

By Taren Grom

► Specialty Pharma Targeted Opportunities

The growing U.S. specialty pharmaceuticals market is comprised of more than 90 companies marketing more than 550 prescription pharmaceutical products, according to MCD Group, and dozens of therapeutic categories, including CNS disorders, pain management, men's and women's health, rare diseases, and urology.

Specialty pharmaceuticals are mainly prescribed by clinical specialists and aimed at well-defined patient groups. In many cases, specialty products address large unmet needs that are inadequately being treated. Examples of specialty indications include but are not limited to infertility, gout, cystic fibrosis, pancreatic insufficiency, vitamin deficiencies, bipolar disorder, multiple sclerosis, and fibromyalgia.

Among the 80 companies MCD Group has identified as actively participating in the specialty pharma space, they range in size, from very small (five employees) to very large (1,000+ employees), but all are actively developing or marketing specialty pharmaceutical products.



ROBERT CHIOINI
CEO and President
Rockwell Medical Inc.

Developing drugs that address unmet needs and improve patient care, while securing reimbursement from both public and private payers, offers the greatest opportunity and challenge to specialty pharmaceutical companies such as Rockwell Medical.

In the current healthcare environment, payers, providers, and pharmaceutical companies are facing unprecedented pressure to reduce costs and increase efficiencies, while providing products that improve patients outcomes.

Rockwell Medical offers and is developing products, targeting end-stage renal disease

(ESRD), chronic kidney disease (CKD), and iron deficiency anemia.

Rockwell is close to bringing two drugs to the market that address unmet patient needs in the renal-dialysis space, offering potential for improved patient care at a lower cost.

This year the company is introducing Calcitriol, the lowest-cost FDA approved generic vitamin D injection.

Next year, the company anticipates the launch of Soluble Ferric Pyrophosphate (SFP), upon FDA approval. SFP is a physiologic non-invasive iron delivery therapy expected to improve patient safety and efficacy at a lower cost than current intravenous (IV) iron therapy. Data suggest SFP has the potential to address the unmet need of preventing functional iron deficiency in dialysis patients.



BILL HEIDEN
CEO
AMAG

The biggest challenge facing specialty pharmaceutical companies is reimbursement for our products.

The challenging regulatory environment for approval of new drugs is a singular and first step in bringing new specialty medicines to market, and the next step of successfully gaining reimbursement can be equally or more daunting. And it's a challenge that is not overcome just once, but on an ongoing basis as the reimbursement landscape is constantly changing and evolving.

The reimbursement changes occur at different times, across many insurance plans — public and private — and are sometimes product specific and other times are across a class of drugs. Specialty pharma companies must continually monitor this ever-changing landscape and be prepared to advocate for appropriate reimbursement for their medicines.

These constant changes affect not only specialty pharma companies, but also the economics of the practice of medicine by individual physicians and hospitals.

Most importantly, these sometimes hard-to-follow, or understand, reimbursement changes affect the patients we all serve because changes can often result in limited access to important medicines.



MICHAEL WYZGA
President and CEO
Radius Health Inc.

The ability of biotech to remain innovative is the biggest singular challenge facing us in the current environment.

This environment has brought intense pressure on the entire healthcare industry to contain and reduce costs while delivering care more efficiently with improved clinical outcomes.

The biggest opportunities for Radius lie in developing therapeutics for underserved patient populations, such as osteoporosis, but the challenges remain in securing the funding to support the pathway from innovation and discovery to bring a drug all the way to market.

These hurdles come from the very inception of a company. Taking a new and innovative idea, chemical entity or even a platform technology to proof of concept can be very costly. Demonstrating that you have an idea or an approach that works — something that will actually save and improve lives — costs enormous amounts of money. It is difficult to obtain funding from government agencies, insurance companies or investors, who want to minimize risk. ^{PV}



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SPECIALTY PHARMA UPDATE

Specialty pharmaceutical companies are generally focused on only a few therapeutic categories, and sometimes have a very narrow focus on one or two “specialty” indications. These companies usually have well-trained sales reps who call on specialty physicians, such as allergists, psychiatrists, endocrinologists, or pain management physicians, according to MCD Group LLC.

Companies active in the specialty pharma sector typically have small to medium-sized salesforces that promote products with annual sales of less than \$200 million, although there are exceptions. Purdue Pharma, for example, is a fully integrated specialty pharmaceutical company focusing on pain management, but its most successful product (OxyContin) currently has annual sales of more than \$3.0 billion. But Purdue still fits into the specialty category as it limits its focus to pain and CNS therapeutics.

Key Players

MCD Group has identified more than 80 companies actively participating in the specialty pharma space. These companies range in size, from very small (five employees) to very large (1,000+ employees), but all are actively developing or marketing specialty pharmaceutical products. The leading specialty pharma companies, based on revenue and company size, are Purdue Pharma, Cephalon, Shire, Allergan, and King Pharmaceuticals. These companies are all fully integrated with wide product offerings, but they still remain focused on select therapeutic areas.

R&D Capabilities

Specialty pharmaceutical companies may or may not have R&D capabilities; the larger companies such as Cephalon, Galderma, and Purdue have extensive in-house capabilities, while other companies such as NextWave Pharmaceuticals and Centrix Pharmaceuticals rely on outsourcing and partnerships to build their product portfolio. Small- to medium-sized

specialty companies tend to in-license late-stage or under-promoted products in therapeutic categories where they see an opportunity for revenue growth. Increasingly, specialty companies are also pursuing new indications or novel drug delivery formulations of existing pharmaceuticals.

Specialty Focus

Drug delivery companies are a key provider of specialty pharmaceutical products, and in fact, several of these companies are transitioning into specialty pharmaceutical companies themselves. As a result, the drug delivery company does not have to rely on partners for marketing of their novel drug delivery product. Examples of drug delivery companies that have changed their corporate strategy to become specialty pharma companies include Depomed and Eurand.

Therapeutic Categories

Specialty pharmaceutical companies are focusing on a number of different therapeutic areas, ranging from pain management and CNS disorders to women’s and men’s health. Some companies focus on only one area such as dermatology, while others have expanded to focus on three or four therapeutic areas. MCD Group has identified specialty pharma companies focused on the following key therapeutic segments:

- » Central Nervous System
- » Pain Management
- » Dermatology
- » Ophthalmics
- » Gastrointestinal
- » Respiratory/Allergy
- » Women’s Health
- » Men’s Health
- » Cardiovascular
- » Urology
- » Diabetes
- » Hospital and Acute Care
- » Rare Diseases

The therapeutic areas targeted most

frequently by specialty pharma companies are CNS (neurology), dermatology, respiratory/allergy, and women’s health. Ophthalmics, pain management and gastrointestinal illnesses are also growing categories, with an increasing number of participant companies.

Market Size

The U.S. specialty pharmaceuticals market had a value of about \$21 billion in 2009. This market value was determined by the U.S. sales of specialty pharma products, as reported by each pharmaceutical company (ex-manufacturer level). In some cases product sales are not available or reported, therefore MCD Group made estimates based on historical sales data, analyst forecasts, or other industry sources.

Drug delivery specialty pharmaceutical products are key contributors to the U.S. specialty pharma market, generating revenue of \$10.6 billion in 2009. Oral modified release specialty products generated revenue of about \$7.0 billion, accounting for one-third of the total specialty pharma market. Examples of the most successful marketed oral drug delivery specialty products currently include OxyContin (controlled-release oxycodone), Adderall XR (extended-release mixed amphetamines), and Asacol (delayed-release mesalamine). Transdermal and pulmonary drug delivery products are also key revenue producers in this market segment.

There are more than 550 specialty pharma products marketed in the United States. The categories with the largest number of marketed products are women’s health (about 90), and dermatology (about 130 products). Overall, the top-selling specialty pharmaceutical products were products for pain, sleep disorders, opioid dependence, and ADHD. With respect to total product revenue, four therapeutic categories generated the highest sales: pain management, CNS, ophthalmics, and dermatology.

Source: MCD Group LLC, Guide to U.S. Specialty Pharmaceutical Market: Companies, Products and Market Data