

Looking Ahead at the Specialty Drug Pipeline After an Active Year of Approvals

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Aimee Tharaldson, PharmD, of Express Scripts, kicked off the Academy of Managed Care Pharmacy (AMCP) 2017 Nexus, held October 16-19 in Dallas, Texas, with a presentation on the pipeline of specialty pharmaceuticals in development.

Tharaldson, who is senior clinical consultant of Emerging Therapeutics at Express Scripts, highlighted the continued trends in the specialty drug market, which include increasing competition from novel molecules, generics, and biosimilars. The latter in particular will take on even greater importance as more regulatory and litigation hurdles are eliminated, she predicted.

This year has yielded additional guidance on the naming of biosimilar drugs, but not on the interchangeability requirements. Once those regulations are known and biosimilar drugs can be deemed interchangeable, Tharaldson anticipates they will produce greater cost savings. Biosimilars “are really acting like competing brands,” she said.

Another trend has been a spike in cancer drug approvals; 13 have been approved so far this year, compared with just 4 in 2016. However, Tharaldson noted, drug prices are not dropping even as more options enter the market. Similarly, orphan drugs, which constitute a growing percentage of the specialty drug pipeline, cost an average of 5 times more than nonorphan drugs. Although orphan drugs are used to treat rare conditions, 30% of drugs in this category are considered “blockbusters” as they bring in \$1 billion or more each year.

Tharaldson provided a brief overview of the current specialty drug market in light of several new approvals. These drugs are responsible for 37% of per-member-per-year pharmacy benefit spending, a proportion that is expected to grow as the roster of available drugs grows longer. She mentioned in particular a handful of specialty drugs that received FDA approval this year but are expected to receive additional indications or be joined by competitors in coming years.

For example, dupilumab was approved for atopic dermatitis this year, but Tharaldson forecasts another approval next year for severe asthma. According to Tharaldson, this is an important drug to keep an eye on, as it is “definitely going to expand in its use.”

She mentioned another “interesting approval,” that of tisagenlecleucel (Kymriah), which became the first approved chimeric antigen receptor (CAR) T-cell treatment for cancer in late August. Its high price tag has made headlines, but its response rates and efficacy are also impressive, leading Tharaldson to predict “there’s going to

be more competition in the CAR T space.”

Year after year, AMCP Nexus attendees rely on Tharaldson to provide insights into the specialty drug pipeline, and she did not disappoint. Experts crowded into a ballroom to hear her discuss the current specialty treatment options and future trends in 9 main classes of conditions.

For example, 7 drugs for inflammatory conditions are expected to receive a decision from the FDA within 2018, with news on golimumab for psoriatic arthritis and ankylosing spondylitis due next week.

In the cancer space, a second CAR T-cell therapy, Juno Therapeutics' JCAR017 for treating B-cell non-Hodgkin lymphoma, should receive an FDA decision sometime in 2018. However, about 40% of the cancer drugs in the current pipeline are oral medications. Similarly, 9 of 10 of the drugs in the pipeline for nonalcoholic steatohepatitis are oral drugs.

Tharaldson also discussed the 3 new drugs in the hemophilia pipeline. One of them, emicizumab, is a monoclonal antibody that replaces the activity of Factor VII, and its manufacturer Genentech is confident it will be approved later this year.

According to Tharaldson, the upcoming pipeline for migraine therapies is dominated by calcitonin gene-related peptide receptor inhibitors, some of which may be delivered in monthly injections.

In contrast, some therapy classes are moving more slowly. The pipeline for hepatitis C therapies is fairly quiet due to market saturation, and any drugs for treating progressive multiple sclerosis are not expected until at least 2020. Alzheimer's disease is another difficult class; its prospective pipeline includes immunotherapies like anti-amyloid β antibodies and oral drugs like BACE inhibitors, but none are up for approval until 2020 and beyond.

Tharaldson also highlighted some miscellaneous specialty drugs that are pending approval, like Intarcia's ITCA 650 with exenatide, which is an implantable minipump for treating type 2 diabetes that is expected to receive approval this November. Benralizumab, an injectable asthma therapy, is due for an approval by December, and its indications could expand to include chronic obstructive pulmonary disease in 2018.

Not all specialty drugs have such a rosy outlook, Tharaldson noted. Ataluren, a protein restoration therapy for the rare disease Duchenne muscular dystrophy, is due for a decision by October 24 after being submitted “under protest”; Tharaldson estimated it had just a 10% chance of approval next week.