PRIME THERAPEUTICS®

Specialty Pipeline **MONTHLY UPDATE**

Critical updates in an ever changing environment

July 2021

NEW DRUG INFORMATION

- **Rylaze™ (asparaginase erwinia chrysanthemi (recombinant)-rywn):** The U.S. Food and Drug Administration (FDA) approval of Rylaze (asparaginase erwinia chrysanthemi [recombinant]-rywn) for use as a component of a multi-agent chemotherapeutic regimen for the treatment of acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL) in pediatric and adult patients one month and older who have developed hypersensitivity to E. coli-derived asparaginase.[1] Rylaze has launched and has an average wholesale price (AWP) of $5,268 per vial.

- **Rezurock™ (belumosudil):** The FDA approved Kadmon Holdings' Rezurock as a 200mg once daily dose for the treatment of adult and pediatric patients 12 years and older with chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy. Rezurock is the first FDA-approved small molecule inhibitor of ROCK2, a signaling pathway that modulates inflammatory responses and fibrotic processes. The FDA approval of Rezurock is based on safety and efficacy results from ROCKstar, a randomized, open-label, multicenter pivotal trial in patients with cGVHD who had received two to five prior lines of systemic therapy. The primary endpoint was an overall response rate (ORR) through cycle 7 day 1 of treatment. Rezurock demonstrated an ORR of 75%, with 6% of patients achieving a complete response and 69% achieving a partial response. The median DOR was 1.9 months and the median time to first response was 1.8 months.[2] Rezurock is expected to be available late August 2021 with a price of $15,500 per 30-count bottle.[3]

- **Bylvay™ (odevixibat):** The FDA has approved Albireo Pharma's Bylvay for the treatment of pruritus in patients three months and older with progressive familial intrahepatic cholestasis (PFIC). Bylvay is a once daily, non-systemic ileal bile acid transport inhibitor (IBATi). Bylvay was approved based on two Phase 3 clinical trials that met its primary endpoint in pruritus and serum bile acid when compared to placebo. Additionally, PEDFIC 2, a long-term, open-label Phase 3 extension study, found Bylvay delivered sustained reductions in serum bile acids as well as improvements in pruritus assessments, growth and other markers of liver function in patients treated up to 48 weeks.[4] Bylvay has launched with a wholesale acquisition cost (WAC) of $385,000 for an 18kg patient.[5]

NEW INDICATIONS

- **Aduhelm™ (aducanumab-avwa):** The FDA has approved the revised indication for Biogen's Aduhelm for treatment of Alzheimer's disease to emphasize that treatment should be initiated only in patients with mild cognitive impairment or the mild dementia stage of disease, the population that started treatment in clinical trials.
- **Padcev® (enfortumab vedotin-ejfy):** The FDA has approved Astellas and Seagen's Padcev for treatment of patients with locally advanced or metastatic urothelial cancer who have received a PD-1/L1 inhibitor and are ineligible for cisplatin.
- **Darzalex Faspro® (daratumumab and hyaluronidase-fihj):** The FDA expanded labeling for Johnson & Johnson Healthcare and Genmab's Darzalex Faspro (daratumumab and hyaluronidase-fihj) for treatment of patients with relapsed or refractory multiple myeloma who have received at least one prior line of therapy, based on the APOLLO trial.
- **Solosec® (secnidazole):** The FDA has approved Lupin Pharm's Solosec as a single-dose oral treatment for trichomoniasis in adults and adolescents.

JULY NEWS

- "When the FDA granted Biogen an accelerated approval for Biogen's Aduhelm, it wasn't just Alzheimer's patients who got their hopes up. There is, for instance, a related condition called cerebral amyloid angiopathy (CAA) — which, like Alzheimer's, is thought to be triggered by accumulation of beta amyloid deposits. The difference is that the amyloid is deposited in vessels in the brain rather than as plaques, potentially leading to bleeding and injury. And patients wanted to know: Since Aduhelm was approved on its ability to clear the very same protein that's causing their disease, should they be taking it off-label?"[6]
- "The FDA's Cardiovascular and Renal Drugs Advisory Committee (CRDAC) has voted 13 to 1 that the benefit-risk profile of roxadustat does not support approval for the treatment of anemia in chronic kidney disease (CKD) in non-dialysis dependent (NDD) adult patients, and 12 to 2 that the benefit-risk profile of roxadustat does not support approval for the treatment of anemia in CKD in dialysis-dependent (DD) adult patients. The FDA will consider the vote, independent opinions and recommendations from experts as it reviews the new drug application (NDA) and is not bound by the Committee's recommendation."[7]
- "With the FDA's JAK hesitations wearing on, atopic dermatitis hopefuls from AbbVie and Eli Lilly will have to keep waiting for a chance to see the market. The companies separately said on Friday that the FDA again delayed decisions over their applications for Rinvoq and Olumiant in moderate to severe atopic dermatitis. Neither provided an updated timeline for the agency's decision. The FDA's deferral is unsurprising given that the agency has already halted verdicts across drugs in the JAK class of medicines for months, Barclays analysts said in a Friday note to clients."[8]

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