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(c) CLARIFICATION OF AVAILABILITY OF INVESTIGATIONAL NEW DRUGS FOR PEDIATRIC STUDY AND USE— (1) AMENDMENT OF THE FEDERAL FOOD, DRUG, AND COSMETIC ACT- Section 505(i)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)(1)) is amended— (A) in subparagraph (B), by striking "and" at the end; (B) in subparagraph (C), by striking the period at the end and inserting "; and"; and (C) by adding at the end the following: "(D) the submission to the Secretary by the manufacturer or the sponsor of the investigation of a new drug of a statement of intent regarding whether the manufacturer or sponsor has plans for assessing pediatric safety and efficacy.". (2) AMENDMENT OF THE PUBLIC HEALTH SERVICE ACT- Section 402(j)(3)(A) of the Public Health Service Act (42 U.S.C. 282(j)(3)(A)) is amended in the first sentence— (A) by striking "trial sites, and" and inserting "trial sites,"; and (B) by striking "in the trial," and inserting "in the trial, and a description of whether, and through what procedure, the manufacturer or sponsor of the investigation of a new drug will respond to requests for protocol exception, with appropriate safeguards, for single-patient and expanded protocol use of the new drug, particularly in children,". (d) REPORT—Not later than January 31, 2003, the Secretary of Health and Human Services, acting through the Commissioner of Food and Drugs and in consultation with the Director of the National Institutes of Health, shall submit to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives a report on patient access to new therapeutic agents for pediatric cancer, including access to single patient use of new therapeutic agents.	(B) by adding at the end the following new paragraph: '(3) CONTINUATION OF OPERATION OF SUBCOMMITTEE—Notwithstanding section 14 of the Federal Advisory Committee Act, the Subcommittee shall continue to operate during the five-year period beginning on the	**Extension of Subcommittee** Extends the Pediatric Subcommittee of the Oncologic Drugs Advisory Committee through October 1, 2012.

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	date of the enactment of the Best Pharmaceuticals for Children Act of 2007.'; and (2) in subsection (d), by striking '2003' and inserting '2009'. **SEC. 503. TRAINING OF PEDIATRIC PHARMACOLOGISTS.** (a) INVESTMENT IN TOMORROW'S PEDIATRIC RESEARCHERS—Section 452G(2) of the Public Health Service Act (42 U.S.C. 285g-10(2)) is amended by adding before the period at the end the following: ', including pediatric pharmacological research'. (b) PEDIATRIC RESEARCH LOAN REPAYMENT PROGRAM—Section 487F(a)(1) of the Public Health Service Act (42 U.S.C. 288-6(a)(1)) is amended by inserting 'including pediatric pharmacological research,' after 'pediatric research,'.	**Pediatric Pharmacologists** Includes pediatric pharmacologists in existing NIH career development and loan repayment programs.

Pediatric Research Equity Act: Improvements to Existing Law		
Existing Law	**2007 Reauthorization**	**Improvements**
	SEC. 401. SHORT TITLE. This title may be cited as the 'Pediatric Research Equity Act of 2007'. **SEC. 402. REAUTHORIZATION OF PEDIATRIC RESEARCH EQUITY ACT.** (a) In General- Section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c) is amended to read as follows:	
SEC. 505B. [21 U.S.C. 355c] RESEARCH INTO PEDIATRIC USES FOR DRUGS AND BIOLOGICAL PRODUCTS.	**'SEC. 505B. RESEARCH INTO PEDIATRIC USES FOR DRUGS AND BIOLOGICAL PRODUCTS.**	
(a) NEW DRUGS AND BIOLOGICAL PRODUCTS—	'(a) NEW DRUGS AND BIOLOGICAL PRODUCTS—	
(1) IN GENERAL—A person that submits an application (or supplement to an application)—	'(1) IN GENERAL- A person that submits, on or after the date of the enactment of the Pediatric Research Equity Act of 2007, an application (or supplement to an application)--	
(A) under section 505 for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration; or	'(A) under section 505 for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration, or	
(B) under section 351 of the Public Health Service Act (42 U.S.C. 262) for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration; shall submit with the application the assessments described in paragraph (2).	'(B) under section 351 of the Public Health Service Act (42 U.S.C. 262) for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration, shall submit with the application the assessments described in paragraph (2).	
(2) ASSESSMENTS—	'(2) ASSESSMENTS—	
(A) IN GENERAL—The assessments referred to in paragraph (1) shall contain data, gathered using appropriate formulations for each age group for which the assessment is required, that are adequate—	'(A) IN GENERAL—The assessments referred to in paragraph (1) shall contain data, gathered using appropriate formulations for each age group for which the assessment is required, that are adequate—	
(i) to assess the safety and effectiveness of the drug or the biological product for the claimed indications in all relevant pediatric subpopulations; and	'(i) to assess the safety and effectiveness of the drug or the biological product for the claimed indications in all relevant pediatric subpopulations; and	
(ii) to support dosing and administration for each pediatric subpopulation for which the drug or the biological product is safe and effective.	'(ii) to support dosing and administration for each pediatric subpopulation for which the drug or the biological product is safe and effective.	
(B) SIMILAR COURSE OF DISEASE OR SIMILAR EFFECT OF DRUG OR BIOLOGICAL PRODUCT—	'(B) SIMILAR COURSE OF DISEASE OR SIMILAR EFFECT OF DRUG OR BIOLOGICAL PRODUCT—	
(i) IN GENERAL—If the course of the disease and the effects of the drug are	'(i) IN GENERAL—If the course of the disease and the effects of the drug are	

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sufficiently similar in adults and pediatric patients, the Secretary may conclude that pediatric effectiveness can be extrapolated from adequate and well-controlled studies in adults, usually supplemented with other information obtained in pediatric patients, such as pharmacokinetic studies.	sufficiently similar in adults and pediatric patients, the Secretary may conclude that pediatric effectiveness can be extrapolated from adequate and well-controlled studies in adults, usually supplemented with other information obtained in pediatric patients, such as pharmacokinetic studies.	
(ii) EXTRAPOLATION BETWEEN AGE GROUPS—A study may not be needed in each pediatric age group if data from 1 age group can be extrapolated to another age group.	'(ii) EXTRAPOLATION BETWEEN AGE GROUPS—A study may not be needed in each pediatric age group if data from one age group can be extrapolated to another age group.	
	'(iii) INFORMATION ON EXTRAPOLATION-A brief documentation of the scientific data supporting the conclusion under clauses (i) and (ii) shall be included in any pertinent reviews for the application under section 505 of this Act or section 351 of the Public Health Service Act (42 U.S.C. 262).	
(3) DEFERRAL—On the initiative of the Secretary or at the request of the applicant, the Secretary may defer submission of some or all assessments required under paragraph (1) until a specified date after approval of the drug or issuance of the license for a biological product if—	'(3) DEFERRAL—	
	'(A) IN GENERAL—On the initiative of the Secretary or at the request of the applicant, the Secretary may defer submission of some or all assessments required under paragraph (1) until a specified date after approval of the drug or issuance of the license for a biological product if—	
(A) the Secretary finds that—	'(i) the Secretary finds that—	
(i) the drug or biological product is ready for approval for use in adults before pediatric studies are complete;	'(I) the drug or biological product is ready for approval for use in adults before pediatric studies are complete;	
(ii) pediatric studies should be delayed until additional safety or effectiveness data have been collected; or	'(II) pediatric studies should be delayed until additional safety or effectiveness data have been collected; or	
(iii) there is another appropriate reason for deferral; and	'(III) there is another appropriate reason for deferral; and	
(B) the applicant submits to the Secretary—	'(ii) the applicant submits to the Secretary--	
(i) certification of the grounds for deferring the assessments;	'(I) certification of the grounds for deferring the assessments;	
(ii) a description of the planned or ongoing studies; and	'(II) a description of the planned or ongoing studies;	
(iii) evidence that the studies are being conducted or will be conducted with due diligence and at the earliest possible time.	'(III) evidence that the studies are being conducted or will be conducted with due diligence and at the earliest possible time; and	
	'(IV) a timeline for the completion of such studies.	
	'(B) ANNUAL REVIEW—	**Annual Deferral Review** Requires an annual review from an applicant who has received a deferral.
	'(i) IN GENERAL—On an annual basis following the approval of a deferral under	

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	subparagraph (A), the applicant shall submit to the Secretary the following information: `(I) Information detailing the progress made in conducting pediatric studies.  `(II) If no progress has been made in conducting such studies, evidence and documentation that such studies will be conducted with due diligence and at the earliest possible time. `(ii) PUBLIC AVAILABILITY—The information submitted through the annual review under clause (i) shall promptly be made available to the public in an easily accessible manner, including through the Web site of the Food and Drug Administration.	**Public Annual Deferral Reviews** Requires annual deferral reviews to be made public.
(4) WAIVERS—	`(4) WAIVERS—	
(A) FULL WAIVER—On the initiative of the Secretary or at the request of an applicant, the Secretary shall grant a full waiver, as appropriate, of the requirement to submit assessments for a drug or biological product under this subsection if the applicant certifies and the Secretary finds that—	`(A) FULL WAIVER—On the initiative of the Secretary or at the request of an applicant, the Secretary shall grant a full waiver, as appropriate, of the requirement to submit assessments for a drug or biological product under this subsection if the applicant certifies and the Secretary finds that--	
(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients is so small or the patients are geographically dispersed);	`(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients is so small or the patients are geographically dispersed);	
(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in all pediatric age groups; or	`(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in all pediatric age groups; or	
(iii) the drug or biological product—	`(iii) the drug or biological product--	
(I) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients; and	`(I) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients; and	
(II) is not likely to be used in a substantial number of pediatric patients.	`(II) is not likely to be used in a substantial number of pediatric patients.	
(B) PARTIAL WAIVER—On the initiative of the Secretary or at the request of an applicant, the Secretary shall grant a partial waiver, as appropriate, of the requirement to submit assessments for a drug or biological product under this subsection with respect to a specific pediatric age group if the applicant certifies and the Secretary finds that—	`(B) PARTIAL WAIVER—On the initiative of the Secretary or at the request of an applicant, the Secretary shall grant a partial waiver, as appropriate, of the requirement to submit assessments for a drug or biological product under this subsection with respect to a specific pediatric age group if the applicant certifies and the Secretary finds that—	
(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed);	`(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed);	

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(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in that age group;	`(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in that age group;	
(iii) the drug or biological product—	`(iii) the drug or biological product--	
(I) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients in that age group; and	`(I) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients in that age group; and	
(II) is not likely to be used by a substantial number of pediatric patients in that age group; or	`(II) is not likely to be used by a substantial number of pediatric patients in that age group; or	
(iv) the applicant can demonstrate that reasonable attempts to produce a pediatric formulation necessary for that age group have failed	`(iv) the applicant can demonstrate that reasonable attempts to produce a pediatric formulation necessary for that age group have failed.	
(C) PEDIATRIC FORMULATION NOT POSSIBLE—If a waiver is granted on the ground that it is not possible to develop a pediatric formulation, the waiver shall cover only the pediatric groups requiring that formulation.	`(C) PEDIATRIC FORMULATION NOT POSSIBLE—If a waiver is granted on the ground that it is not possible to develop a pediatric formulation, the waiver shall cover only the pediatric groups requiring that formulation. An applicant seeking either a full or partial waiver shall submit to the Secretary documentation detailing why a pediatric formulation cannot be developed and, if the waiver is granted, the applicant's submission shall promptly be made available to the public in an easily accessible manner, including through posting on the Web site of the Food and Drug Administration.	**Pediatric Formulations** Requires manufacturers that have tried but been unable to produce a pediatric formulation to submit to FDA the reasons why the formulation cannot be developed.
(D) LABELING REQUIREMENT—If the Secretary grants a full or partial waiver because there is evidence that a drug or biological product would be ineffective or unsafe in pediatric populations, the information shall be included in the labeling for the drug or biological product.	`(D) LABELING REQUIREMENT—If the Secretary grants a full or partial waiver because there is evidence that a drug or biological product would be ineffective or unsafe in pediatric populations, the information shall be included in the labeling for the drug or biological product.	
(b) MARKETED DRUGS AND BIOLOGICAL PRODUCTS—	`(b) MARKETED DRUGS AND BIOLOGICAL PRODUCTS—	
(1) IN GENERAL—After providing notice in the form of a letter and an opportunity for written response and a meeting, which may include an advisory committee meeting, the Secretary may (by order in the form of a letter) require the holder of an approved application for a drug under section 505 or the holder of a license for a biological product under section 351 of the Public Health Service Act (42 U.S.C. 262) to submit by a specified date the assessments described in subsection (a)(2) if the Secretary finds that—	`(1) IN GENERAL—After providing notice in the form of a letter (that, for a drug approved under section 505, references a declined written request under section 505A for a labeled indication which written request is not referred under section 505A(n)(1)(A) to the Foundation of the National Institutes of Health for the pediatric studies), the Secretary may (by order in the form of a letter) require the sponsor or holder of an approved application for a drug under section 505 or the holder of a license for a biological product under section 351 of the Public Health Service Act to submit by a specified date the assessments described in subsection (a)(2), if the Secretary finds that—	
(A)(i) the drug or biological product is used	`(A)(i) the drug or biological product is used	

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for a substantial number of pediatric patients for the labeled indications; and	for a substantial number of pediatric patients for the labeled indications; and	
(ii) the absence of adequate labeling could pose significant risks to pediatric patients; or	'(ii) adequate pediatric labeling could confer a benefit on pediatric patients;	**Post-Market Standard** Changes the criteria for applying PREA to already marketed drugs. New language allows FDA to use a "benefit" standard as opposed to a "risk" standard.
(B)(i) there is reason to believe that the drug or biological product would represent a meaningful therapeutic benefit over existing therapies for pediatric patients for 1 or more of the claimed indications; and	'(B) there is reason to believe that the drug or biological product would represent a meaningful therapeutic benefit over existing therapies for pediatric patients for 1 or more of the claimed indications; or	
(ii) the absence of adequate labeling could pose significant risks to pediatric patients.	'(C) the absence of adequate pediatric labeling could pose a risk to pediatric patients.	
(2) WAIVERS—	'(2) WAIVERS—	
(A) FULL WAIVER—At the request of an applicant, the Secretary shall grant a full waiver, as appropriate, of the requirement to submit assessments under this subsection if the applicant certifies and the Secretary finds that—	'(A) FULL WAIVER—At the request of an applicant, the Secretary shall grant a full waiver, as appropriate, of the requirement to submit assessments under this subsection if the applicant certifies and the Secretary finds that—	
(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed); or	'(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed); or	
(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in all pediatric age groups.	'(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in all pediatric age groups.	
(B) PARTIAL WAIVER—At the request of an applicant, the Secretary shall grant a partial waiver, as appropriate, of the requirement to submit assessments under this subsection with respect to a specific pediatric age group if the applicant certifies and the Secretary finds that—	'(B) PARTIAL WAIVER—At the request of an applicant, the Secretary shall grant a partial waiver, as appropriate, of the requirement to submit assessments under this subsection with respect to a specific pediatric age group if the applicant certifies and the Secretary finds that—	
(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed);	'(i) necessary studies are impossible or highly impracticable (because, for example, the number of patients in that age group is so small or patients in that age group are geographically dispersed);	
(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in that age group;	'(ii) there is evidence strongly suggesting that the drug or biological product would be ineffective or unsafe in that age group;	
(iii)(I) the drug or biological product—	'(iii)(I) the drug or biological product--	
(aa) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients in that age group; and	'(aa) does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients in that age group; and	
(bb) is not likely to be used in a substantial number of pediatric patients in that age group; and	'(bb) is not likely to be used in a substantial number of pediatric patients in that age group; and	
(II) the absence of adequate labeling could	'(II) the absence of adequate labeling could	

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not pose significant risks to pediatric patients; or (iv) the applicant can demonstrate that reasonable attempts to produce a pediatric formulation necessary for that age group have failed.	not pose significant risks to pediatric patients; or '(iv) the applicant can demonstrate that reasonable attempts to produce a pediatric formulation necessary for that age group have failed.	
(C) PEDIATRIC FORMULATION NOT POSSIBLE—If a waiver is granted on the ground that it is not possible to develop a pediatric formulation, the waiver shall cover only the pediatric groups requiring that formulation.	'(C) PEDIATRIC FORMULATION NOT POSSIBLE—If a waiver is granted on the ground that it is not possible to develop a pediatric formulation, the waiver shall cover only the pediatric groups requiring that formulation. An applicant seeking either a full or partial waiver shall submit to the Secretary documentation detailing why a pediatric formulation cannot be developed and, if the waiver is granted, the applicant's submission shall promptly be made available to the public in an easily accessible manner, including through posting on the Web site of the Food and Drug Administration.	**Pediatric Formulations** Requires a manufacturer that has tried but been unable to produce a pediatric formulation to submit to FDA the reasons why the formulation cannot be developed.
(D) LABELING REQUIREMENT—If the Secretary grants a full or partial waiver because there is evidence that a drug or biological product would be ineffective or unsafe in pediatric populations, the information shall be included in the labeling for the drug or biological product.	'(D) LABELING REQUIREMENT—If the Secretary grants a full or partial waiver because there is evidence that a drug or biological product would be ineffective or unsafe in pediatric populations, the information shall be included in the labeling for the drug or biological product. '(3) EFFECT OF SUBSECTION—Nothing in this subsection alters or amends section 301(j) of this Act or section 552 of title 5 or section 1905 of title 18, United States Code.	
(3) RELATIONSHIP TO OTHER PEDIATRIC PROVISIONS— (A) NO ASSESSMENT WITHOUT WRITTEN REQUEST—No assessment may be required under paragraph (1) for a drug subject to an approved application under section 505 unless— (i) the Secretary has issued a written request for a related pediatric study under section 505A(c) of this Act or section 409I of the Public Health Service Act (42 U.S.C. 284m); (ii)(I) if the request was made under section 505A(c)— (aa) the recipient of the written request does not agree to the request; or (bb) the Secretary does not receive a response as specified under section 505A(d)(4)(A); or (II) if the request was made under section 409I of the Public Health Service Act (42 U.S.C. 284m)—		**"Exhaustion" Process** Eliminates much of the "exhaustion" process FDA must now go through before it can mandate a study of a drug already on the market under PREA.

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(aa) the recipient of the written request does not agree to the request; or (bb) the Secretary does not receive a response as specified under section 409I(c)(2) of that Act; and (iii)(I) the Secretary certifies under subparagraph (B) that there are insufficient funds under sections 409I and 499 of the Public Health Service Act (42 U.S.C. 284m, 290b) to conduct the study; or (II) the Secretary publishes in the Federal Register a certification that certifies that— (aa) no contract or grant has been awarded under section 409I or 499 of the Public Health Service Act (42 U.S.C. 284m, 290b); and (bb) not less than 270 days have passed since the date of a certification under subparagraph (B) that there are sufficient funds to conduct the study. (B) NO AGREEMENT TO REQUEST—Not later than 60 days after determining that no holder will agree to the written request (including a determination that the Secretary has not received a response specified under section 505A(d) of this Act or section 409I of the Public Health Service Act (42 U.S.C. 284m), the Secretary shall certify whether the Secretary has sufficient funds to conduct the study under section 409I or 499 of the Public Health Service Act (42 U.S.C. 284m, 290b), taking into account the prioritization under section 409I.		
(c) MEANINGFUL THERAPEUTIC BENEFIT—For the purposes of paragraph (4)(A)(iii)(I) and (4)(B)(iii)(I) of subsection (a) and paragraphs (1)(B)(i) and (2)(B)(iii)(I)(aa) of subsection (b), a drug or biological product shall be considered to represent a meaningful therapeutic benefit over existing therapies if the Secretary estimates that—	'(c) MEANINGFUL THERAPEUTIC BENEFIT—For the purposes of paragraph (4)(A)(iii)(I) and (4)(B)(iii)(I) of subsection (a) and paragraphs (1)(B) and (2)(B)(iii)(I)(aa) of subsection (b), a drug or biological product shall be considered to represent a meaningful therapeutic benefit over existing therapies if the Secretary determines that—	
(1) if approved, the drug or biological product would represent a significant improvement in the treatment, diagnosis, or prevention of a disease, compared with marketed products adequately labeled for that use in the relevant pediatric population; or	'(1) if approved, the drug or biological product could represent an improvement in the treatment, diagnosis, or prevention of a disease, compared with marketed products adequately labeled for that use in the relevant pediatric population; or	
(2) the drug or biological product is in a class of products or for an indication for which there is a need for additional options.	'(2) the drug or biological product is in a class of products or for an indication for which there is a need for additional options.	
(d) SUBMISSION OF ASSESSMENTS—If a person fails to submit an assessment described in subsection (a)(2), or a request	'(d) SUBMISSION OF ASSESSMENTS—If a person fails to submit an assessment described in subsection (a)(2), or a request	

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for approval of a pediatric formulation described in subsection (a) or (b), in accordance with applicable provisions of subsections (a) and (b)—	for approval of a pediatric formulation described in subsection (a) or (b), in accordance with applicable provisions of subsections (a) and (b)—	
(1) the drug or biological product that is the subject of the assessment or request may be considered misbranded solely because of that failure and subject to relevant enforcement action (except that the drug or biological product shall not be subject to action under section 303); but	'(1) the drug or biological product that is the subject of the assessment or request may be considered misbranded solely because of that failure and subject to relevant enforcement action (except that the drug or biological product shall not be subject to action under section 303); but	
(2) the failure to submit the assessment or request shall not be the basis for a proceeding—	'(2) the failure to submit the assessment or request shall not be the basis for a proceeding--	
(A) to withdraw approval for a drug under section 505(e); or	'(A) to withdraw approval for a drug under section 505(e); or	
(B) to revoke the license for a biological product under section 351 of the Public Health Service Act (42 U.S.C. 262).	'(B) to revoke the license for a biological product under section 351 of the Public Health Service Act.	
(e) MEETINGS—Before and during the investigational process for a new drug or biological product, the Secretary shall meet at appropriate times with the sponsor of the new drug or biological product to discuss—	'(e) MEETINGS—Before and during the investigational process for a new drug or biological product, the Secretary shall meet at appropriate times with the sponsor of the new drug or biological product to discuss--	
(1) information that the sponsor submits on plans and timelines for pediatric studies; or	'(1) information that the sponsor submits on plans and timelines for pediatric studies; or	
(2) any planned request by the sponsor for waiver or deferral of pediatric studies.	'(2) any planned request by the sponsor for waiver or deferral of pediatric studies.	
	'(f) REVIEW OF PEDIATRIC PLANS, ASSESSMENTS, DEFERRALS, AND WAIVERS—	**Internal Review** Requires internal committee to review study plans and assessments, as well as deferrals and waivers.
	'(1) REVIEW—Beginning not later than 30 days after the date of the enactment of the Pediatric Research Equity Act of 2007, the Secretary shall utilize the internal committee established under section 505C to provide consultation to reviewing divisions on all pediatric plans and assessments prior to approval of an application or supplement for which a pediatric assessment is required under this section and all deferral and waiver requests granted pursuant to this section.	
	'(2) ACTIVITY BY COMMITTEE—The committee referred to in paragraph (1) may operate using appropriate members of such committee and need not convene all members of the committee.	
	'(3) DOCUMENTATION OF COMMITTEE ACTION—For each drug or biological product, the committee referred to in paragraph (1) shall document, for each activity described in paragraph (4) or (5), which members of the committee	

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	participated in such activity. `(4) REVIEW OF PEDIATRIC PLANS, ASSESSMENTS, DEFERRALS, AND WAIVERS—Consultation on pediatric plans and assessments by the committee referred to in paragraph (1) pursuant to this section shall occur prior to approval of an application or supplement for which a pediatric assessment is required under this section. The committee shall review all requests for deferrals and waivers from the requirement to submit a pediatric assessment granted under this section and shall provide recommendations as needed to reviewing divisions, including with respect to whether such a supplement, when submitted, shall be considered for priority review. `(5) RETROSPECTIVE REVIEW OF PEDIATRIC ASSESSMENTS, DEFERRALS, AND WAIVERS—Not later than 1 year after the date of the enactment of the Pediatric Research Equity Act of 2007, the committee referred to in paragraph (1) shall conduct a retrospective review and analysis of a representative sample of assessments submitted and deferrals and waivers approved under this section since the enactment of the Pediatric Research Equity Act of 2003. Such review shall include an analysis of the quality and consistency of pediatric information in pediatric assessments and the appropriateness of waivers and deferrals granted. Based on such review, the Secretary shall issue recommendations to the review divisions for improvements and initiate guidance to industry related to the scope of pediatric studies required under this section. `(6) TRACKING OF ASSESSMENTS AND LABELING CHANGES—The Secretary, in consultation with the committee referred to in paragraph (1), shall track and make available to the public in an easily accessible manner, including through posting on the Web site of the Food and Drug Administration—  `(A) the number of assessments conducted under this section; `(B) the specific drugs and biological products and their uses assessed under this section;  `(C) the types of assessments conducted under this section, including trial design, the number of pediatric patients studied, and the number of centers and countries involved;	**Tracking** Requires FDA to track the number and type of studies completed, as well as labeling changes and other data resulting from PREA.

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	'(D) the total number of deferrals requested and granted under this section and, if granted, the reasons for such deferrals, the timeline for completion, and the number completed and pending by the specified date, as outlined in subsection (a)(3); '(E) the number of waivers requested and granted under this section and, if granted, the reasons for the waivers; '(F) the number of pediatric formulations developed and the number of pediatric formulations not developed and the reasons any such formulation was not developed; '(G) the labeling changes made as a result of assessments conducted under this section; '(H) an annual summary of labeling changes made as a result of assessments conducted under this section for distribution pursuant to subsection (h)(2); '(I) an annual summary of information submitted pursuant to subsection (a)(3)(B); and '(J) the number of times the committee referred to in paragraph (1) made a recommendation to the Secretary under paragraph (4) regarding priority review, the number of times the Secretary followed or did not follow such a recommendation, and, if not followed, the reasons why such a recommendation was not followed. '(g) LABELING CHANGES— '(1) DISPUTE RESOLUTION— '(A) REQUEST FOR LABELING CHANGE AND FAILURE TO AGREE—If, on or after the date of the enactment of the Pediatric Research Equity Act of 2007, the Commissioner determines that a sponsor and the Commissioner have been unable to reach agreement on appropriate changes to the labeling for the drug that is the subject of the application or supplement, not later than 180 days after the date of the submission of the application or supplement— '(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 does not agree within 30 days after the Commissioner's request to make a labeling change requested by the Commissioner, the Commissioner shall refer the matter to the Pediatric Advisory Committee.	**Dispute Resolution** The dispute resolution provision previously only applied to BPCA. The BPCA dispute resolution process was modified and applied to PREA. The modified language reduces the overall time period for dispute resolution over labeling and removes the provision requiring labeling to be the only remaining open issue before referral for resolution.

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	'(B) ACTION BY THE PEDIATRIC ADVISORY COMMITTEE- Not later than 90 days after receiving a referral under subparagraph (A)(ii), the Pediatric Advisory Committee shall— '(i) review the pediatric study reports; and '(ii) make a recommendation to the Commissioner concerning appropriate labeling changes, if any. '(C) CONSIDERATION OF RECOMMENDATIONS—The Commissioner shall consider the recommendations of the Pediatric Advisory Committee and, if appropriate, not later than 30 days after receiving the recommendation, make a request to the sponsor of the application or supplement to make any labeling changes that the Commissioner determines to be appropriate. '(D) MISBRANDING—If the sponsor of the application or supplement, 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 or supplement to be misbranded. '(E) NO EFFECT ON AUTHORITY—Nothing in this subsection limits the authority of the United States to bring an enforcement action under this Act when a drug lacks appropriate pediatric labeling. Neither course of action (the Pediatric Advisory Committee process or an enforcement action referred to in the preceding sentence) shall preclude, delay, or serve as the basis to stay the other course of action. '(2) OTHER LABELING CHANGES—If, on or after the date of the enactment of the Pediatric Research Equity Act of 2007, the Secretary makes a determination that a pediatric assessment conducted under this section does or does not demonstrate that the drug that is the subject of such assessment is safe and effective in pediatric populations or subpopulations, including whether such assessment results are inconclusive, the Secretary shall order the label of such product to include information about the results of the assessment and a statement of the Secretary's determination. '(h) DISSEMINATION OF PEDIATRIC INFORMATION— '(1) IN GENERAL—Not later than 210 days	

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	after the date of submission of a pediatric assessment under this section, the Secretary shall make available to the public in an easily accessible manner the medical, statistical, and clinical pharmacology reviews of such pediatric assessments, and shall post such assessments on the Web site of the Food and Drug Administration.	**Reviews Made Public** Requires Secretary to make publicly available the medical, statistical, and clinical pharmacology reviews.
	'(2) DISSEMINATION OF INFORMATION REGARDING LABELING CHANGES—Beginning on the date of the enactment of the Pediatric Research Equity Act of 2007, the Secretary shall require that the sponsors of the assessments that result in labeling changes that are reflected in the annual summary developed pursuant to subsection (f)(6)(H) distribute such information to physicians and other health care providers.	**Dissemination Requirements** Requires sponsors to provide physicians and other health care providers with new pediatric labeling information.
	'(3) EFFECT OF SUBSECTION—Nothing in this subsection shall alter or amend section 301(j) of this Act or section 552 of title 5 or section 1905 of title 18, United States Code.	
	'(i) ADVERSE EVENT REPORTING—	**Adverse Event Reporting** Requires drugs studied under PREA to report adverse events and provides for review by the Pediatric Advisory Committee.
	'(1) REPORTING IN YEAR ONE—Beginning on the date of the enactment of the Pediatric Research Equity Act of 2007, during the one-year period beginning on the date a labeling change is made pursuant to subsection (g), the Secretary shall ensure that all adverse event reports that have been received for such drug (regardless of when such report was received) are referred to the Office of Pediatric Therapeutics. In considering such reports, the Director of such Office shall provide for the review of such reports by the Pediatric Advisory Committee, including obtaining any recommendations of such committee regarding whether the Secretary should take action under this Act in response to such reports.	
	'(2) REPORTING IN SUBSEQUENT YEARS—Following the one-year period described in paragraph (1), the Secretary shall, as appropriate, refer to the Office of Pediatric Therapeutics all pediatric adverse event reports for a drug for which a pediatric study was conducted under this section. In considering such reports, the Director of such Office may provide for the review of such reports by the Pediatric Advisory Committee, including obtaining any recommendation of such Committee regarding whether the Secretary should take action in response to such reports.	
	'(3) EFFECT—The requirements of this subsection shall supplement, not supplant, other review of such adverse event reports by the Secretary.	

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(f) SCOPE OF AUTHORITY—Nothing in this section provides to the Secretary any authority to require a pediatric assessment of any drug or biological product, or any assessment regarding other populations or uses of a drug or biological product, other than the pediatric assessments described in this section.	'(j) SCOPE OF AUTHORITY—Nothing in this section provides to the Secretary any authority to require a pediatric assessment of any drug or biological product, or any assessment regarding other populations or uses of a drug or biological product, other than the pediatric assessments described in this section.	
(g) ORPHAN DRUGS—Unless the Secretary requires otherwise by regulation, this section does not apply to any drug for an indication for which orphan designation has been granted under section 526.	'(k) ORPHAN DRUGS—Unless the Secretary requires otherwise by regulation, this section does not apply to any drug for an indication for which orphan designation has been granted under section 526.	
	'(l) INSTITUTE OF MEDICINE STUDY—	**IOM Study** Asks the Institute of Medicine to review past study requests issued by FDA and make recommendations to FDA on scientific framework for future requests.
	'(1) IN GENERAL—Not later than three years after the date of the enactment of the Pediatric Research Equity Act of 2007, the Secretary shall contract with the Institute of Medicine to conduct a study and report to Congress regarding the pediatric studies conducted pursuant to this section or precursor regulations since 1997 and labeling changes made as a result of such studies.	
	'(2) CONTENT OF STUDY—The study under paragraph (1) shall review and assess the use of extrapolation for pediatric subpopulations, the use of alternative endpoints for pediatric populations, neonatal assessment tools, the number and type of pediatric adverse events, and ethical issues in pediatric clinical trials.	
	'(3) REPRESENTATIVE SAMPLE—The Institute of Medicine may devise an appropriate mechanism to review a representative sample of studies conducted pursuant to this section from each review division within the Center for Drug Evaluation and Research in order to make the requested assessment.	
(h) INTEGRATION WITH OTHER PEDIATRIC STUDIES—The authority under this section shall remain in effect so long as an application subject to this section may be accepted for filing by the Secretary on or before the date specified in section 505A(n).	'(m) INTEGRATION WITH OTHER PEDIATRIC STUDIES—The authority under this section shall remain in effect so long as an application subject to this section may be accepted for filing by the Secretary on or before the date specified in section 505A(q).'.	**PREA Extended** PREA linked with expiration of BPCA on October 1, 2012.
	(b) APPLICABILITY—	
	(1) IN GENERAL—Notwithstanding subsection (h) of section 505B of the Federal Food, Drug and Cosmetic Act, as in effect on the day before the date of the enactment of this Act, a pending assessment, including a deferred assessment, required under such section 505B shall be deemed to have been	

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	required under section 505B of the Federal Food, Drug and Cosmetic Act as in effect on or after the date of the enactment of this Act. (2) CERTAIN ASSESSMENTS AND WAIVER REQUESTS—An assessment pending on or after the date that is 1 year prior to the date of the enactment of this Act shall be subject to the tracking and disclosure requirements established under such section 505B, as in effect on or after such date of enactment, except that any such assessments submitted or waivers of such assessments requested before such date of enactment shall not be subject to subsections (a)(4)(C), (b)(2)(C), (f)(6)(F), and (h) of such section 505B.	
	SEC. 403. ESTABLISHMENT OF INTERNAL COMMITTEE. Chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351 et seq.) is amended by inserting after section 505B the following: **'SEC. 505C. INTERNAL COMMITTEE FOR REVIEW OF PEDIATRIC PLANS, ASSESSMENTS, DEFERRALS, AND WAIVERS.** 'The Secretary shall establish an internal committee within the Food and Drug Administration to carry out the activities as described in sections 505A(f) and 505B(f). Such internal committee shall include employees of the Food and Drug Administration, with expertise in pediatrics (including representation from the Office of Pediatric Therapeutics), biopharmacology, statistics, chemistry, legal issues, pediatric ethics, and the appropriate expertise pertaining to the pediatric product under review, such as expertise in child and adolescent psychiatry, and other individuals designated by the Secretary.'.	**Coordinated Internal Review Committee** New language establishes a coordinated internal review committee for BPCA and PREA.
[Original GAO report from PL108-155. Only included BPCA.]		
SEC. 16. REPORT ON PEDIATRIC EXCLUSIVITY PROGRAM. Not later than October 1, 2006, the Comptroller General of the United States, in consultation with the Secretary of Health and Human Services, shall submit to Congress a report that addresses the following issues, using publicly available data or data otherwise available to the Government that may be used and disclosed under applicable law:	**SEC. 404. GOVERNMENT ACCOUNTABILITY OFFICE REPORT.** Not later than January 1, 2011, the Comptroller General of the United States, in consultation with the Secretary of Health and Human Services, shall submit to the Congress a report that addresses the effectiveness of sections 505A and 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a, 355c) and section 409I of the Public Health Service Act (42 U.S.C. 284m) in ensuring that medicines used by children are tested and properly labeled.	**GAO Report** Requires the GAO to produce a report on the results of BPCA and PREA with recommendations for improving the programs.

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(1) The effectiveness of section 505A of the Federal Food, Drug, and Cosmetic Act and section 409I of the Public Health Service Act (as added by this Act) in ensuring that medicines used by children are tested and properly labeled, including— (A) the number and importance of drugs for children that are being tested as a result of this legislation 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 of drugs for children that are not being tested for their use notwithstanding the provisions of this legislation, and possible reasons for the lack of testing; and (C) the number of drugs for which testing is being done, exclusivity granted, and labeling changes required, including the date pediatric exclusivity is granted and the date labeling changes are made and which labeling changes required the use of the dispute resolution process established pursuant to the amendments made by this Act, together with a description of the outcomes of such process, including a description of the disputes and the recommendations of the Pediatric Advisory Subcommittee of the Anti-Infective Drugs Advisory Committee. (2) The economic impact of section 505A of the Federal Food, Drug, and Cosmetic Act and section 409I of the Public Health Service Act (as added by this Act), including an estimate of— (A) the costs to taxpayers in the form of higher expenditures by medicaid and other Government programs; (B) sales for each drug during the 6-month period for which exclusivity is granted, as attributable to such exclusivity; (C) costs to consumers and private insurers as a result of any delay in the availability of lower cost generic equivalents of drugs tested and granted exclusivity under the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 301 et seq.), and loss of revenue by the generic drug industry and retail pharmacies as a result of any such delay; and (D) the benefits to the government, to private insurers, and to consumers resulting from decreased health care costs, including—	Such report shall include— (1) the number and importance of drugs and biological products for children that are being tested as a result of the amendments made by this title and title V and the importance for children, health care providers, parents, and others of labeling changes made as a result of such testing; (2) the number and importance of drugs and biological products for children that are not being tested for their use notwithstanding the provisions of this title and title V and possible reasons for the lack of testing; (3) the number of drugs and biological products for which testing is being done and labeling changes required, including the date labeling changes are made and which labeling changes required the use of the dispute resolution process established pursuant to the amendments made by this title, together with a description of the outcomes of such process, including a description of the disputes and the recommendations of the Pediatric Advisory Committee; (4) any recommendations for modifications to the programs established under sections 505A and 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) and section 409I of the Public Health Service Act (42 U.S.C. 284m) that the Secretary determines to be appropriate, including a detailed rationale for each recommendation; and (5)(A) the efforts made by the Secretary to increase the number of studies conducted in the neonate population; and (B) the results of those efforts, including efforts made to encourage the conduct of appropriate studies in neonates by companies with products that have sufficient safety and other information to make the conduct of the studies ethical and safe.	

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(i) decreased hospitalizations and fewer medical errors, due to more appropriate and more effective use of medications in children as a result of testing and re-labeling because of the amendments made by this Act; (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 type of studies in children for each drug granted exclusivity under the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 301 et seq.), including— (A) a description of the complexity of the studies; (B) the number of study sites necessary to obtain appropriate data; (C) the number of children involved in any clinical studies; and (D) the estimated cost of each of the studies. (4) Any recommendations for modifications to the programs established under section 505A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a) and section 409I of the Public Health Service Act (as added by section 3) that the Secretary determines to be appropriate, including a detailed rationale for each recommendation. (5) The increased private and Government-funded pediatric research capability associated with this Act and the amendments made by this Act. (6) The number of written requests and additional letters of recommendation that the Secretary issues. (7) The prioritized list of off-patent drugs for which the Secretary issues written requests. (8)(A) The efforts made by the Secretary to increase the number of studies conducted in		

Existing Law	2007 Reauthorization	Improvements
the neonate population; and (B) the results of those efforts, including efforts made to encourage the conduct of appropriate studies in neonates by companies with products that have sufficient safety and other information to make the conduct of studies ethical and safe.		

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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REVISED PRIORITY LIST FOR STUDIES INTO OFF-PATENT PAEDIATRIC MEDICINAL PRODUCTS

NOTE and DISCLAIMER

The list includes only products considered to be off-patent, i.e. not covered by a basic patent or a supplementary protection certificate. Information on the off-patent status is not guaranteed by EMEA. It should be noted that information on the authorisation status as well as on available paediatric formulations of medicinal products is very limited and not available for all European Member States. Users of this list are therefore advised to check the patent and authorisation status of the medicinal products of interest.

The methodology used to establish the list was based as much as possible on evidenced based medicine. It is however acknowledged that identification of priorities for research into medicinal products for paediatric use is partly based on subjective criteria and that identified priorities may change over time.

OBJECTIVE OF THE LIST

The aim of Regulation (EC) 1901/2006 of the European Parliament and the Council on Medicinal Products for Paediatric Use, as amended, is to increase availability of medicines authorised for children as well as to increase the information available on the use of medicinal products in the paediatric population. The Regulation includes provisions for funding of studies into off-patent medicinal products. This funding, provided through the EU Framework programmes, should cover the development of off patent medicinal products with a view to the submission of a Paediatric Use Marketing Authorisation (Art. 40:
http://ec.europa.eu/enterprise/pharmaceuticals/eudralex/vol-1/reg_2006_1901/reg_2006_1901_en.pdf).

The objective of the revision of the priority list is to provide the basis for the work programme for the Third Call for Framework Programme 7 of the European Commission. It ensures that funds are directed into research of medicinal products with the highest need in the paediatric population.

The following list of off-patent products has been revised by the Paediatric Committee (PDCO) and was agreed on 29/08/2008.

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