

GERALD S GILCHRIST MD

4248 Linden Hills Blvd
Minneapolis MN 55410
612 920-2280
gilchrist.gerald@mayo.edu

2001 '02 JUL -8 A9:44

July 1, 2002

Dockets Management Branch
(HFA-305)
Food and Drug Administration
5630 Fishers Lane – Room 1061
Rockville, MD 20652

Docket Number: 02N-0152

Dear Sir or Madam:

I am a retired pediatric hematologist-oncologist who served as institutional Principal Investigator for NCI-sponsored therapeutic studies in infants, children and adolescents with various malignancies and as Director of the federally-funded Mayo Comprehensive Hemophilia Center for over 20 years, during which time I was intimately involved in evaluating new therapies. I also served as Chair of the Department of Pediatric and Adolescent Medicine at Mayo Clinic for 12 years. I welcome the opportunity to comment on the relationship between the 1998 Pediatric Rule and the Best Pharmaceuticals for Children Act (P.L. 107-109).

Although significant progress toward pediatric drug studies and labeling has been made over the last five years, much remains to be done if children are to benefit from the phenomenal advances in drug development. The Pediatric Rule provided the necessary incentive for pharmaceutical companies to conduct studies of new and marketed drugs in the pediatric age group.

The proposal by the FDA in March 2002 to suspend the Pediatric Rule would have been a major step backwards in our ability to safely and appropriately treat children with a wide variety of common and uncommon conditions. While this proposal was reversed, this action indicates that children are at risk of losing the ground we have fought so hard to secure for them.

The Pediatric Rule is an essential and successful tool in ensuring that children have access to the drugs they need. Since all new drugs must be studied for pediatric use at the time a drug comes to market unless the FDA grants a waiver, the availability of medications for children has become assured and not merely an option. For too many years we, as pediatric specialists had had to prescribe drugs that had never been studied or approved for use in our patient population

02N-0152

C 47

I believe that all components of the 1998 Pediatric Rule must be preserved as the only comprehensive approach to securing pediatric studies.

The following comments respond to questions and issues raised in the Federal Register notice soliciting public comments:

- Retiring or relaxing any authorities currently in the Pediatric Rule would be to the detriment of children. Also, BPCA is time-limited, voluntary and subject to continuation by the Congress. Those facts speak directly to the need to ensure that the Pediatric Rule remains in place in its entirety.
- Future Congressional authorization of BPCA is uncertain; the Pediatric Rule should mirror the scope of the BPCA and apply to all labeled and potential indications as well as new indications.
- In determining the process of when pediatric studies are conducted, the FDA should rely on the detailed process for requesting pediatric studies of already marketed drugs and securing labeling that is outlined in the BPCA.
- The Pediatric Rule is the only mechanism that ensures that biological products will be studied and available for children. The vast majority of biologics, some of the most innovative new therapies are covered under the Public Health Service Act and are not covered by BPCA.
- Appropriate formulations are an essential for the pediatric population. Failure to require needed formulations for specific age populations negates the intent of the BPCA and the Pediatric Rule.

Thank you for your consideration of these comments.

Sincerely,



Gerald S Gilchrist MD FAAP

4248 Linden Hills Blvd
Minneapolis MN 55410

Dockets Management Branch
(HFA-305)
Food and Drug Administration
5630 Fishers Lane-Room 1061
Rockville MD 20652

20#57/0001

