The Berke Report

Clinical and Commercial Updates for Week Four of April 2023

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- 04.28.23 - Pfizer snags FDA approval for expanded pneumococcal vaccine (pharmamanufacturing)
  - Pfizer announced this week that the FDA has approved its 20-valent pneumococcal conjugate vaccine for use in infants and children aged six weeks to 17 years. The jab, Prevnar 20, protects patients from 20 Streptococcus pneumoniae serotypes, all of which can cause pneumococcal disease (IPD). Prevnar 20 is an upgraded version of Pfizer's Prevnar 13 vaccine, which was first approved in 2010. The updated vaccine now contains an additional seven serotypes (8, 10A, 11A, 12F, 15B, 22F, and 33F) which are associated with antibiotic resistance, severe illness, and are commonly found in pediatric pneumococcal cases. According to a study, these seven serotypes alone are responsible for about 37% of IPD cases in children under five years of age in the U.S.
- 04.28.23 - Tempest Announces Positive Early Results from Global Randomized Phase 1b/2 Combination Study of TPST-1120 in First-Line Hepatocellular Carcinoma (PR)
  - Data from 40 patients in the TPST-1120 arm randomized per protocol against 29 evaluable (30 total) patients in the control arm showed: Unconfirmed responses of 30% for the TPST-1120 triplet arm (12/40) vs. 17.2% for the control arm (5/29), demonstrating a 74.4% relative improvement in objective response rate (ORR); Confirmed responses of 17.5% for the TPST-1120 triplet arm (7/40) vs. 10.3% for the control arm (3/29), demonstrating a 69.9% relative improvement in confirmed ORR; 47.5% (19/40) of the TPST-1120 arm patients are on treatment vs. 23.3% (7/30) in the control arm; 80% (32/40) of the TPST-1120 arm patients are on study vs. 50% (15/30) in the control arm;
- 04.27.23 - AstraZeneca cuts Alexion's PhIII Wilson disease drug, takes $244M writedown (endpts)
  - AstraZeneca is calling it quits with a Phase III rare disease program it obtained via the $39 billion acquisition of Alexion. ALXN1840, or bis-choline tetrathiomolybdate, had in turn come to Alexion from a buyout. In 2018, Alexion offered $855 million in cash to acquire Wilson Therapeutics for the candidate, designed to treat Wilson disease — a rare condition where patients can't remove copper from the body, resulting in excessive amounts of copper accumulating in the liver, brain and eyes.
- 04.27.23 - FDA Approves Otsuka and Lundbeck's ABILIFY ASIMTUFII® (aripiprazole), the First Once-Every-Two-Months Long-acting Injectable (LAI) for the Treatment of Schizophrenia or Maintenance Monotherapy Treatment of Bipolar I Disorder in Adults (PR)
  - ABILIFY ASIMTUFII offers two months of sustained therapeutic concentrations with one dose. Each dose is provided in a single-chamber, prefilled syringe, and is administered by a healthcare professional to appropriate patients via intramuscular injection in the gluteal muscle. ABILIFY ASIMTUFII, a long-acting injectable, provides continuous delivery of medication and can maintain therapeutic plasma concentrations, which may help those living with schizophrenia and bipolar I disorder.
- 04.26.23 - Avenge Bio Announces Successful Completion of First Dose Level Cohort in Phase 1/2 Clinical Trial of AVB-001 for the Treatment of Ovarian Cancer (PR)
  - The dose escalation trial evaluates the safety and tolerability, as well as preliminary efficacy, of AVB-001 administered intraperitoneally across a series of ascending dose-level cohorts. In the first cohort, the administration of AVB-001 has been well tolerated. No dose-limiting toxicities, on-target or off-target toxicities, or other unexpected events were observed. As such, investigators have initiated dosing in the second dose level cohort.
- 04.26.23 - Alnylam and Regeneron Report Positive Interim Phase 1 Clinical Data on ALN-APP, an Investigational RNAi Therapeutic for Alzheimer's Disease and Cerebral Amyloid Angiopathy (PR)
  - Single Doses of ALN-APP Demonstrated Dose-Dependent, Rapid and Sustained Reduction of sAPP**α** and sAPP**β** in Cerebrospinal Fluid, with Up to 90% at Highest Dose to Date. Encouraging Clinical Safety and Tolerability Profile Observed with Single Dosing to Date. Results Provide First Demonstration of Gene Silencing by RNAi Therapeutics in the Human Brain Using Alnylam's Proprietary C16 Platform –
- 04.26.23 - US FDA approves Seres Therapeutics' pill for deadly C. difficile infections (reuters)
  - The drug, branded as Vowst, was approved for treating 18 years and older for recurrent Clostridioides difficile (C. diff) infections (CDI), generally caused by prolonged use of antibiotics, which wipe out friendly colon bacteria and can cause potentially fatal diarrhea and inflammation of the colon. Vowst consists of a certain kind of bacteria made by purifying fecal matter derived from healthy people, while fecal transplants are donated by healthy volunteers and are not purified. The approval for the drug was based on its late-stage trials, in which treatment with the drug helped patients get rid of recurrent CDI.
- 04.25.23 - Insulet Announces FDA Clearance of Omnipod GO™, a First-of-its-Kind Basal-Only Insulin Pod, Further Simplifying Life for People with Type 2 Diabetes (PR)
  - Omnipod GO is a standalone, wearable, insulin delivery system that provides a fixed rate of continuous rapid-acting insulin for 72 hours. The newest addition to the Omnipod brand features a tubeless and waterproof* Pod which is offered in seven different pre-programmed daily rates, ranging from 10 to 40 units per day, and operates without the need for a handheld device to control the Pod. It has been cleared for use with the following U-100 insulins: NovoLog®, Fiasp®, Humalog®, Admelog®, and Lyumjev®.
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- 04.25.23 - InVivyd Announces the FDA Has Cleared Its IND Application for VYD222, a Monoclonal Antibody Candidate for Prevention of COVID-19, and Provides Phase 1 VYD222 Clinical Trial Update (PR)
    - On track for initial readouts in Q2 2023 from ongoing Phase 1 VYD222 clinical trial being conducted in Australia, with cohort 1 dosing complete
- 04.25.23 - Parthenon Therapeutics Announces First Patient Dosed in Phase 1 Clinical Trial of PRTH-101, a Novel DDR1 Inhibitor for the Treatment of Immune-Excluded Solid Tumors (PR)
    - PRTH-101 is a first-in-class Discoidin Domain Receptor 1 (DDR1) antagonist monoclonal antibody that is expressed at very high levels in solid tumors with low levels of T cell infiltration. PRTH-101 is the first development candidate from Parthenon's broad TME-focused pipeline to enter clinical development. PRTH-101 uniquely targets DDR1 and has demonstrated, in preclinical models, anti-tumor activity as a single agent and in combination with an anti-PD-1 checkpoint inhibitor.
- 04.25.23 - Baudax Bio Announces Positive Top-Line Final Results From Phase 2 Randomized Clinical Trial of BX1000 (PR)
    - The BX1000 Phase 2 surgery trial is a randomized, double-blind, active-controlled clinical trial comparing three different doses of BX1000 to a standard dose of 0.6mg/kg rocuronium in a planned clinical trial of 80 adult patients undergoing elective surgery utilizing total intravenous anesthesia. A total of 81 patients were randomized to the four treatment groups. One patient discontinued early and did not receive a study drug. A total of 80 patients were treated. Each BX1000 dose cohort had 20 evaluable patients and the rocuronium cohort had 19 evaluable patients (one subject in this arm experienced a delay in intubating condition assessment.) The primary efficacy endpoint of the study was the proportion of patients that met criteria for Good or Excellent intubating conditions using a standardized scale. Additionally, the clinical trial evaluated the safety and tolerability profile of BX1000 and rocuronium in this patient population.
- 04.24.23 - Atea Pharmaceuticals Announces U.S. FDA Fast Track Designation Granted to Bemnifosbuvir, an Investigational Oral Antiviral, for the Treatment of COVID-19 (PR)
    - Atea Pharmaceuticals, Inc. (Nasdaq: AVIR) ("Atea"), a clinical-stage biopharmaceutical company engaged in the discovery and development of oral antiviral therapeutics for serious viral diseases, today announced that the United States Food and Drug Administration (FDA) has granted Fast Track designation (FTD) to bemnifosbuvir for the treatment of COVID-19. Bemnifosbuvir is an oral, direct-acting antiviral drug candidate being evaluated in the global Phase 3 SUNRISE-3 registrational trial for the treatment of COVID-19 in outpatients at high risk for disease progression regardless of vaccination status. This includes patients over the age of 80, patients 65 years or older with at least one major risk factor, and anyone over the age of 18 who is immunocompromised.
- 04.24.23 - Ionis's presented NEURO-TTRansform Phase III results, showed eplontersen demonstrated consistent and sustained improvement in all measures of disease and quality of life through 66 weeks (PR)
    - At 66 weeks, patients treated with eplontersen demonstrated consistent and sustained benefit on the three co-primary endpoints of serum transthyretin (TTR) concentration, neuropathy impairment and quality of life (QoL). Eplontersen achieved a least squares (LS) mean reduction of 82% in TTR serum concentration from baseline, compared to an 11% reduction from baseline in the external placebo group ($p<0.0001$).1
- 04.23.23 - Apellis Presents Phase 3 Functional Analyses of SYFOVRE™ (pegcetacoplan injection) for Geographic Atrophy (PR)
    - SYFOVRE showed visual function and quality-of-life benefits in patients with extrafoveal lesions (≥0.25 mm from the foveal center). Additionally, SYFOVRE showed a meaningful reduction in the loss of photoreceptor and retinal pigmented epithelial (RPE) cells, which are both required for vision. These analyses utilized data from patients with SPECTRALIS® optical coherence tomography (OCT) images, which allowed for artificial intelligence (AI)-based automated segmentation of the photoreceptor and RPE layers as well as determination of the amount of the central foveal region covered by the GA lesion (foveal occupancy).
- 04.21.23 - CymaBay Therapeutics Announces Publication of Results From the ENHANCE, Phase 3 Study of Seladelpar in Patients with Primary Biliary Cholangitis (PBC) (PR)
    - This double-blind, placebo-controlled, global phase 3 study evaluated the efficacy and safety of seladelpar, a potent, selective, orally active PPAR**δ** agonist, or delpar, after 3 months of treatment in patients with PBC. Patients with elevated alkaline phosphtase (≥ 1.67x upper-limit-of-normal) received treatment as an add-on to first line ursodeoxycholic acid (UDCA), or as monotherapy, if patients were intolerant to UDCA. All pre-specified endpoints were assessed after 3 months in patients receiving oral daily seladelpar 5 mg (n=56), 10 mg (n=55) or placebo (n=56). The primary endpoint was a composite of alkaline phosphatase and bilirubin2 previously accepted by FDA for pivotal studies in PBC. The composite endpoint was achieved in 78.2% of patients on seladelpar 10 mg and 57.1% on seladelpar 5 mg versus 12.5% on placebo (both seladelpar doses with $p<0.0001$).
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