILITY.*—*Any amount appropriated*
7 *under paragraph (1) shall remain available to carry*
8 *out this section until expended.”.*

9 **SEC. 4. *TIMELY LABELING CHANGES FOR DRUGS GRANTED***

10 ***EXCLUSIVITY; DRUG FEES.***

11 (a) *ELIMINATION OF USER FEE WAIVER FOR PEDI-*
12 *ATRIC SUPPLEMENTS.*—*Section 736(a)(1) of the Federal*
13 *Food, Drug, and Cosmetic Act (21 U.S.C. 379h(a)(1)) is*
14 *amended—*

15 (1) *by striking subparagraph (F); and*

16 (2) *by redesignating subparagraph (G) as sub-*
17 *paragraph (F).*

18 (b) *LABELING CHANGES.*—

19 (1) *DEFINITION OF PRIORITY SUPPLEMENT.*—
20 *Section 201 of the Federal Food, Drug, and Cosmetic*
21 *Act (21 U.S.C. 321) is amended by adding at the end*
22 *the following:*

23 “(kk) *PRIORITY SUPPLEMENT.*—*The term ‘pri-*
24 *ority supplement’ means a drug application referred*

1 *to in section 101(4) of the Food and Drug Adminis-*
2 *tration Modernization Act of 1997 (111 Stat. 2298).”.*

3 (2) *TREATMENT AS PRIORITY SUPPLEMENTS.—*
4 *Section 505A of the Federal Food, Drug, and Cos-*
5 *metic Act (21 U.S.C. 355a) is amended by adding at*
6 *the end the following:*

7 *“(l) LABELING SUPPLEMENTS.—*

8 *“(1) PRIORITY STATUS FOR PEDIATRIC SUPPLE-*
9 *MENTS.—Any supplement to an application under*
10 *section 505 proposing a labeling change pursuant to*
11 *a report on a pediatric study under this section—*

12 *“(A) shall be considered to be a priority*
13 *supplement; and*

14 *“(B) shall be subject to the performance*
15 *goals established by the Commissioner for pri-*
16 *ority drugs.*

17 *“(2) DISPUTE RESOLUTION.—If the Commis-*
18 *sioner determines that an application with respect to*
19 *which a pediatric study is conducted under this sec-*
20 *tion is approvable and that the only open issue for*
21 *final action on the application is the reaching of an*
22 *agreement between the sponsor of the application and*
23 *the Commissioner on appropriate changes to the label-*
24 *ing for the drug that is the subject of the*
25 *application—*

1 “(A) not later than 180 days after the date
2 of submission of the application—

3 “(i) the Commissioner shall request
4 that the sponsor of the application make
5 any labeling change that the Commissioner
6 determines to be appropriate; and

7 “(ii) if the sponsor of the application
8 does not agree to make a labeling change re-
9 quested by the Commissioner by that date,
10 the Commissioner shall immediately refer
11 the matter to the Pediatric Advisory Sub-
12 committee of the Anti-Infective Drugs Advi-
13 sory Committee;

14 “(B) not later than 90 days after receiving
15 the referral, the Pediatric Advisory Sub-
16 committee of the Anti-Infective Drugs Advisory
17 Committee shall—

18 “(i) review the pediatric study reports;
19 and

20 “(ii) make a recommendation to the
21 Commissioner concerning appropriate label-
22 ing changes, if any;

23 “(C) the Commissioner shall consider the
24 recommendations of the Pediatric Advisory Sub-
25 committee of the Anti-Infective Drugs Advisory

1 Committee and, if appropriate, not later than 30
2 days after receiving the recommendation, make a
3 request to the sponsor of the application to make
4 any labeling change that the Commissioner de-
5 termines to be appropriate; and

6 “(D) if the sponsor of the application, with-
7 in 30 days after receiving a request under sub-
8 paragraph (C), does not agree to make a labeling
9 change requested by the Commissioner, the Com-
10 missioner may deem the drug that is the subject
11 of the application to be misbranded.”.

12 **SEC. 5. OFFICE OF PEDIATRIC THERAPEUTICS.**

13 (a) *ESTABLISHMENT.*—The Secretary of Health and
14 Human Services shall establish an Office of Pediatric
15 Therapeutics within the Office of the Commissioner of Food
16 and Drugs.

17 (b) *DUTIES.*—The Office of Pediatric Therapeutics
18 shall be responsible for oversight and coordination of all ac-
19 tivities of the Food and Drug Administration that may
20 have any effect on a pediatric population or the practice
21 of pediatrics or may in any other way involve pediatric
22 issues.

23 (c) *STAFF.*—The staff of the Office of Pediatric Thera-
24 peutics shall include—

1 (1) *employees of the Department of Health and*
 2 *Human Services who, as of the date of enactment of*
 3 *this Act, exercise responsibilities relating to pediatric*
 4 *therapeutics;*

5 (2) *1 or more additional individuals with exper-*
 6 *tise concerning ethical issues presented by the conduct*
 7 *of clinical research in the pediatric population; and*

8 (3) *1 or more additional individuals with exper-*
 9 *tise in pediatrics who shall consult and collaborate*
 10 *with all components of the Food and Drug Adminis-*
 11 *tration concerning activities described in subsection*
 12 *(b).*

13 **SEC. 6. NEONATES.**

14 *Section 505A(g) of the Federal Food, Drug, and Cos-*
 15 *metic Act (21 U.S.C. 355a(g)) is amended by inserting*
 16 *“(including neonates in appropriate cases)” after “pedi-*
 17 *atric age groups”.*

18 **SEC. 7. SUNSET.**

19 *Section 505A of the Federal Food, Drug, and Cosmetic*
 20 *Act (21 U.S.C. 355a) is amended by striking subsection (j)*
 21 *and inserting the following:*

22 “(j) *SUNSET.—A drug may not receive any 6-month*
 23 *period under subsection (a) or (c) unless—*

1 “(1) on or before October 1, 2007, the Secretary
2 makes a written request for pediatric studies of the
3 drug;

4 “(2) on or before October 1, 2007, an approvable
5 application for the drug is submitted under section
6 505(b)(1); and

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

8 **SEC. 8. DISSEMINATION OF PEDIATRIC INFORMATION.**

9 Section 505A of the Federal Food, Drug, and Cosmetic
10 Act (21 U.S.C 355a) (as amended by section 4(b)(2)) is
11 amended by adding at the end the following:

12 “(m) DISSEMINATION OF PEDIATRIC INFORMATION.—

13 “(1) IN GENERAL.—Not later than 180 days
14 after the date of submission of a report on a pediatric
15 study under this section, the Commissioner shall make
16 available to the public a summary of the medical and
17 clinical pharmacology reviews of pediatric studies
18 conducted for the supplement, including by publica-
19 tion in the Federal Register.

20 “(2) EFFECT OF SUBSECTION.—Nothing in this
21 subsection alters or amends in any way section 552
22 of title 5 or section 1905 of title 18, United States
23 Code.”.

1 **SEC. 9. CLARIFICATION OF INTERACTION OF MARKET EX-**
 2 **CLUSIVITY UNDER SECTION 505A OF THE**
 3 **FEDERAL FOOD, DRUG, AND COSMETIC ACT**
 4 **AND MARKET EXCLUSIVITY AWARDED TO AN**
 5 **APPLICANT FOR APPROVAL OF A DRUG**
 6 **UNDER SECTION 505(j) OF THAT ACT.**

7 *Section 505A of the Federal Food, Drug, and Cosmetic*
 8 *Act (21 U.S.C. 355a) (as amended by section 8) is amended*
 9 *by adding at the end the following:*

10 *“(n) CLARIFICATION OF INTERACTION OF MARKET EX-*
 11 *CLUSIVITY UNDER THIS SECTION AND MARKET EXCLU-*
 12 *SIVITY AWARDED TO AN APPLICANT FOR APPROVAL OF A*
 13 *DRUG UNDER SECTION 505(j).—*

14 *“(1) IN GENERAL.—If a 180-day period under*
 15 *section 505(j)(5)(B)(iv) overlaps with a 6-month ex-*
 16 *tension under this section, so that the applicant for*
 17 *approval of a drug under section 505(j) entitled to the*
 18 *180-day period under that section loses a portion of*
 19 *the 180-day period to which the applicant is entitled*
 20 *for the drug, the 180-day period shall be extended—*

21 *“(A) if the 180-day period would, but for*
 22 *this subsection, expire after the 6-month exten-*
 23 *sion, by the number of days of the overlap; or*

24 *“(B) if the 180-day period would, but for*
 25 *this subsection, expire during the 6-month exten-*
 26 *sion, by 6 months.*

1 “(2) *EFFECT OF SUBSECTION.*—Under no cir-
2 *cumstances shall application of this section result in*
3 *an applicant for approval of a drug under section*
4 *505(j) being enabled to commercially market the drug*
5 *to the exclusion of a subsequent applicant for ap-*
6 *proval of a drug under section 505(j) for more than*
7 *180 days.”.*

8 **SEC. 10. STUDY CONCERNING RESEARCH INVOLVING CHIL-**
9 **DREN.**

10 (a) *CONTRACT WITH INSTITUTE OF MEDICINE.*—The
11 *Secretary of Health and Human Services shall enter into*
12 *a contract with the Institute of Medicine for—*

13 (1) *the conduct, in accordance with subsection*
14 *(b), of a review of—*

15 (A) *Federal regulations in effect on the date*
16 *of the enactment of this Act relating to research*
17 *involving children;*

18 (B) *federally-prepared or supported reports*
19 *relating to research involving children; and*

20 (C) *federally-supported evidence-based re-*
21 *search involving children; and*

22 (2) *the submission to the appropriate committees*
23 *of Congress, by not later than 2 years after the date*
24 *of enactment of this Act, of a report concerning the*
25 *review conducted under paragraph (1) that includes*

1 *recommendations on best practices relating to re-*
2 *search involving children.*

3 *(b) AREAS OF REVIEW.—In conducting the review*
4 *under subsection (a)(1), the Institute of Medicine shall con-*
5 *sider the following:*

6 *(1) The written and oral process of obtaining*
7 *and defining “assent”, “permission” and “informed*
8 *consent” with respect to child clinical research par-*
9 *ticipants and the parents, guardians, and the indi-*
10 *viduals who may serve as the legally authorized rep-*
11 *resentatives of such children (as defined in subpart A*
12 *of part 46 of title 45, Code of Federal Regulations).*

13 *(2) The expectations and comprehension of child*
14 *research participants and the parents, guardians, or*
15 *legally authorized representatives of such children, for*
16 *the direct benefits and risks of the child’s research in-*
17 *volvement, particularly in terms of research versus*
18 *therapeutic treatment.*

19 *(3) The definition of “minimal risk” with re-*
20 *spect to a healthy child or a child with an illness.*

21 *(4) The appropriateness of the regulations appli-*
22 *cable to children of differing ages and maturity levels,*
23 *including regulations relating to legal status.*

24 *(5) Whether payment (financial or otherwise)*
25 *may be provided to a child or his or her parent,*

1 *guardian, or legally authorized representative for the*
 2 *participation of the child in research, and if so, the*
 3 *amount and type of payment that may be made.*

4 (6) *Compliance with the regulations referred to*
 5 *in subsection (a)(1)(A), the monitoring of such com-*
 6 *pliance (including the role of institutional review*
 7 *boards), and the enforcement actions taken for viola-*
 8 *tions of such regulations.*

9 (7) *The unique roles and responsibilities of insti-*
 10 *tutional review boards in reviewing research involv-*
 11 *ing children, including composition of membership on*
 12 *institutional review boards.*

13 (c) *REQUIREMENTS OF EXPERTISE.—The Institute of*
 14 *Medicine shall conduct the review under subsection (a)(1)*
 15 *and make recommendations under subsection (a)(2) in con-*
 16 *junction with experts in pediatric medicine, pediatric re-*
 17 *search, and the ethical conduct of research involving chil-*
 18 *dren.*

19 **SEC. 11. TECHNICAL AND CONFORMING AMENDMENTS.**

20 *Section 505A of the Federal Food, Drug, and Cosmetic*
 21 *Act (21 U.S.C. 355a) (as amended by sections 2(1), 4(b)(2),*
 22 *8, and 9) is amended—*

23 (1)(A) *by striking “(j)(4)(D)(ii)” each place it*
 24 *appears and inserting “(j)(5)(D)(ii)”;*

1 (B) by striking “(j)(4)(D)” each place it appears
2 and inserting “(j)(5)(D)”; and

3 (C) by striking “505(j)(4)(D)” each place it ap-
4 pears and inserting “505(j)(5)(D)”;

5 (2) by redesignating subsections (a), (g), (h), (i),
6 (j), (k), (l), (m), and (n) as subsections (b), (a), (g),
7 (h), (m), (l), (i), (j), and (k), respectively;

8 (3) by moving the subsections so as to appear in
9 alphabetical order;

10 (4) in paragraphs (1), (2), and (3) of subsection
11 (d), subsection (e), and subsection (m) (as redesign-
12 ated by paragraph (1)), by striking “subsection (a)
13 or (c)” and inserting “subsection (b) or (c)”; and

14 (5) in subsection (g) (as redesignated by para-
15 graph (1)), by striking “subsection (a) or (b)” and in-
16 serting “subsection (b) or (c)”.

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107TH CONGRESS
1ST SESSION

S. 838

[Report No. 107-79]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to improve the safety and efficacy of pharmaceuticals for children.

OCTOBER 4, 2001

Reported with an amendment