

## FDA Commissioner Meets with Patient Advocate Groups

U.S. Food and Drug Administration (FDA) Commissioner Margaret Hamburg, MD, met with leaders and members of approximately 60 patient organizations at the National Organization for Rare Disorders Washington, DC, office in January to share her vision for the FDA and hear patient advocates' hopes, needs and concerns. In a direct, freestyle dialogue, Dr. Hamburg responded to questions on topics such as how the FDA compares benefits and risks of new products and how it communicates its expectations to those developing products. MPS mom and board of directors member Stephanie Bozarth spoke with Dr. Hamburg and left information with the commissioner, including suggestions on how to benefit the review of new drugs and therapies which we believe can make the FDA more responsive to our rare disease.

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## Rare Disease Caucus Update

In 2010 the Rare and Neglected Diseases Congressional Caucus was established to focus on bringing greater congressional attention to the 7,000 known rare diseases that currently have no approved therapy by working to ensure funding for research into these diseases, and providing a forum for members of congress, families and advocacy organizations to exchange ideas and policy concerns. Co-chairs include Rep. John Crowley (D-NY) and Rep. Leonhard Lance (R-NJ). Rep. Fed Upson (R-MI) stepped down from his chair position due to Energy and Commerce Committee rules, however he will continue to actively participate in the Caucus. On Feb. 1, Rep. Crowley and representatives from several advocacy groups, including the Kakkis EveryLife Foundation, met with staff from Rep. Upton, Lance and Crowley's offices to discuss some general issues of concern regarding rare disease drug development.

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## Federal Research Center Will Help Develop Medicines

The Obama administration has become so concerned about the slowing pace of new drugs coming out of the pharmaceutical industry that officials have decided to start a billion-dollar government drug development center to help create medicines. Creating a drug development center is a signature effort of Dr. Francis S. Collins, director of the National Institutes of Health (NIH).

The new effort comes as many large drug makers, unable to find enough new drugs, are paring back research. Promising discoveries in illnesses like depression and Parkinson's that once would have led to clinical trials are instead going unexplored because companies have neither the will nor the resources to undertake the effort.

The initial financing of the government's new drug center is relatively small compared with the \$45.8 billion that the industry estimates it invested in research in 2009. The cost of bringing a single drug to market can exceed \$1 billion, according to some estimates, and drug companies have typically spent twice as much on marketing as on research, a business model that is increasingly suspect.

The NIH has traditionally focused on basic research, such as describing the structure of proteins, leaving industry to create drugs using those compounds. But the drug industry's research productivity has been declining for 15 years, "and it certainly doesn't show any signs of turning upward," said Dr. Collins.

The job of the new center, to be called the National Center for Advancing Translational Sciences, is akin to that of a home seller who spruces up properties to attract buyers in a down market. In this case the center will do as much research as it needs to do so that it can attract drug company investment.

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### Legislative Committee:

Ernie Dummann, Chair  
 Debbie Dummann  
 Jeff Bardsley  
 Stephanie Bozarth  
 Chip Brady  
 Steve Holland  
 Austin Noll  
 MaryEllen Pendleton  
 Kim Whitecotton  
 Roger Chapin  
 Terri Klein  
 Kelly Rose  
 Laurie Turner  
 Barbara Wedehase

This means that, in some cases, the center will use one of the institutes' four new robotic screeners to find chemicals that affect enzymes and might lead to the development of a drug or a cure. In other cases, the center may need to not only discover the right chemicals but also perform animal tests to ensure they are safe and even start human trials to see if they work. All of that has traditionally been done by drug companies, not the government.

"None of this is intended to be competitive with the private sector," Dr. Collins said. "The hope would be that any project that reaches the point of commercial appeal would be moved out of the academic support line and into the private sector."

Whether the government can succeed where private industry has failed is uncertain, officials acknowledge, but they say doing nothing is not an option. The health and human services secretary, Kathleen Sebelius, sent a letter to Congress on Jan. 14 outlining the plan to open the new drug center by October—an unusually rapid turnaround for an idea first released with little fanfare in December.

Creating the center is a signature effort of Dr. Collins, who once directed the agency's Human Genome Project. Dr. Collins has been predicting for years that gene sequencing will lead to a vast array of new treatments, but years of effort and tens of billions of dollars in financing by drug makers in gene-related research has largely been a bust.

As a result, industry has become far less willing to follow the latest genetic advances with expensive clinical trials. Rather than wait longer, Dr. Collins has decided that the government can start the work itself.

"I am a little frustrated to see how many of the discoveries that do look as though they have therapeutic implications are waiting for the pharmaceutical industry to follow through with them," he said.

Dr. Collins' ability to conceive and create such a center in a few short months would have been impossible for most of his predecessors, who had nice offices but little power. But congress in recent years has invested real budgetary and administrative authority in the director's office, and Dr. Collins is the first to fully use these new powers.

Under the plan, more than \$700 million in research projects already under way at various institutes and centers would be brought together at the new center. But officials hope that the prospect of finding new drugs will lure congress into increasing the center's financing well beyond \$1 billion.

Hopes of new money may be optimistic. Republicans in the House have promised to cut the kind of discretionary domestic spending that supports the health institutes, and officials are already bracing for significant cuts this year. But Dr. Collins has hinted that he is willing to cannibalize other parts of the health institutes to bring more resources to the new center.

"There are some people that would say this is not the time to do something bold and ambitious because the budget is so tight," he said. "But we would be irresponsible not to take advantage of scientific opportunity, even if it means tightening in other places." For the plan to go into effect by October, the administration must by law get rid of one of the 27 centers and institutes already in existence at the NIH—something that has never been done before. So the administration plans to downgrade the National Center for Research Resources, in part by giving some of its functions to the new drug center.

*This article is an excerpt from The New York Times, written by Gardiner Harris, published Jan. 22, 2011.*

