NEW DRUG INFORMATION

● **Nubeqa™ (darolutamide):** The U.S. Food and Drug Administration (FDA) approved Bayer’s Nubeqa for the treatment of non-metastatic castration-resistant prostate cancer (nmCRPC). Nubeqa is the third androgen receptor inhibitor to enter the nmCRPC market, joining Pfizer and Astellas’ Xtandi® (enzalutamide) and Johnson & Johnson’s Erleada® (apalutamide). Nubeqa may have similar efficacy to Xtandi and Erleada, but Nubeqa appears to have lower rates of central nervous system (CNS) side effects including: fatigue, falls, fractures and seizures. Nubeqa has a wholesale acquisition cost (WAC) of $11,550 for a 30-day supply of 300mg tablets, and annual WAC of $138,600.¹

● **Turalio™ (pexidartinib):** Daiichi Sankyo's Turalio received FDA approval for treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT) associated with severe morbidity or functional limitations and not responsive to improvement with surgery. It is the first approved therapy for this indication. Turalio is approved with a boxed warning for hepatotoxicity due to the risk of serious and potentially fatal liver injury. Due to this warning, Turalio will be available only through a restricted risk evaluation and mitigation strategy (REMS) program. Distribution is only allowed through exclusive specialty pharmacies directly to the provider.² Turalio will have an annual WAC of around $240,000.³

● **Pretomanid Tablets:** The FDA approved TB Alliance's Pretomanid in combination with bedaquiline and linezolid (BPaL) for the treatment of adult patients with treatment-intolerant or nonresponsive multi-drug resistant pulmonary tuberculosis (MDR-TB) and extensively drug-resistant tuberculosis (XDR-TB). BPaL is an oral, six-month treatment regimen; whereas, current treatment options use a combination of antibiotic drugs for up to two years. Launch plans and pricing are pending.⁴

While the information in this newsletter is from sources we believe to be reliable, we do not warrant that the information in this document is free from error. Use it only as a guide. Statements regarding drugs or manufacturers are not intended as promotion; those statements should not be used to make assumptions about formulary status. Each trademarked drug name is the property of its respective owner.
**Specialty Pipeline Monthly Update: August 2019**

- **Rozlytrek™ (entrectinib):** Genentech/Roche received FDA approval of Rozlytrek for the treatment of adult patients with metastatic non-small cell lung cancer (NSCLC) whose tumors are ROS1-positive. Rozlytrek also received accelerated approval for the treatment of adult and pediatric patients 12 years of age and older with solid tumors that have a neurotrophic tyrosine receptor kinase (NTRK) gene fusion without a known acquired resistance mutation, are metastatic or where surgical resection is likely to result in severe morbidity, and have progressed following treatment or have no satisfactory alternative therapy. The annual WAC will be approximately $205,000. This is about 50% less than Bayer’s Vitrakvi® (larotrectinib), which is also approved for a similar indication for NTRK gene fusion tumors.5

- **Inrebic™ (fedratinib):** The FDA approved Celgene’s Inrebic for the treatment of adult patients with intermediate-2 or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis (MF). Inrebic joins Incyte’s Jakafi® (ruxolitinib) in the myelofibrosis marketplace, but Jakafi covers all intermediate and high-risk adults with MF. Inrebic has a boxed warning in its label for encephalopathy; Jakafi does not contain a boxed warning in its label.

- **Rinvoq™ (upadacitinib):** The FDA approved Abbvie’s Rinvoq for the treatment of adults with moderately to severely active rheumatoid arthritis who have had an inadequate response or intolerance to methotrexate. Rinvoq carries a boxed warning of risk of serious infections, malignancy and thrombosis similar to other Janus kinase inhibitors indicated for rheumatoid arthritis including Eli Lilly & Co.’s Olumiant® (baricitinib) and Pfizer’s Xeljanz® (tofacitinib). Rinvoq is set to launch in late August.

**NEW INDICATIONS**

- **Keytruda® (pembrolizumab):** The FDA expanded Merck’s label of Keytruda to include monotherapy for patients with recurrent locally advanced or metastatic squamous cell carcinoma of the esophagus whose tumors express PD-L1 with disease progression after one or more previous lines of systemic therapy.

- **Sirturo® (bedaquiline):** The FDA granted accelerated approval for Sirturo as part of combination therapy in pediatric patients, those over the age of 12 and younger than 18 and weighing at least 66 lbs (30 kg), with pulmonary multidrug-resistant tuberculosis (MDR-TB) when effective treatment regimen cannot otherwise be provided. Prior to this approval, Sirturo was approved for pulmonary MDR-TB in adults 18 years of age and older.
AUGUST NEWS

● “Additional momentum matters here because Descovy® will likely be going up against generics of Gilead’s own Truvada®, with launches scheduled for next September. Descovy’s advantages over Truvada—substituting TAF, or tenofovir alafenamide, for TDF (tenofovir disoproxil fumarate) in combination with emtricitabine—could appear slight under the best of circumstances. It remains to be seen whether payers will be willing to cover a new branded drug over a generic for safety issues such as bone mineral density.”

● “AveXis, the gene therapy subsidiary of Swiss pharma giant Novartis, was aware of ‘data manipulation’ involving its Zolgensma® gene therapy for spinal muscular atrophy before it was approved in May, but did not inform the FDA until later.”

● “Abeona Therapeutics Inc., a fully integrated leader in gene and cell therapy, today announced positive data from its ongoing Phase 1/2 clinical trial evaluating ABO-102, the Company’s investigational one-time, adeno-associated virus 9 (AAV9) gene therapy for Sanfilippo syndrome type A (MPS IIIA). These new results showed that treatment of the youngest patients with ABO-102, all enrolled in the high-dose cohort 3, resulted in preservation of neurocognitive development 12–18 months post treatment. Robust and sustained improvement observed in biomarkers confers additional evidence of a clear biological effect following ABO-102 administration.”

● “The Institute for Clinical and Economic Review (ICER) released a Final Evidence Report and Report-at-a-Glance assessing the comparative clinical effectiveness and value of the corticosteroid deflazacort (Emflaza®, PTC Therapeutics), and two exon-skipping therapies—eteplirsen (Exondys 51™, Sarepta Therapeutics) and golodirsen (Sarepta Therapeutics). ICER’s report on these three treatments for Duchenne muscular dystrophy (DMD) was reviewed at the July 2019 public meeting of the New England Comparative Effectiveness Public Advisory Council (NE CEPAC), one of ICER’s three independent evidence appraisal committees.”

● “Sarepta Therapeutics, Inc., the leader in precision genetic medicine for rare diseases, announced it had received a Complete Response Letter (CRL) from the FDA regarding the New Drug Application (NDA) seeking accelerated approval of golodirsen injection for the treatment of Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation amenable to exon 53 skipping.”

● “Vertex Pharmaceuticals Incorporated today announced the FDA accepted its NDA for the VX-445 (elexacaftor), tezacaftor and ivacaftor triple combination regimen. The FDA has granted Priority Review of the NDA and assigned a Prescription Drug User Fee Act (PDUFA) target action date of March 19, 2020.”

REFERENCES