NIH, FDA Team Up to Speed New Therapies

BY MARY ELLEN SCHNEIDER

Top scientists at the National Institutes of Health and the Food and Drug Administration will be working together more closely in an effort to improve the regulatory process and bring new treatments to market sooner.

With more new treatments based on emerging technologies, NIH and FDA scientists must communicate with each other earlier and more often, explained Kathleen Sebelius, secretary of the Department of Health and Human Services. From the beginning of a therapy's development, basic scientists at the NIH should share information with the FDA so that FDA regulators can develop appropriate safety and effectiveness standards early on.

At the same time, FDA scientists can help researchers identify possible safety or quality issues earlier in the process, Ms. Sebelius said during a news conference to announce the partnership.

"By communicating throughout the process, it will help researchers navigate the regulatory process and give regulators the scientific tools they need to quickly assess a treatment's risks and benefits," Ms. Sebelius said. "For Americans, this is going to mean that new treatments are available sooner."

With FDA regulators often having to evaluate medicines without the data they need, 'the science that undergirds this kind of regulatory effort could certainly use some additional energy.'

The initiative calls for the creation of a joint FDA-NIH Leadership Council that will include FDA commissioner Dr. Margaret A. Hamburg and NIH director Dr. Francis S. Collins, as well as six senior scientists from each of the two agencies. In addition, the NIH and the FDA have pooled their resources to offer $6.75 million in grants during the next 3 years for research on regulatory science. For example, the agencies are looking for ideas on how the FDA would evaluate the safety and effectiveness of new stem cell therapies.

Government officials will be seeking public comment on how the two agencies can improve their collaboration. The NIH and the FDA will hold a public meeting jointly this spring to gather input from industry, patient advocates, and the public.

To bring safe, effective therapies into the market sooner, the science used to develop new therapeutic compounds must be closely connected to the science that the FDA uses to review those compounds, Dr. Collins said.

But that hasn't always been the case, he added. Researchers have not always shared information with the FDA early in the process, leaving the regulators to evaluate medicines without the data they need. Meanwhile, academics sometimes have expressed concern that the FDA lacks understanding about certain aspects of novel therapies, Dr. Collins noted.

Bridging that gap will be essential, he said, as the FDA is asked to evaluate more combination therapies and medicines developed under the umbrella of personalized medicine.

"The science that undergirds this kind of regulatory effort could certainly use some additional energy," Dr. Collins said. ■

NEWS FROM THE FDA

Orphan Drug Approved for CF

The Food and Drug Administration has approved Cayston to improve respiratory symptoms in cystic fibrosis patients with *Pseudomonas aeruginosa*.

The orphan drug was seen as an urgently needed agent to treat the respiratory and pulmonary symptoms of CF, which led the FDA's Anti-Infective Drugs Advisory Committee to overwhelmingly support approval of Cayston (aztreonam for inhalation solution). The drug is manufactured by Gilead Sciences.

Members of the committee said the bar for approval should be set "quite low" due to the lack of meaningful alternatives, despite misgivings about missing data and negative regimen effects in the two pivotal trials.

The approval was lauded by the Cystic Fibrosis Foundation as "the first new inhaled antibiotic approved for use in cystic fibrosis in more than a decade."

The foundation also is working with Gilead's marketing team to establish the Cayston Access Program, a call center developed with the Cystic Fibrosis Foundation Pharmacy (a wholly owned subsidiary of the Cystic Fibrosis Foundation). The call center will offer help to people with cystic fibrosis and members of their care team with insurance verification, referral to participating specialty pharmacies, claims support, and assistance with copayments.

Gilead also is establishing a program designed to minimize barriers to access for Cayston for uninsured, privately insured, and government-insured people.

New Meningococcal Vaccine Okayed

Physicians now have another meningococcal vaccine option, with the approval of Menveo for patients aged 11-55 years. The new vaccine is indicated for active immunization to prevent invasive meningococcal disease caused by *Neisseria meningitides* serogroups A, C, Y, and W-135. Menveo (Novartis) will be supplied in packages containing five single-dose vials.

The incidence of meningitis is estimated at 1,000-2,000 cases per year in the United States, according to the Centers for Disease Control and Prevention. Meningococcal disease is a leading cause of bacterial meningitis and sepsis. Even with early treatment, the disease may be fatal.

The other two meningococcal vaccines are available in the United States are the polysaccharide vaccine MPSV4 (Menomune, Sanofi-Pasteur) and the conjugate vaccine MCV4 (Menactra, Sanofi-Pasteur).

The CDC's Advisory Committee on Immunization Practices recommends routine immunization with a quadravalent meningococcal conjugate vaccine for all adolescents aged 11-18 years, college freshmen living in dormitories, and those aged 2-10 and 19-55 years who are in high-risk groups.

New Warnings for Oral Chelator

Information about the potential risks of renal impairment, renal failure, hepatic impairment, hepatic failure, and gastrointestinal hemorrhage appear in a boxed warning that has been added to the prescribing information of the iron-chelating agent deferasirox. New contraindications and other safety information also have been added to the label.

Manufacturer Novartis Oncology announced the changes in a Dear Healthcare Provider letter, which was posted on the FDA Web site.

Deferasirox (Exjade) is available in tablets for oral suspension, and was approved in 2005 for treating chronic iron overload due to blood transfusions in patients 2 years of age and older.

The letter says that some reported cases of adverse events were fatal. These reports were more common in people who were elderly or had high-risk myelodysplastic syndromes, underlying renal or hepatic impairment, or low platelet counts. Most of these deaths occurred within 6 months of starting treatment, and "generally involved worsening of the underlying condition," but the possibility that deferasirox "may have contributed to the deaths" has not been ruled out, the revised label says.

The new contraindications for deferasirox include a creatinine clearance less than 40 mL/min, serum creatinine more than twice the age-appropriate upper limit of normal, and platelet counts below 50×10^9/L. The letter says that more studies are being conducted to determine the long-term benefits and risks of deferasirox.

Maalox Product Will Be Renamed

Reports of mix-ups between Maalox products have prompted the manufacturer to change the name of one of the products, according to a notice posted on the FDA's MedWatch site.

The FDA has received five reports of consumers who used Maalox Total Relief, which contains bismuth subsalicylate, when they had intended to use one of the traditional Maalox antacid products, which contain aluminum hydroxide, magnesium hydroxide, and simethicone. Maalox products are manufactured by Novartis.

The Maalox antacid products have packaging similar to that of Maalox Total Relief, which is used to treat diarrhea, upset stomach associated with nausea, heartburn, and gas. Maalox Total Relief should not be used in certain populations, including children and teenagers recovering from a viral infection, and people on oral antidiabetic medications, anticoagulants, or NSAIDs, the FDA statement said.

Because of the "potential for serious adverse events" from confusing these products, the manufacturer has agreed to change the name of Maalox Total Relief to a name that does not include the word "Maalox," to change the graphics on the product's container, and to actively monitor and report adverse events. The manufacturer expects to start selling the renamed product in September 2010.

—From staff reports