

Coverage and Consolidation: Key Issues for Biotech Companies

Manufacturers are concerned about congressional efforts to establish a Medicare pharmacy benefit and to regulate research while FDA shifts regulation of biologics and other drugs

The November elections gave control of both the House and Senate to Republicans who promised to provide pharmaceutical benefit coverage to Medicare beneficiaries. Industry is concerned that crafting such a program will stimulate efforts to rein in prices of new medicines. That could undermine incentives for new product development.

Manufacturers already are filing fewer new drug applications (NDAs) and biologics license applications (BLAs) with FDA. Stringent scientific and regulatory challenges — plus added testing requirements when future reimbursements are uncertain — may limit R&D even more.

FDA's plan to shift oversight of most biotech therapies from the Center for Biologics Evaluation and Research (CBER, www.fda.gov/cber) to the Center for Drug Evaluation and Research (CDER, www.fda.gov/cder) will disrupt the regulatory process during the consolidation effort. Republican leaders in Congress are talking about enacting legislation to curb research involving human embryos. Republican control on Capitol Hill and in the White House raises the prospect that damaging

policies involving scientific issues may become law without any checks and balances.

Full Agenda on Capitol Hill

Congress has approved bioterrorism and user fee legislation that increased resources for FDA and other government public health agencies, but most health policy legislation is still on the table.

Coverage for the uninsured. Democrats call for expanding federal and state healthcare programs. Republicans prefer to give consumers increased tax credits to purchase health insurance. The administration wants to fund a \$90 billion tax credit program (over 10 years) and hopes to gain bipartisan support by setting high standards for the type of insurance eligible for such credits.

Liability reform. Last year the House passed a bill that set caps on malpractice damage awards and limited class action suits, but it died in the Senate (1). Now there will be Senate hearings on the issue, but any kind of liability reform legislation faces a tough fight. Provisions that expand liability protections for vaccine manufacturers in the legislation establishing the Department of Homeland Security (www.whitehouse.gov/deptofhomeland) were nearly eliminated, and the measure may still be modified.

Stem cell research. A major fear is that Republican conservatives may push through legislation to ban human reproductive cloning that also kills stem cell research. Moderates will work to craft a bill that limits only "undesirable" cloning but allows somatic cell nuclear transfer research on embryos to continue.

Genetically modified plants. Recent accidental — and illegal — commingling of bioengineered plants with regular food crops raised an outcry from the food industry and consumer advocates. Food processors want biotech companies to limit "biopharming" to nonfood crops such as tobacco, whereas biotech companies prefer geographic curbs that separate biopharming from major food crop farming regions.

Medicare Rx. Senate Republicans are expected to begin the Medicare debate with last year's compromise bipartisan bill (2). The suggested program is projected to cost \$370 billion over 10 years — half the price tag of the Democrats' more generous plan. Manufacturers back this approach as preferable to a government-run program likely to lead to price controls.

A key concern is how various options for designing a Medicare drug benefit may affect the cost of the program and efforts to influence prescribing and reimbursement for pharmaceuticals. A recent analysis of Medicare prescription program design issues by the Congressional Budget Office predicts a rapid rise (10% a year) in Medicare beneficiaries' drug costs over the next decade, and expanded drug coverage for seniors will boost spending even more (3). These costs will encourage the use of formularies, generic drug substitutes, and other policies to shift cost-sharing to beneficiaries and to limit program expenditures.

Most biotech therapies are administered in clinics and doctors' offices and as such are already covered by Medicare, but adding



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an outpatient pharmacy benefit to the program would have a significant effect on industry. Biotech manufacturers are heavily dependent on capital markets, and the specter of price controls has already dried up investment in the industry. Manufacturers are therefore pressing for a benefit program administered by private entities. Provisions for stop-loss coverage for individuals with catastrophic expenses are important because they ensure access to high-cost breakthrough biotech treatments.

Generic drug access. Efforts to sweeten a Medicare benefit package to build bipartisan support may encourage changes in the Hatch-Waxman Act that governs generic drug regulation. The broad generic reform bill approved by the Senate last July (4) is unlikely to be reconsidered, but there will be interest in adding a moderate proposal that codifies some of the changes recently proposed by FDA. The Biotechnology Industry Organization (BIO, www.bio.org) emphasizes the technical difficulties in developing “equivalent” therapies for products made from living organisms, but cost pressures may open the door to legislative change.

Slowing Innovation

There is mounting concern over the continued decline in the number of new drugs and new molecular entities (NMEs) approved by FDA in recent years. In the mid-1990s, the agency approved more than 120 NDAs annually, including a record 53 NMEs in 1996. In 2001, FDA approved only 66 NDAs and 24 NMEs — and 2002 numbers look even bleaker (5).

Although the decline is not as dramatic for biologics, the application approval pace has slowed. FDA approved 18 new biotech products in each of the past two years, a decline from fiscal year 2000, and the list of approved new BLAs through October 2002 is short. Investigational new drug (IND) applications to test potential new biotech therapies and devices in humans peaked at 674 in fiscal year 2000, but fell to 528 this past year (6).

FDA’s policies may or may not aggravate these trends. Pharmaceutical and biotech manufacturers point out that they continue to invest huge sums in R&D, but their return has been curbed by regulatory demands. Main complaints involve extensive

premarket testing requirements and slower application review procedures.

Agency officials maintain that the numbers are misleading. With fewer applications, a few problematic submissions can boost overall statistics on the time that reviews take. Congress’ delay in approving the 2003 budgets for most government agencies has held up FDA funding increases, but once that impasse is addressed, the agency believes that review operations should improve.

Revising FDA’s Agenda

FDA efforts to ensure that its operations and policies do not prevent approval of useful new products may accelerate now that the agency has a new commissioner. Mark McClellan has been mapping out his agenda, which features initiatives to speed medicines to market, to update drug manufacturing standards, to retain FDA scientists, and to reduce medical errors. McClellan wants to accelerate access to generic drugs and to set standards for medical product bar coding. And he acknowledges the importance of addressing international regulatory issues.

Providing agency systems that are able to review new drugs, devices, and biological treatments as quickly and efficiently as possible is at the top of McClellan’s list and may require “a reexamination and an updating of the way FDA does its job,” the new commissioner told FDA and government officials on 15 November (7).

He emphasized the importance of retaining talented scientists if the agency is to fulfill its critical missions. That will require McClellan to stem the brain drain from FDA’s workforce, a trend that could accelerate with the shift of key staffers from CBER to CDER. Jay Siegel, former director of CBER’s Office of Therapeutics Research and Review and Kathryn Zoon, former director of CBER (see page 13), have already left the agency, and other biologics experts may follow suit.

The new commissioner aims to halt the exodus by establishing a work environment that “encourages creativity, efficiency, and superior performance.” In addition to building a new campus that will house most FDA offices, McClellan supports added educational opportunities and financial incentives for staffers, including salary increases linked to performance and retention allowances for physicians.

He also suggested that FDA professionals should be involved in research activities that are “relevant” to FDA’s mission, touching on one of the more contentious issues related to the CBER–CDER consolidation. Effective regulation of emerging technologies such as cell and gene therapies requires “a close connection to the latest scientific developments.” Such comments are meant to quell fears among CBER scientists that their research programs will be shut down. Addressing this issue and maintaining FDA’s capacity to oversee new biomedical technologies will provide an immediate challenge for the new commissioner. **BPI**

References

- (1) “Help Efficient, Accessible, Low Cost, Timely Healthcare (HEALTH) Act of 2002,” H.R. 4600, approved by the House (26 September 2002).
- (2) “Twenty-first Century Medicare Act,” S. 2729, introduced in the Senate (15 July 2002).
- (3) *Issues in Designing a Prescription Drug Benefit for Medicare* (Congressional Budget Office, Washington DC, October 2002).
- (4) “Greater Access to Affordable Pharmaceuticals Act of 2001,” S. 812, approved by the Senate (31 July 2002).
- (5) CDER, *CDER Report to the Nation: 2001* (FDA, Rockville, MD, 2001), pp. 8–11.
- (6) Elengold, M., “Center for Biologics Evaluation and Research Update,” presented at the ISPE annual meeting (4 November 2002).
- (7) McClellan, M.B., “Phase 2 Groundbreaking at White Oak,” FDA groundbreaking ceremony, White Oak, MD (15 November 2002).