Date: Wednesday, August 13, 1997
FACT SHEET
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PRESIDENT CLINTON ANNOUNCES NEW MEASURES TO INCREASE AVAILABILITY OF INFORMATION ON SAFE USE OF MEDICATIONS USED TO TREAT CHILDREN

Today, President Clinton unveiled a new FDA regulation that will protect children by requiring drug manufacturers to study the safety and appropriate dosage levels of medications for **pediatric** populations. The regulation also requires proper labeling of drugs for use by children. Even though many drugs affect children differently than adults, most drugs have not been tested on **pediatric** populations. Under this rule, manufacturers of prescription drugs likely to be used by children will be required to complete studies and place information on drug labels to help pediatricians and other health care providers make scientifically-based treatment decisions when prescribing drugs to children.

WHY THIS REGULATION IS NEEDED

Most drugs -- even those commonly used in children -- have not been widely tested on pediatric populations. According to the American Academy of Pediatrics, only a small fraction of drugs and biological products marketed in the United States have had clinical trials performed on **pediatric** patients. Despite evidence that drugs affect children differently than adults, 80 percent of all drugs marketed in the United States have not been labeled for use by infants, children, and adolescents. Only forty-two percent of drugs that are widely used in **pediatric** populations have been tested on children.

- Many drugs commonly given to children under the age of six including Prozac, Zoloft, Ritalin, and drugs for asthma, allergic reactions, and ear infections are inadequately tested and labeled for use by children. These drugs, taken together, are given to over five million children each year.
- Less than half of the drugs used in the treatment of HIV infections carry any safety or effectiveness information for children. Of those that do, the data is often incomplete.
- Safety and effectiveness information is especially sparse for the over seven million children under the age of two.
- The percentage of drugs being tested on children decreased by over one-third between 1991 and 1996.

Drugs are likely to have a different impact on children than adults. The appropriate use and dosage levels of medication for children and adults is usually different because of disparities in organs, the immune system, and metabolism.

Children who take prescription drugs that have not been tested on pediatric populations are at serious risk for unexpected adverse reactions. Evidence suggests that prescribing drugs that have not been adequately tested on children can be extremely dangerous. One example of the possible harm is the case of "gray baby syndrome" where a number of babies died from chloramphenicol, an antibiotic that their immature livers were unable to accept. Other children had withdrawl symptoms from prolonged administration of fentanyl, a pain killer used as an adjunct to anesthesia in infants and small children. Still others have suffered seizures and cardiac arrest from bupivacaine, a local anesthetic not adequately tested on **pediatric** populations.

Some physicians are reluctant to prescribe much needed therapies to children because they have not been tested on pediatric populations. Physicians report that they have denied children important new drugs because, in the absence of adequate testing and labeling, they would have to guess at an

appropriate dosage, and they do not want to take that risk. As a result, too many children do not receive the treatment they need and deserve.

SUMMARY OF THE RULE

Pediatric Studies for New Drugs. Under this proposed rule, manufacturers of new drugs would have to do studies on **pediatric** populations under two circumstances: when the product represents a meaningful therapeutic benefit over existing treatments; or when the product is expected to be widely used on **pediatric** patients. The FDA anticipates that about twelve new products each year would meet this requirement. Manufacturers could receive waivers from the requirement to do a **pediatric** study under any one of the following circumstances:

1. The product does not represent meaningful benefits over existing treatments and is not likely to be used on a substantial number of **pediatric** patients as a whole; or

2. Necessary studies are impossible or highly impractical -- i.e., the number of patients is too small or geographically diverse; or

3. There is evidence strongly suggesting that the product would be unsafe or ineffective in **pediatric** populations.

Pediatric Studies for Existing Drugs. For drugs that are already on the market, the new FDA regulation requires additional testing on the **pediatric** population only if there is a "compelling need for more information." The criteria used is:

1. If the product is widely used by **pediatric** populations and the absence of adequate labeling could pose significant risks to **pediatric** populations; or

2. If the product is indicated for very significant or life threatening illnesses, but additional dosing or safety information is needed to permit its safe and effective use in **pediatric** patients.

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FOR IMMEDIATE RELEASE
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FDA PROPOSES TO REQUIRE PEDIATRIC DATA PRIOR TO DRUG AND BIOLOGIC PRODUCT APPROVALS

FDA has proposed a rule to make more medical treatments available to children. Published in today's Federal Register, the proposed rule would require **pediatric**-use data for medicines which may offer improved treatment for children over existing therapies.

Today's proposal would require companies to submit data that could then be put on a drug's **label** to help pediatricians and other health care providers make treatment decisions based on scientific information about their patient population.

"Kids deserve the same access to newly developed drugs that their parents get," said Donna E. Shalala, secretary of Health and Human Services. "With this proposal, we will have the power to ensure pediatricians and other health care providers who treat children have the best scientific information available on which to base their medical decisions."

Even for drugs that are already marketed, this regulation would codify FDA's authority to require, in compelling circumstances, that manufacturers conduct studies to support **pediatric**-use labeling for the approved indications.

In 1994, the Food and Drug Administration issued a regulation to encourage drug manufacturers to submit **pediatric** data voluntarily for review. However, many new drugs are still being approved without information on how they should be used in children.

Without adequate information, physicians may be reluctant to prescribe certain drugs for their **pediatric** patients, or they may prescribe them inappropriately.

"When drug labels do not include **pediatric** information, health care providers are forced to play a guessing game and may not properly dose their patients," said Michael A. Friedman, M.D., FDA lead deputy commissioner. "Not only does this mean sick kids sometimes don't get better, but they also have the potential to get worse as a result of unexpected adverse events."

The proposed rule would allow post-approval submission of **pediatric** data if FDA had safety concerns about testing the drug on children prior to approval.

Likewise, the requirement could be waived if:

- FDA found that the product was likely to be unsafe or ineffective in **pediatric** patients
- that **pediatric** studies were impossible or highly impractical
- that reasonable efforts to develop a **pediatric** formulation had failed.

FDA invites written comments on the proposal from the public and industry, which may be submitted to the Dockets Management Branch, HFA-305, Food and Drug Administration, 12410 Parklawn Drive, Room 1-23, Rockville, MD 20857. The closing date for comments is DATE, 1997. All comments received will be reviewed and considered by the agency in developing the final rule.

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