

# American Academy of Pediatrics



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October 5, 1998

Dockets Management Branch (HFA-305)  
Food and Drug Administration  
12420 Parklawn Drive  
Room 1-23  
Rockville, MD 20857

0353 '98 OCT -5 P2:1407 -5 P2:14

RE: Docket Number 98D-0265  
Guidance for Industry: Qualifying for Pediatric Exclusivity  
Under section 505A of the Federal Food, Drug and Cosmetic Act

Dear Sir or Madam:

The American Academy of Pediatrics, representing 53,000 pediatricians nationally, commends the Food and Drug Administration for the thoughtful and thorough draft Guidance for Industry: Qualifying for Pediatric Exclusivity. The AAP believes this is a document that outlines necessary and appropriate parameters for submissions of certain pediatric studies by the industry while maintaining the flexibility to design pediatric studies on a case-by-case basis.

The AAP wishes to express enthusiastic support for the requirement that reports of pediatric studies be submitted in accordance with requirements for filing of a supplement or new drug application. It is the view of the AAP that this requirement will be a critical component in moving toward the goal of getting more drugs labeled for pediatric populations. FDA's explanation and interpretation of the term *filing* is consistent with the intent of Congress to see that the pediatric exclusivity provisions yield more drugs that are labeled for children. As Representative John Dingell (D-MI), ranking minority member on the House Commerce Committee, noted in remarks on the House floor during consideration of the conference report on the Food and Drug Administration Modernization Act (FDAMA - PL 97-115), "Market incentives are included in the bill to encourage pediatric studies, so that labeling of these products will be useful to pediatricians."

There are several points which the AAP believes would be important for FDA to clarify or emphasize in revising the Guidance:

**The Written Request and its amendments should specify all pediatric populations that need to be studied to qualify for exclusivity.**

**There should be a single Written Request that encompasses both off-label indications that need pediatric studies AND labeled indications that need pediatric studies [sections 505A(a) and 505A(c) new drug applications.]** It is the understanding of the AAP that FDA may be issuing separate Written

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**effects and ineffective outcomes be cited on the label.** Data from studies in children that detect adverse effects should be incorporated into the label for the protection of children and the company. Although usually rare, negative findings should not be allowed to disadvantage children therapeutically through lack of dissemination or by being kept secret. Such information directly benefits the pediatric population by avoiding ineffective medications and providing better information for balancing potential gain and potential adverse effects.

In addition, the AAP asks FDA to explore the possibility of whether another provision of FDAMA (Section 113 – Information Program on Clinical Trials for Serious and Life-threatening Disease) could provide an avenue to disseminate useful information on pediatric studies that are not incorporated into the label.

**The AAP strongly urges FDA to immediately develop a tracking system of drugs on the pediatric list issued May 20, 1998.** Information collected as part of the tracking system would be of great interest to pediatricians and other health professionals. This information would also be essential in preparing FDA’s January 1, 2001 report to Congress on the effectiveness of the pediatric studies exclusivity program in improving information about important pediatric uses for approved drugs, as well as the adequacy of the incentive provided under this section. AAP would be eager to assist FDA in designing an appropriate tracking system.

A key element of the tracking system is identifying the scope, nature and type of labeling changes for various pediatric populations that were accomplished as a result of FDAMA. Other areas that need to be monitored include:

- Label changes
  - aggregate number of actual label changes made for pediatric populations
  - specific label changes made for pediatric populations, identifying the nature and type of changes
  - new formulations
  - expanded age ranges for treatment
  - new dosage guidance
  - newly identified potential adverse effects
- Which drugs have been studied from Docket No. 98N-0056 - the List of Approved Drugs for Which Additional Pediatric Information May Produce Health Benefits in the Pediatric Population (“the List”)
- Number of Written Requests that were rejected and why (if known)
- Proposed Written Requests submitted by industry that were not accepted by FDA.
- How information from studies was disseminated (e.g., labels, publications)
- Drugs granted exclusivity
  - marketed v. new drugs
  - classes
  - diseases
  - use frequency in children
  - number with new formulations
- Number of drugs granted exclusivity for an active ingredient
- Number of additional drugs added to “the List” each year.

The American Academy of Pediatrics appreciates the efforts of the Food and Drug Administration as it moves forward in the development of comprehensive approaches to advance the therapeutics for neonates, infants, children, and adolescents.

Sincerely,

A handwritten signature in black ink that reads "Joseph R. Zanga, MD". The signature is written in a cursive, flowing style.

Joseph R. Zanga, MD, FAAP  
President

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*The following organizations endorse these comments:*

American Pediatric Society  
Association of Medical School Pediatric Department Chairs  
National Association of Children's Hospitals  
Society for Pediatric Research