

# FDA Just Approved Some Highly Anticipated Specialty Drugs; Payers Must Prepare Now

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In late March, the FDA went on an approval tear, paving the way for multiple anticipated specialty drugs to come onto the U.S. market (*SPN 2/17, p. 8*). Some products join already-crowded fields, while others are orphan therapies offering treatments for conditions that previously had none. But either way, payers need to be prepared to determine how they will best manage these new drugs, ensuring appropriate members are able to access them.

Bill Sullivan, principal consultant at Specialty Pharmacy Solutions LLC, called the last week of March “a banner week for the FDA” in a client alert. As the end of the first quarter of 2017 approached, the FDA’s approvals mean 2017 “is finally on track to be [a] substantially more robust approval rate by comparison to 2016 where, at year end, we were only treated to 22 novel drugs,” he observed (see FDA approvals, p. 8).

Here are some of the highlights:

- ***Bavencio (avelumab) for metastatic Merkel cell carcinoma, approved March 23:*** The drug is the first to gain FDA approval for the condition, and the agency gave the drug from Pfizer Inc. and Merck KGaA business EMD Serono Inc. accelerated approval. The programmed death ligand-1 inhibitor is the fourth PD-L1/PD-1 inhibitor to hit the U.S. market, joining Keytruda (pembrolizumab), Opdivo (nivolumab) and Tecentriq (atezolizumab). The product is administered as an infusion over 60 minutes every two weeks.

## MS Drug Is Among New Approvals

“I don’t think this one is going to be very significant” but only because the condition is “very rare,” says April Kunze, senior director, formulary development and trend management strategy at Prime Therapeutics LLC. About 1,600 people in the U.S. are diagnosed annually with the aggressive form of skin cancer, according to the FDA, which gave the drug breakthrough therapy designation, priority review and orphan drug designation.

Because of the low prevalence, “I don’t think it’ll get a ton of uptake,” says Kunze. However, the drug is in clinical trials for multiple other oncologic indications, including urothelial, non-small cell lung, gastric, renal cell and ovarian cancers. If it gains additional approvals, this should allow it to compete with the other PD-L1/PD-1 inhibitors, which all have FDA approval for more than one indication.

Bavencio is priced at \$13,000 per month, or \$156,000 annually, which “is in line with the other PD-1 and PD-L1 inhibitors,” Kunze says. And while much of the media focus on these drugs’ prices is on the per-year amount, Kunze points out that the majority of people taking them can tolerate them for usually only four to six months, so “there are not a ton of people that continue a full year” of treatment.

• ***Zejula (niraparib) for recurrent epithelial ovarian, fallopian tube or primary peritoneal cancer, approved March 27:*** The Tesaro, Inc. drug is a poly ADP-ribose polymerase (PARP) inhibitor and is the third one that the FDA has approved to treat ovarian cancer, joining Lynparza (olaparib) and Rubraca (rucaparib). The agency gave the drug orphan designation for that indication, as well as breakthrough therapy and fast track designation, and priority review.

“Zejula, while an oral therapy, is being released to market through limited distribution using a reported ‘small panel’ of specialty pharmacies,” notes Sullivan.

The company has not disclosed the drug’s price, but analysts expect it to be around \$14,000 per month.

“Like a lot of oncology drugs, Zejula is used after some standard of care with [disease] progression,” Kunze says. However, while Lynparza and Rubraca are indicated for use in BRCA-mutated advanced ovarian cancer, Zejula does not have that specification. “You don’t have to have a BRCA mutation to take Zejula,” but in clinical trials, “it looked like it did a little better in BRCA-positive” people, she says. And it has “a lot of side effects, so it becomes a tolerability” issue, with people likely to weigh “benefits vs. risks.”

• ***Dupixent (dupilumab) for atopic dermatitis, approved March 28:*** The therapy from Sanofi and Regeneron Pharmaceuticals, Inc. is the first and only biologic the FDA has approved to treat adults with moderate-to-severe atopic dermatitis, a form of eczema, whose condition isn’t controlled with topical drugs. The FDA granted it breakthrough therapy designation and priority review, but, as Sullivan points out, “it did not qualify for an orphan designation...so it means that there is a significant base of patients for this condition.”

“Dupixent will not be restricted to limited distribution,” he says, which is “good news for specialty pharmacies. However, there appears to be some inclusion of a small number of select specialty pharmacies in the launch — perhaps as part of a patient support (hub [services]?) program.”

“We’re really watching this closely,” says Kunze. “This is an untreated condition to some extent. There’s nowhere to go once you fail topicals” other than an off-label therapy.

The drug's annual price is \$37,000, which "is in line with some of the other biologics for" psoriasis, an inflammatory skin disease, Kunze observes, adding that Prime anticipates that Dupixent's impact will be between 10 and 30 cents per member per month.

Management of the drug, she says, likely will be "locked down to its labeled indications": moderate-to-severe atopic dermatitis in adults who have failed topicals. "A lot of kids develop" the condition, and about "10% of them keep it as an adult," and about "10% of those would require systemic therapy such as this." She estimates that "about 11 members per 100,000 are appropriate candidates, so you really want to manage" it in line with its indications. It has the "potential for a lot of use because atopic dermatitis is really itchy, and patients are very uncomfortable."

At Express Scripts Holding Co., "Our general thoughts on Dupixent are that Regeneron and Sanofi have been responsible in how they have approached this launch," says spokesperson Jennifer Luddy. "They engaged payers early and worked to get our input. This different tone and tenor is a result of the actions we have taken to change the marketplace dynamic. This is how it should work."

She tells AIS Health that "we had a good amount of education on both sides about the product and about the need to ensure appropriate access for patients. The patient has been the main focus for everyone."

Express Scripts has placed Dupixent on its National Preferred Formulary, says Luddy.

• ***Ocrevus (ocrelizumab) for primary progressive multiple sclerosis (PPMS) and relapsing MS (RMS), approved March 29:*** The infusible from Genentech USA, Inc., a Roche Group company, is the only treatment the FDA has approved for PPMS, which is diagnosed in about 15% of people with MS, says Kunze. "It will likely be approved for primary progressive MS without much pushback from plans," she says. People with PPMS "technically shouldn't be getting treated because nothing is approved for that indication. We assume this is an untreated population, but we know current therapies are being used off label,...but we don't know to what extent," since ICD-9 and ICD-10 codes just indicate a diagnosis of MS.

However, Kunze says, Ocrevus' use in RMS will be "an interesting battle" in that it's "priced pretty competitively to" other self-injected therapies that treat the condition: \$65,000 annually. Sullivan notes that the price is a 25% discount to Rebif, its "primary competitor." Kunze adds that Prime has not heard about any potential rebating for the drug. But since the drug is infused, it likely will fall under the medical benefit, and manufacturers typically do not offer discounts for these products, says Sullivan.

“Self-injectable biologics including Rebif, on the other hand, are often covered under the pharmacy benefit, and their list prices often bake in room for manufacturer discounts” to help secure patient access to the drugs, explains Sullivan.

“So, with a hefty rebate, the price differential can easily be closed. Merck is likely to make that investment to protect its brand share. Although payers are rapidly implementing prior authorizations on drugs administered under the medical benefit, it is not as easy to enforce, especially in the hospital outpatient setting.”

## Trials Showed Superiority Over Rebif

Clinical trials showed Ocrevus is superior to Rebif (interferon beta-1a). “But the hard part about the drug is it’s infused, and there is relatively little safety data,” Kunze says. With MS, when patients are on a therapy and doing well, they tend to stay on that therapy.

Still, an Institute of Clinical and Economic Review report says it is “a cost-effective therapy” and has a “lot of bang for the buck,” says Kunze. “I’m curious to see how it plays out....I think most people won’t start with it but will progress to it.”

“For Ocrevus, we are excited to have a new agent in the market for MS, especially for a subset of patients which are difficult to treat,” says Luddy. “MS is one of the areas [where] we have seen the fastest growth in prices and has been a challenge for patients and plan sponsors. We are currently analyzing the clinical data and economics.”

Sullivan notes that Ocrevus is available through a limited-distribution program. “Sales to physicians (and presumably home care) would predominantly be...patient-specific/white bag/pharmacy-benefit-adjudicated. Volume is not expected to be high.” However, “With strong clinical trial outcomes, physicians will seriously consider and write for Ocrevus — especially since it brings additional revenue back into the office or outpatient clinic.”

In addition, he says, “Ocrevus is an IV-infused medication, so there might be ‘some’ opportunity for home infusion. Another infused drug in the MS category is Tysabri, which payers commonly include as a pharmacy benefit and is not infrequently sold to home care providers.” The drug is administered every six months over three-and-a-half hours.

According to Sullivan, “Making smart marketing decisions is critical to creating demand for any product, especially when a new product (in this case Ocrevus, an infused therapy) is launched into an already-crowded category like multiple sclerosis with over a dozen established brands.

Differentiation, therefore, is essential to elbow your way into the pack and gain market share. Roche

has been able to achieve that differentiation in several important ways: price, route of administration and indication.”

• ***Austedo (deutrabenazine) for chorea associated with Huntington’s disease, approved April***

**3:** The Teva Pharmaceutical Industries Ltd. product, which received orphan drug designation, treats the writhing movements that affect about 90% of people with Huntington’s.

“The condition clearly qualifies for an orphan designation with only 35,000 potential patients in the U.S.,” explains Sullivan. “Approval was based on a very small trial that included only 90 patients, underscoring the need for a new, more efficacious, therapeutic option for this condition.”

He points out that “Austedo is yet another therapy coming to market through limited distribution. Given that it is such a small population and has a very serious side-effects profile (depression and suicidality),” Teva’s use of a limited-distribution program “is not surprising.” And while pricing information was not available by press time, “with such a small patient population, one can safely assume that it won’t be cheap, especially given Teva’s track record on pricing (and regular price increases) for its high-end products like Copaxone.”

Analysts estimate the annual price will be around \$60,000. That compares with the \$152,000 annual price of Xenazine (tetrabenazine), the only other drug approved for the indication, and generic forms of the drug that cost around \$96,000 per year.

“It’s a very interesting strategy in a sense” with respect to Xenazine’s and the generics’ availability, says Kunze. The anticipated pricing will “make it easy” for people on that drug to transition to the newer product, which is supposed to alleviate some of the side effects of the older drug.

What’s also interesting about this drug is that Teva has submitted a supplemental new drug application for Austedo to treat tardive dyskinesia that the agency could make a decision on by August, notes Kunze.

But on April 11, the FDA approved the first drug to treat that condition, Neurocrine Biosciences, Inc.’s Ingrezza (valbenazine) (see briefs, below). That company will not disclose the price until May, but analysts expect it will be between \$20,000 and \$60,000 annually — most likely closer to Austedo’s anticipated \$60,000 price tag.

In light of this, “it feels a little like [Austedo is] trying to compete in both markets,” she observes, adding that tardive dyskinesia has a bigger patient population than Huntington’s chorea. If Austedo gains that additional indication, this offers “a potential preferred product” strategy for payers.