

Medical & Surgical Devices for Children:

Public Policy Approaches to Solving Access

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American Academy of Pediatrics

DEDICATED TO THE HEALTH OF ALL CHILDREN™



American Academy of Pediatrics

- The American Academy of Pediatrics (AAP) is a professional organization of 60,000 primary care pediatricians, pediatric medical subspecialists, and pediatric surgical specialists
- Dedicated to the health, safety, and well-being of infants, children, adolescents, and young adults since its founding in 1930
- AAP maintains numerous organized sections with device expertise:

Allergy and Immunology

Anesthesiology and Pain Medicine

Cardiology and Cardiac Surgery

Clinical Pharmacology and
Therapeutics

Gastroenterology

Genetics and Birth Defects

Hematology/Oncology

Hepatology and Nutrition

Infectious Diseases

Nephrology

Neurological Surgery

Neurology

Ophthalmology

Orthopaedics

Otolaryngology

Pediatric Dentistry and Oral Health

Pediatric Pulmonology

Perinatal Pediatrics

Plastic Surgery

Radiology

Rheumatology

Urology

Surgery

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Defining Advocacy

“It should be our aim to discover neglected problems and, so far as in our power, to correct evils and introduce reform.”

**--Issac Abt, MD
First AAP President, 1930**



Children and Device Regulation

- Children are a small and vulnerable population
- Since 1977, the AAP has advocated strongly that medicines used in children should be tested in children
- The Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) have been hugely successful innovations for children
- Just as in drugs, children need medical and surgical devices made with their unique needs in mind and studied in pediatric populations
- Children have unique medical and surgical device needs related to size, growth, and physiological differences
- Differences between drug and device markets call for unique policy solutions for children





The Pediatric Devices Initiative

- Beginning in June 2004, a series of stakeholder meetings yielded recommendations for improving the availability of pediatric medical and surgical devices. The meetings were hosted by:
 - American Academy of Pediatrics
 - Elizabeth Glaser Pediatric AIDS Foundation
 - National Organization for Rare Disorders (NORD)
 - National Association of Children's Hospitals
 - Advanced Medical Technology Association (AdvaMed)
- Representatives from government, industry, medicine and patient groups participated



Themes from Stakeholders Meetings

- In contrast to the market for drugs, the market for devices is complex, variable, and short-term
- Significant barriers exist to pediatric device development, including high costs, small markets, few incentives, lack of information, and difficulty connecting innovators with manufacturers
- Data on pediatric devices is scarce, there is a need to identify and prioritize unmet pediatric device needs



FDA Opinion



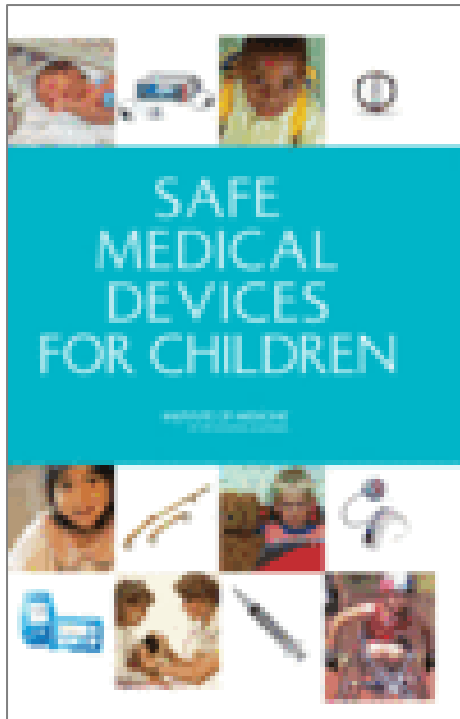
In October 2004, the Food and Drug Administration released a report that identified numerous barriers to the development and approval of devices for children.



The Institute of Medicine Report

In July 2005, the Institute of Medicine (IOM) issued a report on the adequacy of post-market surveillance of pediatric medical devices.

The IOM found significant gaps in safety monitoring and recommended expanding the FDA's ability to require post-market studies of certain products and improving public access to information about post-market pediatric studies.



On to Capitol Hill

The issues identified by the stakeholder meetings and the IOM report on pediatric devices led to a legislative proposal, introduced first in 2006 as the Pediatric Medical Device Safety and Improvement Act.



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Key Pediatrician Champions



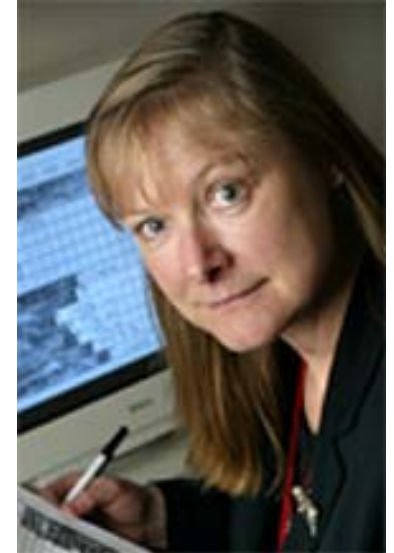
Dr. Jon Abramson

Wake Forest University
School of Medicine



Dr. Robert Campbell

Children's Hospital of
Philadelphia



Dr. Ann Halbower

University of Colorado
School of Medicine

Key Legislative Champions

Sen. Chris Dodd (D-CT)



Rep. Ed Markey (D-MA)



Sen. Mike DeWine (R-OH)



Rep. Mike Rogers (R-MI)





PUBLIC LAW 110-85—SEPT. 27, 2007

121 STAT. 823

Public Law 110-85
110th Congress

An Act

To amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user fee programs for prescription drugs and for medical devices, to enhance the postmarket authorities of the Food and Drug Administration with respect to the safety of drugs, and for other purposes.

Sept. 27, 2007
[H.R. 3540]

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

Food and Drug
Administration
Amendments Act
of 2007.
21 USC 901 note.

SECTION 1. SHORT TITLE.

This Act may be cited as the "Food and Drug Administration Amendments Act of 2007".

SEC. 2. TABLE OF CONTENTS.

The table of contents for this Act is as follows:

Sec. 1. Short title.
Sec. 2. Table of contents.

TITLE I—PRESCRIPTION DRUG USER FEE AMENDMENTS OF 2007

Sec. 101. Short title; references in title; finding.
Sec. 102. Definitions.
Sec. 103. Authority to assess and use drug fees.
Sec. 104. Fees relating to advisory review of prescription drug television advertising.
Sec. 105. Reauthorization; reporting requirements.
Sec. 106. Sunset dates.
Sec. 107. Effective date.
Sec. 108. Savings clause.
Sec. 109. Technical amendment; conforming amendment.

TITLE II—MEDICAL DEVICE USER FEE AMENDMENTS OF 2007

Sec. 201. Short title; references in title; finding.

Subtitle A—Fees Related to Medical Devices
Sec. 211. Definitions.
Sec. 212. Authority to assess and use device fees.
Sec. 213. Reauthorization; reporting requirements.
Sec. 214. Savings clause.
Sec. 215. Additional authorization of appropriations for postmarket safety information.
Sec. 216. Effective date.
Sec. 217. Sunset clause.

Subtitle B—Amendments Regarding Regulation of Medical Devices

Sec. 221. Extension of authority for third party review of premarket notification.
Sec. 222. Registration.
Sec. 223. Filing of lists of drugs and devices manufactured, prepared, packaged, and accompanied by registrative statements; accompanying disclosures.
Sec. 224. Electronic registration and listing.
Sec. 225. Report by Government Accountability Office.
Sec. 226. Unique device identification system.
Sec. 227. Frequency of reporting for certain devices.

The Food and Drug Administration Amendments Act of 2007

Title III: Pediatric Medical Device Safety and Improvement Act

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Pediatric Device Law Passed in 2007

- In 2007, Congress passed the **Pediatric Medical Device Safety and Improvement Act** along with the Medical Device User Fee Act (MDUFA) reauthorization in FDAAA
- Success stemmed from multi-stakeholder support, key champions in Congress and a compelling articulation of the need
- The law was the first-ever piece of legislation passed exclusively on pediatric medical devices



Pediatric Medical Device Safety and Improvement Act of 2007

The law:

- Increased FDA authority for tracking of pediatric device approvals
- Incentivized device development by removing the humanitarian device exemption (HDE) profit cap for pediatric devices
 - At least one device approved under the revised program so far
- Established non-profit consortia to stimulate device development
 - Four consortia currently up and running, program funded at \$3 million/year
 - Office of Orphan Products Development (OOPD) successfully overseeing
- Enhanced postmarket surveillance for pediatric devices
- Enhanced the federal response for pediatric device needs



SEC. 302. TRACKING PEDIATRIC DEVICE APPROVALS

- Amends Chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351 et seq.) to create a mechanism to allow FDA to **track the number and types of devices approved specifically for children or for conditions that occur in children**, as well as the approval times for premarket approvals and humanitarian device exemptions. Requires annual reports to Congress on the results beginning 18 months after enactment.
- Provides specific authority to FDA to allow the **extrapolation** of adult data to support a pediatric indication, as appropriate.



SEC. 303. MODIFICATION TO HUMANITARIAN DEVICE EXEMPTION

- Amends existing provision to **allow profit for devices approved as humanitarian device exemptions (HDE)** that are specifically designed to meet a pediatric need.
- Already approved adult HDEs upon date of enactment are eligible for the HDE profit modification but only if they meet the conditions of this section.
- Requires adverse event reports for pediatric HDE devices to be referred to the Office of Pediatric Therapeutics.
- Requires a GAO report no later than January 1, 2012 to assess whether the HDE profit exemption has increased the availability of pediatric devices.
- Directs FDA to issue **guidance** within 180 days to **institutional review committees** for responding to HDEs.



SEC. 304. ENCOURAGING PEDIATRIC MEDICAL DEVICE RESEARCH

- Amends section 402(b) of the Public Health Service Act (42 U.S.C. 282(b)) to designate a **contact point** of office to help innovators and physicians access existing funding for pediatric medical device development.
- Directs the **NIH, FDA, and AHRQ to submit a plan** within 180 days of enactment for pediatric medical device research that identifies gaps and proposes a research agenda for addressing them.



SEC. 305. DEMONSTRATION GRANTS FOR IMPROVING PEDIATRIC DEVICE AVAILABILITY

- Establishes demonstration grants for non-profit consortia to facilitate the development, production, and distribution of pediatric medical devices by:
 - **encouraging innovation** and connecting qualified individuals with pediatric device ideas with potential manufacturers,
 - **mentoring and managing** pediatric device projects through the development process,
 - **connecting** innovators and physicians to existing Federal and non-Federal resources, and
 - **providing assistance and advice** as needed on business development, personnel training, prototype development, and postmarket needs.



SEC. 305. DEMONSTRATION GRANTS FOR IMPROVING PEDIATRIC DEVICE AVAILABILITY

- Requires consortia to coordinate with the **NIH** to identify research issues that require further study and with the **FDA** to facilitate approval of pediatric indications.
- Authorizes **\$6 million** for each of fiscal years 2008 through 2012.



Funding for Pediatric Device Consortia

- AAP, in partnership with EGPAF and others, launched a successful campaign that resulted in funding for the device consortia:
 - FY2009: \$2 million
 - FY2010: \$3 million
 - FY2011: \$3 million (proposed)

Year 1 Grantees:

James Geiger, M.D. and the Michigan Pediatric Device Consortium

Pedro DelNido, M.D. and The Pediatric Cardiovascular Device Consortium

Michael Harrison, M.D. and the University of California, San Francisco Pediatric Device Consortium

Year 2 Grantees:

James Geiger, M.D. and the Michigan Pediatric Device Consortium

Pedro DelNido, M.D. and The Pediatric Cardiovascular Device Consortium

Michael Harrison, M.D. and the University of California, San Francisco Pediatric Device Consortium

Pablo Garcia and Sanjeev Dutta and the MISTRAL Device Consortium



Increased Pediatric Focus at CDRH

- FDA and its Center for Devices and Radiological Health (CDRH) have responded to Congress and pediatric advocates by increasing pediatric device activities
- New position created and filled, Chief Pediatric Medical Officer in CDRH director's office: Susan Cummins, MD, FAAP
- Multi-specialty pediatric device clinical trials meeting held in October 2009
- Other specialty-specific pediatric device meetings have been held
- CDRH considered pediatrics in its review of regulatory science



Looking Forward

- Significant progress has been made but we're still just getting started – the law was an important first step
- Pediatric tracking provisions of the law are not implemented
- Need for continued federal investment in the pediatric device consortia program
- Portions of the Pediatric Medical Device Safety and Improvement Act will need to be reauthorized
- Inclusion of pediatric issues in current regulatory and possibly legislative initiatives such as 510(k) reform



FDA 510(k) Initiative



“One factor that complicates our science-based decision making, in the 510(k) context and more broadly, is that we operate within an **ever-changing scientific landscape**. As new scientific information emerges about the risks and benefits of a given device type, we must be prepared to modify our treatment of that device type accordingly. At the same time, to facilitate innovation, we seek to maintain **predictability in our regulatory pathways**.”

-- Jeff Shuren, MD, JD
Director, Center for Devices
and Radiological Health, FDA



Pediatric Opportunities in FDA Device Reform

- Important opportunities to understand off-label uses of 510(k) devices in children, especially in the absence of pediatric tracking information
- Possibilities for better understanding of predicate devices and any associated safety concerns in pediatrics
- Opportunities to improve pediatric post-market surveillance and condition of clearance studies
- Faster and better labeling information available to providers and consumers



Next Steps

- Continuing to increase capacity and expertise in CDRH on child- and adolescent-specific device development and regulatory issues
- Fostering connections across Centers of FDA on pediatrics through the Office of Pediatric Therapeutics (OPT), the Pediatric Advisory Committee (PAC) and other opportunities
- Permanently integrating children's issues into the FDA regulatory paradigm for devices and other therapeutics



Next Steps



- Opportunities for improvements to the development of safe medical devices for children through Congressional action on FDA user fee and other programs in 2012
- Consideration of new methods, opportunities, incentives for pediatric device development





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