

107TH CONGRESS
2D SESSION

H. R. 5594

To amend the Federal Food, Drug, and Cosmetic Act to require labeling containing information applicable to pediatric patients.

IN THE HOUSE OF REPRESENTATIVES

OCTOBER 9, 2002

Mrs. MORELLA (for herself and Ms. PRYCE of Ohio) introduced the following bill; which was referred to the Committee on the Energy and Commerce

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to require labeling containing information applicable to pediatric patients.

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. PEDIATRIC LABELING OF DRUGS AND BIOLOGI-**
2 **CAL PRODUCTS.**

3 (a) IN GENERAL.—Subchapter A of chapter V of the
4 Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351
5 et seq.) is amended by inserting after section 505A the
6 following:

7 **“SEC. 505B. PEDIATRIC LABELING OF DRUGS AND BIOLOGI-**
8 **CAL PRODUCTS.**

9 “(a) NEW DRUGS AND BIOLOGICAL PRODUCTS.—

10 “(1) IN GENERAL.—A person that submits an
11 application (or supplement to an application)—

12 “(A) under section 505 for a new active in-
13 gredient, new indication, new dosage form, new
14 dosing regimen, or new route of administration;
15 or

16 “(B) under section 351 of the Public
17 Health Service Act (42 U.S.C. 262) for a bio-
18 logical product license;

19 shall submit with the application the assessments de-
20 scribed in paragraph (2).

21 “(2) ASSESSMENTS.—

22 “(A) IN GENERAL.—The assessments re-
23 ferred to in paragraph (1) shall contain data,
24 gathered using appropriate formulations, that
25 are adequate—

1 “(i) to assess the safety and effective-
2 ness of the drug, or the biological product
3 licensed under section 351 of the Public
4 Health Service Act (42 U.S.C. 262), for
5 the claimed indications in all relevant pedi-
6 atric subpopulations; and

7 “(ii) to support dosing and adminis-
8 tration for each pediatric subpopulation for
9 which the drug, or the biological product li-
10 censed under section 351 of the Public
11 Health Service Act (42 U.S.C. 262), is
12 safe and effective.

13 “(B) SIMILAR COURSE OF DISEASE OR
14 SIMILAR EFFECT OF DRUG OR BIOLOGICAL
15 PRODUCT.—If the course of the disease and the
16 effects of the drug are sufficiently similar in
17 adults and pediatric patients, the Secretary may
18 conclude that pediatric effectiveness can be ex-
19 trapolated from adequate and well-controlled
20 studies in adults, usually supplemented with
21 other information obtained in pediatric patients,
22 such as pharmacokinetic studies.

23 “(3) DEFERRAL.—On the initiative of the Sec-
24 retary or at the request of the applicant, the Sec-
25 retary may defer submission of some or all assess-

1 ments required under paragraph (1) until a specified
2 date after approval of the drug or issuance of the li-
3 cense for a biological product if—

4 “(A) the Secretary finds that—

5 “(i) the drug or biological product is
6 ready for approval for use in adults before
7 pediatric studies are complete; or

8 “(ii) pediatric studies should be de-
9 layed until additional safety or effective-
10 ness data have been collected; and

11 “(B) the applicant submits to the
12 Secretary—

13 “(i) a certified description of the
14 planned or ongoing studies; and

15 “(ii) evidence that the studies are
16 being conducted or will be conducted with
17 due diligence.

18 “(4) WAIVERS.—

19 “(A) FULL WAIVER.—At the request of an
20 applicant, the Secretary shall grant a full waiv-
21 er, as appropriate, of the requirement to submit
22 assessments under this subsection if—

23 “(i) necessary studies are impossible
24 or highly impractical;

1 “(ii) there is evidence strongly sug-
2 gesting that the drug or biological product
3 would be ineffective or unsafe in all pedi-
4 atric age groups; or

5 “(iii) the drug or biological product—

6 “(I) does not represent a mean-
7 ingful therapeutic benefit over existing
8 therapies for pediatric patients; and

9 “(II) is not likely to be used in a
10 substantial number of pediatric pa-
11 tients.

12 “(B) PARTIAL WAIVER.—At the request of
13 an applicant, the Secretary shall grant a partial
14 waiver, as appropriate, of the requirement to
15 submit assessments under this subsection with
16 respect to a specific pediatric age group if—

17 “(i) necessary studies are impossible
18 or highly impractical;

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

23 “(iii) the drug or biological product—

24 “(I) does not represent a mean-
25 ingful therapeutic benefit over existing

1 therapies for pediatric patients in that
2 age group; and

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

6 “(iv) the applicant demonstrates that
7 reasonable attempts to produce a pediatric
8 formulation necessary for that age group
9 have failed.

10 “(C) LABELING REQUIREMENT.—If the
11 Secretary grants a full or partial waiver because
12 there is evidence that a drug or biological prod-
13 uct would be ineffective or unsafe in pediatric
14 populations, the information shall be included
15 in the labeling for the drug or biological prod-
16 uct.

17 “(b) MARKETED DRUGS AND BIOLOGICAL PROD-
18 UCTS.—

19 “(1) IN GENERAL.—After providing notice and
20 an opportunity for written response and a meeting,
21 which may include an advisory committee meeting,
22 the Secretary may by order require the holder of an
23 approved application relating to a drug under sec-
24 tion 505 or the holder of a license for a biological
25 product under section 351 of the Public Health

1 Service Act (42 U.S.C. 262) to submit by a specified
2 date the assessments described in subsection (a) if
3 the Secretary finds that—

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

7 “(ii) the absence of adequate labeling could
8 pose significant risks to pediatric patients; or

9 “(B)(i) there is reason to believe that the
10 drug or biological product would represent a
11 meaningful therapeutic benefit over existing
12 therapies for pediatric patients for 1 or more of
13 the claimed indications; and

14 “(ii) the absence of adequate labeling could
15 pose significant risks to pediatric patients.

16 “(2) WAIVERS.—

17 “(A) FULL WAIVER.—At the request of an
18 applicant, the Secretary shall grant a full waiv-
19 er, as appropriate, of the requirement to submit
20 assessments under this subsection if—

21 “(i) necessary studies are impossible
22 or highly impractical; or

23 “(ii) there is evidence strongly sug-
24 gesting that the drug or biological product

1 would be ineffective or unsafe in all pedi-
2 atric age groups.

3 “(B) PARTIAL WAIVER.—At the request of
4 an applicant, the Secretary shall grant a partial
5 waiver, as appropriate, of the requirement to
6 submit assessments under this subsection with
7 respect to a specific pediatric age group if—

8 “(i) necessary studies are impossible
9 or highly impractical;

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

14 “(iii)(I) the drug or biological product
15 does not represent a meaningful thera-
16 peutic benefit over existing therapies for
17 pediatric patients in that age group;

18 “(II) the drug or biological product is
19 not likely to be used in a substantial num-
20 ber of pediatric patients in that age group;
21 and

22 “(III) the absence of adequate label-
23 ing could not pose significant risks to pedi-
24 atric patients; or

1 “(iv) the applicant demonstrates that
2 reasonable attempts to produce a pediatric
3 formulation necessary for that age group
4 have failed.

5 “(C) LABELING REQUIREMENT.—If the
6 Secretary grants a full or partial waiver because
7 there is evidence that a drug or biological prod-
8 uct would be ineffective or unsafe in pediatric
9 populations, the information shall be included
10 in the labeling for the drug or biological prod-
11 uct.

12 “(3) RELATIONSHIP TO OTHER PEDIATRIC PRO-
13 VISIONS.—

14 “(A) NO ASSESSMENT WITHOUT WRITTEN
15 REQUEST.—No assessment may be required
16 under paragraph (1) for a drug subject to an
17 approved application under section 505
18 unless—

19 “(i) the Secretary has issued a written
20 request for related pediatric studies under
21 section 505A(d) or section 409I of the
22 Public Health Service Act; and

23 “(ii)(I) if the request was made under
24 section 505A(d)—

1 “(aa) the recipient of the written
2 request does not agree to the request;
3 or

4 “(bb) the Secretary does not re-
5 ceive a response as specified under
6 section 505A(d)(4)(A); or

7 “(II) if the request was made under
8 section 409I of the Public Health Service
9 Act—

10 “(aa) the recipient of the written
11 request does not agree to the request;
12 or

13 “(bb) the Secretary does not re-
14 ceive a response as specified under
15 section 409I(c)(2) of that Act.

16 “(B) NO EFFECT ON OTHER AUTHOR-
17 ITY.—Nothing in this subsection shall be con-
18 strued to alter any requirement under section
19 505A(d)(4) or section 409I of the Public
20 Health Service Act. Subject to paragraph
21 (2)(A), nothing in this subsection, section
22 505A(d)(4), or section 409I or 499 of the Pub-
23 lic Health Service Act shall be construed to pre-
24 clude the Secretary from exercising the author-
25 ity of the Secretary under this subsection.

1 “(c) DELAY IN SUBMISSION OF ASSESSMENTS.—If a
2 person delays the submission of assessments relating to
3 a drug or biological product beyond a date specified in
4 subsection (a) or (b)—

5 “(1) the drug or biological product—

6 “(A) may be considered by the Secretary
7 to be misbranded and subject to action under
8 sections 302 and 304; and

9 “(B) shall not be subject to action under
10 section 303; and

11 “(2) the delay shall not be the basis for a pro-
12 ceeding to withdraw approval for a drug under sec-
13 tion 505(e) or revoke the license for a biological
14 product under section 351 of the Public Health
15 Service Act (42 U.S.C. 262).

16 “(d) MEETINGS.—The Secretary shall meet at appro-
17 priate times in the investigational new drug process with
18 the sponsor to discuss background information that the
19 sponsor shall submit on plans and timelines for pediatric
20 studies, or any planned request for waiver or deferral of
21 pediatric studies.”.

22 (b) CONFORMING AMENDMENTS.—

23 (1) Section 505(b)(1) of the Federal Food,
24 Drug, and Cosmetic Act (21 U.S.C. 355(b)(1)) is
25 amended in the second sentence—

1 (A) by striking “and (F)” and inserting
2 “(F)”; and

3 (B) by striking the period at the end and
4 inserting “, and (G) any assessments required
5 under section 505B.”.

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

8 (A) in the subsection heading, by striking
9 “REGULATIONS” and inserting “PEDIATRIC
10 STUDY REQUIREMENTS”; and

11 (B) by striking “pursuant to regulations
12 promulgated by the Secretary” and inserting
13 “by a provision of law (including a regulation)
14 other than this section”.

15 (3) Section 351(a)(2) of the Public Health
16 Service Act (42 U.S.C. 262(a)(2)) is amended—

17 (A) by redesignating subparagraph (B) as
18 subparagraph (C); and

19 (B) by inserting after subparagraph (A)
20 the following:

21 “(B) PEDIATRIC STUDIES.—A person that
22 submits an application for a license under this
23 paragraph shall submit to the Secretary as part
24 of the application any assessments required

1 under section 505B of the Federal Food, Drug,
2 and Cosmetic Act.”.

3 (c) FINAL RULE.—Except to the extent that the final
4 rule is inconsistent with the amendment made by sub-
5 section (a), the final rule promulgating regulations requir-
6 ing manufacturers to assess the safety and effectiveness
7 of new drugs and biological products in pediatric patients
8 (63 Fed. Reg. 66632 (December 2, 1998)), shall be con-
9 sidered to implement the amendment made by subsection
10 (a).

11 (d) NO EFFECT ON AUTHORITY.—Section 505B of
12 the Federal Food, Drug, and Cosmetic Act (as added by
13 subsection (a)) does not affect whatever existing authority
14 the Secretary of Health and Human Services has to re-
15 quire pediatric assessments regarding the safety and effi-
16 cacy of drugs and biological products in addition to the
17 assessments required under that section. The authority,
18 if any, of the Secretary of Health and Human Services
19 regarding specific populations other than the pediatric
20 population shall be exercised in accordance with the Fed-
21 eral Food, Drug, and Cosmetic Act (21 U.S.C. 301 et
22 seq.) as in effect on the day before the date of enactment
23 of this Act.

1 **SEC. 2. TECHNICAL CORRECTION.**

2 Section 505A of the Federal Food, Drug, and Cos-
3 metic Act (21 U.S.C. 355a) is amended in subparagraphs
4 (A) and (B) of subsection (b)(2) and subparagraphs (A)
5 and (B) of subsection (c)(2) by striking “505(j)(4)(B)”
6 and inserting “505(j)(5)(B)”.

○