File Pediatric labeling

Draft 8/11/97 12:30pm

PRESIDENT WILLIAM J. CLINTON
REMARKS ON PEDIATRIC-DOSE LABELING
THE WHITE HOUSE
AUGUST 14, 1997

Thank you, Mrs. TK. Thank you for sharing TK's story with us.

It is simply unacceptable that our children do not have the benefit of all of the medical advances science has offered our adults. And that is why I will take executive action to put an end to this disparity. This Administration will act to make prescription medicines safe for America's children.

TK's story is not unique. Far too many medicines -- a majority, in fact -- lack adequate information on safe and effective doses for children. Far too many pediatricians are forced to guess the proper doses or forego what might be the very best treatment available. Far too many parents are left to worry that their children are not getting the best possible care.

Small children are particularly vulnerable to problems when doctors don't know precisely how much of an important medicine to give. I'll give you just one example. A number of years ago, 33 infants died after being given what looked like reasonably small doses of a crucial antibiotic commonly used in adults. It turned out that the infants were unable to clear the medicine from their bodies and deadly amounts built up in their livers. The antibiotic simply hadn't been studied adequately in young children. Guessing -- even educated guessing -- had deadly results. ~~And today, There are still many drugs today~~

This looks fine

The rule we are making public today will put an end to the guesswork and give parents and pediatricians the dose information they desperately need. This new rule will require the manufacturers of medicines needed urgently by children to study the effects in young people and provide information on dosages. And the rule will apply not only to innovative new medicines now in development but also to medicines already on the market. But keep in mind, in both cases the rule will target only the medicines for which a lack of dose information puts children at risk.

And let me be clear on another point: If a manufacturer fails to do the studies in children, the FDA will not reject a new drug or pull an older one from the shelves. That, of course, might deprive adult patients of important therapies. Instead, we will seek a federal court order to ensure that the proper studies are done.

Senator Dodd has also introduced legislation that would provide financial incentives for drug manufacturers to perform the needed studies in children. I applaud Senator Dodd's

approach. It is compatible with the rule we are introducing today, and I hope that when the Congress returns from recess and gets back to work on ~~streamlining the FDA~~ comprehensive FDA reform, ~~Congress will send me a bill that contains this provision and I can sign it into law.~~ I look forward to ~~[illegible]~~ work w/ Senator Dodd & others on this issue.

Through the common-sense initiative I have announced today, we will take one more significant step toward assuring quality health care for our children. Last week, I signed a balanced budget agreement with the largest new commitment to health care for children since the Medicaid was enacted, 32 years ago. And now we are working to ensure that the treatments we give our children are safe and that cutting-edge medicines will be available to children just as soon as they are available to adults.

We owe this much to parents like TK and Elizabeth Glazer, whom the First Lady just spoke of a few minutes ago. We owe this to every parent and every child. As the First Lady says, children are not rugged individualists; they depend upon us to give them love, guidance, discipline and the benefit of the best medical care. Their future and ours depends on how well we do our job.

Draft 8/12/97 8pm

PRESIDENT WILLIAM J. CLINTON
REMARKS ON PEDIATRIC-DOSE LABELING
THE WHITE HOUSE
AUGUST 13, 1997

Acknowledgments: V.P. Gore, the First Lady; Rep. Jim Greenwood; Dr. David Kessler, who was instrumental in preparing this rule; Dr. Michael Friedman, Acting Commissioner of the FDA; Reagan Ralph, who introduces you.

Little Samuel's story is not unique. According to the American Academy of Pediatrics, more than 50% of medicines that have proved helpful for children have not been adequately tested for children's use. That is simply unacceptable. Today, I will take executive action to ensure that parents and pediatricians will have the safety information they need.

Doctors have long known that children react differently from adults to prescription drugs. In many cases, children must have vastly scaled down doses. In some cases, their bodies have not developed enough to take any quantity of a medicine that has been proven safe for adults.

We are aware of these dangers, and yet we still do not have good information about medications for some of the most common childhood illnesses -- like asthma, allergic reactions, and ear infections. We also don't know enough about medications to treat life-threatening diseases. Less than half of the drugs used to treat the estimated 12,000 children with HIV infection have been tested for use in children. Information is particularly sparse for children under two.

Without clear guidance, pediatricians sometimes decide not to prescribe for children drugs used successfully by adults. That means children are sometimes deprived of what might be the very best treatment available.

The pediatrician's other alternative is to guess. But guessing -- even educated guessing -- can have grave consequences. Some time ago, doctors gave infants small doses of a crucial antibiotic commonly used by adults. It turned out that the infants were unable to clear the drug from their bodies and large amounts built up in their livers. Because needed dosage studies had not been done, 23 infants died.

The rule I am announcing today will put an end to the guesswork. The new rule I am announcing today will require the manufacturers of all medicines needed by children to study the drugs' effects in young people. The results will then be displayed on drug labels to help pediatricians and other health-care professionals make good decisions about how to treat their young patients. I am pleased to report that groups representing patients, physicians, nurses,

pharmacists, and drug manufacturers have all indicated their willingness to help implement this new rule.

I want to applaud Senators Dodd and DeWine and Congressmen Greenwood and Waxman, who have introduced legislation that would provide additional incentives for drug manufacturers to perform the needed dosage studies in children. Their approach is compatible with the rule we are introducing today. I look forward to working with them on this issue as the Congress continues our efforts to pass comprehensive FDA reform this fall.

Through the common-sense initiative I have announced today, we will take one more significant step toward assuring quality health care for our children. Last week, I signed a balanced budget agreement with the largest new commitment to health care since Medicaid was enacted, more than three decades ago. Under this law, as many as 5 million uninsured children will get health care coverage.

Now we are working to ensure that the treatments we give our children are safe and that cutting-edge medicines are available to children just as soon as they are available to adults. We owe this much to parents like Reagan Ralph and Elizabeth Glaser, whom the First Lady just spoke of a few minutes ago. We owe this to every parent and every child. As the First Lady says, children are not rugged individualists; they depend upon us to give them love, guidance, discipline, and the benefit of good medical care. Their future and ours depends on how well we do our job.

FAX Transmission

U.S. Department of Health and Human Services
Office of the Secretary

DATE: 8/12/97

TO: Jennifer Klein

TO: SARAH
FROM: JEN

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PHONE:

FROM: Delia Ibarra & Laura Schiller
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TOTAL NUMBER OF PAGES TRANSMITTED:

COMMENTS:

Secretary's remarks for tomorrow.

Mr. President, Mr. Vice President, Mrs. Clinton, Mrs. [parent], other distinguished guests:

Today we announce another dose of good medicine for our children's health -- and another victory for good government.

As our kids get ready to head back to school, their parents are worrying about whether they'll have the right books, the right classes, and, even the right clothes.

But, when a child gets sick there's one thing parents should never have to worry about -- and that's whether their children will have the right medicines -- safe medicines. And, that's why we're here.

Over the last four years, we have fought hard to make sure that all citizens have access to promising new drugs that are safe, effective, and arrive on time.

The results? More cancer drugs are reaching people who need them -- when they need them.

Drug approval times have been slashed in half -- and are now as fast or even faster than every other nation.

More specific

Miles of red tape have been cut. Piles of regulations have been thrown away.

And we're working with the Congress to reach consensus on balanced FDA reform.

But, even as we work to make the FDA more efficient, there is one thing we must never do: We must never lose sight of -- or compromise -- its fundamental mission:

... To promote the public health, to maintain the public trust, and to improve the lives of all Americans -- especially children.

Every once in awhile, we have an extraordinary opportunity to do that.

When an FDA doctor named Francis Kelsey was faced with a decision about approving Thalidomide more than 30 years ago, she knew she had an opportunity to prevent birth defects in countless children.

And she did it.

When this Administration was faced with a decision about whether to regulate tobacco use by children, we knew we had the opportunity to save an entire generation from a deadly addiction. And we did it.

And now, once again, we see too many children placed in harm's way.

Too many children with asthma, depression, and other ailments and not enough information about what medicines and what dosages will help them.

Too many pediatricians forced to play a high-stakes guessing game about whether to forgo an effective treatment or risk prescribing one that could hurt -- not heal -- a child.

Now, once again, as a government and as a nation, we have an opportunity -- and a responsibility -- to act.

We have an opportunity to place our most exciting scientific discoveries firmly within the grasp of every American child. And today we are.

Now, I'm very pleased to introduce a leader who has dedicated his entire life to keeping our earth in the balance, to ensuring that our science and technology are on the cutting-edge, and to always putting our public health first.

Through his words and deeds, he has created a vision for good government that can protect our health today and meet our challenges tomorrow. It is my great honor to introduce Vice President Al Gore.

Date: August 12, 1997

RE: The proposed FDA regulation to provide health care professionals with more information regarding pediatric doses drugs.

While your intentions may be noble, the proposed regulation will further confuse the public by allowing health professionals and, in turn, parents to assume that the FDA has required a careful well-controlled study before making recommendations for drug doses intended for infants and children.

Any regulation, intended to protect the safety and well-being of infants and children, by providing health care professionals with more information regarding pediatric doses of a drug, must include a requirement that the drug manufacturer disclose whether or not those dosages have been determined by well-controlled, randomized trials and approved by the FDA. The information should also discuss whether or not a follow up has been carried out to determine the long-term effects of the drug on child development.

Respectively submitted,

Doris Haire

Doris Haire, President

FOR IMMEDIATE RELEASE
August 13, 1997

Contact: Mark Grayson
(202) 835-3465

PHARMACEUTICAL INDUSTRY COMMITTED TO CHILDREN'S HEALTH; WILL WORK WITH CLINTON ADMINISTRATION TO PROMOTE THAT GOAL

PhRMA President Alan F. Holmer issued the following statement today in response to a White House proposal mandating clinical studies of drugs in children:

As a parent -- more specifically, as the father of two children with cystic fibrosis -- I want more than anything in the world to see more and better medicines developed to help and heal children.

The pharmaceutical industry is committed to this goal. A 1997 survey found 146 new medicines in development for children, including 36 for cancer, the leading disease killer of children; 9 for AIDS, the prime cause of death among young children in some cities; and 5 for cystic fibrosis, the most common fatal genetic disease in the United States. All 146 medicines have been or are being tested in children; all of the 75 companies developing these medicines intend to seek the Food and Drug Administration's approval for pediatric use of these drugs.

In view of all the ongoing testing and studying of medicines in children, we question whether a government mandate is needed. However, we are committed to working with the President and others in the Administration, on a collaborative and constructive basis, regarding their proposed regulation to advance the goal of better medicine for children -- a goal we all share.

- more -

With that goal in mind, any regulation should:

- First and foremost, protect the safety of children and avoid exposing them to premature or unnecessary risk.

  For example, before beginning testing on kids, we want to ensure that a potential drug is truly viable, and that we adequately understand how adult patients respond to it.

- Recognize the enormous practical difficulties of testing drugs in children and developing formulations of medicines appropriate for children.

  For example, one manufacturer has tried and failed more than 1,000 times to develop a pediatric formulation of one antibiotic drug. Another company unsuccessfully spent more time, money, and human resources on a failed syrup than on the adult formulation and the chewable version combined.

- Be precisely worded and flexibly applied so that testing in children does not delay the drug development and approval process.

  We need to get safe and effective medicines to all Americans -- children and adults -- as rapidly as possible. But under no circumstances should a drug be withheld from the market after it has been found to be safe and effective for either adults or children.

Pharmaceutical companies are leading the way in the search for cures for the diseases that kill and disable both children and adults. We believe the best way the government can help patients is to enact S. 830, the FDA Modernization and Accountability Act of 1997, which will ensure that all patients -- children, their parents, their grandparents, other adults -- have timely access to the fruits of biomedical research.

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Devil in Detail

My name is Annie Cefaratti. I am an American Lung Association volunteer and parent of a child with cystic fibrosis. My son, Anthony was first diagnosed with cystic fibrosis as an infant. For the past 12 years, Anthony and our family have learned how to live with cystic fibrosis. I love Anthony. I count every day Anthony's life a gift.

Anthony's gift of life would not be possible without medical care and the prescription drugs that have allowed him live a happy and fulfilling life. One of the drugs my son uses to manage his cystic fibrosis is a class of drugs called quintalones. While I am grateful for the prescription drugs that have helped my son live, I also have questions.

How will this drug effect my child?

Are there any risks in this drug unique to a child?

Is the recommended dosage based on the needs of child?

Clearly these are questions a parent would want answered. For some drugs prescribed, like the quintalones that Anthony takes, doctors do not really know the answers to these questions. Without this information, I worry that we are essentially, "dosing in the dark."

Anthony needs drugs to keep his CF in check. I would not want any action taken that would slow the development of new drugs to treat Anthony's CF. But I also want information that will help our physician and my family make informed choices about Anthony's health.

I am pleased that President Clinton has announced the new requirements for pediatric drug labeling. The information that the proposed FDA rule provides will help parents and physicians make treatment decisions based on scientific studies. The rule will help ensure my Anthony and all children receive the safest and most effective medicine that science can provide.

Again, Mr. President, thank you for your leadership in ensuring the health and safety of our nation's children.

62578

Juvenile Diabetes Foundation International
The Diabetes Research Foundation
Government Relations

August 12, 1997

Contact: Eric Schutt (202) 371-2760

Statement Provided by the Juvenile Diabetes Foundation International (JDFI) Regarding Proposed Regulation on Pediatric Labeling:

The Juvenile Diabetes Foundation International (JDFI) applauds President Clinton's leadership in attempting to improve the health care of all children, particularly those who suffer from diabetes. This announcement demonstrates an important understanding that children are not simply small adults, and therefore may require additional measures to ensure that they receive the best possible treatments currently available.

1400 I Street N.W. · Suite 500 · Washington, D.C. 20005 · 202-371-9746 · Fax: 202-371-2760

"A physician's clinical decision's must be based on scientific evidence, and the Administration's action will take us another step towards evidence-based medical practice." American College of Physicians.

National Association of Pediatric
Nurse Associates & Practitioners, Inc.

August 12, 1997

President William Jefferson Clinton
1600 Pennsylvania Avenue, N.W.
Washington, D.C. 20500

Dear President Clinton:

On behalf of the 5,300 members of the National Association of Pediatric Nurse Associates and Practitioners and the children they serve, I commend you for your leadership in addressing pediatric pharmaceutical research and labeling.

As providers of health care for children, we understand the great importance of pharmaceutical research that recognizes children are not "little adults" -- their metabolism, response rates, and tolerance levels are different from adults.

We greatly appreciate the Administration's effort to ensure that pharmaceuticals are adequately tested in children and that such studies are used to support better labeling for pediatric drugs. Giving health care providers adequate and appropriate information about usage is crucial in ensuring the health and safety of our nation's children.

Thank you again for keeping our children's interests at the forefront of national health policy. We look forward to assisting the Administration in development of these new regulations.

Sincerely,

Patricia Franklin

Patricia Franklin, MSN, RN, CPNP
President

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E-mail: 74224.51@Compuserve.Com Internet: www.pcnap.org

CENTER FOR MEDICAL CONSUMERS

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(212) 674 7105 ▫ FAX (212) 674 7100

August 12, 1997

President Bill Clinton
The White House
Washington, D.C.

Dear President Clinton:

I am writing to express my support for the FDA's proposed requirement that drug manufacturers provide more labeling information about the safety and efficacy of their products for use in pediatric populations.

Prescription drugs are used to treat all segments of the population, yet the clinical trial experience on which their approval is based often excludes the very young and the very old. Once a drug is marketed, however, it is widely prescribed even though the thresholds for safety and effectiveness may differ among age groups.

It is time to stop putting children unnecessarily in harm's way by improving the information required on drug labeling pertaining to pediatric use.

Sincerely,

Arthur Aaron Levin, MPH
Director

Fran Du Melle
Director
Government Relations

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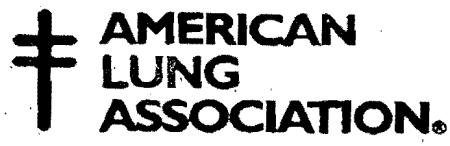
Marilyn Hansen
Executive Director, ATS

AMERICAN THORACIC SOCIETY



August 12, 1997

William J. Clinton
1600 Pennsylvania Avenue, NW
Washington, DC 20500

Mr. President:

On behalf of the American Lung Association and its medical section, the American Thoracic Society, we want to congratulate you for the Food and Drug Administration's (FDA) proposed rule on Pediatric Drug Labeling requirements. The proposed rule you have announced today will help the FDA, health care providers, the pharmaceutical industry and patients reach a needed consensus on labeling and information requirements for pediatric prescription drugs.

The pharmaceutical industry has improved the lives of millions of American children by developing new drugs to treat the array of pediatric diseases. However, some of the prescription drugs were initially developed with adults in mind and are used to treat children as almost an after-thought. Drugs are often used to treat children without understanding the unique health and dosing needs of a child. Our nation's children are not after-thoughts.

Today the goal should be for health care providers, pharmaceutical companies and patients to work together to collect and distribute information on the pediatric application of prescription drugs. Mr. President, I applaud your announcement today as being a first step in reaching that goal.

Sincerely,

Donald I. Clark
President,
The American Lung Association

Talmadge E. King
President,
The American Thoracic Society

When You Can't Breathe, Nothing Else Matters®

Founded in 1904, the American Lung Association includes affiliated associations throughout the U.S., and a medical section, the American Thoracic Society.

Official Journals

American Journal of Respiratory and Critical Care Medicine

American Journal of Respiratory Cell and Molecular Biology

File Pediatric labeling

PRESS RELEASE

For Immediate Release

Contact:

Diane Dorman
Manager, Communications
202.833.9070

GPIA'S TILL TO ATTEND WHITE HOUSE CEREMONY ON PEDIATRIC DRUG LABELING

Washington, DC, Tuesday, August 12, 1997— Dr. Alice Till, President of the Generic Pharmaceutical Industry Association, has been invited to attend a Rose Garden ceremony at the White House tomorrow when President Clinton will announce an Administration initiative to identify the proper pediatric dosage for drugs commonly used by children.

Based on the FDA's proposed criteria that a drug must be used widely by children and have a "meaningful therapeutic benefit," FDA officials say there may be about 12 new drugs a year that require pediatric studies. For those drugs already on the market, additional studies will be needed only if a drug is widely used by children and continued use of the drug by the pediatric population "poses a significant health risk."

"Up until now, definitive information for the administration of certain drugs to children has not been available to pediatricians and other healthcare providers. So doctors have been hesitant to prescribe these drugs without the assurance that they are safe and effective in this important group of patients," said Till. "The FDA is to be commended for taking the initiative to propose regulations to address these issues. The Administration has taken an important first step towards ensuring that American children receive the very best pharmaceutical care in the world. The generic industry applauds the White House for its strong public support of this initiative," continued Dr. Till.

GPIA represents the leading manufacturers and distributors of generic medicines, and related companies. Its mission is to insure that the American consumer is provided with the highest quality generic drugs at the lowest possible cost.

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C. EVERETT KOOP, M.D.

August 12, 1997

Honorable William J. Clinton
The President
The White House
1600 Pennsylvania Avenue
Washington, DC

Dear Mr. President:

I am writing to applaud the efforts of you and your Administration on children's pharmaceuticals. After long years of working with inadequate data, the nation's physicians, pharmacists, and parents will finally have solid information about the safety and dosing of medicines for children. I am very pleased that you have moved to require that drugs be tested for pediatric patients unless there's a good reason not to do so. This is a common-sense and workable policy that will do a lot of good.

Throughout my medical career of working with children one-by-one and my public health career of working with the children of America, I know how desperate families can become when their sons and daughters are sick. The current drug approval system usually leaves them with only two choices: Give the children drugs that have not been tested for safety or withhold a treatment that works in adults. It is an unsatisfactory choice.

Instead, the policy you are proposing would create a new and better choice: drugs that have been studied for pediatric use. It is the right policy to choose. We should ensure that children benefit from biomedical research and medical progress as much as adults do. You should be congratulated on taking this step.

Sincerely yours,

C. Everett Koop, M.D., Sc.D.

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Juvenile Diabetes Foundation International
The Diabetes Research Foundation

Government Relations

August 12, 1997

Contact: Eric Schutt (202) 371-2760

Statement Provided by the Juvenile Diabetes Foundation International (JDFI)
Regarding Proposed Regulation on Pediatric Labeling:

The Juvenile Diabetes Foundation International (JDFI) applauds President Clinton's leadership in attempting to improve the health care of all children, particularly those who suffer from diabetes.. This announcement demonstrates an important understanding that children are not simply small adults, and therefore may require additional measures to ensure that they receive the best possible treatments currently available.

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National Organization for Rare Disorders, Inc.®

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. . out of the darkness, into the light . . .

Member Organizations:
Alliance of Patients Support Groups
Alpha 1 Antitrypsin Deficiency National Association
ALS Association
American Brain Tumor Association
American Porphyria Foundation
American Society of Adults with Pseudo-obstruction, Inc. (ASAP)
American Syringomyelia Alliance Project
Aplastic Anemia Foundation of America
Association for Glycogen Storage Disease
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation, Inc.
Charcot-Marie-Tooth Association
Chromosome 18 Registry and Research Society
Cornelia de Lange Syndrome Foundation, Inc.
Cystinosis Foundation, Inc.
Dysautonomia Foundation, Inc.
Dystonia Medical Research Foundation
Dystrophic Epidermolysis Bullosa Research Assoc. (D.E.B.R.A.)
Ehlers-Danlos National Foundation
Epilepsy Foundation of America
Families of Spinal Muscular Atrophy
Fanconi Anemia Research Fund, Inc.
Foundation Fighting Blindness
Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)
Guillain-Barre Syndrome Foundation International
HHT Foundation International, Inc.
Hemochromatosis Foundation, Inc.
Hereditary Disease Foundation
Histiocytosis Association of America
Human Growth Foundation
Huntington's Disease Society of America, Inc.
Immune Deficiency Foundation
International Fibrodysplasia Ossificans Progressiva (F.O.P.) Assoc., Inc.
International Joseph Diseases Foundation, Inc.
International Rett Syndrome Association
Interstitial Cystitis Association of America, Inc.
Lowe's Syndrome Association
Malignant Hyperthermia Association of the United States
Myasthenia Gravis Foundation
Myeloproliferative Disease Center
Myositis Association of America
Mucolipidosis Type IV Foundation (ML4)
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National Adrenal Diseases Foundation
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National Hemophilia Foundation
National Leigh's Disease Foundation
National Marfan Foundation
National Mucopolysaccharidoses Society, Inc.
National Multiple Sclerosis Society
National Neurofibromatosis Foundation
National PKU News
National Sjogren's Syndrome Association
National Spasmodic Torticollis Association (NSTA)
National Tay-Sachs & Allied Diseases Association, Inc.
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National Urea Cycle Disorders Foundation
Neurofibromatosis, Inc.
Obsessive Compulsive Foundation
Osteogenesis Imperfecta Foundation
Oxalosis and Hyperoxaluria Foundation
Paget Foundation
Parkinson's Disease Foundation, Inc.
Prader-Willi Syndrome Association
Reflex Sympathetic Dystrophy Syndrome Association
Scleroderma Federation, Inc.
Scleroderma Info Exchange, Inc.
Sickle Cell Disease Association of America, Inc.
Tourette Syndrome Association, Inc.
Trigeminal Neuralgia Association
United Leukodystrophy Foundation, Inc.
United Parkinson Foundation
United Patients' Association for Pulmonary Hypertension, Inc.
Vestibular Disorders Association
VHL Family Alliance
Wegener's Granulomatosis Support Group, Inc.
Williams Syndrome Association
Wilson's Disease Association

August 12, 1997

President Bill Clinton
The White House
1600 Pennsylvania Ave.
Washington, DC
Attention: Barbara Wooley

Via FAX: 202-456-6218

Dear President Clinton:

The National Organization for Rare Disorders (NORD), representing an estimated 20 million Americans with more than 5,000 rare "orphan diseases," applauds your important initiative to ensure that pediatric information is included on pharmaceutical labeling. As you are keenly aware, until now most prescription drugs have not been tested on children, and appropriate dosage information for pediatricians is absent from product labeling.

Your new FDA regulation requiring pediatric data on new drugs is long overdue and essential to the well being of ill children throughout the nation. It is vitally important that henceforth manufacturers will be required to include children in clinical trials, and that data from pediatric studies will be included on pharmaceutical labeling. The inclusion of an enforcement mechanism is vital to the success of the new regulation, ensuring that all manufacturers will obey the new rule.

Mr. President, on behalf of the millions of American children who are afflicted by rare disorders, most of which are genetic, we are truly grateful for your leadership and concern for improving the lives of America's children.

Very truly yours,

Abbey S. Meyers
President

Associate Members

A-T Project
Angelman Syndrome Newsletter, Inc.
ALS Association/Greater Philadelphia Chapter
American Autoimmune Related Diseases Assoc.
American Behcet's Disease Association, Inc.
American Laryngeal Papilloma Foundation
American Pseudo-obstruction & Hirschsprung's Disease Society, Inc.
American Self-help Clearinghouse
Androgen Insensitivity Group
Angel View Crippled Children's Foundation
Arc of Ohio
Ataxia Telangiectasia Children's Project
Canadian Organization for Rare Disorders
Center for Research in Sleep Disorders
Children's Leukemia Foundation/MI
Children's PKU Network
Chronic Granulomatous Disease Assoc. Inc.
Consortium of Multiple Sclerosis Centers
Cooley's Anemia Foundation
Cushing Support & Research Foundation
Earl J. Goldberg Aplastic Anemia Foundation
Family Caregiver Alliance
Footsteps Inside
Freeman-Sheldon Parent Support Group
Help Hospitalized Children's Fund
Hydrocephalus Association
International Foundation for Alternating Hemiplegia of Childhood
J.J.J.s.P. Foundation
Klippel-Trenaunay Support Group
Lake Chase Tay-Sachs Foundation
Lyme Disease Foundation
National Association for Pseudoxanthoma Elasticum
National Head Injury Foundation
National Incontinentia Pigmenti Foundation
National Lymphedema Network
National Niemann-Pick Disease Foundation
National Patient Air Transport Helline
National Spasmodic Dysphonia Association
Neuropathic Support Association
Osteoporosis and Related Bone Diseases National Resource Center
PXE International, Inc.
Parent to Parent of GA, Inc.
Parent to Parent of New Zealand
Research Trust for Metabolic Diseases in Children/UK
Restless Legs Syndrome Foundation
Serious Nephrotic Association
Shwachman Syndrome Support
Shy-Drager Syndrome Support Group
Sickle Cell Association of the Texas Gulf Coast
Society for Progressive Supranuclear Palsy, Inc.
Soto's Syndrome Support Group
Sturge-Weber Foundation
Tourette Syndrome Association of MO, DC & VA
Treacher Collins Foundation
Tuberous Sclerosis Association of IL
United Scleroderma Foundation, Inc.
Valnoir Ms Metabolic Lysosomales/ France

*Associations are joining continuously. For newest listing, please contact the NORD office.

C. EVERETT KOOP, M.D.

August 12, 1997

Honorable William J. Clinton
The President
The White House
1600 Pennsylvania Avenue
Washington, DC

Dear Mr. President:

I am writing to applaud the efforts of you and your Administration on children's pharmaceuticals. After long years of working with inadequate data, the nation's physicians, pharmacists, and parents will finally have solid information about the safety and dosing of medicines for children. I am very pleased that you have moved to require that drugs be tested for pediatric patients unless there's a good reason not to do so. This is a common-sense and workable policy that will do a lot of good.

Throughout my medical career of working with children one-by-one and my public health career of working with the children of America, I know how desperate families can become when their sons and daughters are sick. The current drug approval system usually leaves them with only two choices: Give the children drugs that have not been tested for safety or withhold a treatment that works in adults. It is an unsatisfactory choice.

Instead, the policy you are proposing would create a new and better choice: drugs that have been studied for pediatric use. It is the right policy to choose. We should ensure that children benefit from biomedical research and medical progress as much as adults do. You should be congratulated on taking this step.

Sincerely yours,

C. Everett Koop, M.D., Sc.D.

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SENT VIA FACSIMILE TO 202/456-6218

August 12, 1997

The White House
Office of Public Liaison
Attention: Ms. Barbara Wooley
122 Old Executive Office Building
Washington, D.C. 20502

Re: Proposed Regulation on Pediatric Labeling

Dear Ms. Wooley:

Throughout its 102 year history, the National Medical Association has been a leading voice to eliminate disparities in health, particularly among the poor, minorities, and underserved populations. Because of limitations in testing of many drugs in pediatric populations and labeling for pediatric use of specific drugs, children can be considered an underserved population for certain drug therapies. The proposed regulation requiring pediatric-use data on new drugs, with provisions pertaining to existing drugs, has the potential for improving available treatments and increasing the effectiveness and safety of treatments for this vulnerable population.

Sincerely,

Nathaniel H. Murdock

Nathaniel H. Murdock, M.D.
President

BARBARA BOXER
CALIFORNIA

COMMITTEES:
APPROPRIATIONS
BANKING, HOUSING, AND URBAN AFFAIRS
BUDGET
ENVIRONMENT AND PUBLIC WORKS

United States Senate

HART SENATE OFFICE BUILDING
SUITE 112
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(202) 224-3553
senator@boxer.senate.gov
http://www.senate.gov/~boxer

August 11, 1997

The President
The White House
Washington, DC 20500

Dear Mr. President:

I write in support of your Administration's proposed regulations on children's pharmaceuticals. These new regulations have the potential to do much to improve child health in America. Your actions to raise the visibility of them will also educate parents across the nation regarding important health issues involved in giving prescription drugs to children.

For too long the routine course has been that drugs will be tested only for adult use and that parents, pediatricians, and pharmacists will be left without solid data on which to base decisions regarding treatments for a sick child. I am informed that 80 percent of drugs on the market have never been tested for safety in children, even drugs that are routinely prescribed for them. I am pleased that these regulations will finally address this deficiency.

When the FDA regulations become final, I am informed that the new presumption will instead be that drugs will be tested for children's use unless there is a reason not to. This is clearly the appropriate way to proceed.

I am particularly touched that much of this action has been undertaken in the spirit of Elizabeth Glaser, for whom I had the highest regard. From the first moment that I met her, to the time she testified before Congress at my invitation, to the last days of her life, Elizabeth worked tirelessly to get drugs and drug research for children. She was near desperation when she first discovered that the drug that she herself was taking was not available to treat her daughter, Ariel. She and the Pediatric AIDS Foundation, which she co-founded, set themselves a goal of ensuring that this does not happen again--for children with AIDS or for children with any serious disease.

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SAN FRANCISCO, CA 94111
(415) 403-0100

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SUITE 545
EL SEGUNDO, CA 90245
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2500 TULARE STREET
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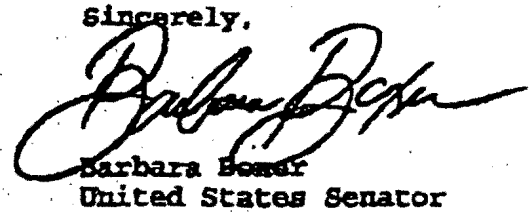
210 NORTH E STREET
SUITE 210
SAN BERNARDINO, CA 92401
(909) 889-8575

PRINTED ON RECYCLED PAPER

I know I speak for the Foundation--and, I am somehow sure, for Elizabeth--when I thank you for your actions, congratulate you on the new proposals, and express every hope and wish that the broadest possible number of children will benefit.

Sincerely,



Barbara Boxer
United States Senator

BB/jo

NACDS

National Association of Chain Drug Stores

Thomas M. Ryan
Chairman of the Board

Ronald L. Ziegler
President & CEO

August 11, 1997

The President
The White House
Washington DC. 20500

Dear Mr. President:

On behalf of the members of the National Association of Chain Drug Stores, we are writing to support the intent of a new proposed regulation to be issued by the Food and Drug Administration that would provide pharmacists and other health care providers with additional information on prescription drugs that can be used in treating child illnesses.

The National Association of Chain Drug Stores (NACDS) membership consists of more than 130 retail chain community pharmacy companies. Collectively, chain community pharmacy comprises the largest component of pharmacy practice with over 86,000 pharmacists. These pharmacists are responsible for filling approximately 60% of the more than 2.6 billion prescriptions dispensed annually in the United States.

Pharmacists are considered one of the most accessible and trusted health care professionals, and frequently the only health professional available in many small towns and rural areas. On a daily basis, community retail pharmacists counsel patients on proper use of their medications and answer questions about prescription drugs prescribed for them or their family members.

Product labeling information that would provide all health professionals with readily accessible information about proper dosage for the use of adult medications by children would further assist pharmacists and other providers in determining the appropriate use of these medications for children. We strongly support efforts that would increase the amount of useful information available to health care providers in treating and counseling patients.

Sincerely,

Ron Ziegler

Ronald L. Ziegler
President and Chief Executive Officer

AmFAR

Fax 212-682-9812

August 8, 1997

The President
The White House
Washington, DC

Dear Mr. President:

The American Foundation for AIDS Research (AmFAR) endorses your support of the proposed rule by the Food and Drug Administration to require safety and dosing studies for new drugs that will be used in children. It is a clear statement that this nation is concerned about the health of its children. This decision will be an additional milestone in FDA progress, increasing the certainty that all individuals will benefit from the best of our therapeutic discoveries. Life-saving treatment must be available to men, women and children simultaneously. No one should be denied treatment because of age. No parent should be concerned about the safety of a drug for their child.

For years we have watched as life-saving drugs were approved for adults but not for children. Nowhere has this been more dramatic and visible that in the HIV/AIDS epidemic. Currently, there are 11 drugs approved for adults to treat HIV and only 5 for children. Parents have pleaded for these new drugs for their children as they have watched their own health or that of their adult friends improve while their children worsened.

We at AmFAR have strongly supported FDA rules that have accelerated the availability of promising treatments for patients with HIV infection and other diseases. We have seen new treatments return people to healthy and productive lives. We also strongly support equal access for all individuals to these therapies. Your decision to support this new FDA rule has the potential to bring closure to an inequity in health care by bringing life-saving treatments to this and future generations of our children.

Sincerely,

Mathilde Krim, Ph.D.
Founding Co-Chair and
Chairman of the Board

Arthur J. Ammann, M.D.
President

Elizabeth Taylor
Founding National Chairman

Mathilde Krim, Ph.D.
Founding Co-Chair
Chairman of the Board

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The Rev. Dr. Randolph Nugent
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Alan D. Schwartz
Michael D. Shriver
Mervyn F. Silverman, M.D., M.P.H.
Peter R. Staley
The Rt. Rev. William E. Swing

Directors Emeriti
Jonathan M. Mann, M.D., M.P.H.
Abigail Van Buren
Joel D. Weisman, D.O.

Sharon Stone
Chairman
The Campaign for AIDS Research

Arthur J. Ammann, M.D.
President

Jerome J. Radwin
Executive Vice-President and
Chief Executive Officer

American Academy of Family Physicians

The doctors who specialize in you

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capitol@aafp.org
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August 8, 1997

The Honorable William J. Clinton
The White House
Washington, DC 20500

Dear President Clinton:

On behalf of the 85,000 members of the American Academy of Family Physicians, I would like to commend you for your initiative to improve labeling of drugs prescribed for children. Your proposed regulation will help ensure that our nation's children receive safe and effective medications.

Family physicians in the United States see millions of children each year. For example, in 1993, more than 37 million office visits to family doctors included patients under the age of 17, which means that 25% of children's visits to a doctor were to a family physician. Consequently, we are highly supportive of an initiative that will provide family physicians with additional information to use as we prescribe drugs for children.

Again, thank you for initiating this important proposal. America's family physicians stand ready to assist you in implementing an initiative that will be of enormous benefit to our youngest patients.

Sincerely,

Douglas E. Henley, MD
Chair, Board of Directors

President
Patrick B. Harr, MD
Maryville, Missouri

Vice President
Susan Black, MD
Lowell, Massachusetts

President-elect
Neil H. Brooks, MD
Rockville, Connecticut

Board Chair
Douglas E. Henley, MD
Fayetteville, North Carolina

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Grand Junction, Colorado
Jimmie H. Smith, Jr., MD
(Resident Member)
Albany, Georgia
David Hutcheson-Tipton
(Student Member)
Denver, Colorado

aafp
50 YEARS OF CARING
1947 - 199

FDA

OFFICE OF THE COMMISSIONER
OFFICE OF POLICY
5600 FISHERS LANE Rm. 14-81
ROCKVILLE, MD 20857
PHONE - (301)827-3370
FAX - (301)443-5930

Date: 8/8/97

To: Chris Jennings

From: D. Sullivan-Ford

FAX #: 202 456-5557

Of Pages Not Including Cover: 4

Comments:
Q's & A's for Pediatric Labeling Attached.

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PEDIATRIC LABELING Qs and As

1. Why are you doing this now?

Despite efforts in recent years to address this issue, most drugs still lack adequate pediatric labeling. Every year about one half of all new drug and biologic products approved, that are likely to be used in children, lack information to support their safe and effective use in children. Pediatricians initially lack information necessary to prescribe these products to children who may need them. For products already on the market, information is eventually made available to physicians by journal articles, handbooks and other references, but usually years after a drug is approved (and even then the information isn't available on the label).

2. Can't you achieve the same effect through voluntary compliance?

FDA has taken a number of steps in recent rears to address inadequate pediatric labeling, including issuing a rule in 1994 requiring drug manufactures to survey existing data and determine whether those data are sufficient to give information on how to use a drug in pediatric populations. Most drugs, however, still lack adequate pediatric labeling.

3. Given that the draft FDA reform legislation, pending in Congress, contains financial incentives to encourage voluntary compliance, why is this rule necessary?

This rule is intended to complement the financial incentives for market exclusivity that are currently being proposed in Congress. Because financial incentives may work in some cases, but offer no guarantees, this rule is needed to give FDA the authority to ensure that products widely used or offering a clinically significant use in children carry adequate labeling for their safe and effective use in children.

4. Have children been at risk in the past?

The lack of adequate pediatric labeling has resulted in the use of many products that have only been tested for adults, causing an increased risk of inappropriate and unexpected adverse effects. Also, sometimes useful drugs not have been used in children because their effects in kids were uncertain..

5. How many drugs that are commonly used in children lack this data?

FDA estimates that one-half of the drugs approved in the past five years that had potential usefulness in kids had no pediatric labeling at the time of approval.

6. How many products will be affected by the rule?

FDA approves approximately 30 major new drug advances each year. About half of those products will need pediatric studies under this rule. The agency will also review drugs already on the market to determine which ones should have pediatric studies. The effect of this rule will that in a few years most drugs important to children will have directions for use in kids on their labels.

7. What kinds of drugs are commonly missing this pediatric data?

Drugs such as anti-asthmatics, steroids, drugs to treat gastrointestinal problems, strong pain medications, antidepressants, and antihypertensives commonly lack appropriate pediatric labeling.

8. What do doctors do when they don't have this information?

Many physicians rely on referenced pediatric handbooks for dosing and use information. Others are reluctant to prescribe certain products to children without adequate pediatric labeling on the product.

9. When can parents expect that information to support safe and effective use of products in children will become available.

We believe that, in some cases, the information already exists and the drug companies merely need to analyze and compile it. In these cases, the information can be made available on the labeling of the products fairly quickly. In other cases, studies need to be conducted. Under the requirements of our 1994 regulation, where the effects of the product and the disease for which it is indicated are sufficiently similar in both adults and children, these studies can be done within one year.

10. What drugs now have the best pediatric data?

Vaccines and antibiotics are the most adequately labeled drugs for children.

11. Why haven't companies provided this information in the past?

Some companies do provide this information, however, pediatric labeling has not been required until now. Lack of familiarity with pediatric studies, need for a pediatric formulation, and cost may account for the lack of adequate pediatric information.

12. How much will this cost drug manufacturers?

The costs of pediatric studies will be less than 1% of the total costs of developing a drug. FDA estimates that the total annual industry costs will be less than $20 million per year (for a multi-billion dollar industry).

13. Will drug prices increase as a result of this regulation?

Because the cost of pediatric studies to manufacturers is expected to be small, it is anticipated that there will be little or no price increases to patients

14. Does this regulation cover all drugs?

It covers all drugs that are widely used in, or important to, children.

15. Will this requirement hold up drug approvals?

No, the proposal allows for postapproval submission of pediatric data if concerns about pediatric testing arise prior to approval. Additionally, the requirement may be waived in certain cases.

16. How will these waivers work and won't they make the regulation meaningless?

The waivers are only intended to be used in those cases in which the drug would not be widely used and would not represent a meaningful benefit over existing treatment or where the studies are impossible or highly impractical or pose undue risks to pediatric patients.

17. Why doesn't it cover medical devices? Do we not have this kind of problem with medical device pediatric data?

Once the agency gains experience in requiring pediatric studies for drugs and biologics, it will assess the need for such labeling for devices.

18. What kind of improvement can we expect in our childrens' health as a result of this regulation?

We expect safer and more effective medicines for children. The primary benefits expected are the reductions in avoidable adverse drug reactions and under or over treatments that would result from better informing health care practitioners about whether, and in what dosages, a given drug was safe and effective for use in children.

19. When will this regulation go into effect?

There is a 90 day period for comment on the proposed rule after which the agency will evaluate and respond to the comments and publish a final rule. The final rule will take effect 3 months after issuance. At that time, for drugs and biologics already on the market, FDA, in compelling circumstances, may request that pediatric studies be initiated. Manufacturers of new drug and biologic products, under review at the agency, will have 2 years to comply with the pediatric study requirement. Manufacturers of new products, not yet submitted for review, will have 18 months to comply with the requirement.

20. What kind of "legal action" can FDA take to force companies to provide this data on approved drugs?

FDA can go to court and ask the court to order the company to comply with the regulations. If the company does not comply, the court can impose penalties.

21. Would pediatric study participants' lives be at risk without having all the adult data from the start?

Pediatric participants are not enrolled in studies until enough data on the drug have been obtained so that the risk to the pediatric participants is low.

This section contains a sunset provision that states that no market exclusivity will be granted based on pediatric studies begun after January 1, 2004. In addition, the Secretary must complete a study and report to Congress no later than January 1, 2003, the agency's experience under the program. The report must address the program's effectiveness, the adequacy of its incentives, the program's economic impact, and any suggestions for the program's modification.

Sec. 619. Positron Emission Tomography.

Section 619 amends the FFDCA to include the regulation of compounded positron emission tomography (PET) drugs. The provision defines compounded PET drugs to mean drugs that exhibit spontaneous disintegration of unstable nuclei; includes nonradioactive reagents, nuclide generators, accelerators, electronic synthesizers, or associated software used to prepare any such drug; and, which have been compounded in accordance with State law by or on the order of a practitioner licensed in that State or in a federal facility in accordance with the laws of the State in which it is located. The Act is amended to stipulate that a compounded PET drug is adulterated, and thus subject to regulatory and/or legal action by FDA if it is not compounded, processed, packed, or held in accordance with the PET compounding standards and official monographs of the United States Pharmacopoeia (USP).

The act is further amended to provide that neither a New Drug Application (NDA) nor an Abbreviated New Drug Application (ANDA) is required by a licensed practitioner to produce a compounded PET product in accordance with USP standards. Within 30 days of enactment, the Secretary must publish in the *Federal Register* a notice revoking all previously published efforts by FDA to provide industry guidance and regulatory standards for PET products.

TITLE VII-FEES RELATING TO DRUGS

Sec. 701. Short Title.

Section 701 provides that this title be cited as the "Prescription Drug Users Fee Reauthorization Act of 1997."

Sec. 702. Findings.

Section 702 sets forth four congressional findings: (1) the prompt approval of safe and effective new drugs and other therapies is critical to improve public health; (2) additional resources augmenting the Food and Drug Administration's (FDA) review of human drug applications serve the public health; (3) the successful Prescription Drug User Fee Act of 1992 (PDUFA) program reduced drug review times; therefore it should be reauthorized for an additional 5 years and should be carried out by FDA with more ambitious and comprehensive regulatory goals; (4) fees authorized by amendments will be used to expedite the drug

is no different for food contact substances being marketed subject to a PMN.

Section 617(c) specifies that this legislation shall be effective following 18 months from the date of its enactment. PMNs may be filed after this period (and become effective 120 days after their receipt by FDA) without regard to whether FDA has issued regulations implementing this legislation.

Sec. 617. Health Claims For Food Products.

Section 617 amends section 403(r)(3) (21 USC 343(r)(3)) of the FFDCA. It provides an alternative to the current standard and review process by allowing health claims to be made based on information published by authoritative U.S. government scientific bodies. The new provision will allow a health claim in food labeling without FDA authorization, if it consists of or will otherwise summarize or reflect information contained in a publication of a Federal Government scientific organization or some component of the National Academy of Sciences. If any such health claim is made, it must be submitted to FDA, along with the published information on which it is based, at least 120 days prior to its appearance in the marketplace. A claim meeting the requirements may be made until a final regulation, prohibiting or modifying the claim, becomes effective, or a U.S. District Court determines that the nutritional claims requirements have not been met.

Sec. 618. Pediatric Studies Marketing Exclusivity.

Section 618 amends Chapter V (21 USC 351 et. seq.) of the FFDCA by creating new section 505A--Pediatric Studies of Drugs. If, prior to the approval of a new drug, the Secretary determines that information about the drug will produce health benefits in a pediatric population, and makes a written request for pediatric studies, and the studies are completed and accepted, then the sponsor or manufacturer can qualify for up to 6 months of extra market exclusivity. If the Secretary makes a written request for pediatric studies of an already marketed drug, and those studies are completed, then the manufacturer can be granted up to 6 months of increased market exclusivity as well.

Within 180 days of enactment, the Secretary, after consultation with experts, must develop and publish an initial list of approved drugs for which additional pediatric information may produce health benefits. When the Secretary has formally requested pediatric studies those studies must be conducted by a written protocol agreed to by the sponsor, patent holder, and the Secretary. Less than 60 days after the pediatric studies have been submitted, the Secretary must determine whether the studies were done properly and notify the sponsor or holder. In addition, the provision contains a section describing other means by which the study protocol requirements can be met.

American Academy of Pediatrics

Department of Government Liaison
Suite 400 North
601 13th Street, N.W.
Washington, D.C. 20005

Phone: 800/336-5475 or 202/347-8600
Fax: 202/393-6137

To: Chris Jennings

Fax #: 456-5557 **Phone #:**

From: Elaine Holland
Assistant Director (x3005)

Date: August 6, 1997

Page 1 of

Re: Per our conversation. Talk with you tomorrow.

Company Name	Address	Phone Number	Fax Number
American Lecithin Company	PO Box 1908 33 Turner Road Danbury, CT 06813-1908	203/790-2700	203/790-2705
Beiersdorf Inc.	PO Box 5529 Norwalk, CT 06856-5529	203/583-8008	
Boehringer Ingelheim Pharmaceuticals, Inc.	900 Ridgebury Road PO Box 368 Ridgefield, CT 06877-0368	203/791-6194	
Gray Pharmaceuticals Co.	*Affiliate, The Purdue Frederick Company* 100 Connecticut Ave Norwalk, CT 06850-3590	203/853-0123	
Eli Lilly and Company *Regional Sales Office*	300 First Stamford Place Stamford, CT 06902	203/357-7228	
Miles Inc. Pharmaceutical Division	400 Morgan Lane West Haven, CT 06516	800/468-0894 203/937-2000	
The Purdue Frederick Company	100 Connecticut Ave Norwalk, CT 06850-3590	203/853-0123	

Pete Wilson → 703 684 1355
Elaine Hollard - 347-8600

Rick Pollack -
Dr Harold Varmus

Date: Wednesday, August 6th

Caller	Phone	Time	Message	Response
Rick Pollack	1800-913-3188	12:45	pls call	
Jen Klein	6-2599	12:15	pls. Call her ASAP	
Rich Boxer	414/527-3000	11:55	Pls call	
Rick Khan,	703/299-2065	12:00	Is Newt Gingrich invited to this event? The speakers' ofc called and expected to be invited.	
Jay Hoover	67103		Medicare Commission	
Michelle in Rahm's office	62531	1:30	pls. call	
Vicki Radd	6-7176	2:10	Pls. Call her -- she just has a quick question.	

Sandoz → Doug Cornell
v. Pres.
828-8811

Steve Laughton (Hogan & Hartson)
637-8615

Pete [illegible] Tedey → Amgen
289-4307 PhRMA FDA work group

= Aquarion

Jerry [illegible] Kimpel = Glaxo-Wellcome

Ted Oliver = Merck

– Jeff Peterson
Dave Williams = Connaught

Schering: Pfizer – Bob Lowey
463-7372

Pharmacia →

File Pediatric Labeling

Date: Friday, March 14, 1997
FOR IMMEDIATE RELEASE
Contact: Food and Drug Administration Jason Brodsky:(301)827-3417
Broadcast Media: (301) 827-3434, Consumer Hotline:(800) 532-4440

FDA Approves First Protease Inhibitors With Labeling For Use In Children

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The Food and Drug Administration today approved two HIV protease inhibitors -- some of the most powerful medicines against the infection -- for use in treating children.

Nelfinavir received its initial approval today -- including information on use in both adults and children -- approximately 3 months after its application was received. The labeling for nelfinavir includes a "pediatric use" statement, giving doctors specific dosage recommendations for patients 2 to 13 years old. In addition, ritonavir, a previously approved protease inhibitor, also received "pediatric use" labeling today.

"Today's actions not only add another powerful weapon to our arsenal for treating HIV infection, but provide us with critical information on using these cutting edge drugs to help HIV-positive children," said HHS Secretary Donna E. Shalala.

"With each approval, we are providing more options in designing individualized treatment programs for adults and children," said Dr. Michael A. Friedman, FDA Lead Deputy Commissioner.

The "pediatric use" section in the drug labeling provides pecific recommendations for the use of the drugs in children. A

regulatory reform initiated in 1994 eased the process of including label information that helps physicians in treating pediatric patients -- particularly in serious or life-threatening situations. Now, such information can be provided when evidence suggests that the course of the disease and the effects of the drug are sufficiently similar in the pediatric and adult populations to permit extrapolation from adult efficacy data to pediatric patients.

Nelfinavir received accelerated approval, a regulatory mechanism under which FDA bases early marketing approval for a product on laboratory markers such as plasma HIV RNA (a measure of viral load) and CD4 cell counts until information about clinical endpoints such as disease progression or mortality is available.

FDA based its approval of nelfinavir on studies of up to 24 weeks in duration showing that the drug was active in combination with other antiretroviral drugs for the treatment of HIV or if administered alone. However, because the antiviral activity of nelfinavir is increased when used with other drugs approved for treatment of HIV, combination therapy is recommended.

The most frequent adverse event associated with the use of nelfinavir is diarrhea, which can usually be controlled with nonprescription drugs. Other adverse events reported during clinical trials included nausea, vomiting, and asthenia.

Nelfinavir should not be administered concurrently with terfenadine, astemizole, cisapride, trizolam or

midazolam, because these drugs may inhibit its metabolism and create the potential for serious and/or life-threatening cardiac arrhythmias or prolonged sedation. FDA has worked with the manufacturer to ensure that these for Children drug interactions with nelfinavir are clearly highlighted on the package label and that patient education materials are made available to health care professionals and patients.

Agouron Pharmaceuticals is marketing nelfinavir under the trade name Viracept. Ritonavir is marketed under the trade name Norvir by Abbott Laboratories.

Note: HHS press releases are available on the World Wide Web at: http://www.dhhs.gov.