



Not Your Father's Drug Approval Process: FDAAA and the Evolving Pharmaceutical Marketplace

FORUM SESSION ANNOUNCEMENT

A DISCUSSION FEATURING:

Cole Werble and Michael McCaughan

*Senior Editors, The RPM Report
Founding Members, Prevision Policy*

David Dorsey

*Acting Deputy Commissioner for Policy, Planning & Budget
U.S. Food and Drug Administration*

Peter K. Honig, MD

*Managing Director
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Andrea Masciale

*Senior Director, Global Regulatory Affairs Strategic Policy & Support
Johnson & Johnson Pharmaceuticals*

FRIDAY, MARCH 12, 2010

11:45AM–12:15PM—Lunch

12:15PM–2:00PM—Discussion

LOCATION

Reserve Officers Association
One Constitution Avenue, NE
Congressional Hall of Honor
Fifth Floor
*(Across from the Dirksen
Senate Office Building)*

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The U.S. Food and Drug Administration (FDA) is responsible for the regulation and oversight of more than \$1 trillion worth of products. In its purview is everything from aspirin to vaccines, cardiac defibrillators to X-ray machines, cat food to genetically engineered chickens, peanut butter to baby bottles, and lipstick to cigarettes. Most people probably cannot get through the day without touching or ingesting something that FDA has reviewed or inspected or is monitoring.

For human drugs, FDA is responsible for reviewing, approving, inspecting, and monitoring millions of prescription and over-the-counter products. The agency reviews thousands of applications every year, such as those to test new drugs in humans, allow new drugs to enter the marketplace, make generic drugs, switch from prescription to non-prescription status, and change the manufacturing or labeling of drugs. Annual spending on prescription drugs alone is approaching \$250 billion and generally accounts for about 10 percent of total health spending.

The regulation of drugs and the requirements for drug applications have evolved steadily since passage of the Food and Drugs Act of 1906, most dramatically when the Federal Food, Drug, and Cosmetic Act of 1938 authorized the FDA to demand evidence of safety for new drugs, and again later when the Kefauver-Harris Amendments of 1962 added the requirement for manufacturers to prove their drugs' effectiveness.

Most recently, the Food and Drug Administration Amendments Act of 2007 (FDAAA) added significantly to FDA's authorities, reauthorizing and expanding existing programs as well as adding new ones.¹ FDAAA includes numerous provisions, many of which apply directly or indirectly to new drugs.² These provisions are generally aimed at strengthening FDA's science base, accelerating innovation, and enhancing safety. Some of the specific provisions are highlighted below.

For example, FDAAA

- Reauthorizes the **Prescription Drug User Fee Act (PDUFA)**, which requires that manufacturers pay fees for certain new drug applications in order to provide some of the resources that FDA needs to review them; expands the amount of fees that can be collected and what the funds can be used for, including manufacturer's voluntary submission of direct-to-consumer television drug advertisements for advisory review by FDA and for increased activities to ensure drug safety once a drug has been approved and entered the market
- Reauthorizes the **Best Pharmaceuticals for Children Act** and the **Pediatric Research Equity Act**, both intended to encourage more research and development of treatments for children

- Gives FDA additional authorities to oversee the safety of drugs after they have been approved and are on the market, including the authority to require a study or clinical trial after approval, to require a labeling change if called for, and to require a **risk evaluation and mitigation strategy (REMS)** at any point that the expected or actual safety profile of the drug warrants it, including as part of the initial application for approval
- Requires the agency to conduct regular, biweekly screening of the existing **Adverse Event Reporting System (AERS)** database and post a quarterly report of any new safety information or potential signal of a serious risk identified within the last quarter; and directs the agency to collaborate with public, academic, and private entities to develop an **Active Postmarket Risk Identification and Analysis system** using electronic databases
- Expands existing requirements for **clinical trial registration** in order to increase public availability of the information, mandating registration of a broader range of trials, providing an enforcement mechanism, and requiring posting of trial results
- Authorizes the creation of the **Reagan-Udall Foundation** as well as collaborative agreements with educational or tax-exempt organizations to implement the **FDA Critical Path Initiative**, both intended to foster innovation, accelerate development, and enhance safety of medical products
- Requires that any drug that includes a new active ingredient be referred to an FDA advisory committee (the agency uses numerous **advisory committees** to provide independent advice from outside experts) and revises FDA's approach to preventing **conflicts of interest** among committee members, requiring broader outreach for members, stricter exclusion criteria, and greater transparency

Although most of the provisions of FDAAA build on existing features and requirements of the process for reviewing and approving new drugs, they nonetheless represent substantial additional responsibilities and requirements for both FDA and drug manufacturers. They also present major opportunities for collaboration, for advancing drug science, and for promoting the public health. Each provision has its advocates and its detractors, and discussion and debate has continued during more than two years of implementation about whether each goes far enough, or too far.

KEY QUESTIONS

The speakers for this session collectively bring decades' worth of experience in drug industry analysis, FDA legislation, agency operations,



and drug development. They will describe and discuss the implementation of FDAAA and address the following questions:

- What are the goals of FDAAA, and what challenges has it presented to FDA and the industry?
- How is implementation of FDAAA progressing? What has been the legislation's impact on FDA's operations?
- What has been the impact on the pharmaceutical industry? On public health? Have there been unintended consequences? If so, what are they?
- Are any of the provisions lagging in implementation? If so, why? What might be done to improve the situation?
- What lessons might be drawn from the experience with FDAAA that could be helpful in thinking about the next round of FDA legislation?

SPEAKERS

Cole Werble and Michael McCaughan are senior editors of *The RPM Report*, a respected source of drug industry analysis, and founding members of Prevision Policy, which provides health care policy analysis for business executives and investors. Together they have more than 55 years' worth of experience in watching and analyzing the pharmaceutical industry. They will provide an overview of drug regulation, focusing on FDAAA and its impact on the drug industry.

David Dorsey is acting deputy commissioner for policy, planning and budget at the FDA. From 2001 to 2009, he served as a detailee from FDA to Sen. Edward Kennedy's staff on the Senate Committee on Health, Education, Labor and Pensions. He will discuss FDAAA from both the congressional and agency perspectives.

Peter Honig, MD, currently serves as a pharmaceutical consultant, the Pharmaceutical Research and Manufacturers of America's representative to the International Conference on Harmonization (ICH) Steering Committee, and the co-chair of the ICH Global Cooperation Group. He has held senior leadership positions at the FDA and Merck Research Laboratories.

Andrea Masciale is senior director in the global regulatory affairs strategic policy and support organization of Johnson & Johnson Pharmaceuticals Group. Prior to joining Johnson & Johnson in 2003, she served at FDA in the Center for Drug Evaluation as regulatory

counsel in the Office of Regulatory Policy and then as the director of the Division of Information Disclosure Policy.

Dr. Honig and Ms. Masciale will discuss how the industry is responding to the challenges of implementing FDAAA and how the marketplace is changing as a result.

ENDNOTES

1. Food and Drug Administration Amendments Act of 2007 (FDAAA), P. L. 110-85, September 27, 2007.
2. For more information about FDAAA, see FDA's Web site, at www.fda.gov/RegulatoryInformation/Legislation/FederalFoodDrugandCosmeticAct/FDCAct/SignificantAmendmentstotheFDCAct/FoodandDrugAdministrationAmendmentsActof2007/default.htm. In addition, congressional staff can access the Congressional Research Service's report "FDA Amendments Act of 2007 (P.L. 110-85)," by Erin D. Williams and Susan Thaul, Order Code RL34465, published April 28, 2008.