



**United States  
General Accounting Office  
Washington, D.C. 20548**

---

**Office of the General Counsel**

B-281648

December 21, 1998

The Honorable James M. Jeffords  
Chairman  
The Honorable Edward M. Kennedy  
Ranking Minority Member  
Committee on Labor and Human Resources  
United States Senate

The Honorable Thomas J. Bliley, Jr.  
Chairman  
The Honorable John D. Dingell  
Ranking Minority Member  
Committee on Commerce  
House of Representatives

**Subject:** Department of Health and Human Services, Food and Drug Administration: Regulations Requiring Manufacturers to Assess the Safety and Effectiveness of New Drugs and Biological Products in Pediatric Patients

Pursuant to section 801(a)(2)(A) of title 5, United States Code, this is our report on a major rule promulgated by the Department of Health and Human Services, Food and Drug Administration (FDA), entitled "Regulations Requiring Manufacturers to Assess the Safety and Effectiveness of New Drugs and Biological Products in Pediatric Patients" (RIN: 0910-AB20). We received the rule on December 4, 1998. It was published in the Federal Register as a final rule on December 2, 1998. 63 Fed. Reg. 66632.

The final rule requires pediatric studies of certain new and marketed drugs and biological products. The rule will partially address the lack of pediatric use information by requiring that manufacturers of certain products provide sufficient data and information to support directions for pediatric use for the claimed indications.

Enclosed is our assessment of the FDA's compliance with the procedural steps required by section 801(a)(1)(B)(i) through (iv) of title 5 with respect to the rule. Our review indicates that the FDA complied with the applicable requirements.

If you have any questions about this report, please contact James Vickers, Assistant General Counsel, at (202) 512-8210. The official responsible for GAO evaluation work relating to the Department of Health and Human Services, Food and Drug Administration, is Bernice Steinhardt, Director, Health Services Quality and Public Health Issues. Ms. Steinhardt can be reached at (202) 512-7119.

Robert P. Murphy  
General Counsel

Enclosure

cc: The Honorable Donna E. Shalala  
The Secretary of Health and  
Human Services

ANALYSIS UNDER 5 U.S.C. § 801(a)(1)(B)(i)-(iv) OF A MAJOR RULE  
ISSUED BY  
THE DEPARTMENT OF HEALTH AND HUMAN SERVICES,  
FOOD AND DRUG ADMINISTRATION  
ENTITLED  
"REGULATIONS REQUIRING MANUFACTURERS TO ASSESS  
THE SAFETY AND EFFECTIVENESS OF NEW DRUGS AND  
BIOLOGICAL PRODUCTS IN PEDIATRIC PATIENTS"  
(RIN: 0910-AB20)

(i) Cost-benefit analysis

The FDA conducted a cost-benefit analysis of the final rule. FDA estimates the annual cost of the rule to be \$46.7 million--based on the annual costs of the rule from 1993 to 1997 if it had been effective in 1993.

FDA states that it could not develop a quantifiable estimate of the benefits of the rule. However, FDA estimates that for five illnesses (asthma, HIV/AIDS, cancer, pneumonia, and kidney infections) the reduction in hospitalization rate differentials (pediatric vs. adult) would yield annual savings of \$76 million.

(ii) Agency actions relevant to the Regulatory Flexibility Act, 5 U.S.C. §§ 603-605, 607, and 609

The Secretary of HHS has certified that the final rule will not have a significant economic impact on a substantial number of small entities.

(iii) Agency actions relevant to sections 202-205 of the Unfunded Mandates Reform Act of 1995, 2 U.S.C. §§ 1532-1535

The final rule does not contain an intergovernmental or private sector mandate, as defined by title II of the act, of over \$100 million a year.

(iv) Other relevant information or requirements under acts and executive orders

Administrative Procedure Act, 5 U.S.C. §§ 551 *et seq.*

The final rule was issued using the notice and comment procedures contained in 5 U.S.C. § 553. On August 15, 1997, FDA published a Notice of Proposed Rulemaking in the Federal Register. 62 Fed. Reg. 43900. FDA received 54 written comments in response to the proposal and also held a public hearing on October 27, 1997, with experts in the field, members of the pharmaceutical industry, and other

interested parties. FDA responds to the comments received in the preamble to the final rule.

#### Paperwork Reduction Act, 44 U.S.C. §§ 3501-3520

The final rule contains an information collection which is subject to review and approval by the Office of Management and Budget (OMB) under the Paperwork Reduction Act.

The preamble to the final rule contains the information required by the act, including the title of the collection, a description of the respondents, and the annual estimated burden hours. Based upon comments received and a request by OMB to consider all comments regarding the reporting burden, FDA has increased the annual burden hours to 40,571.

#### Statutory authorization for the rule

The FDA cites as authority for the issuance of the final rule sections 201(n), 201(p), 301(a), 301(d), 502(a), 502(f), 502(j), 505(a), 505(d)(7), 505(i), 505(k), and 701(a) of the Federal Food, Drug, and Cosmetic Act and section 351 of the Public Health Service Act.

#### Executive Order No. 12866

The final rule was determined to be an "economically significant" regulatory action and was reviewed and approved by the Office of Management and Budget as complying with the requirements of the order.