PRIME THERAPEUTICS®

# Specialty Pipeline MONTHLY UPDATE

Critical updates in an ever changing environment

October 2020

## NEW DRUG INFORMATION

- **Veklury® (remdesivir):** The U.S. Food and Drug Administration (FDA) granted approval of Gilead's Veklury for use in adult and pediatric patients 12 years of age and older for the treatment of COVID-19 requiring hospitalization. Veklury should only be administered in a hospital or in a health care setting capable of providing acute care comparable to inpatient hospital care. Veklury is the first treatment for COVID-19 to receive FDA approval. Veklury is an inhibitor of the SARS-CoV-2 RNA-dependent RNA polymerase, which is essential for viral replication. The approval of Veklury was based on three randomized, controlled clinical trials that included patients hospitalized with mild-to-severe COVID-19. In the largest study, (ACTT-1; N-1062), the median time to recovery from COVID-19 was 10 days for the Veklury group compared to 15 days for the placebo group, which was statistically significant.[1] All-cause mortality did not show statistical significance (11.4% with Veklury versus 15.2% with placebo).Veklury has a wholesale acquisition cost (WAC) of $520 per vial with most patients expected to receive a five-day treatment course using six vials ($3,120/patient).[2] Prior to Veklury's FDA approval, it was authorized for use under an Emergency Use Authorization (EUA) for the treatment of hospitalized adult and pediatric patients with suspected or laboratory-confirmed COVID-19.

- **Xeljanz™ (tofacitinib):** The FDA granted approval of Pfizer's Xeljanz for the treatment of children and adolescents two years and older with active polyarticular course juvenile idiopathic arthritis (pcJIA). Two formulations were approved, a tablet and an oral solution, both are dosed based on weight. Xeljanz is the first janus kinase (JAK) inhibitor approved in the U.S. for the treatment of pcJIA. Xeljanz was approved based on a Phase 3 study that met its primary endpoint of decreasing disease flares; patients treated with Xeljanz had 31% compared to 55% with placebo patients.[3] Xeljanz 5 mg tablets are already available; Xeljanz oral solution is anticipated to be available by the end of Q1 2021 with pricing to follow.

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- **Inmazeb™ (atoltivimab, maftivimab and odesivimab-ebgn):** Regeneron's Inmazeb was approved by the FDA for the treatment of infection caused by Zaire ebolavirus in adult and pediatric patients, including neonates born to a mother who is RT-PCR positive for Zaire ebolavirus infection. Inmazeb is administered as a single, weight-based intravenous infusion (50 mg atoltivimab, 50 mg maftivimab and 50 mg odesivimab per kg). Inmazeb efficacy and safety was observed in the PALM trial, which demonstrated patients treated with Inmazeb had a one-third mortality rate (33.8%) compared to 51% mortality with control treated patients.[4] According to the World Health Organization (WHO), the average ebola case fatality rate is around 50%.[5] Regeneron is working with the WHO to supply Inmazeb doses over six years under compassionate use protocol. Regeneron plans to continue to offer the therapy for free in response to outbreaks in the Democratic Republic of Congo through the protocol for compassionate use.[5]

## NEW INDICATIONS

- **Nucala® (mepolizumab):** The FDA granted approval of GlaxoSmithKline's Nucala for adults and children aged 12 years and older with hypereosinophilic syndrome (HES) for six months or longer without another identifiable non-blood related cause of the disease.
- **Dovato® (dolutegravir and lamivudine):** The FDA expanded the indication of ViiV Healthcare's Dovato to include it as a switch treatment for HIV-1 infection in virologically suppressed adults on a stable antiretroviral regimen with no treatment failure.
- **Xeljanz® (tofacitinib):** The FDA has approved a new indication of Pfizer's Xeljanz tablet to include the treatment of active polyarticular course juvenile idiopathic arthritis (pcJIA) in children aged two years and older.
- **Kalydeco® (ivacaftor):** Vertex's Kalydeco has been approved by the FDA to expanded population (ages four months to less than six months old) for the combination regimen of the cystic fibrosis transmembrane conductance regulator (CFTR) corrector and the CFTR potentiator for treatment of cystic fibrosis patients with additional rare CFTR mutations.
- **Opdivo® (nivolumab) and Yervoy® (ipilimumab):** The FDA has approved Bristol-Myers Squibb's Opdivo and Yervoy to include first-line treatment of adults with malignant pleural mesothelioma that cannot be removed by surgery.
- **Simponi Aria® (golimumab):** Johnson and Johnson's Simponi Aria has been approved by the FDA to include patients two years of age and older for the treatment of active psoriatic arthritis (PsA) or active polyarticular juvenile idiopathic arthritis (pJIA).
- **Haegarda® (C1 Esterase Inhibitor Subcutaneous (Human)):** The FDA has approved a new indication for CSL Behring's Haegarda for routine prophylaxis of hereditary angioedema attacks in patients six years of age and older.
- **Wakix® (pitolisant):** The FDA has approved a new indication for Harmony Biosciences' Wakix for treatment of cataplexy in adults with narcolepsy.
- **Keytruda® (pembrolizumab):** Merck's Keytruda has a new indication for the PD-1 inhibitor immuno-oncologic as monotherapy for treatment of adults with relapsed or refractory classical Hodgkin lymphoma (cHL).

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## OCTOBER NEWS

- "Johnson and Johnson said that it had paused further dosing (on pivotal COVID-19 vaccine study), noting that 'the participant's illness is being reviewed and evaluated by the ENSEMBLE independent Data Safety Monitoring Board as well as our internal clinical and safety physicians.'"[6]
- "The French biotech Inventiva announced that they had won breakthrough status, becoming — by their count — the first company to receive the designation for a NASH drug since Intercept did 5 years ago. Inventiva announced that its lead drug, lanifibranor, was successful in a Phase II trial. Across nearly 250 volunteers, 49% of those who received the high dose of the drug saw their liver cell inflammation and ballooning decline by at least two points on a standard scale and didn't see their fibrosis worsen, compared to 27% on placebo. The trial also hit secondary endpoints at low and high dose for NASH resolution and improvement of fibrosis and NASH."[7]
- "Remdesivir, the only antiviral drug authorized for treatment of Covid-19 in the United States, fails to prevent deaths among patients, according to a study of more than 11,000 people in 30 countries sponsored by the World Health Organization. The drug was granted emergency authorization by the Food and Drug Administration after a trial by the National Institutes of Health found that remdesivir modestly reduced the time to recovery in hospitalized patients. 'This puts the issue to rest — there is certainly no mortality benefit,' said Dr. Ilan Schwartz, an infectious disease physician at the University of Alberta in Canada."[8]

## REFERENCES


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