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# SPECIALTY PHARMACY NEWS

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## Outlook 2010

### **2010 May See New Drugs for MS, Lupus; Personalized Therapies Will See Growth**

With hundreds of specialty drugs in the pipeline, expect to see a wide variety hitting the U.S. marketplace in 2010, say industry experts. And personalized medicine will continue to play a growing role in drug development, as more companies look to create products for specialized populations.

Stephen Cichy, executive vice president of managed care, marketing and product development for BioScrip, Inc., says there are three general patterns within the specialty drug pipeline:

**(1) Continued emergence of new products with new formulations:** "Specialty pharmacy is a marketplace no longer dominated by self-injectables," he says. "We'll see the continued emergence of infused and oral products. There will be quite a bit of oral penetration in 2010, which continues to challenge the definition of specialty pharmacy."

**(2) Emergence of new products to treat conditions with no therapeutic options:** One example he cites is Auxilium Pharmaceuticals, Inc.'s Xiaflex (collagenase clostridium histolyticum) for the treatment of Dupuytren's contracture. Renee Rayburg, director of clinical services for BioScrip, also points to InterMune's pirfenidone, an oral therapy for the treatment of idiopathic pulmonary fibrosis. Approval is expected in May, she says. And lupus, which has not had a new therapeutic option in 50 years, may finally see a new drug in Benlysta (belimumab), formerly LymphoStat-B, in late 2010. The drug is co-developed by Human Genome Sciences (HGS) and GlaxoSmithKline.

**(3) Continued expansion of indications for products already on the marketplace:** This represents a "pipeline within the pipeline," says Cichy. With many specialty drugs, "the mechanism of action is targeting common biological processes," he says. For example, an immunomodulatory drug "can impact any condition with an immune component." Rayburg tells *SPN* that "many oral oncology agents are being studied in multiple types of cancer."

Rayburg says there are a few additional pipeline events to watch for in 2010:

◆ **Significant drug approvals in multiple sclerosis (MS):** Acorda Therapeutics Inc. submitted a new drug application to the FDA earlier this year for Amaya (fampridine), with a proposed indication of improving walking ability in people with MS. Approval is expected in January, says Rayburg, who adds that there "could be significant utilization" among the 350,000+ people in the U.S. with MS. And Novartis may submit an application for its oral therapy FTY720 (fingolimod) to the FDA by the end of this month (*SPN 10/09, p. 1*). Approval is expected in fourth-quarter 2010, she says, and the drug could be the first oral MS drug to hit the marketplace.

◆ **FDA approval of Zalbin (albinterferon alfa-2b)** for the treatment of chronic hepatitis C is expected in third-quarter 2010, Rayburg says. Administered by subcutaneous injection once every two weeks, the therapy is a long-acting formulation of interferon alfa 2b, she says. It is being developed by HGS and Novartis.

◆ **FDA approval of the Amgen Inc. therapy Prolia (denosumab)** for the treatment of osteoporosis is expected in mid-2010, says Rayburg. The therapy is a subcutaneous injection every six months, which is "very favorable for compliance — oral therapies range from daily to once a month," she explains.

#### **Personalized Medicine, Genetic Testing on Rise**

Drugs that are effective only in people with a specific genetic makeup will continue to hit the marketplace. A recently released PricewaterhouseCoopers report estimates the personalized medicine market in the U.S. at \$232 billion, with 11% annual growth projected. By 2015, says the report, the market will nearly double to more than \$450 billion.

"We expect practical application of genetic testing will evolve in 2010 to give us a greater understanding of which tests are useful in impacting clinical decision making," says Ed Pezalla, M.D., national medical director and chief clinical officer for Aetna Pharmacy Management. Cichy notes that "as the biologic segment continues to evolve, there is continued

investment among several manufacturers toward personalized medicine. 2010 will see several products potentially approved."

For example, PTC Therapeutics' Ataluren (PTC124) is the "first investigational new drug developed to enable a functioning protein in patients with a genetic disorder because of a nonsense mutation," says Rayburg. The company is seeking approval for the treatment of nonsense mutation Duchenne muscular dystrophy. Rayburg notes that only a specific genetic test can identify a nonsense mutation — an alteration in the genetic code that prematurely stops the development of an essential protein — and the test's estimated cost is \$3,000 to \$4,000. Duchenne muscular dystrophy occurs in about 1 in 3,500 males annually, and approximately 15% of cases arise from the nonsense mutation, she says. With more drugs such as Ataluren being indicated for an ultra-orphan population, "the level of innovation these products bring to the marketplace creates an environment for a high price point," maintains Cichy.

According to F. Randy Vogenberg, Ph.D., co-founder of pharmaceutical consulting firm Employer-based Pharmaceutical Strategies, LLC, genetic testing will experience "continued interest and speeding up of product development leading into 2012. Diagnostic applications will continue to grow over the next two years prior to the next wave of drug therapy expansion. It's still not clear how drug therapy and companion diagnostics will be handled under future benefit plans or designs."

Other industry experts share this concern about genetic testing. "It is a very interesting landscape," says Domenick Bertelli, a partner with Putnam Associates, a pharmaceutical and biotechnology consulting firm. He says his company has been particularly focused on how personalized medicine applies to oncology. The science is moving quickly, he tells *SPN*, but there are some hurdles to leveraging the data. A Putnam survey showed that "thought leaders are way ahead of physicians," although the HER2 test for Herceptin (trastuzumab) "is helping train them," he says.

Two trends are emerging in genetic testing, Bertelli says:

**(1) Tumor typing:** More and more treatments will be based on where the mutation occurs, he says, noting that the KRAS mutation may be in the skin, lung and colon. "Oncologists are slowly starting to understand they need to do tumor typing earlier rather than wait," he explains. But "science is way ahead of the practice" in some instances, such as with gastrointestinal stromal tumors.

**(2) Blood-level monitoring:** Bertelli points to the cytochrome P450 enzyme system, which affects drug metabolism. "A lot of different drugs use different pathways" within the system, he notes. Some people may metabolize a drug very quickly, while others may metabolize that drug very slowly. But "there's a reasonable chance the doctor is not checking blood levels" because many of these drugs don't have titration information on their labels, he says. He notes that physicians will titrate immunosuppressant drugs in transplant patients very closely. "The same principle applies in general with oncology," he explains.

For these trends to gain wider acceptance, in addition to getting physicians on board, issues with payers and the regulatory environment need to be addressed. For example, payers need to take a look at their reimbursement. "If oncologists want to do sophisticated tumor typing, payers almost always OK this," he says. About 10% of doctors now are doing this, but "it's going to be a lot more expensive if 50% of doctors are doing this." In addition, "the FDA should be thinking hard on how to get this information onto drug labels," says Bertelli, because manufacturers are reluctant to do so. According to Elan Rubinstein, Pharm.D., founder and principal of consulting firm EB Rubinstein Associates, manufacturers of these tests have a role to play in health plan coverage as well. They will need to provide "a tighter link between the test and the value of its result, because test manufacturers are under a lot of pressure to demonstrate value before plans cover and doctors prescribe."

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