PRIME THERAPEUTICS®

Specialty Pipeline **MONTHLY UPDATE**

Critical updates in an ever changing environment

November 2020

NEW DRUG INFORMATION

- **Bronchitol™** (mannitol inhalation powder, for oral inhalation use): The U.S. Food and Drug Administration (FDA) granted approval of Chiesi Group and Pharmaxis' Bronchitol as add-on maintenance therapy to improve pulmonary function in cystic fibrosis (CF) patients 18 years of age and older. Bronchitol is the first formulation of mannitol that is an inhaled dry powder administered by 10 capsules twice daily. Bronchitol's approval was based on three large-scale Phase 3 clinical trials that demonstrated sustained improvement in forced expiratory volume in one second (FEV1) compared to control.[1] Patients seeking to use Bronchitol must first pass a mannitol tolerance test, and the first dose must be administered under medical supervision to monitor risk of bronchospasm. Bronchitol is scheduled to launch in March 2021 with pricing to follow.

NEW INDICATIONS

- **Keytruda® (pembrolizumab):** The FDA has expanded the indication of Merck's Keytruda (pembrolizumab) to include use in combination with chemotherapy for treatment of patients with locally recurrent unresectable or metastatic triple negative breast cancer (TNBC) whose tumors express PD-L1 (CPS ≥10).

NOVEMBER NEWS

- "Biogen had dodged a bullet when the FDA recused longtime Alzheimer's researcher David Knopman from this week's expert panel review of aducanumab. As Knopman made clear in comments filed at the FDA and in a separate paper he co-authored that appeared recently, he's flat opposed to an OK at this point. He insisted that Biogen's data don't meet the FDA's standards for an approval, relying on hopelessly biased post hoc data sorties, and that the big biotech can easily run a timely follow-up trial to prove or disprove the drug's ability to help patients. By most accounts, though certainly not all, any drug that does get approved for bending the curve on the progression of Alzheimer's will land in the middle of a gold mine opportunity. There are no drugs approved for that. And Biogen desperately needs this one."[2]

- "Pfizer Inc. said that its COVID-19 vaccine may be a remarkable 95% effective, based on early and incomplete test results that nevertheless brought a big burst of optimism to a world desperate for the means to finally bring the catastrophic outbreak under control. Pfizer, which is developing the vaccine with its German partner BioNTech, now is on track to apply later this month for emergency-use approval from the U.S. Food and Drug Administration, once it has the necessary safety information in hand. Even if all goes well, authorities have stressed it is unlikely any vaccine will arrive much before the end of the year, and the limited initial supplies will be rationed. Dr. Anthony Fauci, the U.S. government's top infectious-disease expert, said the results suggesting 90% effectiveness are "just extraordinary," adding: "Not very many people expected it would be as high as that."[3]
- "A second COVID-19 vaccine now also appears highly effective in preventing illness following exposure to the virus that causes the disease. The biotech company Moderna Inc. said Monday that its experimental vaccine was 94.5% effective in preventing disease, according to an analysis of its clinical trial. The news comes a week after Pfizer and BioNTech said their vaccine was more than 90% effective."[4]
- "The Food and Drug Administration has granted Priority Review to pegcetacoplan (Apellis Pharmaceuticals) for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Pegcetacoplan is a synthetic cyclic peptide conjugated to a polyethylene glycol polymer that inhibits the complement cascade centrally at C3 and C3b. The NDA is supported by data from the Phase 3 PEGASUS study that assessed the efficacy and safety of pegcetacoplan in 80 adults with PNH. Patients were randomized to receive either pegcetacoplan (n=41) or eculizumab (n=39) for 16 weeks. The primary end point was the change from baseline in hemoglobin level to week 16. Key secondary end points included transfusion avoidance, absolute reticulocyte count and Functional Assessment of Chronic Illness Therapy (FACIT)-fatigue score. Results showed that pegcetacoplan met the primary end point demonstrating superiority to eculizumab with a statistically significant improvement in hemoglobin levels at week 16 (P <.0001)."[5]
- "The Peripheral and Central Nervous System Drugs Advisory Committee overwhelmingly voted no on a number of counts regarding the efficacy and evidence supporting the use of aducanumab in Alzheimer's disease (AD). In response to the question of whether two studies – EMERGE and ENGAGE – provide 'strong evidence' that aducanumab is effective at treating AD, eight experts voted no – with one voting yes and two 'uncertain'."[6]

REFERENCES

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