107TH CONGRESS
1st Session
HOUSE OF REPRESENTATIVES
REPORT
107–277

BEST PHARMACEUTICALS FOR CHILDREN ACT

NOVEMBER 9, 2001.—Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

Mr. TAUZIN, from the Committee on Energy and Commerce, submitted the following

R E P O R T

together with

DISSENTING VIEWS

[To accompany H.R. 2887]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 2887) to amend the Federal Food, Drug, and Cosmetic Act to improve the safety and efficacy of pharmaceuticals for children, having considered the same, report favorably thereon with an amendment and recommend that the bill as amended do pass.

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99–006

AMENDMENT

The amendment is as follows:
Strike all after the enacting clause and insert the following:

SECTION 1. SHORT TITLE.

This Act may be cited as the "Best Pharmaceuticals for Children Act".

SEC. 2. PEDIATRIC STUDIES OF ALREADY-MARKETED DRUGS.

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

(1) by striking subsection (b); and

(2) by redesignating subsections (c) through (k) as subsections (b) through (j), respectively.

(b) CONFORMING AMENDMENTS.—Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is amended in subsection (b) (as redesignated by subsection (a)(2) of this section)—

(1) by inserting after "the Secretary" the following: "determines that information relating to the use of an approved drug in the pediatric population may produce health benefits in that population and"; and

(2) by striking "concerning a drug identified in the list described in subsection (b)".

SEC. 3. RESEARCH FUND FOR THE STUDY OF DRUGS LACKING EXCLUSIVITY.

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

(1) by redesignating the second section 409C (relating to clinical research) as section 409G;

(2) by redesignating the second section 409D (relating to enhancement awards) as section 409H; and

(3) by adding at the end the following:

"SEC. 409I. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS LACKING EXCLUSIVITY.

"(a) LIST OF DRUGS LACKING EXCLUSIVITY FOR WHICH PEDIATRIC STUDIES ARE NEEDED.—

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

"(A)(i) there is an approved application under section 505(j) of the Federal Food, Drug, and Cosmetic Act;

"(ii) there is a submitted application that could be approved under the criteria of section 505(j) of the Federal Food, Drug, and Cosmetic Act;

"(iii) there is no patent protection or market exclusivity protection under the Federal Food, Drug, and Cosmetic Act; or

"(iv) there is, under section 505A(c)(4)(C) of the Federal Food, Drug, and Cosmetic Act, a referral for inclusion on such list; and

"(B) additional studies are needed to assess the safety and effectiveness of the use of the drug in the pediatric population.

"(2) CONSIDERATION OF AVAILABLE INFORMATION.—In developing the list under paragraph (1), the Secretary shall consider, for each drug on the list—

"(A) the availability of information concerning the safe and effective use of the drug in the pediatric population;

"(B) whether additional information is needed;

"(C) whether new pediatric studies concerning the drug may produce health benefits in the pediatric population; and

"(D) whether reformulation of the drug is necessary;

"(b) CONTRACTS FOR PEDIATRIC STUDIES.—The Secretary shall award contracts to entities that have the expertise to conduct pediatric clinical trials (including qualified universities, hospitals, laboratories, contract research organizations, federally funded programs such as pediatric pharmacology research units, other public or private institutions, or individuals) to enable the entities to conduct pediatric studies concerning one or more drugs identified in the list described in subsection (a).

"(c) PROCESS FOR CONTRACTS AND LABELING CHANGES.—

"(1) WRITTEN REQUEST TO HOLDERS OF APPROVED APPLICATIONS FOR DRUGS LACKING EXCLUSIVITY.—

"(A) IN GENERAL.—The Commissioner of Food and Drugs, in consultation with the Director of National Institutes of Health, may issue a written request (which shall include a timeframe for negotiations for an agreement) for pediatric studies concerning a drug identified in the list described in subsection (a) to all holders of an approved application for the drug under section 505 of the Federal Food, Drug, and Cosmetic Act. Such a written request shall be made in a manner equivalent to the manner in which a written request is made under subsection (a) or (b) of section 505A of the Federal Food, Drug, and Cosmetic Act, including with respect to information provided on the pediatric studies to be conducted pursuant to the request.

"(B) PUBLICATION OF REQUEST.—If the Commissioner of Food and Drugs does not receive a response to a written request issued under subparagraph (A) within 30 days of the date on which a request was issued, the Secretary, acting through the Director of National Institutes of Health and in consultation with the Commissioner of Food and Drugs, shall publish a request for contract proposals to conduct the pediatric studies described in the written request.

"(C) DISQUALIFICATION.—A holder that receives a first right of refusal shall not be entitled to respond to a request for contract proposals under subparagraph (B).

"(D) GUIDANCE.—Not later than 270 days after the date of enactment of this section, the Commissioner of Food and Drugs shall promulgate guidance to establish the process for the submission of responses to written requests under subparagraph (A).

"(2) CONTRACTS.—A contract under this section may be awarded only if a proposal for the contract is submitted to the Secretary in such form and manner, and containing such agreements, assurances, and information as the Secretary determines to be necessary to carry out this section.

"(3) REPORTING OF STUDIES.—

"(A) Upon completion of a pediatric study in accordance with a contract awarded under this section, a report concerning the study shall be submitted to the Director of National Institutes of Health and the Commissioner of Food and Drugs. The report shall include all data generated in connection with the study.

"(B) AVAILABILITY OF REPORTS.—Each report submitted under subparagraph (A) shall be considered to be in the public domain, and shall be assigned a docket number by the Commissioner of Food and Drugs. An interested person may submit written comments concerning such pediatric studies to the Commissioner of Food and Drugs, and the written comments shall become part of the docket file with respect to each of the drugs.

"(C) ACTION BY COMMISSIONER.—The Commissioner of Food and Drugs shall take appropriate action in response to the reports submitted under subparagraph (A) in accordance with paragraph (4).

"(4) REQUEST FOR LABELING CHANGES.—During the 180-day period after the date on which a report is submitted under paragraph (3)(A), the Commissioner of Food and Drugs shall—

"(A) review the report and such other data as are available concerning the safe and effective use in the pediatric population of the drug studied; and

"(B) negotiate with the holders of approved applications for the drug studied for any labeling changes that the Commissioner of Food and Drugs determines to be appropriate and requests the holders to make; and

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

"(ii) publish in the Federal Register a summary of the report and a copy of any requested labeling changes.

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

"(A) the Commissioner of Food and Drugs shall immediately refer the request to the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee; and

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

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

"(ii) make a recommendation to the Commissioner of Food and Drugs as to appropriate labeling changes, if any.

"(6) FDA DETERMINATION.—Not later than 30 days after receiving a recommendation from the Subcommittee under paragraph (5)(B)(ii) with respect to a drug, the Commissioner of Food and Drugs shall consider the recommendation and, if appropriate, make a request to the holders of approved applications for the drug to make any labeling change that the Commissioner of Food and Drugs determines to be appropriate.

"(7) FAILURE TO AGREE.—If a holder of an approved application for a drug, within 30 days after receiving a request to make a labeling change under paragraph (6), does not agree to make a requested labeling change, the Commissioner may deem the drug to be misbranded under the Federal Food, Drug, and Cosmetic Act.

"(8) RECOMMENDATION FOR FORMULATION CHANGES.—If a pediatric study completed under public contract indicates that a formulation change is necessary and the Secretary agrees, the Secretary shall send a nonbinding letter of recommendation regarding that change to each holder of an approved application.

"(d) CONFIDENTIAL COMMERCIAL INFORMATION; TRADE SECRETS.—Nothing in this section requires or authorizes the use or disclosure of confidential commercial information or trade secrets.

"(e) AUTHORIZATION OF APPROPRIATIONS.—

"(1) IN GENERAL.—For the purpose of carrying out this section, there are authorized to be appropriated $200,000,000 for fiscal year 2002, and such sums as may be necessary for each of the fiscal years 2003 through 2007.

"(2) AVAILABILITY.—Any amount appropriated under paragraph (1) shall remain available to carry out this section until expended.".

SEC. 4. WRITTEN REQUEST TO HOLDERS OF APPROVED APPLICATIONS FOR DRUGS THAT HAVE MARKET EXCLUSIVITY.

Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is amended in subsection (c) (as redesignated by section 2(a)(2) of this Act) by adding at the end the following:

"(4) WRITTEN REQUEST TO HOLDERS OF APPROVED APPLICATIONS FOR DRUGS THAT HAVE MARKET EXCLUSIVITY.—

"(A) REQUEST AND RESPONSE.—If the Secretary makes a written request for pediatric studies under subsection (b) to the holder of an application approved under section 505(b)(1), the holder, not later than 180 days after receiving the written request, shall respond to the Secretary as to the intention of the holder to act on the request by—

"(i) indicating when the pediatric studies will be initiated, if the holder agrees to the request; or

"(ii) indicating that the holder does not agree to the request.

"(B) NO AGREEMENT TO REQUEST.—

"(i) REFERRAL.—If the holder does not agree to a written request within the time period specified in subparagraph (A), and if the Secretary determines that there is a continuing need for information relating to the use of the drug in the pediatric population (including neonates as appropriate), the Secretary shall refer the drug to the Foundation for Pediatric Research established under section 499A of the Public Health Service Act (referred to in this paragraph as the 'Foundation') for consideration for the conduct of the pediatric studies described in the written request.

"(ii) PUBLIC NOTICE.—The Secretary shall give public notice of a referral under clause (i), including notice of the name of the drug, the name of the manufacturer, and the indication to be studied.

"(C) LACK OF FUNDS.—If, on referral of a drug under subparagraph (B)(i), the Foundation certifies to the Secretary that the Foundation does not have funds available to conduct the requested studies, the Secretary shall refer the drug for inclusion on the list established under section 409I of the Public Health Service Act for the conduct of the studies.

"(D) CONFIDENTIAL COMMERCIAL INFORMATION; TRADE SECRETS.—Nothing in this paragraph requires or authorizes the use or disclosure of confidential commercial information or trade secrets.

"(E) NO REQUIREMENT TO REFER.—Nothing in this subsection shall be construed to require that every declined written request shall be referred to the Foundation.".

SEC. 5. TIMELY LABELING CHANGES FOR DRUGS GRANTED EXCLUSIVITY; DRUG FEES.

(a) ELIMINATION OF USER FEE WAIVER FOR PEDIATRIC SUPPLEMENTS.—Section 736(a)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 379h(a)(1)) is amended—

(1) by striking subparagraph (F); and

(2) by redesignating subparagraph (G) as subparagraph (F).

(b) LABELING CHANGES.—

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

"(kk) PRIORITY SUPPLEMENT.—The term 'priority supplement' means a drug application referred to in section 101(4) of the Food and Drug Administration Modernization Act of 1997 (111 Stat. 2298).".

(2) TREATMENT AS PRIORITY SUPPLEMENTS.—Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a), as amended by section 2(a)(2) of this Act, is amended by adding at the end the following:

"(k) LABELING SUPPLEMENTS.—

"(1) PRIORITY STATUS FOR PEDIATRIC SUPPLEMENTS.—Any supplement to an application under section 505 proposing a labeling change pursuant to a report on a pediatric study under this section—

"(A) shall be considered to be a priority supplement; and

"(B) shall be subject to the performance goals established by the Commissioner for priority drugs.

"(2) DISPUTE RESOLUTION.—If the Commissioner determines that an application with respect to which a pediatric study is conducted under this section is approvable and that the only open issue for final action on the application is the reaching of an agreement between the sponsor of the application and the Commissioner on appropriate changes to the labeling for the drug that is the subject of the application—

"(A) not later than 180 days after the date of submission of the application—

"(i) the Commissioner shall request that the sponsor of the application make any labeling change that the Commissioner determines to be appropriate; and

"(ii) if the sponsor of the application does not agree to make a labeling change requested by the Commissioner by that date, the Commissioner shall immediately refer the matter to the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee;

"(B) not later than 90 days after receiving the referral, the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee shall—

"(i) review the pediatric study reports; and

"(ii) make a recommendation to the Commissioner concerning appropriate labeling changes, if any;

"(C) the Commissioner shall consider the recommendations of the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee and, if appropriate, not later than 30 days after receiving the recommendation, make a request to the sponsor of the application to make any labeling change that the Commissioner determines to be appropriate; and

"(D) if the sponsor of the application, within 30 days after receiving a request under subparagraph (C), does not agree to make a labeling change requested by the Commissioner, the Commissioner may deem the drug that is the subject of the application to be misbranded.".

SEC. 6. OFFICE OF PEDIATRIC THERAPEUTICS.

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

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

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

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

(2) 1 or more additional individuals with expertise concerning ethical issues presented by the conduct of clinical research in the pediatric population; and

(3) 1 or more additional individuals with expertise in pediatrics who shall consult and collaborate with all components of the Food and Drug Administration concerning activities described in subsection (b).

SEC. 7. NEONATES.

Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is amended in subsection (f) (as redesignated by section 2(a)(2) of this Act) by inserting "(including neonates in appropriate cases)" after "pediatric age groups".

SEC. 8. SUNSET.

Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is amended by striking subsection (i) (as redesignated by section 2(a)(2) of this Act) and inserting the following:

"(i) SUNSET.—A drug may not receive any 6-month period under subsection (a) or (b) unless—

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

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

"(3) all requirements of this section are met.".

SEC. 9. DISSEMINATION OF PEDIATRIC INFORMATION.

Section 505A of the Federal Food, Drug, and Cosmetic Act, as amended by section 5(b)(2) of this Act, is amended by adding at the end the following:

"(l) DISSEMINATION OF PEDIATRIC INFORMATION.—

"(1) IN GENERAL.—Not later than 180 days after the date of submission of a report on a pediatric study under this section, the Commissioner shall make available to the public a summary of the medical and clinical pharmacology reviews of pediatric studies conducted for the supplement, including by publication in the Federal Register.

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

SEC. 10. CLARIFICATION OF INTERACTION OF MARKET EXCLUSIVITY UNDER SECTION 505A OF THE FEDERAL FOOD, DRUG, AND COSMETIC ACT AND MARKET EXCLUSIVITY AWARDED TO AN APPLICANT FOR APPROVAL OF A DRUG UNDER SECTION 505(j) OF THAT ACT.

Section 505A of the Federal Food, Drug, and Cosmetic Act, as amended by section 9 of this Act, is amended by adding at the end the following:

"(m) CLARIFICATION OF INTERACTION OF MARKET EXCLUSIVITY UNDER THIS SECTION AND MARKET EXCLUSIVITY AWARDED TO AN APPLICANT FOR APPROVAL OF A DRUG UNDER SECTION 505(j).—

"(1) IN GENERAL.—If a 180-day period under section 505(j)(5)(B)(iv) overlaps with a 6-month extension under this section, so that the applicant for approval of a drug under section 505(j) entitled to the 180-day period under that section loses a portion of the 180-day period to which the applicant is entitled for the drug, the 180-day period shall be extended—

"(A) if the 180-day period would, but for this subsection, expire after the 6-month extension, by the number of days of the overlap; or

"(B) if the 180-day period would, but for this subsection, expire during the 6-month extension, by 6 months.

"(2) EFFECT OF SUBSECTION.—Under no circumstances shall application of this section result in an applicant for approval of a drug under section 505(j) being enabled to commercially market the drug to the exclusion of a subsequent applicant for approval of a drug under section 505(j) for more than 180 days.".

SEC. 11. PROMPT APPROVAL OF GENERIC DRUGS WHEN PEDIATRIC INFORMATION ADDED TO LABELING.

(a) IN GENERAL.—Section 505A of the Federal Food, Drug, and Cosmetic Act, as amended by section 10 of this Act, is amended by adding at the end the following subsection:

"(n) PROMPT APPROVAL OF GENERIC DRUGS WHEN PEDIATRIC INFORMATION ADDED TO LABELING.—

"(1) IN GENERAL.—A drug for which an application has been submitted or approved under section 505(j) and which otherwise meets all other applicable requirements under that section shall be considered eligible for approval and shall not be considered misbranded under section 502 even when its labeling omits a pediatric indication or other aspect of labeling pertaining to pediatric use that is protected by patent or by market exclusivity pursuant to clause (iii) or (iv) of section 505(j)(5)(D).

"(2) LABELING OF GENERIC DRUG.—Notwithstanding the provisions of clause (iii) or (iv) of section 505(j)(5)(D), the Secretary may require that the labeling of a drug approved under section 505(j) that omits pediatric labeling pursuant to paragraph (1) include—

"(A) a statement that the drug is not labeled for the protected pediatric use; and

"(B) any warnings against unsafe pediatric use that the Secretary considers necessary.

"(3) RULE OF CONSTRUCTION.—Paragraphs 1 and 2 of this subsection do not affect—

"(A) the availability or scope of exclusivity under this section;

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

"(C) except as expressly provided in paragraph (1) and (2), the operation of section 505.".

(b) EFFECTIVE DATE.—The amendments made by subsection (a) take effect on the date of the enactment of this Act, including with respect to applications under section 505(j) of the Federal Food, Drug, and Cosmetic Act that are approved or pending on that date.

SEC. 12. ADVERSE-EVENT REPORTING.

(a) TOLL-FREE NUMBER IN LABELING.—Not later than one year after the date of the enactment of this Act, the Secretary of Health and Human Services shall promulgate a final rule requiring that the labeling of each drug for which an application is approved under section 505 of the Federal Food, Drug, and Cosmetic Act (regardless of the date on which approved) include the toll-free number maintained by the Secretary for the purpose of receiving reports of adverse events regarding drugs. With respect to the final rule:

(1) The rule shall provide for the implementation of such labeling requirement in a manner that the Secretary considers to be most likely to reach the broadest consumer audience.

(2) In promulgating the rule, the Secretary shall seek to minimize the cost of the rule on the pharmacy profession.

(3) The rule shall take effect not later than 60 days after the date on which the rule is promulgated.

(b) DRUGS WITH PEDIATRIC MARKET EXCLUSIVITY.—

(1) IN GENERAL.—During the one-year beginning on the date on which a drug receives a period of market exclusivity under 505A of the Federal Food, Drug, and Cosmetic Act, any report of an adverse event regarding the drug that the Secretary of Health and Human Services receives shall be referred to the Office of Pediatric Therapeutics established under section 6 of this Act. In considering the report, the Director of such Office shall provide for the review of the report by the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee, including obtaining any recommendations of such Subcommittee regarding whether the Secretary should take action under the Federal Food, Drug, and Cosmetic Act in response to the report.

(2) RULE OF CONSTRUCTION.—Paragraph (1) may not be construed as restricting the authority of the Secretary of Health and Human Services to continue carrying out the activities described in such paragraph regarding a drug after the one-year period described in such paragraph regarding the drug has expired.

SEC. 13. FOUNDATION FOR PEDIATRIC RESEARCH.

Title IV of the Public Health Service Act (42 U.S.C. 281 et seq.) is amended by adding at the end the following part:

"PART J—FOUNDATION FOR PEDIATRIC RESEARCH

"SEC. 499A. ESTABLISHMENT AND DUTIES OF FOUNDATION.

"(a) IN GENERAL.—The Secretary, acting through the Director of NIH and in consultation with the Commissioner of Food and Drugs, shall establish a nonprofit corporation to be known as the Foundation for Pediatric Research (hereafter in this section referred to as the 'Foundation'). The Foundation shall not be an agency or instrumentality of the United States Government.

"(b) PURPOSE OF FOUNDATION.—The purpose of the Foundation shall be to collect funds and award grants for research on drugs listed by the Secretary pursuant to section 409I(a)(1)(A).

"(c) CERTAIN ACTIVITIES OF FOUNDATION.—

"(1) IN GENERAL.—In carrying out subsection (b), the Foundation may solicit and accept gifts, grants, and other donations, establish accounts, and invest and expend funds in support of a program to encourage donations for the conduct of studies of drugs referred to in subsection (b).

"(2) FEES.—The Foundation may assess fees for the provision of professional, administrative and management services by the Foundation in amounts determined reasonable and appropriate by the Executive Director.

"(3) AUTHORITY OF FOUNDATION.—The Foundation shall be the sole entity responsible for carrying out the activities described in this subsection.

"(d) BOARD OF DIRECTORS.—

"(1) COMPOSITION.—

"(A) The Foundation shall have a Board of Directors (hereafter referred to in this section as the 'Board'), which shall be composed of ex officio and appointed members in accordance with this subsection. Appointed members of the Board shall be the voting members.

"(B) The ex officio members of the Board shall be—

"(i) the Chairman and ranking minority member of the Subcommittee on Health (Committee on Energy and Commerce) or their designees, in the case of the House of Representatives;

"(ii) the Chairman and ranking minority member of the Committee on Health, Education, Labor and Pensions or their designees, in the case of the Senate;

"(iii) the Director of NIH; and

"(iv) the Commissioner of Food and Drugs.

"(C) The ex officio members of the Board under subparagraph (B) shall appoint to the Board 11 individuals from among a list of candidates to be provided by the National Academy of Science. Of such appointed members—

"(i) 5 shall be representative of the experts in pediatric medicine and research field;

"(ii) 1 shall be a biomedical ethicist; and

"(iii) 5 shall be representatives of the general public, which may include representatives of affected industries.

"(D)(i) Not later than 30 days after the date of the enactment of the Best Pharmaceuticals for Children Act, the Director of NIH shall convene a meeting of the ex officio members of the Board to—

"(I) incorporate the Foundation and establish the general policies of the Foundation for carrying out the purposes of subsection (b), including the establishment of the bylaws of the Foundation; and

"(II) appoint the members of the Board in accordance with subparagraph (C).

"(ii) Upon the appointment of the members of the Board under clause (i)(II), the terms of service of the ex officio members of the Board as members of the Board shall terminate.

"(E) The agreement of not less than three-fifths of the members of the ex officio members of the Board shall be required for the appointment of each member to the initial Board.

"(F) No employee of the National Institutes of Health shall be appointed as a member of the Board.

"(2) CHAIR.—

"(A) The ex officio members of the Board under paragraph (1)(B) shall designate an individual to serve as the initial Chair of the Board.

"(B) Upon the termination of the term of service of the initial Chair of the Board, the appointed members of the Board shall elect a member of the Board to serve as the Chair of the Board.

"(3) TERMS AND VACANCIES.—

"(A) The term of office of each member of the Board appointed under paragraph (1)(C) shall be 5 years, except that the terms of offices for the initial appointed members of the Board shall expire as determined by the ex officio members and the Chair.

"(B) Any vacancy in the membership of the Board shall be filled in the manner in which the original position was made and shall not affect the power of the remaining members to execute the duties of the Board.

"(C) If a member of the Board does not serve the full term applicable under subparagraph (A), the individual appointed to fill the resulting vacancy shall be appointed for the remainder of the term of the predecessor of the individual.

"(D) A member of the Board may continue to serve after the expiration of the term of the member until a successor is appointed.

"(4) COMPENSATION.—Members of the Board may not receive compensation for service on the Board. Such members may be reimbursed for travel, subsistence, and other necessary expenses incurred in carrying out the duties of the Board, as set forth in the bylaws issued by the Board.

"(5) MEETINGS AND QUORUM.—A majority of the members of the Board shall constitute a quorum for purposes of conducting the business of the Board.

"(6) CERTAIN BYLAWS.—

"(A) In establishing bylaws under this subsection, the Board shall ensure that the following are provided for:

"(i) Policies for the selection of the officers, employees, and agents of the Foundation.

"(ii) Policies, including ethical standards, for the acceptance, solicitation, and disposition of donations and grants to the Foundation and for the disposition of the assets of the Foundation. Policies with respect to ethical standards shall ensure that officers, employees and agents of the Foundation (including members of the Board) avoid encumbrances that would result in a conflict of interest, including a financial conflict of interest or a divided allegiance. Such policies shall include requirements for the provision of information concerning any ownership or controlling interest in entities related to the activities of the Foundation by such officers, employees and agents and their spouses and relatives.

"(iii) Policies for the conduct of the general operations of the Foundation.

"(B) In establishing bylaws under this subsection, the Board shall ensure that such bylaws (and activities carried out under the bylaws) do not—

"(i) reflect unfavorably upon the ability of the Foundation to carry out its responsibilities or official duties in a fair and objective manner; or

"(ii) compromise, or appear to compromise, the integrity of any governmental agency or program, or any officer or employee involved in such program.

"(e) INCORPORATION.—The initial members of the Board shall serve as incorporators and shall take whatever actions necessary to incorporate the Foundation.

"(f) NONPROFIT STATUS.—The Foundation shall be considered to be a corporation under section 501(c) of the Internal Revenue Code of 1986, and shall be subject to the provisions of such section.

"(g) EXECUTIVE DIRECTOR.—

"(1) IN GENERAL.—The Foundation shall have an Executive Director who shall be appointed by the Board and shall serve at the pleasure of the Board. The Executive Director shall be responsible for the day-to-day operations of the Foundation and shall have such specific duties and responsibilities as the Board shall prescribe.

"(2) COMPENSATION.—The rate of compensation of the Executive Director shall be fixed by the Board.

"(h) POWERS.—In carrying out subsection (b), the Foundation shall operate under the direction of its Board, and may—

"(1) adopt, alter, and use a corporate seal, which shall be judicially noticed;

"(2) provide for 1 or more officers, employees, and agents, as may be necessary, define their duties, and require surety bonds or make other provisions against losses occasioned by acts of such persons;

"(3) hire, promote, compensate, and discharge officers and employees of the Foundation, and define the duties of the officers and employees;

"(4) with the consent of any executive department or independent agency, use the information, services, staff, and facilities of such in carrying out this section;

"(5) sue and be sued in its corporate name, and complain and defend in courts of competent jurisdiction;

"(6) modify or consent to the modification of any contract or agreement to which it is a party or in which it has an interest under this part;

"(7) establish a process for the selection of candidates for positions under subsection (c);

"(8) solicit, accept, hold, administer, invest, and spend any gift, devise, or bequest of real or personal property made to the Foundation;

"(9) enter into such other contracts, leases, cooperative agreements, and other transactions as the Executive Director considers appropriate to conduct the activities of the Foundation; and

"(10) exercise other powers as set forth in this section, and such other incidental powers as are necessary to carry out its powers, duties, and functions in accordance with this part.

"(i) ADMINISTRATIVE CONTROL.—No participant in the program established under this part shall exercise any administrative control over any Federal employee, nor shall the Foundation attempt to influence an executive branch agency or employee.

"(j) GENERAL PROVISIONS.—

"(1) FOUNDATION INTEGRITY.—The members of the Board shall be accountable for the integrity of the operations of the Foundation and shall ensure such integrity through the development and enforcement of criteria and procedures relating to standards of conduct (including those developed under subsection (d)(6)(A)(ii), financial disclosure statements, conflict of interest rules, recusal and waiver rules, audits and other matter determined appropriate by the Board.

"(2) FINANCIAL CONFLICTS OF INTEREST.—Any individual who is an officer, employee, or member of the Board of the Foundation may not (in accordance with policies and requirements developed under subsection (d)(6)(A)(ii) personally or substantially participate in the consideration or determination by the Foundation of any matter that would directly or predictably affect any financial interest of the individual or a relative (as such term is defined in section 109(16) of the Ethics in Government Act of 1978) of the individual, of any business organization or other entity, or of which the individual is an officer or employee, or is negotiating for employment, or in which the individual has any other financial interest.

"(3) AUDITS; AVAILABILITY OF RECORDS.—The Foundation shall—

"(A) provide for annual audits of the financial condition of the Foundation; and

"(B) make such audits, and all other records, documents, and other papers of the Foundation, available to the Secretary and the Comptroller General of the United States for examination or audit.

"(4) REPORTS.—

"(A) Not later than 5 months following the end of each fiscal year, the Foundation shall publish a report describing the activities of the Foundation during the preceding fiscal year. Each such report shall include for the fiscal year involved a comprehensive statement of the operations, activities, financial condition, and accomplishments of the Foundation.

"(B) With respect to the financial condition of the Foundation, each report under subparagraph (A) shall include the source, and a description of, all gifts or grants to the Foundation of real or personal property, and the source and amount of all gifts or grants to the Foundation of money. Each such report shall include a specification of any restrictions on the purposes for which gifts or grants to the Foundation may be used.

"(C) The Foundation shall make copies of each report submitted under subparagraph (A) available for public inspection, and shall upon request provide a copy of the report to any individual for a charge not exceeding the cost of providing the copy.

"(D) The Board shall annually hold a public meeting to summarize the activities of the Foundation and distribute written reports concerning such activities and the scientific results derived from such activities.

"(5) SERVICE OF FEDERAL EMPLOYEES.—Federal employees may serve on committees advisory to the Foundation and otherwise cooperate with and assist the Foundation in carrying out its function, so long as the employees do not direct or control Foundation activities.

"(6) RELATIONSHIP WITH EXISTING ENTITIES.—The Foundation may, pursuant to appropriate agreements, acquire the resources of existing nonprofit private corporations with missions similar to the purposes of the Foundation.

"(7) INTELLECTUAL PROPERTY RIGHTS.—The Board may adopt written standards with respect to the ownership of any intellectual property rights derived from the collaborative efforts of the Foundation prior to the commencement of such efforts.

"(8) NATIONAL INSTITUTES OF HEALTH AMENDMENTS OF 1990.—The activities conducted in support of the National Institutes of Health Amendments of 1990 (Public Law 101–613), and the amendments made by such Act, shall not be nullified by the enactment of this section.

"(9) LIMITATION OF ACTIVITIES.—The Foundation shall exist solely as an entity to collect funds and award grants for research on drugs listed by the Secretary pursuant to section 409I(a)(1)(A).

"(10) TRANSFER OF FUNDS.—The Foundation may transfer funds to the National Institutes of Health. Any funds transferred under this paragraph shall be subject to all Federal limitations relating to federally-funded research.

"(k) DUTIES OF THE DIRECTOR.—

"(1) APPLICABILITY OF CERTAIN STANDARDS TO NON-FEDERAL EMPLOYEES.—In the case of any individual who is not an employee of the Federal Government and who serves in association with the National Institutes of Health, with respect to financial assistance received from the Foundation, the Foundation may not provide the assistance of, or otherwise permit the work at the National Institutes of Health to begin until a memorandum of understanding between the individual and the Director of NIH, or the designee of such Director, has been executed specifying that the individual shall be subject to such ethical and procedural standards of conduct relating to duties performed at the National Institutes of Health, as the Director of NIH determines is appropriate.

"(2) SUPPORT SERVICES.—The Director of NIH shall provide facilities, utilities and support services to the Foundation.

"(l) REPORTS OF STUDIES; LABELING CHANGES.—

"(1) IN GENERAL.—Upon completion of a pediatric study conducted pursuant to this section, a report concerning the study shall be submitted to the Director of National Institutes of Health and the Commissioner of Food and Drugs. The report shall include all data generated in connection with the study.

"(2) AVAILABILITY OF REPORTS; ACTION BY FOOD AND DRUG ADMINISTRATION; LABELING CHANGES.—With respect to a report submitted under paragraph (1), the provisions of paragraphs (3)(B) through (8) of section 409I(c) apply to such report to the same extent and in the same manner as such provision apply to a report submitted under section 409I(c)(3)(A).

"(m) FUNDING.—

"(1) AUTHORIZATION OF APPROPRIATIONS.—For the purpose of carrying out this part, there are authorized to be appropriated such sums as may be necessary for fiscal year 2002 and each subsequent fiscal year.

"(2) LIMITATION REGARDING OTHER FUNDS.—Amounts appropriated under any provision of law other than paragraph (1) may not be expended to establish or operate the Foundation.".

SEC. 14. STUDY CONCERNING RESEARCH INVOLVING CHILDREN.

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

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

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

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

(C) federally-supported evidence-based research involving children; and

(2) the submission to the appropriate committees of Congress, by not later than 2 years after the date of enactment of this Act, of a report concerning the review conducted under paragraph (1) that includes recommendations on best practices relating to research involving children.

(b) AREAS OF REVIEW.—In conducting the review under subsection (a)(1), the Institute of Medicine shall consider the following:

(1) The written and oral process of obtaining and defining "assent", "permission" and "informed consent" with respect to child clinical research participants and the parents, guardians, and the individuals who may serve as the legally authorized representatives of such children (as defined in subpart A of part 46 of title 45, Code of Federal Regulations).

(2) The expectations and comprehension of child research participants and the parents, guardians, or legally authorized representatives of such children, for the direct benefits and risks of the child's research involvement, particularly in terms of research versus therapeutic treatment.

(3) The definition of "minimal risk" with respect to a healthy child or a child with an illness.

(4) The appropriateness of the regulations applicable to children of differing ages and maturity levels, including regulations relating to legal status.

(5) Whether payment (financial or otherwise) may be provided to a child or his or her parent, guardian, or legally authorized representative for the participation of the child in research, and if so, the amount and type of payment that may be made.

(6) Compliance with the regulations referred to in subsection (a)(1)(A), the monitoring of such compliance (including the role of institutional review boards), and the enforcement actions taken for violations of such regulations.

(7) The unique roles and responsibilities of institutional review boards in reviewing research involving children, including composition of membership on institutional review boards.

(c) REQUIREMENTS OF EXPERTISE.—The Institute of Medicine shall conduct the review under subsection (a)(1) and make recommendations under subsection (a)(2) in conjunction with experts in pediatric medicine, pediatric research, and the ethical conduct of research involving children.

SEC. 15. STUDY ON EFFECTS OF THIS ACT.

Not later than October 1, 2006, the Comptroller General of the United States shall submit to the Congress and the Secretary of Health and Human Services a report that describes the following:

(1) The effectiveness of the amendments made by this Act in ensuring that all drugs used by children are tested and properly labeled, including—

(A) the number and importance for children of drugs that are being tested as a result of such amendments, and the importance for children, health care providers, parents, and others of labeling changes made as a result of such testing;

(B) the number and importance for children of drugs that are not being tested for their use notwithstanding the amendments, and possible reason for this; and

(C) the number of drugs for which pediatric testing has been done, for which a period of market exclusivity has been granted, and for which labeling changes required the use of the dispute resolution process established pursuant to the amendments, together with a description of the outcomes of such process, including a description of the disputes and the recommendations of the advisory committee.

(2) The economic impact of the amendments made by this Act, including an estimate of—

(A) costs to taxpayers in the form of higher expenditures by Medicaid and other government programs;

(B) costs to consumers as a result of any delay in the availability of lower cost generic equivalents of drugs tested and granted exclusivity pursuant to such amendments, and loss of revenue by the generic drug industry and any other affected industry as a result of any such delay; and

(C) benefits to the government, to private insurers, and to consumers resulting from decreased health care costs, including—

(i) decreased hospitalizations, due to more appropriate and more effective use of medications in children as a result of testing and re-labeling because of such amendments;

(ii) direct and indirect benefits associated with fewer physician visits not related to hospitalization;

(iii) benefits to children from missing less time at school and being less affected by chronic illnesses, thereby allowing a better quality of life;

(iv) benefits to consumers from lower health insurance premiums due to lower treatment costs and hospitalization rates; and

(v) benefits to employers from reduced need for employees to care for family members.

(3) The nature and types of studies in children of drugs granted a period of market exclusivity pursuant to the amendments made by this Act, including a description of the complexity of such studies, the number of study sites necessary to obtain appropriate data, and the numbers of children involved in any clinical studies, and the cost of such studies for each type of study identified.

(4) The increased pediatric research capability, both private and government-funded, associated with the amendments made by this Act.

SEC. 16. MINORITY CHILDREN AND PEDIATRIC-EXCLUSIVITY PROGRAM.

(a) PROTOCOLS FOR PEDIATRIC STUDIES.—Section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) is amended in subsection (c)(2) (as redesignated by section 2(a)(2) of this Act) by inserting after the first sentence the following: "In reaching an agreement regarding written protocols, the Secretary shall take into account adequate representation of children of ethnic and racial minorities.".

(b) STUDY BY GENERAL ACCOUNTING OFFICE.—

(1) IN GENERAL.—The Comptroller General of the United States shall conduct a study for the purpose of determining the following:

(A) The extent to which children of ethnic and racial minorities are adequately represented in studies under section 505A of the Federal Food, Drug, and Cosmetic Act; and to the extent ethnic and racial minorities are not adequately represented, the reasons for such under representation and recommendations to increase such representation.

(B) Whether the Food and Drug Administration has appropriate management systems to monitor the representation of the children of ethnic and racial minorities in such studies.

(C) Whether drugs used to address diseases that disproportionately affect racial and ethnic minorities are being studied for their safety and effectiveness under section 505A of the Federal Food, Drug, and Cosmetic Act.

(2) DATE CERTAIN FOR COMPLETING STUDY.—Not later than January 10, 2003, the Comptroller General shall complete the study required in paragraph (1) and submit to the Congress a report describing the findings of the study.

SEC. 17. TECHNICAL AND CONFORMING AMENDMENTS.

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

(1)(A) by striking "(j)(4)(D)(ii)" each place such term appears and inserting "(j)(5)(D)(ii)"; and

(B) by striking "(j)(4)(D)" each place such term appears and inserting "(j)(5)(D)"; and

(2)(A) in subsection (c) (as redesignated by section 2(a)(2) of this Act), in each of paragraphs (1) through (3), by striking "subsection (a) or (c)" and inserting "subsection (a) or (b)"; and

(B) in subsection (d) (as so redesignated), in the last sentence, by striking "subsection (a) or (c)" and inserting "subsection (a) or (b)".

PURPOSE AND SUMMARY

The purpose of H.R. 2887, the Best Pharmaceuticals for Children Act, is to ensure that the pediatric exclusivity provision from the Food and Drug Administration Modernization Act, which will expire at the end of this calendar year, is reauthorized. This legislation reauthorizes the provision through FY 2007.

Further, the legislation creates a research fund within the NIH for drugs that are off-patent or lacking in remaining exclusivity and for certain on-patent drugs for which the application holder does not study under pediatric exclusivity. Through this fund the NIH, with consultation from the FDA, shall contract with researchers to study these drugs in children. As part of this effort the NIH, in consultation with the FDA and experts in pediatric research, shall publish an annual list of these drugs that merit the highest priority for study.

Because there is concern that pediatric exclusivity as presently constituted does not lead to quick changes in drug labels, H.R. 2887 ensures that pediatric supplemental NDAs will be given priority status, and subjected to a user fee (under current law, pediatric applications are not subject to the PDUFA application user fee). Further, the legislation provides for a dispute resolution mechanism for labeling changes that have not been resolved within 180 days of supplement submission. At the end of this process, should the manufacturer not agree to comply with the Secretary's proposed label, the Secretary is given the authority to "deem" the drug misbranded.

BACKGROUND AND NEED FOR LEGISLATION

Children suffer many of the same diseases as adults, but according to the American Academy of Pediatrics, only a small fraction (roughly 25%) of all drugs have been studied in pediatric patients. While most drugs have not been studied for use in children, many of these drugs are nonetheless prescribed for the benefit of children. Generally, the dosage for the child is determined by a physician based upon the child's age, height and weight. The lack of pediatric testing and labeling can place children at risk of under- and

overdosing, and the lack of age appropriate formulations (i.e., liquids or chewable tablets) may result in the improper administration of drugs.

To encourage the study of drugs in children and the development of pediatric labeling information, the Food and Drug Administration Modernization Act of 1997 contained a provision which provides six months of drug marketing exclusivity to be attached to existing patent protections or other exclusivity in exchange for conducting pediatric studies, at the request of the FDA. This provision, known as pediatric exclusivity, expires at the end of this calendar year.

To qualify for the six months of exclusivity, the FDA must first issue a Written Request to a manufacturer to conduct pediatric studies. If the FDA does not request that a drug be studied, there is no way for that drug to have its exclusivity extended under this provision. If the manufacturer does all of the studies required by the FDA in the Written Request, the manufacturer will then submit the results of those studies, as well as proposed label changes, in the form of a NDA or supplemental NDA. The FDA then has 90 days to determine whether the studies conducted by the manufacturer comport with the Written Request. If the studies do comply with the Written Request, the manufacturer is entitled to an additional six months of exclusivity for all drug products containing the active moiety which was studied.

In its January 2001 Report to Congress, the FDA found that "the pediatric exclusivity provision has done more to generate clinical studies and useful prescribing information for the pediatric population than any other regulatory or legislative process to date." While in the six years prior to the enactment of the pediatric exclusivity provision only six studies were conducted on the pediatric population at the request of the FDA, in the four years since enactment there have been 197 Written Requests issued to conduct 400+ studies on the pediatric population.

While the incentive has worked to increase the number of drugs being studied in the pediatric population, the FDA found that the incentive is not adequate for certain drugs. For example, drugs with no patent protection or marketing exclusivity remaining are not eligible for any exclusivity. Further there is little or no economic incentive to study on-patent drugs with very little patent protection remaining, or with low sales volumes. Also, the incentive is frequently ineffectual in producing studies in the neonatal population, because drugs must first be studied in older age cohorts in order to develop the body of evidence required to test the drugs in neonates.

Also, while the FDA found that pediatric exclusivity has resulted in pediatric studies being conducted at a high volume, they also found that the provision will increase certain health care expenditures, and decrease others. For example, due to the delay in generic competition resulting from an additional six months of brand exclusivity, the FDA estimates a yearly cost of $695 million to the nation's pharmaceutical consumers. This amounts to an additional cost of one half of one percent to the nation's pharmaceutical bill. As far as savings are concerned, the FDA calculated that eliminating just 25% of the difference in hospitalization rates between children and middle-aged adults for five diseases would save $228

million annually. Also, a Tufts Center for the Study of Drug Development report estimates that the pediatric exclusivity provision saves up to $7 billion per year by making treatments more effective for pediatric patients ("If even one-hundredth of the annual economic impact from these leading causes of death and disability was reduced by providing more effective treatments to children, making for healthier adults in the process, the $7 billion saved each year would be 10 times more than the nearly $700 million that FDA has estimated as the yearly cost to society from the pediatric exclusivity awards.")

HEARINGS

The Subcommittee on Health held a hearing on Evaluating the Effectiveness of the Food and Drug Administration Act on Thursday, May 3, 2001. The Subcommittee received testimony from: Ms. Linda Suydam, D.P.A., Senior Food Commissioner, Food and Drug Administration; Dr. Gregory L. Kearns, Professor and Chief, Division of Clinical Pharmacology and Medical Toxicology, Children's Mercy Hospital and Clinics; Ms. Carole Ben-Maimon, President and C.E.O., Barr Laboratories, on behalf of The General Pharmaceutical Association; Dr. Richard Gorman, M.D., Incoming Chair, Committee on Drugs, American Academy of Pediatrics; Ms. Abbey Meyers, President, National Organization for Rare Disorders; Mr. Travis Plunket, Legislative Director, Consumer Federation of America, on behalf of Patient and Consumer Coalition; and Dr. Timothy R, Franson, M.D., Vice President, Clinical Research and Regulatory Affairs, U.S., Lilly Research Laboratories.

COMMITTEE CONSIDERATION

On Thursday, October 4, 2001, the Subcommittee on Health met in open markup session and approved H.R. 2887, the Best Pharmaceuticals for Children Act, for Full Committee consideration, as amended, by a record vote of 24 yeas and 5 nays. On Thursday, October 11, 2001, the Committee on Energy and Commerce met in open markup session and favorably ordered reported H.R. 2887, as amended, by a record vote of 41 yeas and 6 nays.

COMMITTEE VOTES

Clause 3(b) of rule XIII of the Rules of the House of Representatives requires the Committee to list the record votes on the motion to report legislation and amendments thereto. The following record votes were taken in connection with ordering H.R. 2887 reported. A motion by Mr. Tauzin to order H.R. 2887 reported to the House, as amended, was agreed to by a record vote of 41 yeas to 6 nays.

COMMITTEE ON ENERGY AND COMMERCE -- 107TH CONGRESS
ROLL CALL VOTE # 19

BILL: H.R. 2887, the Best Pharmaceuticals for Children Act.

AMENDMENT: Amendment to the amendment in the nature of a substitute offered by Mr. Brown, #1a.

DISPOSITION: **NOT AGREED TO**, as amended by unanimous consent, by a roll call vote of 12 yeas to 36 nays.

REPRESENTATIVE	YEAS	NAYS	PRESENT	REPRESENTATIVE	YEAS	NAYS	PRESENT
Mr. Tauzin		X		Mr. Dingell	X		
Mr. Bilirakis		X		Mr. Waxman	X		
Mr. Barton				Mr. Markey			
Mr. Upton		X		Mr. Hall		X	
Mr. Stearns		X		Mr. Boucher			
Mr. Gillmor		X		Mr. Towns		X	
Mr. Greenwood		X		Mr. Pallone	X		
Mr. Cox		X		Mr. Brown	X		
Mr. Deal		X		Mr. Gordon			
Mr. Largent		X		Mr. Deutsch	X		
Mr. Burr				Mr. Rush		X	
Mr. Whitfield		X		Ms. Eshoo		X	
Mr. Ganske				Mr. Stupak	X		
Mr. Norwood		X		Mr. Engel	X		
Mrs. Cubin				Mr. Sawyer	X		
Mr. Shimkus		X		Mr. Wynn		X	
Mrs. Wilson		X		Mr. Green	X		
Mr. Shadegg		X		Ms. McCarthy		X	
Mr. Pickering		X		Mr. Strickland		X	
Mr. Fossella		X		Ms. DeGette		X	
Mr. Blunt				Mr. Barrett	X		
Mr. Davis		X		Mr. Luther	X		
Mr. Bryant		X		Ms. Capps	X		
Mr. Ehrlich		X		Mr. Doyle		X	
Mr. Buyer		X		Mr. John		X	
Mr. Radanovich		X		Ms. Harman			
Mr. Bass		X					
Mr. Pitts		X					
Ms. Bono		X					
Mr. Walden		X					
Mr. Terry		X					

COMMITTEE ON ENERGY AND COMMERCE -- 107TH CONGRESS
ROLL CALL VOTE # 20

BILL: H.R. 2887, the Best Pharmaceuticals for Children Act.

AMENDMENT: Amendment to the amendment in the nature of a substitute offered by Mr. Brown, #1b.

DISPOSITION: NOT AGREED TO, by a roll call vote of 13 yeas to 33 nays.

REPRESENTATIVE	YEAS	NAYS	PRESENT	REPRESENTATIVE	YEAS	NAYS	PRESENT
Mr. Tauzin		X		Mr. Dingell	X		
Mr. Bilirakis		X		Mr. Waxman	X		
Mr. Barton		X		Mr. Markey			
Mr. Upton		X		Mr. Hall		X	
Mr. Stearns		X		Mr. Boucher			
Mr. Gillmor		X		Mr. Towns		X	
Mr. Greenwood		X		Mr. Pallone	X		
Mr. Cox		X		Mr. Brown	X		
Mr. Deal				Mr. Gordon			
Mr. Largent		X		Mr. Deutsch	X		
Mr. Burr		X		Mr. Rush		X	
Mr. Whitfield		X		Ms. Eshoo		X	
Mr. Ganske				Mr. Stupak	X		
Mr. Norwood		X		Mr. Engel	X		
Mrs. Cubin				Mr. Sawyer	X		
Mr. Shimkus		X		Mr. Wynn		X	
Mrs. Wilson		X		Mr. Green			
Mr. Shadegg		X		Ms. McCarthy		X	
Mr. Pickering		X		Mr. Strickland	X		
Mr. Fossella				Ms. DeGette	X		
Mr. Blunt				Mr. Barrett	X		
Mr. Davis		X		Mr. Luther	X		
Mr. Bryant		X		Ms. Capps	X		
Mr. Ehrlich				Mr. Doyle		X	
Mr. Buyer		X		Mr. John		X	
Mr. Radanovich		X		Ms. Harman			
Mr. Bass		X					
Mr. Pitts		X					
Ms. Bono		X					
Mr. Walden		X					
Mr. Terry		X					

COMMITTEE ON ENERGY AND COMMERCE -- 107TH CONGRESS
ROLL CALL VOTE # 21

BILL: H.R. 2887, the Best Pharmaceuticals for Children Act.

AMENDMENT: Amendment to the amendment in the nature of a substitute offered by Mr. Stupak, #1c.

DISPOSITION: NOT AGREED TO, by a roll call vote of 17 yeas to 27 nays.

REPRESENTATIVE	YEAS	NAYS	PRESENT	REPRESENTATIVE	YEAS	NAYS	PRESENT
Mr. Tauzin		X		Mr. Dingell	X		
Mr. Bilirakis		X		Mr. Waxman	X		
Mr. Barton		X		Mr. Markey			
Mr. Upton		X		Mr. Hall			
Mr. Stearns				Mr. Boucher			
Mr. Gillmor		X		Mr. Towns	X		
Mr. Greenwood		X		Mr. Pallone	X		
Mr. Cox		X		Mr. Brown	X		
Mr. Deal		X		Mr. Gordon			
Mr. Largent				Mr. Deutsch	X		
Mr. Burr		X		Mr. Rush		X	
Mr. Whitfield				Ms. Eshoo		X	
Mr. Ganske				Mr. Stupak	X		
Mr. Norwood		X		Mr. Engel	X		
Mrs. Cubin		X		Mr. Sawyer	X		
Mr. Shimkus				Mr. Wynn		X	
Mrs. Wilson		X		Mr. Green	X		
Mr. Shadegg		X		Ms. McCarthy		X	
Mr. Pickering		X		Mr. Strickland	X		
Mr. Fossella		X		Ms. DeGette	X		
Mr. Blunt				Mr. Barrett	X		
Mr. Davis		X		Mr. Luther	X		
Mr. Bryant				Ms. Capps	X		
Mr. Ehrlich				Mr. Doyle	X		
Mr. Buyer		X		Mr. John	X		
Mr. Radanovich		X		Ms. Harman			
Mr. Bass		X					
Mr. Pitts		X					
Ms. Bono		X					
Mr. Walden		X					
Mr. Terry		X					

COMMITTEE ON ENERGY AND COMMERCE -- 107TH CONGRESS
ROLL CALL VOTE # 22

BILL: H.R. 2887, the Best Pharmaceuticals for Children Act.

AMENDMENT: Motion by Mr. Tauzin to order H.R. 2887 reported to the House, amended.

DISPOSITION: **AGREED TO**, by a roll call vote of 41 yeas to 6 nays.

REPRESENTATIVE	YEAS	NAYS	PRESENT	REPRESENTATIVE	YEAS	NAYS	PRESENT
Mr. Tauzin	X			Mr. Dingell		X	
Mr. Bilirakis	X			Mr. Waxman		X	
Mr. Barton	X			Mr. Markey			
Mr. Upton	X			Mr. Hall	X		
Mr. Stearns				Mr. Boucher			
Mr. Gillmor				Mr. Towns	X		
Mr. Greenwood	X			Mr. Pallone		X	
Mr. Cox	X			Mr. Brown		X	
Mr. Deal	X			Mr. Gordon			
Mr. Largent	X			Mr. Deutsch		X	
Mr. Burr	X			Mr. Rush			
Mr. Whitfield	X			Ms. Eshoo	X		
Mr. Ganske				Mr. Stupak	X		
Mr. Norwood	X			Mr. Engel	X		
Mrs. Cubin	X			Mr. Sawyer	X		
Mr. Shimkus	X			Mr. Wynn	X		
Mrs. Wilson	X			Mr. Green	X		
Mr. Shadegg	X			Ms. McCarthy	X		
Mr. Pickering	X			Mr. Strickland	X		
Mr. Fossella	X			Ms. DeGette	X		
Mr. Blunt				Mr. Barrett		X	
Mr. Davis	X			Mr. Luther	X		
Mr. Bryant	X			Ms. Capps	X		
Mr. Ehrlich	X			Mr. Doyle	X		
Mr. Buyer	X			Mr. John	X		
Mr. Radanovich				Ms. Harman			
Mr. Bass	X						
Mr. Pitts	X						
Ms. Bono	X						
Mr. Walden	X						
Mr. Terry	X						

COMMITTEE OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII of the Rules of the House of Representatives, the Committee held an oversight hearing.

STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

The objective of H.R. 2887 is to ensure that drugs used in children are properly studied and labeled for pediatric use.

NEW BUDGET AUTHORITY, ENTITLEMENT AUTHORITY, AND TAX EXPENDITURES

In compliance with clause 3(c)(2) of rule XIII of the Rules of the House of Representatives, the Committee finds that H.R. 2887, the Best Pharmaceuticals for Children Act, would result in no new or increased budget authority, entitlement authority, or tax expenditures or revenues.

COMMITTEE COST ESTIMATE

While the Committee adopts the Congressional Budget Office ("CBO") estimate submitted pursuant to section 402 of the Congressional Budget Act with regard to changes in direct spending and revenues, the Committee believes that certain aspects of CBO's estimate with respect to spending subject to appropriation is significantly overstated. For this reason, the Committee adopts its own estimate with regard to spending subject to appropriation.

Basis of Estimate

In its revised Cost Estimate issued on November 6, 2001, CBO estimates that H.R. 2887 would increase federal outlays for discretionary programs by $11 million in 2002, and by $698 million over the 2002–2006 period. Additionally, CBO estimates the bill would lower direct spending by about $7 million over the 2002–2006 period, and increase federal revenues by $6 million in 2002, and by $33 million over the 2002–2006 period.

Of the $698 million increase in federal outlays for discretionary programs over the 2002–2006 period, the CBO estimates that $660 million of this increase is needed to pay for Section 3 of H.R. 2887, Research Fund for the Study of Drugs Lacking Exclusivity. To arrive at this figure, the CBO assumes that 150 drugs lacking market exclusivity or patent protection, and certain biologics, would be studied under this new provision. CBO also assumes that 15 drugs with remaining patent protection or market exclusivity would be studied under this section after referral from the Foundation for Pediatric Research, also created by H.R. 2887. The CBO estimates that the average cost of conducting the studies requested by the Food and Drug Administration would be about $4 million per drug. Multiplying the 165 drugs and biologics identified by CBO as eligible for study by the $4 million average cost per study, the CBO estimates that this provision would result in a $660 million increase in federal outlays for discretionary programs over the 2002–2006 period.

The Committee believes that nothing in this legislation would allow for the study of biologics under the Research Fund for the Study of Drugs Lacking Exclusivity. The legislation requires the

Director of the National Institutes of Health, in consultation with the Commissioner of the Food and Drug Administration and experts in pediatric research, to "develop, prioritize, and publish an annual list of approved drugs" (emphasis added). Only the "drugs (emphasis added) identified in the list" would be eligible to be studied under this program. Clearly, the study of biologics under this new fund is not contemplated or allowed.

The Committee adopts CBO's estimate that 15 drugs referred to the Research Fund for the Study of Drugs Lacking Exclusivity by the Foundation for Pediatric Research would be studied with public funds. However, the Committee strongly disagrees with CBO that 150 drugs might qualify for study under the new fund. The American Academy of Pediatrics, perhaps the foremost authority on the subject, estimates that between 30–50 drugs will need study under the fund.

The goal of this section of the legislation is to establish a list of drugs commonly used in pediatric populations and for which information is inadequate either with respect to formulation, dose, effectiveness, or safety. In developing the list of drugs eligible for study under the new fund, the Secretary is required to take into account the following factors: (a) the availability of information concerning the safe and effective use of the drug in the pediatric population; (b) whether additional information is needed; (c) whether new pediatric studies concerning the drug may produce health benefits in the pediatric population; and (d) whether reformulation of the drug is necessary. It is not enough that the drug either be lacking market exclusivity or patent protection; rather, there must be a real need for study.

While many drugs lacking market exclusivity or patent protection are used to treat children, published studies, pediatric handbooks, and wide clinical experience provide adequate data for their use. Such drugs would therefore not qualify for study. However, in such situations where information (such as formulation development, dose, special effectiveness or safety information) is needed for the safe use of the medicine in children, then these drugs would qualify for study under the fund. In addition, careful attention should be given to utilization patterns, since older drugs are frequently replaced by newer drugs.

Further, even if 150 drugs lacking market exclusivity or patent protection do indeed qualify for study under the new fund, investigative capacity for pediatric medicine studies is limited, as are pediatric patients required for studies. There are only 13 Pediatric Pharmacology Research Units in this country, and there are limited numbers of pediatric subspecialists. To be sure, the numbers of researchers and research units are growing due to the great success of the pediatric exclusivity provision, however the capacity does not exist to conduct the numbers of studies identified by CBO.

As noted above, the American Academy of Pediatrics estimates that between 30–50 drugs would need to be studied by the new fund created by section 3. Adopting the $4 million per study estimate identified by CBO, and multiplying this by the midpoint estimate (40 drugs) identified by the American Academy of Pediatrics, $160 million would be needed to study such drugs. Adding to this the cost of studying the 15 drugs which would be referred to the new fund by the Foundation for Pediatric Research ($60 million),

$220 million of spending subject to appropriation for the 2002–2006 period is necessary, not the $660 million identified by CBO. Subtracting the $440 million difference between these two figures from the $698 million CBO estimate, $258 million of spending subject to appropriation for the 2002–2006 period would be required.

For the aforementioned reasons, the Committee believes that its estimate more accurately reflects the costs associated with the enactment of H.R. 2887 than the CBO estimate reprinted below.

CONGRESSIONAL BUDGET OFFICE ESTIMATE

Pursuant to clause 3(c)(3) of Rule XIII of the Rules of the House of Representatives, the following is the cost estimate provided by the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974:

U.S. CONGRESS,
CONGRESSIONAL BUDGET OFFICE,
Washington, DC, November 6, 2001.

Hon. W.J. "BILLY" TAUZIN,
Chairman, Committee on Energy and Commerce,
House of Representatives, Washington, DC.

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed revised cost estimate for H.R. 2887, the Best Pharmaceuticals for Children Act. On November 2, 2001, CBO prepared an estimate for H.R. 2887 that did not treat spending of revenue collected by the Foundation for Pediatric Research as direct spending. This estimate corrects that error.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contact is Julia Christensen.

Sincerely,

STEVEN LIEBERMAN
(For Dan L. Crippen, Director).

Enclosure.

Summary: H.R. 2887 would extend expiring pediatric exclusivity provisions of the Food and Drug Administration (FDA) Modernization Act of 1997. Pediatric exclusivity refers to a six-month period during which the FDA will not permit another manufacturer to market a generic version of a drug. Such exclusivity is granted in exchange for the manufacturer conducting studies, requested by the FDA, of the effect of drugs when taken by children.

The bill would create a new research fund within the National Institutes of Health (NIH) to test the use for children of drugs that lack patent or other market exclusivity protections. It would also create a non-profit foundation to collect funds and awards grants for research on pediatric uses of qualifying drugs. The bill would modify the review and labeling processes associated with pediatric supplements and would promote the reporting and collecting of information on adverse reactions to drugs.

H.R. 2887 would clarify the interaction of market exclusivity awarded to certain generic manufacturers and pediatric exclusivity awarded to innovator drug companies when the two periods of market exclusivity overlap. It would also amend the approval process for generic drugs when pediatric information is added to the labeling. In addition, the bill would establish an Office of Pediatric

Therapeutics within the FDA and would authorize several studies related to the pediatric exclusivity program and pediatric research.

Assuming the appropriation of the necessary funds, CBO estimates that H.R. 2887 would increase federal outlays for discretionary programs by $11 million in 2002 and by $698 million over the 2002–2006 period. Those costs consist of amounts required to implement and administer the activities authorized under the bill and the effect of H.R. 2887 on the costs of certain discretionary programs that purchase drugs or contribute toward the pharmacy costs of beneficiaries.

The bill would result in higher prices for certain drugs that would be granted an extended period of market exclusivity, but would also accelerate the entry of generic versions of some drugs, which would lead to lower prices. CBO estimates that the net effect of the bill would be to reduce the average price of prescription drugs slightly through 2007 and to increase prices in subsequent years.

In the near term, lower drug prices would reduce the costs of federal programs that purchase prescription drugs or provide health insurance that covers prescription drugs. CBO estimates that savings to programs subject to appropriation—such as health insurance provided to active workers through the Federal Employees Health Benefits (FEHB) program, the Coast Guard, the Public Health Service (PHS), and health programs of the Departments of Veterans Affairs (VA) and Defense (DoD)—would total $3 million in 2002 and $33 million over the 2002–2006 period.

Lower prices would also reduce direct spending—for Medicaid and for health insurance provided to annuitants by FEHB, DoD, and the Coast Guard—by $2 million in 2002 and by $32 million over the 2002–2006 period. However, H.R. 2887 would increase federal direct spending on those programs by $160 million over the 2002–2011 period, reflecting higher average drug prices, on balance, in later years.

Grants made by the newly created foundation would be direct spending, because they would not be subject to the availability of appropriations. CBO expects expenditures by the foundation for grants would begin in 2003; therefore, there would be no direct spending in 2002. CBO estimates that awards made by the foundation would increase direct spending by $25 million over the 2002–2006 period and by $59 million over the 2002–2011 period.

The bill would also affect revenues in two ways. First, donations and gifts received by the foundation would increase federal revenues. Secondly, CBO assumes that part of the savings or costs from changes in health insurance costs would be passed on to workers as increases or decreases, respectively, in taxable compensation. Lower prices for prescription drugs under the bill would initially reduce premiums for private health insurance (compared with premiums under current law). Higher drug prices would subsequently push premiums higher. CBO estimates the bill would increase federal revenues by $6 million in 2002, by $33 million over the 2002–2006 period, and by $15 million over the 2002–2011 period. Because enacting H.R. 2887 would affect direct spending and revenues, pay-as-you-go procedures would apply.

H.R. 2887 contains no intergovernmental mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local and trib-

al governments, as administrators of the Medicaid program and as providers of health care coverage for their employees, may realize both costs and savings as a result of provisions in the bill. Provisions affecting market and pediatric exclusivity would result in added costs, and requirements for prompt approval of some generic drugs would result in savings.

The bill would impose several requirements on pharmacists and on manufacturers of both generic and brand-name drugs that would be considered private-sector mandates under UMRA. CBO estimates that the direct cost of the mandates would not exceed the threshold specified in UMRA ($113 million 2001, adjusted annually for inflation) in any of the first five years during which the mandates would be effective.

Estimated cost to the Federal Government: The estimated budgetary impact of H.R. 2887 is shown in Table 1. The costs of this legislation would fall within budget functions 050 (national defense), 550 (health), and 700 (veterans' benefits and services.)

Basis of estimate: For this estimate, CBO assumes that the bill will be enacted in the fall of 2001 and that outlays will follow historical spending rates for the authorized activities. Where H.R. 2887 specifies the amounts authorized to be appropriated, CBO assumes that such appropriations will be made. Where appropriations of such sums as necessary are authorized, CBO assumes that the estimated amounts will be provided for each fiscal year.

Spending subject to appropriations

Assuming appropriation of the necessary amounts, CBO estimates that enacting H.R. 2887 would result in higher outlays for discretionary federal programs of $11 million in 2002 and $698 million over the 2002–2006 period. The NIH and the FDA are the agencies responsible for carrying out most of the provisions in H.R. 2887. CBO estimates that implementing the bill would cost FDA $11 million in 2002 and $154 million over the 2002–2006 period (net of collections of user fees), assuming the appropriation of the necessary amounts. Costs to NIH would increase by $1 million in 2002 and by $571 million over the 2002–2006 period. Table 2 shows the estimated authorization levels and outlays under H.R. 2887 for fiscal years 2002 through 2006.

TABLE 1.—ESTIMATED BUDGETARY IMPACT OF H.R. 2887

	By fiscal year, in millions of dollars—					
	2001	2002	2003	2004	2005	2006
SPENDING SUBJECT TO APPROPRIATION						
Spending Under Current Law:						
Estimated Budget Authority [1]	21,482	22,024	22,504	22,987	23,468	23,974
Estimated Outlays	18,341	20,322	21,537	22,362	23,093	23,152
Proposed Changes:						
Estimated Authorization Level [2]	0	217	94	246	161	109
Estimated Outlays	0	11	94	216	213	165
Spending Under H.R. 2887:						
Estimated Authorization Level	21,482	22,241	22,598	23,233	23,629	24,083
Estimated Outlays	18,341	20,333	21,631	22,578	23,306	23,317
CHANGES IN DIRECT SPENDING						
Estimated Revenues	0	4	3	0	−2	−6
Estimated Outlays	0	−2	1	2	−1	−6

TABLE 1.—ESTIMATED BUDGETARY IMPACT OF H.R. 2887—Continued

	By fiscal year, in millions of dollars—					
	2001	2002	2003	2004	2005	2006
CHANGES IN REVENUES						
Estimated Revenues	0	6	6	6	6	9

[1] The 2001 level is the amount appropriated for that year for the National Institutes of Health (NIH) and the Food and Drug Administration (FDA). The NIH and the FDA are the agencies responsible for implementing and administering the activities authorized in the bill. Current-law amounts for those programs during the 2002–2006 period assume appropriations remain at 2001 levels, with adjustments for inflation.

[2] The estimated amounts reflect the costs to the NIH and the FDA for implementing and administering activities authorized under H.R. 2887 and the effects of the bill on pharmacy costs of other federal discretionary programs.

Research Fund for the Study of Drugs Lacking Exclusivity. Section 3 of H.R. 2887 would create a research fund to pay for pediatric studies of certain drugs lacking market exclusivity. Market exclusivity refers to the exclusive rights conveyed to manufacturers on their drugs. Those rights may stem either from patent protection or through the marketing approval process governed by the FDA.

Under certain circumstances, if manufacturers fail to pursue pediatric testing requested in writing by the FDA, the fund could award contracts to pay for studies on drugs with market exclusivity remaining. The fund would be administered by the NIH.

H.R. 2887 would authorize the appropriation of $200 million for the fund in 2002, and such sums as necessary each year until 2007. CBO estimates that the combined outlays for FDA and NIH activities to set up the fund, make awards from the fund, and process the pediatric supplements under new program requirements would be about $1 million in 2002 and $639 million during the 2002–2006 period, assuming appropriation of the necessary funds. (Pediatric supplements are the applications filed by manufacturers to amend the information provided to the FDA for its use in approving the use of the product by children.)

TABLE 2.—ESTIMATED AUTHORIZATIONS AND OUTLAYS UNDER H.R. 2887

	By fiscal year, in millions of dollars—					
	2001	2002	2003	2004	2005	2006
SPENDING SUBJECT TO APPROPRIATION						
National Institutes of Health (NIH):						
Estimated Authorization Level	0	200	43	204	133	91
Estimated Outlays	0	1	73	182	178	137
Food and Drug Administration (FDA):						
Estimated Authorization Level	0	16	53	48	35	30
Estimated Outlays	0	11	23	38	42	39
Other Programs:						
Veterans' Administration (VA) Health Program:						
Estimated Authorization Level	0	−1	−1	−3	−4	−6
Estimated Outlays	0	−1	−1	−3	−4	−6
Department of Defense (DoD) Health Program:						
Estimated Authorization Level	0	−1	−1	−2	−3	−4
Estimated Outlays	0	−1	−1	−2	−3	−4
Federal Employees Health Benefits (FEHB) Program:						
Estimated Authorization Level	0	(1)	(1)	−1	−1	−1
Estimated Outlays	0	(1)	(1)	−1	−1	−1
Public Health Service and Other Programs, Excluding NIH and FDA:						
Estimated Authorization Level	0	3	(1)	(1)	(1)	−1
Estimated Outlays	0	1	1	(1)	1	(1)
Total Changes:						
Estimated Authorization Level	0	217	94	246	161	109

TABLE 2.—ESTIMATED AUTHORIZATIONS AND OUTLAYS UNDER H.R. 2887—Continued

	By fiscal year, in millions of dollars—					
	2001	2002	2003	2004	2005	2006
Estimated Outlays	0	11	94	216	213	165

[1] Less than $500,000 in costs or savings.

Under the bill, the NIH, in consultation with the FDA, would establish a priority list of drugs without market exclusively that warrant additional testing for children. Certain drugs with market exclusivity could also be referred to that list by the Secretary of Health and Human Services (HHS) for study financed by the fund. Except in those special referral cases, the bill would set up a contracting process that allows the holders of the approved application for the drug the right of first refusal to receive payment from the fund to conduct the requested studies. If no response is received to FDA's request within 30 days, a competitive contracting process outlined by the bill would be set in motion. H.R. 2887 would specify the reporting procedures for data resulting from the studies and the process for incorporating any necessary new information on drug labels.

CBO expects that roughly 150 non-referral drugs ultimately might qualify for study financed by the new fund. That estimate is based on data showing that 170 drugs on the FDA's May 2000 List of Approved Drugs for Which Additional Pediatric Information May Produce Health Benefits in the Pediatric Population currently lack patent or other market exclusivity protections. Additional candidates for study under the fund would include drugs coming off patent or otherwise losing market exclusivity in the next few years. Moreover, one interpretation of the provision may allow a broader group of biologicals to qualify for study financed by the fund. CBO assumes that given the rapid advancement in therapies, some products potentially qualifying for study ultimately would not be studied. CBO also estimates that up to 15 drugs that retain market exclusivity protections would likely be studied over the 2002–2006 period because of referral by the Secretary of HHS. CBO estimates that the average cost of conducting the studies requested by the FDA would be about $4 million per drug. In total, CBO estimates that about $660 million in contracts to study drugs would be awarded from the fund over the 2002–2006 period.

Changes to Written Request and Response Procedure for Drugs that Have Market Exclusivity. Section 4 of H.R. 2887 would change the written request procedure for drugs that have market exclusivity by requiring a response by the manufacturer to the FDA request within 180 days of receiving the request. If the Secretary of HHS determined there is a continuing need for information on a drug for which the manufacturer did not agree to conduct the requested studies, the Secretary would have to refer the drug to the newly created Foundation for Pediatric Research for consideration. If the foundation certified to the Secretary of HHS that insufficient funds are available to conduct the requested studies, the Secretary would be required to refer the drug for inclusion on the priority list associated with the fund established under section 3. CBO expects that the FDA would process fewer than 10 pediatric supplements

over the 2002–2006 period as a result of referred studies funded by foundation grants.

CBO estimates that referral and coordination activities plus costs associated with processing supplements associated with foundation-sponsored studies would increase the administrative costs of the FDA and NIH by less than $500,000 in 2002 and by $4 million during the 2002–2006 period.

Modifications to the Existing Pediatric Exclusivity Program. It is unclear how the sunset provisions of the pediatric exclusivity program authorized under the FDA Modernization Act of 1997 will apply after January 1, 2002. For the purposes of this estimate, CBO assumes that the authority to grant pediatric exclusivity to certain targeted drugs will continue under current law. For any drug (active moiety) for which both a new drug application is submitted and a written request received by January 1, 2002, CBO assumes that FDA will have the authority under current law to grant pediatric exclusivity if the standard requirements set forth by the existing program are met.

Furthermore, CBO assumes that FDA will retain authority under current law to issue written requests and grant pediatric market exclusivity beyond January 1, 2002, to certain drugs if FDA perceives a continuing need for information relating to the drug. To qualify, the drug must meet the following criteria:

- The drug must have been in commercial distribution as of November 21, 1997;
- The drug must appear on the FDA's January 1, 2002, "List"; and
- The drug must meet the standard requirements set forth by the program.

Section 5 of the bill would affect the review and labeling processes associated with pediatric supplements. Such modifications include eliminating the waiver of user fees for pediatric supplements, identifying all pediatric supplements as priority supplements, and defining a process for timely pediatric labeling changes. Taken together, these provisions would increase FDA's costs for administering the existing program and processing supplements anticipated under current law. CBO estimates that fulfilling these new requirements for current law supplements would increase FDA's costs, on net, by $2 million in 2002 and by $34 million during the 2002–2006 period, assuming appropriation of the necessary funds.

CBO's estimate reflects collections from user fees only in fiscal year 2002 because the authority to collect fees under the Prescription Drug User Fee Act (PDUFA) of 1992, as amended by the FDA Modernization Act of 1997, will expire at the end of fiscal year 2002. CBO also assumes that manufacturers submitting supplements for studies conducted under both the new research fund and the foundation would not be required to pay any user fees because the supplements would refer to that clinical data "by reference."

Office of Pediatric Therapeutics. H.R. 2887 would establish an Office of Pediatric Therapeutics within the FDA. The office would be responsible for oversight and coordination of FDA's activities involving pediatric issues. CBO estimates that the office would consist of five full-time employees. We estimate that the new office would cost less than $500,000 in 2002 and $2 million over the

2002–2006 period, assuming appropriation of the necessary amounts.

Reauthorization of the Pediatric Exclusivity Program. The bill would grant an additional six months of market exclusivity to pharmaceutical manufacturers that conduct pediatric studies on certain drugs. In total, CBO estimates that the reauthorized program would cost $6 million in 2002 and $63 million over the 2002–2006 period, subject to the appropriation of the necessary funds. (This reauthorization would also cause an increase of $28 million in direct spending over the 2002–2006 period. That effect is discussed later.)

The reauthorized program would grant a six-month extension for a drug provided that: (1) FDA has issued a written request for pediatric studies on the drug on or before October 1, 2007; (2) an approvable new drug application for the drug has been submitted on or before October 1, 2007; and (3) the requirements of the program have been met. The benefit under reauthorization generally would accrue to approved drugs introduced since November 22, 1997, that have not yet received a written request from the FDA for pediatric studies, and to new drugs pending marketing approval.

CBO expects that manufacturers would conduct pediatric trials and receive pediatric exclusivity on upwards of 100 drugs under the reauthorized program. Assuming appropriation of the necessary funds, CBO estimates that FDA's cost to administer the reauthorized program under the new requirements outlined in section 5 of the bill would be $5 million in 2002 and $34 million over the 2002–2006 period.

Extending market exclusivity under the reauthorized program would increase costs for discretionary federal programs by less than $500,000 in 2002 and $29 million over the 2002–2006 period, assuming appropriation of the necessary funds. Programs of the PHS and the VA would be affected, as would pharmacy costs incurred by FEHB, DoD, and the Coast Guard for active workers.

To estimate the costs associated with higher drug prices paid by federal purchasers, CBO identified a set of about 30 approved drugs that would qualify for pediatric exclusivity under the reauthorized program. Using 2000 sales data and the date of market approval for those products, CBO projected sales for each drug based on an average drug sales curve calculated by FDA for its January 2001 Status Report to the Congress on the Pediatric Exclusivity Provision. CBO identified sales in the year of anticipated expiration of market exclusivity and estimated the reduction in pharmaceutical costs to federal programs that would accrue to government purchasers at generic entry under current law. The amount of such savings lost to the federal government would be the cost of extending pediatric exclusivity to each drug. CBO's methodology incorporated recent market trends that suggest a more rapid loss of market share to generics in the first months after generic entry than previously estimated by the CBO. Pending further study of these market dynamics, CBO assumes that generic products, on average, account for roughly 30 percent of total market volume and cost about 70 percent of the brand price after three months on the market. After six months, CBO assumes that generic drugs would account for roughly 40 percent of total market volume and cost about 60 percent of the brand price.

To estimate the cost of new drugs obtaining pediatric extensions under the reauthorized program, CBO assumed that 30 new drugs would be introduced each year and one-half of them would qualify for pediatric exclusivity. CBO estimated the average first full-year sales by inflating FDA's estimate of $125 million per drug in 1999. (CBO assumed an average annual rate of increase in launch price of about 10 percent since 1999.) Using data from several industry sources. CBO assumed that roughly one out of five new drugs getting pediatric exclusivity extensions under the reauthorized program would lose market exclusivity between 2002 and 2011. After identifying sales in the year of anticipated expiration of market exclusivity protections, CBO estimated the cost associated with new drugs receiving an additional six months of exclusivity in the same manner as outlined above for existing drugs.

Dissemination of Pediatric Information. H.R. 2887 would require the FDA to make available to the public a summary of the medical and clinical pharmacological reviews of pediatric studies conducted under the program. CBO estimates that this provision would cost the FDA an additional $1 million in 2002 and $7 million during the 2002–2006 period.

Clarification of the Interaction between Certain Market Exclusivity Periods. H.R. 2887 would clarify Congressional intent regarding the interaction between 180-day generic exclusivity and pediatric exclusivity when the two periods of market exclusivity overlap. CBO estimates that this provision would increase the costs of certain federal discretionary programs by $1 million in 2002 and by $5 million over the 2002–2006 period. CBO estimates that the FDA would need to spend less than $500,000 over the 2002–2006 period to implement the provision.

Under certain conditions, the first generic manufacturer that files a substantially complete abbreviated new drug application (ANDA) challenging an innovator's patent claim under a "paragraph IV" filing may be awarded 180 days of generic market exclusivity. During the 180-day generic exclusivity period, the FDA cannot approve a subsequently filed ANDA for a generic version of that specific drug product. This provision of law may provide the first generic "paragraph IV" filer an opportunity to recoup some of the risk litigation costs by providing that manufacturer with market exclusivity for its version during the first 180 days of generic marketing.

The 180-day generic exclusivity period begins after a court decision finding the challenged patent invalid, unenforceable, or not infringed, or the date of first commercial marking of the ANDA product, whichever is earlier. In the event that the 180-day generic exclusivity period overlaps with the pediatric exclusivity period, the bill would specify the amount of time that is restored to the generic manufacturer's 180-day exclusivity period.

Under the bill, if the 180-day generic exclusivity period expires at some point after the pediatric exclusivity period, the 180-day period would be extended by the number of days of the overlap. Alternatively, if the 180-day generic period expires during the pediatric exclusivity period, the 180-day generic exclusivity would be extended by six months. CBO assumes that any portion of overlap between the 180-day generic exclusivity and a valid patent that re-

mains in force would not be restored to the generic manufacturer under the bill.

Restoring a portion of the effective 180-day generic exclusivity would allow the first generic "paragraph IV" filer to charge higher prices during that period because of the lack of pricing competition from other generic companies. CBO assumes that the generic manufacturer enjoying market exclusivity would charge, on average, 10 percent more for the generic version during the effective period of market exclusivity. As a result, the costs to public and private purchasers of drugs would be slightly higher during the restored period because of this provision.

However, CBO assumes that a significant overlap in the periods of market exclusivity would occur relatively infrequently. The most likely scenario would occur when a first generic "paragraph IV" challenger wins a court case on one patent—and that patent is declared invalid, unenforceable, or not infringed—while at least one other patent on the drug product remains in force after the decision. To date, only one similar situation has been identified surrounding a drug patent case argued before the courts in 2000.

CBO anticipates that the recent case may be an indicator of the potential for overlaps of 180-day generic and pediatric periods of market exclusivity in the future. We assumed that there was a 50 percent probability that the same percent of sales for brand drugs losing market exclusivity in future years (as seen in 2001 associated with the recent case) may be subject to an overlap scenario. CBO further assumed that an average of three effective months of the 180-day generic exclusivity for the generic "paragraph IV" challenger would be restored under the provision. (Under the bill, CBO assumes that there would be no guarantee in any particular case that a generic manufacturer would be able to commercially market with effective market exclusivity if overlap remains between pediatric exclusivity and existing patent or other market exclusivity protection.) For this estimate, CBO assumed generics generally would gain about 30 percent of market share after three months and be priced at roughly 70 percent of the brand version.

Amendments to the Generic Drug Approval Process. H.R. 2887 would amend the approval process for generic drugs when pediatric information is added to the labeling. The bill would require prompt approval of a generic drug that otherwise meets all other applicable requirements even when its labeling omits pediatric information that is protected by patent or other market exclusivity protections. The bill would allow the Secretary of HHS to require certain statements and warnings on the affected generic labels. That provision would take effect immediately upon enactment with respect to all new applications and to those that are approved or pending. CBO estimates that implementing these provisions would cost the FDA less than $500,000 over the 2002–2006 period.

In directing the FDA to approve generic applications lacking pediatric labeling under certain circumstances, these provisions would accelerate entry of lower-cost generic products onto the market. Under current law, CBO assumes an average delay of three years for the generic products that might face a moratorium on their marketing approval because of pediatric labeling exclusivity. To estimate the savings associated with this provision, CBO assumed that at the end of the three years, generics would constitute

roughly 70 percent of market volume and cost about 50 percent of the brand product's price. CBO estimates that eliminating the delay in the entry of lower-priced generics would result in savings to federal discretionary health programs of about $4 million in 2002 and $67 million over 2002–2006 period.

Adverse Event Reporting. H.R. 2887 would require manufacturers to label all drugs with the toll-free number maintained by HHS for the reporting of adverse drug events. In addition, the bill would require that all manufacturers receiving pediatric exclusivity report any adverse event to the FDA during the one-year period following the granting of such exclusivity. Those reports would have to be reviewed by the Office of Pediatric Therapeutics and reported to the Pediatric Advisory Subcommittee of the Anti-infective Drugs Advisory Committee. CBO estimates that implementing this provision would cost the FDA less than $500,000 in 2002 and $1 million over the 2002–2006 period.

Foundation for Pediatric Research. The bill would create a nonprofit corporation called the "Foundation for Pediatric Research" to collect funds and award grants for pediatric research on drugs that are on the priority list established under section 3. It would require that all reporting, labeling, and other requirements specified under section 3 be applicable to drugs studied with foundation grants. The bill would authorize the appropriation of such sums as necessary for 2002 and subsequent years to carry out the activities associated with the foundation.

CBO expects that donations and gifts collected by the foundation would be considered revenues to the federal government. Grants made by the foundation would be direct spending, because they would not be subject to the availability of appropriations. We expect that, on average, the foundation would collect amounts sufficient to sponsor the study of one to two drugs annually.

The bill also would direct the NIH to provide support services to the foundation. H.R. 2887 would require annual reports on the activities of the foundation and would allow the foundation to assess fees for the provision of specific types of services in amounts determined reasonable. CBO estimates that establishing and administering the foundation would cost almost $1 million in 2002 and $3 million over the 2002–2006 period, assuming appropriation of the necessary funds. NIH's costs associated with the foundation would be less than $500,000 in 2002 and $1 million over the 2002–2006 period.

Studies on Pediatric Exclusivity Program and Pediatric Research. H.R. 2887 would require the Secretary of HHS to contract with the Institute of Medicine to conduct a study on federal regulations and issues surrounding pediatric research. CBO estimates the cost of implementing this provision would total about $1.5 million from 2002 through 2003. In addition, the bill would require the General Accounting Office to conduct two studies—one evaluating the effectiveness and economic impact of amendments to the pediatric exclusivity program made by H.R. 2887 and one evaluating the representation of ethnic and racial minorities in pediatric studies under the program. CBO estimates that those studies would cost almost $1 million in 2002 and $3 million over the 2002–2006 period.

Effect on direct spending: H.R. 2887 would increase federal direct spending over the 2002–2011 period by $219 million, CBO estimates, but direct spending would be lower in 2002 (by about $2 million) and over the 2002–2006 period (by about $7 million). The three provisions of the bill that would affect the price of drugs for discretionary health programs discussed earlier would also affect direct spending by federal health programs characterized as mandatory (that is, not requiring appropriation action). Reauthorizing the pediatric exclusivity program would increase direct spending (for Medicaid and for annuitants covered by health insurance offered through FEHB, DoD, and the Coast Guard) by less than $500,000 in 2002, $28 million over the 2002–2006 period, and $320 million over the 2002–2011 period. Clarifying the interaction between the 180-day generic market exclusivity and pediatric exclusivity periods when they overlap would increase federal direct spending for health programs by about $1 million in 2002, $5 million over the 2002–2006 period, and $10 million over the 2002–2011 period. However, CBO estimates that significant savings would be generated by requiring prompt approval of generic applications under certain circumstances. That provision would save those federal health programs about $4 million in 2002, $65 million over the 2002–2006 period, and about $170 million over the 2002–2011 period.

Grants made by the newly created Foundation for Pediatric Research would be direct spending, because they would not be subject to the availability of appropriations. CBO expects that expenditures by the foundation for grants would begin in 2003; therefore, there would be no direct spending in 2002. CBO estimates that awards made by the foundation would increase direct spending by $25 million over the 2002–2006 period and by $59 million over the 2002–2011 period.

Effect on revenue: CBO estimates that H.R. 2887 would increase federal revenues by $6 million in 2002, by $33 million over the 2002–2006 period, and by $15 million over the 2002–2011 period.

The bill would affect federal revenues in two ways. First, donations and gifts collected by the foundation, averaging an estimated $6 million to $7 million a year, would be considered revenues to the federal government.

Secondly, CBO assumes that changes in drug prices would affect the costs of private health insurance premiums, and a portion of those amounts would be returned to workers through changes in taxable compensation. H.R. 2887 would increase costs for employer-sponsored health plans because of the changes in the costs of pharmacy benefits resulting from the extension of pediatric exclusivity to some drugs and from clarifying the interaction of any overlap between 180-day generic market exclusivity and pediatric exclusivity. However, the savings generated by promoting prompt approval of generics would lead to overall lower costs in certain years, mostly during the earlier part of the 2002–2011 period. After 2007, however, pharmacy costs, on net, would be higher as a result of H.R. 2887. Higher net pharmacy costs translate into higher premium payments for employer-sponsored plans during those years, and thus lower taxable compensation for employees.

CBO assumes that 60 percent of the change in the cost of health premiums would be offset by changes in profits and by behavioral

responses of employers and employees. The remaining 40 percent would be passed through to workers as changes in taxable compensation and would lead to changes in federal tax revenues.

From 2002 through 2007, federal tax revenues would increase slightly under the bill. However, CBO estimates that federal tax revenues would begin to fall starting in 2009 when the effect of declining revenues from lower taxable income overwhelms the effect of higher revenues from incoming donations and gifts to the foundation.

Pay-as-you-go considerations: The Balanced Budget and Emergency Deficit Control Act sets up pay-as-you-go procedures for legislation affecting direct spending and receipts. The following table displays CBO's estimate of the effects of H.R. 2887 on direct spending and receipts. For the purposes of enforcing pay-as-you-go procedures, only the effects in the budget year and the succeeding four years are counted.

	By fiscal year, in millions of dollars—									
	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011
Change in Outlays	−2	1	2	−1	−6	−3	20	49	70	89
Change in Revenues	6	6	6	6	9	7	3	−3	−9	−16

Estimated impact on state, local, and tribal governments: H.R. 2887 contains no intergovernmental mandates as defined in UMRA. Because the bill would delay the entry into the marketplace of some generic drugs, CBO estimates that costs would increase for the Medicaid programs and for health care for state, local, and tribal employees. However, the bill also would require prompt approval of generics in certain cases. Those provisions would result in savings for the same programs. CBO estimates that state spending for Medicaid would decrease by a net of about $18 million over the 2002–2006 period, but that over the 2002–2011 period, states would incur net costs for Medicaid of about $95 million. CBO has not completed estimates of the effect of the provisions on health care programs offered to employees of state, local, and tribal governments. However, those programs would similarly realize net savings over the 2002–2006 period and incur net costs over the 2002–2011 period.

Estimated impact on the private sector: The bill contains a number of private-sector mandates on manufacturers of both generic and brand-name drugs and on pharmacists. First, it would prohibit generic drug manufacturers, under certain conditions, from producing generic versions of drugs for a period of six months. Based on expected patent expirations and current rates of new drug development, CBO estimates that the number of drugs receiving new pediatric exclusivity under the provision would be relatively small in any of the first five years the mandate would be effective. The forgone profits from sales of generic drugs over the six-month period also would be small in each of those years.

Second, the bill would remove a provision enacted under PDUFA that waives user fees for all applications for pediatric supplements, thereby imposing a new private-sector mandate on sponsors of those applications. PDUFA will expire at the end of fiscal year 2002, so the mandate would have no effect after that date. CBO es-

timates that total costs in fiscal year 2002 for all such supplements would be less than $10 million.

Third, brand-name drug companies that receive pediatric exclusivity would effectively be required to comply with any changes in labeling requested by the Food and Drug Administration. Failure to comply could cause the drug to be deemed as mislabeled and removed from the market. The cost of this requirement to affected companies would be minimal.

Finally, the bill would require all drug manufacturers to include on all labels the toll-free telephone number maintained by HHS for reporting adverse drug events. That requirement would necessitate a one-time change in labels and could also require pharmacists to include the phone number with all prescriptions. Those required changes constitute private-sector mandates, but the added costs would be small.

CBO estimates that the direct cost of the mandates contained in the bill—on both generic and brand-name drug manufacturers—would not exceed the annual threshold specified in UMRA ($113 million in 2001, adjusted annually for inflation) in any of the first five years the mandates would be effective.

Comparison with previous estimates: On November 2, 2001, CBO prepared an estimate for H.R. 2887 that did not treat spending of revenue collected by the Foundation for Pediatric Research as direct spending. This estimate corrects that error.

Estimate prepared by: Federal Costs: Julia Christensen. Impact on State, Local, and Tribal Governments: Leo Lex. Impact on the Private Sector: Judith Wagner.

Estimate approved by: Robert A. Sunshine, Assistant Director for Budget Analysis.

FEDERAL MANDATES STATEMENT

The Committee adopts as its own the estimate of Federal mandates prepared by the Director of the Congressional Budget Office pursuant to section 423 of the Unfunded Mandates Reform Act.

ADVISORY COMMITTEE STATEMENT

No advisory committees within the meaning of section 5(b) of the Federal Advisory Committee Act were created by this legislation.

CONSTITUTIONAL AUTHORITY STATEMENT

Pursuant to clause 3(d)(1) of rule XIII of the Rules of the House of Representatives, the Committee finds that the Constitutional authority for this legislation is provided in Article I, section 8, clause 3, which grants Congress the power to regulate commerce with foreign nations, among the several States, and with the Indian tribes.

APPLICABILITY TO LEGISLATIVE BRANCH

The Committee finds that the legislation does not relate to the terms and conditions of employment or access to public services or accommodations within the meaning of section 102(b)(3) of the Congressional Accountability Act.

SECTION-BY-SECTION ANALYSIS OF THE LEGISLATION

Section 1. Short title

Section 1 provides the short title of the legislation, the Best Pharmaceuticals for Children Act.

Section 2. Pediatric studies of already-marketed drugs

In its January 2001 Status Report to Congress, the FDA noted that it "believes that there is no longer a benefit in maintaining a prioritized list of drugs, as currently required under section 505A(b)." This section follows this recommendation by eliminating the requirement for the pediatric list.

Section 3. Research fund for the study of drugs lacking exclusivity

As noted previously, while the pediatric exclusivity program has been highly successful in ensuring that drugs with patent life or market exclusivity remaining are studied, it has provided no incentive for the study of drugs which are already subject to generic competition, and for certain on-patent drugs.

This section addresses this gap in the statute through the creation of a "Program for Pediatric Studies of Drugs Lacking Exclusivity" within the Public Health Service. Under this program, not later than one year after the date of enactment, the Director of the National Institutes of Health, in consultation with the Commissioner of Food and Drugs and experts in pediatric research will develop and prioritize a list of drugs with no patent protection or market exclusivity remaining for which additional studies are needed to assess the safety and effectiveness of the use of the drug in pediatric populations. Also, certain on-patent drugs, or drugs with remaining exclusivity, which are not studied by the Foundation for Pediatric Research will be referred to this public program for inclusion on the list.

Under this program, for drugs on the list with no remaining patent life or market exclusivity, the Secretary shall first issue "written requests" to all holders of approved applications. These written requests will detail the studies which need to be conducted in order to develop information about use of the drug in pediatric populations. Should the holders of approved applications decide not to study these drugs, the Secretary will then publish a request for contract proposals to conduct the pediatric studies detailed in the written requests. Entities such as universities, teaching hospitals, hospitals, laboratories, contract research organizations, Pediatric Pharmacology Research Units, among others, will be eligible to bid for these contracts. In contracting for the study of drugs on the priority list, the Secretary shall ensure that there is no duplication of effort or other wasting of public funds. Further, drugs referred to the public program by the Foundation for Pediatric Research will be eligible for study under this public program.

Once the studies are completed, a report will be provided to the Director of the NIH and the Commissioner of the FDA, and such reports shall be considered to be in the public domain. The Commissioner of the FDA shall then consider the reports, and negotiate with all application holders for labeling changes deemed necessary. Should the holders of the applications not agree to the labeling changes proposed, then the Commissioner will refer his rec-

ommendation to the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee, which will review this recommendation and forward back to the Commissioner its recommendation as to appropriate labeling changes, if any. Upon reviewing the recommendation, the Commissioner shall forward to application holders his final request for labeling changes. The application holders will then have 30 days to make all requested changes, and should they decide not to make the changes, the Commissioner will have the authority to deem the drug misbranded. Nothing in this section authorizes any use or disclosure of confidential commercial information or trade secrets.

This public program is authorized at $200,000,000 in FY 2002, and such sums as may be necessary through FY 2007.

Section 4. Written request to holders of approved applications for drugs that have market exclusivity

If a written request for studies is issued to the holder of an application with remaining patent life or market exclusivity, the holder will have 180 days to decide whether or not to study the drugs pursuant to the written request. Should the sponsor decide not to study the drug under the pediatric exclusivity program, the drug may then be referred to the Foundation for Pediatric Research for study. Should the Foundation for Pediatric Research certify that it does not have funds available to conduct the requested studies, the drug will then be referred to the Program for Pediatric Studies of Drugs Lacking Exclusivity for prioritization and study.

Section 5. Timely labeling changes for drugs granted exclusivity; drug fees

First, this section eliminates the automatic user fee waiver for pediatric supplements, and ensures that pediatric supplements proposing labeling changes shall be considered priority supplements, subject to the performance goals established for priority drugs.

If the Commissioner believes that a pediatric supplement which has been submitted subsequent to studies being conducted under section 505A is approvable, but the only open issue for final action is reaching agreement on labeling changes, the Commissioner shall negotiate labeling changes with the application holder. Should the holder of the application not agree to the labeling changes proposed, then the Commissioner will refer his recommendation to the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee, which will review this recommendation and forward back to the Commissioner its recommendation as to appropriate labeling changes, if any. Upon reviewing the recommendation, the Commissioner shall forward to the application holder his final request for labeling changes. The application holder will then have 30 days to make all requested changes, and should the holder decide not to make the changes, the Commissioner will have the authority to deem the drug misbranded. It is important to note that nothing in this section authorizes any use or disclosure of confidential commercial information or trade secrets.

Section 6. Office of Pediatric Therapeutics

Section 6 creates an Office of Pediatric Therapeutics within the Commissioner's Office at the FDA. Such Office will be responsible

for oversight and coordination of all activities of the FDA that may have any effect on a pediatric population or the practice of pediatrics or involve pediatric issues.

Section 7. Neonates

The FDA noted in its Status Report to Congress the need to ensure that the neonatal pediatric population be studied. Though other provisions in H.R. 2887 will help to ensure that this population is studied, this provision reaffirms the importance of studying the neonatal population by inserting the word "neonates" in the "Definitions" paragraph in section 505A.

Section 8. Sunset

This section specifies that this reauthorization expires at the end of FY 2007.

Section 9. Dissemination of pediatric information

For studies conducted pursuant to section 505A, the Commissioner shall make available in the public a summary of the medical and clinical pharmacology reviews of pediatric studies conducted.

Section 10. Clarification of interaction of market exclusivity under section 505a of the Federal Food, Drug, and Cosmetic Act and market exclusivity awarded to an applicant for approval of a drug under section 505(j) of that act

This section ensures that any generic manufacturer which is entitled to the 180 day generic exclusivity under section 505(j)(5)(B)(iv) for successfully challenging a patent will not see that period of exclusivity nullified or abrogated by the six month grant of exclusivity pursuant to section 505A.

This section is necessary because earlier this year litigation resulted in a situation wherein the generic and pediatric exclusivities could have overlapped. This section ensures what many have expressed in writing to either the Secretary or the Acting Commissioner of the FDA: the clear intent of the Congress is to have the exclusivities run consecutively, not concurrently. That is, in situations where the exclusivities could overlap, the Pediatric Exclusivity six month period is to run first, and then the 180 day generic exclusivity is to run upon the expiration of the six month period. It is the Committee's belief that any manufacturer entitled to the 180 day generic exclusivity should not see any of that period eviscerated by Pediatric Exclusivity.

Section 11. Prompt approval of generic drugs when pediatric information added to labeling

This section ensures that any drug which has been submitted or approved under section 505(j) shall not be considered ineligible for approval or misbranded when its labeling omits a pediatric indication or other aspect of pediatric labeling pertaining to a pediatric use when such indication or aspect of pediatric labeling pertaining to a pediatric use is protected by exclusivity under sections 505(j)(5)(D)(iii) or (iv).

Pursuant to a written request, the FDA can ask that manufacturers conduct pediatric studies which could give rise not only to the six months of exclusivity provided for in section 505A, but also

three years of exclusivity pursuant to the Hatch-Waxman Act. This Section does not prevent any manufacturer from earning six months of exclusivity and then claiming three years of supplemental exclusivity pursuant to section 505(j). However, it does make clear that if a manufacturer does claim supplemental exclusivity under section 505(j), the terms of that exclusivity will not prevent generic competition for the indications or aspects of labeling which are not protected. This provision allows the Secretary to require that drugs approved under section 505(j) and that omit protected pediatric labeling include a statement that the drug is not labeled for the protected pediatric use and any warnings against unsafe pediatric use.

Section 12. Adverse-event reporting

This section allows the Secretary one year to promulgate a final rule which will require the drug labeling for each drug approved under section 505(b) to include the toll-free number maintained by the Secretary for the purpose of receiving adverse event reports regarding drugs.

The language in this provision expressly requires the Secretary to adopt a rule which ensures that the toll-free number reach the "broadest consumer audience." The Committee believes that the best way to reach the "broadest consumer audience" is to include the toll-free number on the prescription bottle, vial, etc., perhaps in the form of auxiliary labels. In addition, the Secretary through rulemaking can decide whether to require the toll-free to be included by manufacturers on unit-of-use packaging, such as ointments, creams, opthalmic or otic products, or any other items for which pharmacists can dispense the product without repackaging.

The language directs the Secretary to promulgate the rule in a way which minimizes the cost of the rule on the pharmacy profession. It is very important to the Committee that pharmacists, who are already overburdened, do not have their workload increased by the rule the Secretary will promulgate. Therefore, the Secretary is free to consider all creative alternatives which will result in the 1–800 number reaching the hands of the ultimate consumers, without increasing burdens on pharmacists.

Also, for each drug which receives exclusivity under section 505A, during the one year period following the grant of exclusivity all adverse event reports shall be forwarded to the Office of Pediatric Therapeutics. The Office of Pediatric Therapeutics shall review and forward the reports to the Anti-Infective Drugs Advisory Subcommittee, which shall hold a yearly public hearing to consider whether to forward recommendations to the Secretary about the drugs which have been granted exclusivity during the preceding year.

Section 13. Foundation for Pediatric Research

This section creates a private, nongovernmental Foundation which will collect funds and award grants for research on drugs listed by the Secretary pursuant to section 409I of the Public Health Service Act. The Foundation will have the right of first refusal for studying drugs with patent life or existing market exclusivity remaining but for which written requests did not result in the sponsors conducting studies.

This private Foundation is intended to supplement the work which will be done by the public fund within the NIH. While the Committee will stridently work to ensure that enough public funds are available to study the drugs which need to be studied, the Committee recognizes that during tight budgetary times it is essential to create a private source of funding for the drugs which are used in children yet nonetheless have not been studied for pediatric use.

Like the public fund, all studies conducted pursuant to grants by the private Foundation will be forwarded to the Director of the NIH and the Commissioner of Food and Drugs. Also like the public fund, the same mechanisms will be in place to ensure that the drugs studied are ultimately labeled for use in pediatric populations.

Section 14. Study concerning research involving children

This section requires the Secretary to enter into a contract with the Institute of Medicine for the conduct of a study of the Federal regulations in effect relating to research involving children, paying particular attention to the process of obtaining informed consent, the expectations and comprehension of child research participants and their parents, and the definition of "minimal risk," among other things.

Section 15. Study of effects of this act

This section requires a comprehensive cost-benefit study to be conducted by the Comptroller General. In conducting this study, the Secretary is to take into consideration the costs of section 505A to consumers and the government, as well as the effectiveness of the amendments made to the Act in ensuring that all drugs used by children are tested and properly labeled. Also, the study is to consider the benefits to consumers, the government, and private insurers resulting from decreased health care costs through lower hospitalization rates, fewer physician visits, and lower insurance rates. In its January Status Report to Congress, the FDA quantified savings from reducing the difference in hospitalization rates between adults and pediatric patients for five diseases alone. The Secretary quantified these savings as more than $200 million per year. A Tufts University study, on the other hand, did a much more comprehensive study and estimated that section 505A may save up to $7 billion per year. The study to be conducted by the GAO is to consider the costs and the benefits of the provision in considerable depth.

Section 16. Minority Children and Pediatric-Exclusivity Program

This provision amends Section 505A to have the Secretary take into consideration in reaching agreement on pediatric protocols the "adequate representation in of children of ethnic and racial minorities."

Further, the Section requires the Comptroller General to conduct a study which will consider the extent to which children of racial and ethnic minorities are included in studies pursuant to section 505A. If such pediatric patients are not included in studies pursuant to section 505A, the GAO shall make recommendations to increase such representation, among other things.

Section 17. Technical and conforming amendments

This section makes certain technical changes to existing law.

CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

In compliance with clause 3(e) of rule XIII of the Rules of the House of Representatives, changes in existing law made by the bill, as reported, are shown as follows (existing law proposed to be omitted is enclosed in black brackets, new matter is printed in italic, existing law in which no change is proposed is shown in roman):

FEDERAL FOOD, DRUG, AND COSMETIC ACT

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CHAPTER II—DEFINITIONS

* * * * * * *

SEC. 201. For the purposes of this Act—

(a) * * *

* * * * * * *

(kk) PRIORITY SUPPLEMENT.—The term "priority supplement" means a drug application referred to in section 101(4) of the Food and Drug Administration Modernization Act of 1997 (111 Stat. 2298).

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CHAPTER V—DRUGS AND DEVICES

SUBCHAPTER A—DRUGS AND DEVICES

* * * * * * *

SEC. 505A. PEDIATRIC STUDIES OF DRUGS.

(a) MARKET EXCLUSIVITY FOR NEW DRUGS.—If, prior to approval of an application that is submitted under section 505(b)(1), the Secretary determines that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the Secretary makes a written request for pediatric studies (which shall include a timeframe for completing such studies), and such studies are completed within any such timeframe and the reports thereof submitted in accordance with subsection (d)(2) or accepted in accordance with subsection (d)(3)—

(1)(A)(i) the period referred to in subsection (c)(3)(D)(ii) of section 505, and in subsection **[**(j)(4)(D)(ii)**]** *(j)(5)(D)(ii)* of such section, is deemed to be five years and six months rather than five years, and the references in subsections (c)(3)(D)(ii) and **[**(j)(4)(D)(ii)**]** *(j)(5)(D)(ii)* of such section to four years, to forty-eight months, and to seven and one-half years are deemed to be four and one-half years, fifty-four months, and eight years, respectively; or

(ii) the period referred to in clauses (iii) and (iv) of subsection (c)(3)(D) of such section, and in clauses (iii) and (iv) of subsection **[**(j)(4)(D)**]** *(j)(5)(D)* of such section, is deemed to be three years and six months rather than three years; and

* * * * * * *

[(b) SECRETARY TO DEVELOP LIST OF DRUGS FOR WHICH ADDITIONAL PEDIATRIC INFORMATION MAY BE BENEFICIAL.—Not later than 180 days after the date of enactment of the Food and Drug Administration Modernization Act of 1997, the Secretary, after consultation with experts in pediatric research shall develop, prioritize, and publish an initial list of approved drugs for which additional pediatric information may produce health benefits in the pediatric population. The Secretary shall annually update the list.]

[(c)] *(b)* MARKET EXCLUSIVITY FOR ALREADY-MARKETED DRUGS.—If the Secretary *determines that information relating to the use of an approved drug in the pediatric population may produce health benefits in that population and* makes a written request to the holder of an approved application under section 505(b)(1) for pediatric studies (which shall include a timeframe for completing such studies) [concerning a drug identified in the list described in subsection (b)], the holder agrees to the request, the studies are completed within any such timeframe, and the reports thereof are submitted in accordance with subsection (d)(2) or accepted in accordance with subsection (d)(3)—

(1)(A)(i) the period referred to in subsection (c)(3)(D)(ii) of section 505, and in subsection [(j)(4)(D)(ii)] *(j)(5)(D)(ii)* of such section, is deemed to be five years and six months rather than five years, and the references in subsections (c)(3)(D)(ii) and [(j)(4)(D)(ii)] *(j)(5)(D)(ii)* of such section to four years, to forty-eight months, and to seven and one-half years are deemed to be four and one-half years, fifty-four months, and eight years, respectively; or

(ii) the period referred to in clauses (iii) and (iv) of subsection (c)(3)(D) of such section, and in clauses (iii) and (iv) of subsection [(j)(4)(D)] *(j)(5)(D)* of such section, is deemed to be three years and six months rather than three years; and

* * * * * * *

[(d)] *(c)* CONDUCT OF PEDIATRIC STUDIES.—

(1) AGREEMENT FOR STUDIES.—The Secretary may, pursuant to a written request from the Secretary under subsection (a) or [(c)] *(b)*, after consultation with—

(A) * * *

* * * * * * *

(2) WRITTEN PROTOCOLS TO MEET THE STUDIES REQUIREMENT.—If the sponsor or holder and the Secretary agree upon written protocols for the studies, the studies requirement of subsection (a) or [(c)] *(b)* is satisfied upon the completion of the studies and submission of the reports thereof in accordance with the original written request and the written agreement referred to in paragraph (1). *In reaching an agreement regarding written protocols, the Secretary shall take into account adequate representation of children of ethnic and racial minorities.* Not later than 60 days after the submission of the report of the studies, the Secretary shall determine if such studies were or were not conducted in accordance with the original written request and the written agreement and reported in accordance with the requirements of the Secretary for filing and so notify the sponsor or holder.

(3) OTHER METHODS TO MEET THE STUDIES REQUIREMENT.—If the sponsor or holder and the Secretary have not agreed in writing on the protocols for the studies, the studies requirement of subsection (a) or [(c)] *(b)* is satisfied when such studies have been completed and the reports accepted by the Secretary. Not later than 90 days after the submission of the reports of the studies, the Secretary shall accept or reject such reports and so notify the sponsor or holder. The Secretary's only responsibility in accepting or rejecting the reports shall be to determine, within the 90 days, whether the studies fairly respond to the written request, have been conducted in accordance with commonly accepted scientific principles and protocols, and have been reported in accordance with the requirements of the Secretary for filing.

(4) WRITTEN REQUEST TO HOLDERS OF APPROVED APPLICATIONS FOR DRUGS THAT HAVE MARKET EXCLUSIVITY.—

(A) REQUEST AND RESPONSE.—If the Secretary makes a written request for pediatric studies under subsection (b) to the holder of an application approved under section 505(b)(1), the holder, not later than 180 days after receiving the written request, shall respond to the Secretary as to the intention of the holder to act on the request by—

(i) indicating when the pediatric studies will be initiated, if the holder agrees to the request; or

(ii) indicating that the holder does not agree to the request.

(B) NO AGREEMENT TO REQUEST.—

(i) REFERRAL.—If the holder does not agree to a written request within the time period specified in subparagraph (A), and if the Secretary determines that there is a continuing need for information relating to the use of the drug in the pediatric population (including neonates as appropriate), the Secretary shall refer the drug to the Foundation for Pediatric Research established under section 499A of the Public Health Service Act (referred to in this paragraph as the "Foundation") for consideration for the conduct of the pediatric studies described in the written request.

(ii) PUBLIC NOTICE.—The Secretary shall give public notice of a referral under clause (i), including notice of the name of the drug, the name of the manufacturer, and the indication to be studied.

(C) LACK OF FUNDS.—If, on referral of a drug under subparagraph (B)(i), the Foundation certifies to the Secretary that the Foundation does not have funds available to conduct the requested studies, the Secretary shall refer the drug for inclusion on the list established under section 409I of the Public Health Service Act for the conduct of the studies.

(D) CONFIDENTIAL COMMERCIAL INFORMATION; TRADE SECRETS.—Nothing in this paragraph requires or authorizes the use or disclosure of confidential commercial information or trade secrets.

(E) NO REQUIREMENT TO REFER.—Nothing in this subsection shall be construed to require that every declined written request shall be referred to the Foundation.

[(e)**]** *(d)* DELAY OF EFFECTIVE DATE FOR CERTAIN APPLICATION.—If the Secretary determines that the acceptance or approval of an application under section 505(b)(2) or 505(j) for a new drug may occur after submission of reports of pediatric studies under this section, which were submitted prior to the expiration of the patent (including any patent extension) or the applicable period under clauses (ii) through (iv) of section 505(c)(3)(D) or clauses (ii) through (iv) of section 505**[**(j)(4)(D)**]** *(j)(5)(D)*, but before the Secretary has determined whether the requirements of subsection (d) have been satisfied, the Secretary shall delay the acceptance or approval under section 505(b)(2) or 505(j) until the determination under subsection (d) is made, but any such delay shall not exceed 90 days. In the event that requirements of this section are satisfied, the applicable six-month period under subsection (a) or **[**(c)**]** *(b)* shall be deemed to have been running during the period of delay.

[(f)**]** *(e)* NOTICE OF DETERMINATIONS ON STUDIES REQUIREMENT.—The Secretary shall publish a notice of any determination that the requirements of subsection (d) have been met and that submissions and approvals under subsection (b)(2) or (j) of section 505 for a drug will be subject to the provisions of this section.

[(g)**]** *(f)* DEFINITIONS.—As used in this section, the term "pediatric studies" or "studies" means at least one clinical investigation (that, at the Secretary's discretion, may include pharmacokinetic studies) in pediatric age groups *(including neonates in appropriate cases)* in which a drug is anticipated to be used.

[(h)**]** *(g)* LIMITATIONS.—A drug to which the six-month period under subsection (a) or (b) has already been applied—

(1) * * *

* * * * * * *

[(i)**]** *(h)* RELATIONSHIP TO REGULATIONS.—Notwithstanding any other provision of law, if any pediatric study is required pursuant to regulations promulgated by the Secretary and such study meets the completeness, timeliness, and other requirements of this section, such study shall be deemed to satisfy the requirement for market exclusivity pursuant to this section.

[(j) SUNSET.—A drug may not receive any six-month period under subsection (a) or (c) unless the application for the drug under section 505(b)(1) is submitted on or before January 1, 2002. After January 1, 2002, a drug shall receive a six-month period under subsection (c) if—

[(1) the drug was in commercial distribution as of the date of enactment of the Food and Drug Administration Modernization Act of 1997;

[(2) the drug was included by the Secretary on the list under subsection (b) as of January 1, 2002;

[(3) the Secretary determines that there is a continuing need for information relating to the use of the drug in the pediatric population and that the drug may provide health benefits in that population; and

[(4) all requirements of this section are met.**]**

(i) SUNSET.—A drug may not receive any 6-month period under subsection (a) or (b) unless—

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

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

(3) all requirements of this section are met.

[(k)**]** *(j)* REPORT.—The Secretary shall conduct a study and report to Congress not later than January 1, 2001, based on the experience under the program established under this section. The study and report shall examine all relevant issues, including—

(1) * * *

* * * * * * *

(k) LABELING SUPPLEMENTS.—

(1) PRIORITY STATUS FOR PEDIATRIC SUPPLEMENTS.—Any supplement to an application under section 505 proposing a labeling change pursuant to a report on a pediatric study under this section—

(A) shall be considered to be a priority supplement; and

(B) shall be subject to the performance goals established by the Commissioner for priority drugs.

(2) DISPUTE RESOLUTION.—If the Commissioner determines that an application with respect to which a pediatric study is conducted under this section is approvable and that the only open issue for final action on the application is the reaching of an agreement between the sponsor of the application and the Commissioner on appropriate changes to the labeling for the drug that is the subject of the application—

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

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

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

(B) not later than 90 days after receiving the referral, the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee shall—

(i) review the pediatric study reports; and

(ii) make a recommendation to the Commissioner concerning appropriate labeling changes, if any;

(C) the Commissioner shall consider the recommendations of the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee and, if appropriate, not later than 30 days after receiving the recommendation, make a request to the sponsor of the application to make any labeling change that the Commissioner determines to be appropriate; and

(D) if the sponsor of the application, within 30 days after receiving a request under subparagraph (C), does not agree to make a labeling change requested by the Commissioner,

the Commissioner may deem the drug that is the subject of the application to be misbranded.

(l) DISSEMINATION OF PEDIATRIC INFORMATION.—

(1) IN GENERAL.—Not later than 180 days after the date of submission of a report on a pediatric study under this section, the Commissioner shall make available to the public a summary of the medical and clinical pharmacology reviews of pediatric studies conducted for the supplement, including by publication in the Federal Register.

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

(m) CLARIFICATION OF INTERACTION OF MARKET EXCLUSIVITY UNDER THIS SECTION AND MARKET EXCLUSIVITY AWARDED TO AN APPLICANT FOR APPROVAL OF A DRUG UNDER SECTION 505(j).—

(1) IN GENERAL.—If a 180-day period under section 505(j)(5)(B)(iv) overlaps with a 6-month extension under this section, so that the applicant for approval of a drug under section 505(j) entitled to the 180-day period under that section loses a portion of the 180-day period to which the applicant is entitled for the drug, the 180-day period shall be extended—

(A) if the 180-day period would, but for this subsection, expire after the 6-month extension, by the number of days of the overlap; or

(B) if the 180-day period would, but for this subsection, expire during the 6-month extension, by 6 months.

(2) EFFECT OF SUBSECTION.—Under no circumstances shall application of this section result in an applicant for approval of a drug under section 505(j) being enabled to commercially market the drug to the exclusion of a subsequent applicant for approval of a drug under section 505(j) for more than 180 days.

(n) PROMPT APPROVAL OF GENERIC DRUGS WHEN PEDIATRIC INFORMATION ADDED TO LABELING.—

(1) IN GENERAL.—A drug for which an application has been submitted or approved under section 505(j) and which otherwise meets all other applicable requirements under that section shall be considered eligible for approval and shall not be considered misbranded under section 502 even when its labeling omits a pediatric indication or other aspect of labeling pertaining to pediatric use that is protected by patent or by market exclusivity pursuant to clause (iii) or (iv) of section 505(j)(5)(D).

(2) LABELING OF GENERIC DRUG.—Notwithstanding the provisions of clause (iii) or (iv) of section 505(j)(5)(D), the Secretary may require that the labeling of a drug approved under section 505(j) that omits pediatric labeling pursuant to paragraph (1) include—

(A) a statement that the drug is not labeled for the protected pediatric use; and

(B) any warnings against unsafe pediatric use that the Secretary considers necessary.

(3) RULE OF CONSTRUCTION.—Paragraphs 1 and 2 of this subsection do not affect—

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

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

(C) except as expressly provided in paragraph (1) and (2), the operation of section 505.

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CHAPTER VII—GENERAL AUTHORITY

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SUBCHAPTER C—FEES

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PART 2—FEES RELATING TO DRUGS

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SEC. 736. AUTHORITY TO ASSESS AND USE DRUG FEES.

(a) TYPES OF FEES.—Beginning in fiscal year 1998, the Secretary shall assess and collect fees in accordance with this section as follows:

(1) HUMAN DRUG APPLICATION AND SUPPLEMENT FEE.—

(A) * * *

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[(F) EXCEPTION FOR SUPPLEMENTS FOR PEDIATRIC INDICATIONS.—A supplement to a human drug application proposing to include a new indication for use in pediatric populations shall not be assessed a fee under subparagraph (A).**]**

[(G)**]** *(F)* REFUND OF FEE IF APPLICATION WITHDRAWN.—If an application or supplement is withdrawn after the application or supplement was filed, the Secretary may refund the fee or a portion of the fee if no substantial work was performed on the application or supplement after the application or supplement was filed. The Secretary shall have the sole discretion to refund a fee or a portion of the fee under this subparagraph. A determination by the Secretary concerning a refund under this paragraph shall not be reviewable.

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PUBLIC HEALTH SERVICE ACT

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TITLE IV—NATIONAL RESEARCH INSTITUTES

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PART B—GENERAL PROVISIONS RESPECTING NATIONAL RESEARCH INSTITUTES

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SEC. [409C.] *409G.* CLINICAL RESEARCH.

(a) IN GENERAL.—The Director of National Institutes of Health shall undertake activities to support and expand the involvement of the National Institutes of Health in clinical research.

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SEC. [409D.] *409H.* ENHANCEMENT AWARDS.

(a) MENTORED PATIENT-ORIENTED RESEARCH CAREER DEVELOPMENT AWARDS.—

(1) * * *

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SEC. 409I. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS LACKING EXCLUSIVITY.

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

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

(A)(i) there is an approved application under section 505(j) of the Federal Food, Drug, and Cosmetic Act;

(ii) there is a submitted application that could be approved under the criteria of section 505(j) of the Federal Food, Drug, and Cosmetic Act;

(iii) there is no patent protection or market exclusivity protection under the Federal Food, Drug, and Cosmetic Act; or

(iv) there is, under section 505A(c)(4)(C) of the Federal Food, Drug, and Cosmetic Act, a referral for inclusion on such list; and

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

(2) CONSIDERATION OF AVAILABLE INFORMATION.—In developing the list under paragraph (1), the Secretary shall consider, for each drug on the list—

(A) the availability of information concerning the safe and effective use of the drug in the pediatric population;

(B) whether additional information is needed;

(C) whether new pediatric studies concerning the drug may produce health benefits in the pediatric population; and

(D) whether reformulation of the drug is necessary;

(b) CONTRACTS FOR PEDIATRIC STUDIES.—The Secretary shall award contracts to entities that have the expertise to conduct pediatric clinical trials (including qualified universities, hospitals, laboratories, contract research organizations, federally funded programs such as pediatric pharmacology research units, other public or private institutions, or individuals) to enable the entities to conduct pediatric studies concerning one or more drugs identified in the list described in subsection (a).

(c) PROCESS FOR CONTRACTS AND LABELING CHANGES.—

(1) WRITTEN REQUEST TO HOLDERS OF APPROVED APPLICATIONS FOR DRUGS LACKING EXCLUSIVITY.—

(A) IN GENERAL.—The Commissioner of Food and Drugs, in consultation with the Director of National Institutes of Health, may issue a written request (which shall include a timeframe for negotiations for an agreement) for pediatric studies concerning a drug identified in the list described in subsection (a) to all holders of an approved application for the drug under section 505 of the Federal Food, Drug, and Cosmetic Act. Such a written request shall be made in a manner equivalent to the manner in which a written request is made under subsection (a) or (b) of section 505A of the Federal Food, Drug, and Cosmetic Act, including with respect to information provided on the pediatric studies to be conducted pursuant to the request.

(B) PUBLICATION OF REQUEST.—If the Commissioner of Food and Drugs does not receive a response to a written request issued under subparagraph (A) within 30 days of the date on which a request was issued, the Secretary, acting through the Director of National Institutes of Health and in consultation with the Commissioner of Food and Drugs, shall publish a request for contract proposals to conduct the pediatric studies described in the written request.

(C) DISQUALIFICATION.—A holder that receives a first right of refusal shall not be entitled to respond to a request for contract proposals under subparagraph (B).

(D) GUIDANCE.—Not later than 270 days after the date of enactment of this section, the Commissioner of Food and Drugs shall promulgate guidance to establish the process for the submission of responses to written requests under subparagraph (A).

(2) CONTRACTS.—A contract under this section may be awarded only if a proposal for the contract is submitted to the Secretary in such form and manner, and containing such agreements, assurances, and information as the Secretary determines to be necessary to carry out this section.

(3) REPORTING OF STUDIES.—

(A) Upon completion of a pediatric study in accordance with a contract awarded under this section, a report concerning the study shall be submitted to the Director of National Institutes of Health and the Commissioner of Food and Drugs. The report shall include all data generated in connection with the study.

(B) AVAILABILITY OF REPORTS.—Each report submitted under subparagraph (A) shall be considered to be in the public domain, and shall be assigned a docket number by the Commissioner of Food and Drugs. An interested person may submit written comments concerning such pediatric studies to the Commissioner of Food and Drugs, and the written comments shall become part of the docket file with respect to each of the drugs.

(C) ACTION BY COMMISSIONER.—The Commissioner of Food and Drugs shall take appropriate action in response to the reports submitted under subparagraph (A) in accordance with paragraph (4).

(4) REQUEST FOR LABELING CHANGES.—During the 180-day period after the date on which a report is submitted under paragraph (3)(A), the Commissioner of Food and Drugs shall—

(A) review the report and such other data as are available concerning the safe and effective use in the pediatric population of the drug studied; and

(B) negotiate with the holders of approved applications for the drug studied for any labeling changes that the Commissioner of Food and Drugs determines to be appropriate and requests the holders to make; and

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

(ii) publish in the Federal Register a summary of the report and a copy of any requested labeling changes.

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

(A) the Commissioner of Food and Drugs shall immediately refer the request to the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee; and

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

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

(ii) make a recommendation to the Commissioner of Food and Drugs as to appropriate labeling changes, if any.

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

(7) FAILURE TO AGREE.—If a holder of an approved application for a drug, within 30 days after receiving a request to make a labeling change under paragraph (6), does not agree to make a requested labeling change, the Commissioner may deem the drug to be misbranded under the Federal Food, Drug, and Cosmetic Act.

(8) RECOMMENDATION FOR FORMULATION CHANGES.—If a pediatric study completed under public contract indicates that a formulation change is necessary and the Secretary agrees, the Secretary shall send a nonbinding letter of recommendation regarding that change to each holder of an approved application.

(d) CONFIDENTIAL COMMERCIAL INFORMATION; TRADE SECRETS.—Nothing in this section requires or authorizes the use or disclosure of confidential commercial information or trade secrets.

(e) AUTHORIZATION OF APPROPRIATIONS.—

(1) IN GENERAL.—For the purpose of carrying out this section, there are authorized to be appropriated $200,000,000 for fiscal

year 2002, and such sums as may be necessary for each of the fiscal years 2003 through 2007.

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

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PART J—FOUNDATION FOR PEDIATRIC RESEARCH

SEC. 499A. ESTABLISHMENT AND DUTIES OF FOUNDATION.

(a) IN GENERAL.—The Secretary, acting through the Director of NIH and in consultation with the Commissioner of Food and Drugs, shall establish a nonprofit corporation to be known as the Foundation for Pediatric Research (hereafter in this section referred to as the "Foundation"). The Foundation shall not be an agency or instrumentality of the United States Government.

(b) PURPOSE OF FOUNDATION.—The purpose of the Foundation shall be to collect funds and award grants for research on drugs listed by the Secretary pursuant to section 409I(a)(1)(A).

(c) CERTAIN ACTIVITIES OF FOUNDATION.—

(1) IN GENERAL.—In carrying out subsection (b), the Foundation may solicit and accept gifts, grants, and other donations, establish accounts, and invest and expend funds in support of a program to encourage donations for the conduct of studies of drugs referred to in subsection (b).

(2) FEES.—The Foundation may assess fees for the provision of professional, administrative and management services by the Foundation in amounts determined reasonable and appropriate by the Executive Director.

(3) AUTHORITY OF FOUNDATION.—The Foundation shall be the sole entity responsible for carrying out the activities described in this subsection.

(d) BOARD OF DIRECTORS.—

(1) COMPOSITION.—

(A) The Foundation shall have a Board of Directors (hereafter referred to in this section as the Board"), which shall be composed of ex officio and appointed members in accordance with this subsection. Appointed members of the Board shall be the voting members.

(B) The ex officio members of the Board shall be—

(i) the Chairman and ranking minority member of the Subcommittee on Health (Committee on Energy and Commerce) or their designees, in the case of the House of Representatives;

(ii) the Chairman and ranking minority member of the Committee on Health, Education, Labor and Pensions or their designees, in the case of the Senate;

(iii) the Director of NIH; and

(iv) the Commissioner of Food and Drugs.

(C) The ex officio members of the Board under subparagraph (B) shall appoint to the Board 11 individuals from among a list of candidates to be provided by the National Academy of Science. Of such appointed members—

(i) 5 shall be representative of the experts in pediatric medicine and research field;

(ii) 1 shall be a biomedical ethicist; and

(iii) 5 shall be representatives of the general public, which may include representatives of affected industries.

(D)(i) Not later than 30 days after the date of the enactment of the Best Pharmaceuticals for Children Act, the Director of NIH shall convene a meeting of the ex officio members of the Board to—

(I) incorporate the Foundation and establish the general policies of the Foundation for carrying out the purposes of subsection (b), including the establishment of the bylaws of the Foundation; and

(II) appoint the members of the Board in accordance with subparagraph (C).

(ii) Upon the appointment of the members of the Board under clause (i)(II), the terms of service of the ex officio members of the Board as members of the Board shall terminate.

(E) The agreement of not less than three-fifths of the members of the ex officio members of the Board shall be required for the appointment of each member to the initial Board.

(F) No employee of the National Institutes of Health shall be appointed as a member of the Board.

(2) CHAIR.—

(A) The ex officio members of the Board under paragraph (1)(B) shall designate an individual to serve as the initial Chair of the Board.

(B) Upon the termination of the term of service of the initial Chair of the Board, the appointed members of the Board shall elect a member of the Board to serve as the Chair of the Board.

(3) TERMS AND VACANCIES.—

(A) The term of office of each member of the Board appointed under paragraph (1)(C) shall be 5 years, except that the terms of offices for the initial appointed members of the Board shall expire as determined by the ex officio members and the Chair.

(B) Any vacancy in the membership of the Board shall be filled in the manner in which the original position was made and shall not affect the power of the remaining members to execute the duties of the Board.

(C) If a member of the Board does not serve the full term applicable under subparagraph (A), the individual appointed to fill the resulting vacancy shall be appointed for the remainder of the term of the predecessor of the individual.

(D) A member of the Board may continue to serve after the expiration of the term of the member until a successor is appointed.

(4) COMPENSATION.—Members of the Board may not receive compensation for service on the Board. Such members may be reimbursed for travel, subsistence, and other necessary expenses

incurred in carrying out the duties of the Board, as set forth in the bylaws issued by the Board.

(5) MEETINGS AND QUORUM.—A majority of the members of the Board shall constitute a quorum for purposes of conducting the business of the Board.

(6) CERTAIN BYLAWS.—

(A) In establishing bylaws under this subsection, the Board shall ensure that the following are provided for:

(i) Policies for the selection of the officers, employees, and agents of the Foundation.

(ii) Policies, including ethical standards, for the acceptance, solicitation, and disposition of donations and grants to the Foundation and for the disposition of the assets of the Foundation. Policies with respect to ethical standards shall ensure that officers, employees and agents of the Foundation (including members of the Board) avoid encumbrances that would result in a conflict of interest, including a financial conflict of interest or a divided allegiance. Such policies shall include requirements for the provision of information concerning any ownership or controlling interest in entities related to the activities of the Foundation by such officers, employees and agents and their spouses and relatives.

(iii) Policies for the conduct of the general operations of the Foundation.

(B) In establishing bylaws under this subsection, the Board shall ensure that such bylaws (and activities carried out under the bylaws) do not—

(i) reflect unfavorably upon the ability of the Foundation to carry out its responsibilities or official duties in a fair and objective manner; or

(ii) compromise, or appear to compromise, the integrity of any governmental agency or program, or any officer or employee involved in such program.

(e) INCORPORATION.—The initial members of the Board shall serve as incorporators and shall take whatever actions necessary to incorporate the Foundation.

(f) NONPROFIT STATUS.—The Foundation shall be considered to be a corporation under section 501(c) of the Internal Revenue Code of 1986, and shall be subject to the provisions of such section.

(g) EXECUTIVE DIRECTOR.—

(1) IN GENERAL.—The Foundation shall have an Executive Director who shall be appointed by the Board and shall serve at the pleasure of the Board. The Executive Director shall be responsible for the day-to-day operations of the Foundation and shall have such specific duties and responsibilities as the Board shall prescribe.

(2) COMPENSATION.—The rate of compensation of the Executive Director shall be fixed by the Board.

(h) POWERS.—In carrying out subsection (b), the Foundation shall operate under the direction of its Board, and may—

(1) adopt, alter, and use a corporate seal, which shall be judicially noticed;

(2) provide for 1 or more officers, employees, and agents, as may be necessary, define their duties, and require surety bonds

or make other provisions against losses occasioned by acts of such persons;

(3) hire, promote, compensate, and discharge officers and employees of the Foundation, and define the duties of the officers and employees;

(4) with the consent of any executive department or independent agency, use the information, services, staff, and facilities of such in carrying out this section;

(5) sue and be sued in its corporate name, and complain and defend in courts of competent jurisdiction;

(6) modify or consent to the modification of any contract or agreement to which it is a party or in which it has an interest under this part;

(7) establish a process for the selection of candidates for positions under subsection (c);

(8) solicit, accept, hold, administer, invest, and spend any gift, devise, or bequest of real or personal property made to the Foundation;

(9) enter into such other contracts, leases, cooperative agreements, and other transactions as the Executive Director considers appropriate to conduct the activities of the Foundation; and

(10) exercise other powers as set forth in this section, and such other incidental powers as are necessary to carry out its powers, duties, and functions in accordance with this part.

(i) ADMINISTRATIVE CONTROL.—No participant in the program established under this part shall exercise any administrative control over any Federal employee, nor shall the Foundation attempt to influence an executive branch agency or employee.

(j) GENERAL PROVISIONS.—

(1) FOUNDATION INTEGRITY.—The members of the Board shall be accountable for the integrity of the operations of the Foundation and shall ensure such integrity through the development and enforcement of criteria and procedures relating to standards of conduct (including those developed under subsection (d)(6)(A)(ii), financial disclosure statements, conflict of interest rules, recusal and waiver rules, audits and other matter determined appropriate by the Board.

(2) FINANCIAL CONFLICTS OF INTEREST.—Any individual who is an officer, employee, or member of the Board of the Foundation may not (in accordance with policies and requirements developed under subsection (d)(6)(A)(ii) personally or substantially participate in the consideration or determination by the Foundation of any matter that would directly or predictably affect any financial interest of the individual or a relative (as such term is defined in section 109(16) of the Ethics in Government Act of 1978) of the individual, of any business organization or other entity, or of which the individual is an officer or employee, or is negotiating for employment, or in which the individual has any other financial interest.

(3) AUDITS; AVAILABILITY OF RECORDS.—The Foundation shall—

(A) provide for annual audits of the financial condition of the Foundation; and

(B) make such audits, and all other records, documents, and other papers of the Foundation, available to the Secretary and the Comptroller General of the United States for examination or audit.

(4) REPORTS.—

(A) Not later than 5 months following the end of each fiscal year, the Foundation shall publish a report describing the activities of the Foundation during the preceding fiscal year. Each such report shall include for the fiscal year involved a comprehensive statement of the operations, activities, financial condition, and accomplishments of the Foundation.

(B) With respect to the financial condition of the Foundation, each report under subparagraph (A) shall include the source, and a description of, all gifts or grants to the Foundation of real or personal property, and the source and amount of all gifts or grants to the Foundation of money. Each such report shall include a specification of any restrictions on the purposes for which gifts or grants to the Foundation may be used.

(C) The Foundation shall make copies of each report submitted under subparagraph (A) available for public inspection, and shall upon request provide a copy of the report to any individual for a charge not exceeding the cost of providing the copy.

(D) The Board shall annually hold a public meeting to summarize the activities of the Foundation and distribute written reports concerning such activities and the scientific results derived from such activities.

(5) SERVICE OF FEDERAL EMPLOYEES.—Federal employees may serve on committees advisory to the Foundation and otherwise cooperate with and assist the Foundation in carrying out its function, so long as the employees do not direct or control Foundation activities.

(6) RELATIONSHIP WITH EXISTING ENTITIES.—The Foundation may, pursuant to appropriate agreements, acquire the resources of existing nonprofit private corporations with missions similar to the purposes of the Foundation.

(7) INTELLECTUAL PROPERTY RIGHTS.—The Board may adopt written standards with respect to the ownership of any intellectual property rights derived from the collaborative efforts of the Foundation prior to the commencement of such efforts.

(8) NATIONAL INSTITUTES OF HEALTH AMENDMENTS OF 1990.—The activities conducted in support of the National Institutes of Health Amendments of 1990 (Public Law 101–613), and the amendments made by such Act, shall not be nullified by the enactment of this section.

(9) LIMITATION OF ACTIVITIES.—The Foundation shall exist solely as an entity to collect funds and award grants for research on drugs listed by the Secretary pursuant to section 409I(a)(1)(A).

(10) TRANSFER OF FUNDS.—The Foundation may transfer funds to the National Institutes of Health. Any funds transferred under this paragraph shall be subject to all Federal limitations relating to federally-funded research.

(k) DUTIES OF THE DIRECTOR.—

(1) APPLICABILITY OF CERTAIN STANDARDS TO NON-FEDERAL EMPLOYEES.—In the case of any individual who is not an employee of the Federal Government and who serves in association with the National Institutes of Health, with respect to financial assistance received from the Foundation, the Foundation may not provide the assistance of, or otherwise permit the work at the National Institutes of Health to begin until a memorandum of understanding between the individual and the Director of NIH, or the designee of such Director, has been executed specifying that the individual shall be subject to such ethical and procedural standards of conduct relating to duties performed at the National Institutes of Health, as the Director of NIH determines is appropriate.

(2) SUPPORT SERVICES.—The Director of NIH shall provide facilities, utilities and support services to the Foundation.

(l) REPORTS OF STUDIES; LABELING CHANGES.—

(1) IN GENERAL.—Upon completion of a pediatric study conducted pursuant to this section, a report concerning the study shall be submitted to the Director of National Institutes of Health and the Commissioner of Food and Drugs. The report shall include all data generated in connection with the study.

(2) AVAILABILITY OF REPORTS; ACTION BY FOOD AND DRUG ADMINISTRATION; LABELING CHANGES.—With respect to a report submitted under paragraph (1), the provisions of paragraphs (3)(B) through (8) of section 409I(c) apply to such report to the same extent and in the same manner as such provision apply to a report submitted under section 409I(c)(3)(A).

(m) FUNDING.—

(1) AUTHORIZATION OF APPROPRIATIONS.—For the purpose of carrying out this part, there are authorized to be appropriated such sums as may be necessary for fiscal year 2002 and each subsequent fiscal year.

(2) LIMITATION REGARDING OTHER FUNDS.—Amounts appropriated under any provision of law other than paragraph (1) may not be expended to establish or operate the Foundation.

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DISSENTING VIEWS

We agree with the American Academy of Pediatrics, the Food and Drug Administration (FDA), and the sponsors of H.R. 2887 that proper testing and labeling of drugs for children is an important health matter. For example, we believe that pediatric testing should simply be required in appropriate cases. The Federal Food, Drug, and Cosmetic Act (the Act) requires that drugs be safe and effective for their intended use. The Act does not say safe and effective, except for children.

But, we oppose H.R. 2887 because it imposes unnecessary costs on the consuming and taxpaying public. Even the American Academy of Pediatrics recognizes that the exclusivity incentive "may be providing a monetary windfall for a limited number of drugs," and "believes it is important to determine whether some monetary limits are needed for certain drugs . . . ."

All programs should be evaluated for their cost effectiveness, including this one. In comments submitted with respect to the Department of Health and Human Services' (HHS) January 2001 report, "The Pediatric Exclusivity Provision" (the Report), the American Academy of Pediatrics "urged the development of different incentive levels depending on the need for information on specific drugs, to mitigate the tendency to conduct studies on those drugs with the greatest profits rather than those with the greatest need." It should be noted that many well known consumer advocacy organizations oppose H.R. 2887 because of the unnecessary costs of the exclusivity incentive.

In the four years of this program's existence, it has already cost consumers and taxpayers billions of dollars while producing only 19 new drug labels. One drug netted its owner, Astra Zeneca, a windfall of $1.4 billion, or approximately 350 times the cost of an average pediatric study. Eli Lilly gained a $900 million windfall during the extra six-months monopoly pricing on Prozac. In neither case has the American public, who has paid these higher prices, received any additional information of the pediatric uses of the drugs. If H.R. 2887 becomes law, these and virtually all other brand name drug companies with "blockbuster drugs" will continue to extract billions of excess profits in return for studies that cost, on average, less than $4 million to conduct.

The Committee rejected cost-effective alternative incentives such as the Waxman/Brown Substitute, that would have replaced the six-month exclusivity incentive with direct reimbursement of the costs of the studies, plus a 100 percent profit.

According to the Report, "the impact of the lack of lower cost generic drugs on some patients, especially those without health insurance and the elderly, may be significant." The Report also concluded that "[t]he greatest burden will fall on consumers with no private or public insurance support, which may disproportionately

affect lower income purchasers" and that "the pediatric exclusivity provision . . . imposes substantial costs on consumers and taxpayers."

A survey of twenty-five drugs that may be eligible for pediatric exclusivity during the five-year authorization period of H.R. 2887 indicates that the sponsors of these products will receive additional sales revenues of more than $11 billion. The profit margins on these revenues are quite high since the drugs have already been in existence for many years having already recovered development costs many fold. Without the pediatric exclusivity, these drugs would face price competition and all would benefit.

Moreover, the exclusivity program does not always yield the labeling for which consumers have paid. The FDA has identified approximately 400 drugs for which pediatric testing and labeling is needed. Five years later, 38 drugs have received exclusivity and only half of these products have labeling that reflects the results of those tests.

When the systematic exclusion and underrepresentation of women in drug testing was brought to light years ago, none suggested that the situation could only be addressed with an economic incentive to industry. Nor are we aware of anyone ever suggesting that the only way to address racial and ethnic disparities in drug product research was through an economic incentive for product sponsors. The same should be the case for our children. In fact, FDA's new drug approval regulations, 21 CFR Part 314, specifically require that both effectiveness and safety data be presented by gender, age, and racial subgroups and effectiveness data shall identify any modifications of dose or dose interval needed for specific subgroups.

It was a mistake to ever allow drug product sponsors to systematically exclude children from the protection of the Act's safety and efficacy standard. This mistake was compounded in 1997 by the creation of pediatric exclusivity, a program that says that proper testing and labeling of drugs used by children will depend upon a product sponsor's evaluation of the adequacy of a financial incentive. The Report stated that the incentive "has naturally tended to produce pediatric studies on those products where the exclusivity has the greatest value. This has left some drugs of importance to children, but for which the incentive has little or no value, unstudied."

According to the FDA, "the absence of pediatric labeling information poses significant risks for children." For example, the FDA has noted that inadequate dosing information exposes pediatric patients to the risk of adverse reactions that could be avoided. FDA has noted reports of injuries and deaths in children resulting from use of drugs that were not adequately tested in the pediatric population. Furthermore, as FDA has noted, lack of pediatric testing can expose patients to ineffective treatment or deny them the benefits of therapeutic advances.

Aside from H.R. 2887's reliance on the six-month exclusivity incentive, the bill fails to condition that incentive on actual labeling that reflects the results of studies. Prescribing physicians, parents, and older children do not read studies, they read product labels. There should be no exclusivity unless and until the product is la-

beled. Our colleague, Representative Bart Stupak, offered an amendment that would have closed this dangerous loophole in the law. Unfortunately, this amendment was rejected.

Giving credit where credit is due, it should be noted that some improvements have been made in the bill. These include suggestions and amendments offered by our colleagues Representatives Stupak, Pallone, and DeGett. These views reflect our concern with the bill's core provision, namely the provision that conditions proper testing and labeling of products for children on a product sponsor's evaluation of the adequacy of a financial incentive. Public health decisions should not depend on such hit-or-miss inducements whose cost is often borne by those least able to pay.

JOHN D. DINGELL.
SHERROD BROWN.
HENRY A. WAXMAN.
PETER DEUTSCH.
FRANK PALLONE, Jr.
TOM BARRETT.
BART STUPAK.

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