

American Academy of Pediatrics



Reply To:

Department of Federal Affairs
American Academy of Pediatrics
The Homer Building
601 Thirteenth Street, NW
Suite 400 North
Washington, DC 20005
202/347-8600
800/336-5475
Fax: 202/393-6137
e-mail: kids1st@aap.org
http://www.aap.org

Executive Committee

President

Donald E. Cook, MD

Vice President

Steve Berman, MD

Executive Director

Joe M. Sanders, Jr, MD

Board of Directors

Eileen M. Ouellette, MD, JD
Salem, Massachusetts

Robert M. Corwin, MD
Syracuse, New York

Susan S. Aronson, MD
Narberth, Pennsylvania

E. Stephen Edwards, MD
Raleigh, North Carolina

Stanford A. Singer, MD
Bloomfield Hills, Michigan

Ordean L. Torstenson, MD
Madison, Wisconsin

L. Leighton Hill, MD
Houston, Texas

Jon R. Almquist, MD
Federal Way, Washington

Lucy S. Crain, MD, MPH
San Francisco, California

Charles W. Linder, MD
Augusta, Georgia

00D-1336
Immediate Past President

Joel J. Alpert, MD

September 18, 2000

Dockets Management Branch (HFA-305)
Food and Drug Administration
5630 Fishers Lane
Room 1061
Rockville, MD 20852

RE: Docket Number 00D-1336
Draft Guidance for Industry: Pediatric Oncology Studies in Response to
a Written Request; Availability

Dear Sir or Madam:

The American Academy of Pediatrics, representing 55,000 pediatricians nationally, commends the Food and Drug Administration for the thoughtful and thorough draft Guidance for Industry: Pediatric Oncology Studies in Response to a Written Request. The AAP believes this is a document that outlines necessary and appropriate parameters for submissions of certain pediatric oncology studies by the industry while maintaining the flexibility to design pediatric studies on a case-by-case basis.

The Academy has been extremely active on the issue of identifying ways to improve the study of oncology drugs in children. In February 2000 the American Academy of Pediatrics hosted a small (25 individuals) invitational scientific meeting to discuss the therapeutic needs of children suffering from cancer. Invited guests included pediatricians, pediatric oncologists, clinical pharmacologists, and individuals from the federal government, industry and the parent/patient advocacy community. Among the issues identified are the delay in acquiring investigational new drugs for Phase I & II pediatric clinical trials and the implementation of the pediatric studies provision of the Food and Drug Administration Modernization Act (FDAMA) as it relates to oncology drugs.

The participants identified several changes in process to improve the treatment of children's cancer through increased study of new agents earlier in the drug development and approval process.

A clear consensus by participants was that the decisions about which drugs should be studied in children should be based upon the judgment of pediatric oncology scientists and clinicians with knowledge of the actions of the drugs and the biology of tumors in children. It is important to preserve the collaborative study of children's cancer therapy through research protocols, which have produced unprecedented improvements in outcomes for children over the last three decades. This process would avoid enrolling the limited number of children with cancer in trials of medications with little chance of success, thus avoiding an unethical exposure to a drug with limited likelihood of

0454 00 SEP 18 P2:09

C2

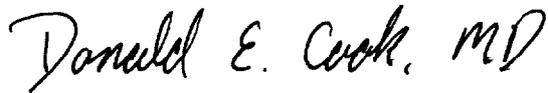
success and delaying study of more promising drugs. This would provide a solid basis of science for “directing” the study of oncology drugs in children.

Recommendation:

Based on the consensus reached at the February 2000 meeting, the Academy has only one suggestion to propose as FDA finalizes the draft guidance. In the introduction to the guidance, FDA should specifically encourage industry to collaborate directly with children’s cancer study groups to initiate a proposal for written requests to FDA. This would provide a solid basis of science for the study of oncology drugs in children. It would also provide FDA with a document that has the approval of children’s cancer study groups who can provide the best assessment of the types of oncology drugs and studies that are needed in the pediatric populations.

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 "Donald E. Cook, MD". The signature is written in a cursive style with a large, prominent "D" at the beginning.

Donald E. Cook, MD, FAAP
President

DEC:ev

The following organizations endorse these comments:

Ambulatory Pediatric Society
American Pediatric Society
Association of Medical School Pediatric Department Chairs
Society for Pediatric Research