Mid-Term Election Results 2010

November's mid-term elections are said to bring many changes in the nation's capital. Congress returned on Nov. 15, 2010, for what is known as a lame-duck session, lasting just a few weeks. (A "lame-duck session" is one that occurs after an election, but before the new Congress is sworn in.) Many of the substantive legislation, such as funding for government agencies, expiring tax cuts, stem cells and other important issues are being taken up in 2011.

Further delay of final congressional decisions on government funding could be a mixed bag at best for research. It is possible that Congress could decide to fund the entire Fiscal Year 2011 through a "continuing resolution" at 2010 levels. Unfortunately, that would mean the National Institutes of Health (NIH) would not receive its proposed $1 billion increase and the new Cures Acceleration Network would not get its planned $50 million to get the program started.

In regards to the appropriations committees, both the House and Senate will need to appoint several new members in the coming weeks. Sen. Tom Harkin (D-IA), a friend of NIH research, will remain as chairman of the Senate Appropriations Subcommittee.

With Republicans taking control of the House, one of the Rare Disease Caucus co-chairs, Rep. Fred Upton (R-MI), is in line to become chairman of the House Energy and Commerce Committee, which has jurisdiction over a great deal of health legislation. Sen. Harkin will remain as chairman of the Senate Health, Education, Labor & Pensions Committee, which handles health issues in the Senate.

We will be saying goodbye to Sen. Arlen Specter (D-PA) who lost in the May 2010 election primary from Pennsylvania. Sen. Specter, his Legislative Health Liaison Regina Campbell, and his staff will be missed. We look forward to meeting newly elected Sen. Pat Toomey (R-PA) in 2011.

Alabama	Shelby	R	Iowa	Grassley	R	Ohio	Portman	R
Alaska	Murkowski	R	Kansas	Moran	R	Oklahoma	Coburn	R
Arizona	McCain	R	Kentucky	Paul	R	Oregon	Wyden	D
Arkansas	Boozman	R	Louisiana	Vitter	R	Pennsylvania	Toomey	R
California	Boxer	D	Maryland	Mikulski	D	South Carolina	DeMint	R
Colorado	Bennet	D	Missouri	Blunt	R	South Dakota	Thune	R
Connecticut	Blumenthal	D	Nevada	Reid	D	Utah	Lee	R
Delaware	Coons	D	New Hampshire	Ayotte	R	Vermont	Leahy	D
Florida	Rubio	R	New York	Schumer	D	Washington	Murray	D
Georgia	Isakson	R	New York *Special Election*	Gillibrand	D	West Virginia	Manchin	D
Hawaii	Inouye	D	North Carolina	Burr	R	Wisconsin	Johnson	R
Idaho	Crapo	R	North Dakota	Hoeven	R			
Illinois	Kirk	R						
Indiana	Coats	R						

President Obama Signs Improving Access to Clinical Trials Act

On Oct. 5, President Obama signed legislation to enable people with rare diseases to participate in clinical trials without losing eligibility for public healthcare benefits. The Improving Access to Clinical Trials Act had passed the Senate on Aug. 5 and the House on Sept. 23.

Rare Disease Investigators Training Course Draws More Than 100 Participants

National Organization for Rare Disorders (NORD) co-sponsored a successful three-day course for rare disease investigators Oct. 18–20 in Rockville, MD. More than 100 researchers attended. The course was organized by the U.S. Food and Drug Administration (FDA) Center for Drug Evaluation and Research, the NIH Office of Rare Diseases Research, the NIH National Institute for Neurological Disorders and Stroke, and Duke University Medical Center.

Janet Woodcock, MD, director of the Center for Drug Evaluation and Research (CDER), opened the conference by stating the FDA's commitment to advancing orphan products through the regulatory system. The same commitment to orphan product development was voiced by other FDA and NIH speakers, including Christopher Austin, MD, director of the Clinical Genomics Center at NIH and NIH Therapeutics for Rare and Neglected Diseases.

NORD President Peter L. Saltonstall said that NORD is seeking ways to replicate the course next year. He particularly thanked Anne Pariser, MD, associate director for Rare Diseases, CDER, for her leadership in organizing the course.

Do We Need A War on Rare Diseases?

Is it time to declare a national "war on rare diseases," comparable to the famous "war on cancer" that began in the 1970s?

The Institute of Medicine report released in October stops short of recommending a full martial assault, but it does call for the creation of an aggressive national strategy to accelerate drug development. Policymakers, Congress, the NIH, FDA and patient advocates are all taking a hard look at how to improve research and development for rare diseases, which affect about 25 million people a year.

A number of innovative approaches are already being tried, including a program at NIH to get more involved in the development of drugs for rare diseases and an effort by the FDA to encourage orphan drug filings. The report lauds these new approaches but says that the sheer number of rare diseases, the different priorities of various researchers and advocacy groups and limited available resources require a national, integrated strategy.

One key recommendation is national task force, set up by the head of U.S. Department of Health and Human Services, to make sure NIH, FDA and patient-advocate efforts are better coordinated and monitored.

Other suggestions include setting up a repository of publicly available animal models for rare disorders and another public repository of biological data on rare diseases. Both could potentially be used by patient groups as well as investigators.

Thomas Boat, chairman of the Institute of Medicine, who wrote the report, tells the Wall Street Journal Health Blog that up until now, rare disease care and research has been disorder-specific, so "efforts have really been segmented." Boat says the recommendations are designed to increase cross-fertilization of ideas and sharing of resources such as repositories and specimens. He adds that committee members briefed NIH and FDA officials on the findings and also hope to talk to legislators.

Timothy Coté, director of the FDA's orphan products development office, says that a congressionally established committee he chairs is assessing the new report. Its recommendations for how FDA ought to tackle rare diseases are due to Congress by next March, and the report's ideas will be a big part of that strategy.

"They are right. We feel we already launched a war, and now it's time to escalate," he says.

By Amy Dockser Marcus, Wall Street Journal