What Healthcare Reform Means to Biotechnology

After passage of "The Patient Protection and Affordable Care Act of 2009" by the US House of Representatives in March, Biotechnology Industry Organization (BIO) president and CEO Jim Greenwood released a statement in support of the bill on behalf of biotech's main lobbying organization.

He stated that the bill provides "real solutions for our nation's health care challenges and real hope for patients living with debilitating diseases." He added that it should lead to "new and improved treatments, cures, and cost-savings for patients while driving job growth in our industry and maintaining our nation's global leadership in biotechnology innovation."

Thanks to bipartisan efforts, the bill enables the FDA to create a pathway for approval of biosimilar products, providing 12 years of market exclusivity to brand-name products. "According to the Congressional Budget Office, this provision will save patients tens of billions of dollars over the coming decade," said Greenwood. And it includes incentives that should "attract the massive investment required to speed the discovery and development of the next generation of breakthrough therapies and potential cures for the world's most debilitating diseases."

The bill should also provide financial relief to research-intensive, small biotechnology companies suffering from tight credit markets. According to the *San Francisco Business Times,* biotech and venture capitalists are two groups generally considered "winners" in the reform passage. As Ron Leuty reported in the *Times* on 22 March, "Health care reform expands the crowd of paying consumers that all drug makers — both biotech and traditional pill and tablet makers — can tap for good, old-fashioned revenue."

Clinical Trials: Use Caution Before Calling It Good

An international study of nearly 100 clinical trials that were stopped early after positive results has found that many of the therapeutic effects were exaggerated. In the *Journal of the American Medical Association,* the authors recommend that drug researchers resist pressures to end clinical trials early and instead continue them longer before even considering premature termination.

"In most cases, early stopping of clinical trials resulted in misleading estimates of treatment effects . . . likely to result in misguided decisions about the trade-off between risks and benefits of a therapy," said corresponding author Victor Montori of the Mayo Clinic. "On average, treatments with no effect would show a reduction in relative risk of almost 30% in stopped early trials. Treatments with a true relative risk reduction of 20% would show a reduction of over 40%."

Clinical trials end early because of a convincing (usually large) apparent difference between an experimental treatment and an existing standard therapy. Studies end so participants taking a placebo or less effective medication can also take the studied drug if it seems to work well. Doctors can prescribe a therapy sooner if it reaches the market earlier. Montori says almost everyone benefits from a trial ending early — doctors, researchers, funding sources, pharmaceutical companies, scientific journals, even reporters — everyone except patients, who may ultimately take the wrong drugs.

The study authors recommend that researchers use restraint and truncate clinical trials only near their end — and only with very good reason. Otherwise, says Dr. Montori, patients and physicians could be making treatment choices based on inaccurate information, or worse, choosing one treatment when another may be more appropriate.

Contamination Difficulties for Two Big Biotechs

While Genzyme Corporation faces a probable consent decree for its Cerezyme and Fabrazyme production troubles, the FDA asked physicians to temporarily stop using a GlaxoSmithKline vaccine because of contamination with a porcine virus.

Genzyme's chairman Henri Termeer said in a March conference call, "We are truly advancing in the recovery of the Allston Landing plant," even though the FDA plans to "take enforcement action to ensure that products manufactured at the plant are made in compliance with good manufacturing practice regulations." The agency may also levy fines against the company. The Allston plant has failed regulatory inspections since 2008, and in June 2009 a virus contamination issue forced its temporary closure.

Meanwhile, GSK's oral Rotarix vaccine (for protecting infants against rotavirus) was found in March 2010 to be contaminated with traces of type-I porcine circovirus (PCV-1). This DNA virus infects pigs but is not infectious to humans. It appears to have come from cell cultures used to make the vaccine.

Independent researchers found the contaminant while testing a new way to screen vaccines for impurities. Although this issue doesn't pose a safety risk, an FDA official said it would be smart to "pause and try to determine what this virus was doing in the vaccine and how it got there."

Pediatricians were advised to use Merck's RotaTeq rotavirus vaccine rather than GSK's product for the time being. A GSK spokesperson said the company "is now reviewing how best to replace, in a timely way, the cell bank and virus seeds used as base production material." The vaccine is made at a plant in Belgium. Preliminary tests of GSK's inactivated polio vaccine, also made in cell culture, found no virus contamination.