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Payers struggle to keep up with high rate of FDA drug approvals

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By Bryant Furlow

FDA incentives to encourage development of rare disease therapies have paid dividends, with an unprecedented number of novel treatments in the orphan drugs pipeline. But expedited regulatory review has also raised concerns about a lowered evidentiary bar when it comes to drug efficacy, cautioned speakers at an October 19 session of the Academy of Managed Care Pharmacy (AMCP) 2017 Nexus in Dallas.

"Rare diseases have come to the forefront and have captured everybody's attention," said Alexandra Lin, PharmD, manager, pharmacy clinical programs & customer support at Blue Cross Blue Shield of Michigan, in Detroit.

In 2016, the FDA approved 22 new drugs, of which 41% had an orphan drug designation, she reported.

"This year, the FDA's already approved 29 novel drugs, of which 38% are orphan-drug designated," she said. "The pipeline has over 560 investigational drugs with orphan designations in development and we expect this number to continue growing."

But those advances have come at the expense of a strong evidence base for approvals.

"The FDA has been approving drugs faster and faster," cautioned Elizabeth Saltzman, PharmD, manager, specialty pharmacy utilization management at Blue Cross Blue Shield of Michigan. "It's not always with clear evidence proving the medication is both safe and efficacious."

Post-approval studies lacking

The FDA has not always demanded follow-up by manufacturers; post-approval studies have not been conducted, in some cases, several years after expedited drug approvals based on preliminary clinical studies that used surrogate endpoints instead of survival rates, for example.

"There must be some type of standardization created for post-approval studies," Lin urged. "Follow-up by the manufacturers and FDA should be guaranteed. The FDA should be prepared to revoke approval of novel drugs if no superior efficacy of clinical endpoints is found."

Informally polling the NEXUS audience, Saltzman asked, "Has your organization had a difficult time forecasting and keeping up with new products coming to market?" Nine in 10 audience members answered in the affirmative.

Nodding at their response, she said that Blue Cross Blue Shield of Michigan has found it difficult as well.

"We do pipeline monitoring and have an Emerging Therapies Workgroup," she said. "At BCBSM, our overall clinical approach for drug formulary approvals is to look at safety, efficacy and cost—and cost? That's been huge lately."

The team of pharmacists, actuaries, physicians, and data analysts also examines treatment guidelines, expert opinion, and "pipeline, pipeline, pipeline," Saltzman said.

The team examines approved alternative medications but those are few and far between for rare diseases, Saltzman noted. That makes it all the more important to understand other investigational drugs that might come to market in the future, she explained.

Next: What payers should do

Expect the unexpected

Payers need increased transparency and access to specialists, and must pay "more and more" attention to the drug-development pipeline, Saltzman said.

FDA's priority reviews and other hastened approval mechanisms mean that payers have to "expect the unexpected," Saltzman said, citing the previous afternoon's surprise FDA approval of Kite/Gilead's CAR-T therapy axicabtagene ciloleucel (KTE-C19) for non-Hodgkin lymphoma—a month earlier than expected, despite lingering concerns about cost and toxicity.

Investigational CAR-T (chimeric antigen receptor T-cell) immunotherapies are definitely on the Emerging Therapies Workgroup's radar. CAR-T cells are the patient's own cells, genetically engineered at a remote lab to recognize and attack tumor cells. Blood is taken from the patient, cryopreserved and shipped to the specialty lab for genetic reprogramming and amplification, then shipped back to the treating hospital for infusion into the patient.

"Vein-to-vein," the process can take between 16 to 22 days, Lin said.

The expense of CAR-Ts' highly-personalized manufacturing process—an anticipated $475,000 per treatment for Novartis's tisagenlecleucel (Kymriah), a treatment for children and young adults with relapsed acute lymphoblastic leukemia (ALL)—has made headlines.

The Institute for Clinical and Economic Review (ICER) recommends that payers develop "internal sophistication in knowledge about gene therapies," Lin said. "They recommend early dialogue with manufacturers of promising new gene therapies about making clinical studies as robust as possible—particularly if randomized, controlled trials will not be performed."

Payers should also work with clinicians, patient groups, regulators, and manufacturers to develop robust patient registries, Lin added.

They should also explore and negotiate outcomes-based payment agreements, she said.