The Berke Report

Clinical and Commercial Updates for the First Week of April 2023

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- 04.06.23 - Enanta Pharmaceuticals Receives FDA Fast Track Designation for EDP-323, its Oral, L-Protein Inhibitor in Development for the Treatment of Respiratory Syncytial Virus (PR)
  - Enanta Pharmaceuticals, Inc. (NASDAQ:ENTA), a clinical-stage biotechnology company dedicated to creating small molecule drugs for viral infections, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for EDP-323, Enanta's L-protein inhibitor in development for the treatment of respiratory syncytial virus (RSV). EDP-323 is being evaluated in a Phase 1 double-blind, placebo-controlled study designed to assess its safety, tolerability, and pharmacokinetics (PK). Enanta plans to present new preclinical PK data at the European Congress of Clinical Microbiology and Infectious Diseases. Expects to report topline data from the Phase 1 study this quarter.
- 04.05.23 - MAPS PBC Announces Positive Topline Results from Long-Term Observational Follow-Up Study on MDMA-Assisted Therapy for Treatment of PTSD (PR)
  - MAPS Public Benefit Corporation ("MAPS PBC"), a clinical-stage company dedicated to changing the way mental health conditions are treated, announced positive topline results from an observational follow-up study evaluating the long-term safety and efficacy of MDMA-assisted therapy for post-traumatic stress disorder ("PTSD"). Preliminary findings show that participants in this study demonstrated a durable response at least six months, and in some cases a year or more, after their final MDMA-assisted therapy session during the Phase 3 study.
- 04.04.23 - Seagen, Merck's Padcev-Keytruda combo snags FDA accelerated approval for bladder cancer (endpts)
  - Seagen's Padcev and Merck's Keytruda have been cleared by the FDA as a combo for first-line treatment of bladder cancer, potentially pushing Padcev into a multibillion-dollar market just as Pfizer prepares to consummate its $43 billion buyout of Seagen. The FDA handed out the accelerated approval based on positive data from a multi-cohort study with Padcev (enfortumab vedotin) and Keytruda (pembrolizumab) in patients with locally advanced or metastatic urothelial cancer who are ineligible for cisplatin-containing chemotherapy. The new OK could propel Padcev, which reported $451 million in 2022 sales, "to a multi-blockbuster franchise," according to William Blair analysts, who estimate the cisplatin-ineligible urothelial carcinoma market to be worth $2.5 billion to $3 billion globally. Topline results for a confirmatory Phase III trial could come as early as the end of 2023 and "expand the global (total addressable market) to over $5 billion," they added.
- 04.04.23 - Perfuse Therapeutics Announces FDA Clearance of IND Application for Phase 1/2a Clinical Trial of PER-001 Intravitreal Implant in Patients with Glaucoma
  - The first-in human single-arm, open label, Phase 1 portion of the study will evaluate the safety and tolerability of PER-001 Intravitreal Implant in patients with advanced glaucoma. The Phase 2a trial, is a patient masked, randomized, sham-controlled study that will evaluate the safety, tolerability, and pharmacodynamics of two doses of PER-001 in patients with progressing glaucoma. This milestone marks transition of Perfuse Therapeutics to a clinical-stage company
- 04.04.23 - Gossamer halts work on BTK inhibitor after 2 patient deaths (fiercebiotech)
  - Gossamer Bio is ending development of a lymphoma med after reports of two deaths and other serious adverse events in a phase 2 trial that was paused in March citing the "benefit/risk profile observed to date." Gossamer revealed GB5121, a central nervous system-penetrant Bruton's tyrosine kinase (BTK) inhibitor, in October 2021, hoping to patch up a hole left in its pipeline by a two-time failed lead asset. At the time, CEO Faheem Hasnain called BTK a "validated target" but one that has yet to prove its abilities in the CNS.
- 04.03.23 - Citius Pharmaceuticals Completes Enrollment in Phase 2b Study of Halo-Lido for the Prescription Treatment of Hemorrhoids (PR)
  - the last patient has been enrolled in the Company's Phase 2b clinical study of Halo-Lido (CITI-002), a topical formulation for the relief of hemorrhoids. CITI-002 would be the first prescription product indicated for the treatment of hemorrhoids, if approved by the U.S. Food and Drug Administration (FDA). Approximately 300 adults with a clinical diagnosis of symptomatic hemorrhoids were enrolled in the Halo-Lido Phase 2b study (NCT05348200), a multi-center, randomized, dose-ranging, double-blind, parallel group comparison clinical trial. The primary outcome of the study is the change in hemorrhoidal symptoms as reported by the patients following treatment. Efficacy and safety data are recorded by patients utilizing a proprietary mobile-enabled Patient Reported Outcome (ePRO) instrument. The results of the study are anticipated by the end of the second quarter of 2023 and are expected to provide the foundation for development of the Phase 3 study.
- 04.03.23 - Amid dispute with FDA, Akebia proposes new dosing for oral anemia drug in kidney disease (fiercepharma)
  - Akebia Therapeutics isn't giving up on its oral anemia drug vadadustat despite an FDA rejection and a partnership gone awry. Now, just as the company challenges the FDA decision, Akebia has offered top-line data for a reduced dosing regimen. Vadadustat, given three times a week, matched up to Roche and CSL Vifor's erythropoiesis-stimulating agent (ESA) Mircera at controlling hemoglobin levels in dialysis patients with anemia that's caused by chronic kidney disease. Akebia unveiled the phase 3 win from the FO2CUS trial on Monday. Vadadustat was non-inferior to Mircera at two different dosing levels when measured during two evaluation periods, according to the company. Vadadustat is currently available in Japan as a once-daily med for treating chronic kidney disease-related anemia. Akebia's local partner, Mitsubishi Tanabe Pharma, scored that approval in 2020 and has been selling it under the brand name Vafseo. The European Commission is also expected to hand out a green light in dialysis-dependent patients soon following a backing from drug reviewers at the European Medicines Agency.

- 04.03.23 -Travere's Filspari reduces urine protein levels in adults with IgAN (clinicaltrialarean)
  - The interim analysis showed that patients treated with 400mg of Filspari achieved a mean reduction of 49.8% in proteinuria compared to baseline. Participants in the active control arm received 300 mg of irbesartan and achieved a mean reduction of 15.1%% in proteinuria. The trial (NCT03762850) enrolled 404 patients.Additionally, 20.8% of the treatment cohort achieved complete remission, urine protein excretion <0.3 g/day, compared to 7.9% participants in the control arm. Partial remission, urine protein excretion <1.0 g/day, was achieved in 70.3% of participants in Filspari arm compared to 44.1% of comparator cohort. The trial's secondary endpoints are measuring estimated glomerular filtration rate (eGFR) over a 52-, 104-, and 110-week periods.
- 04.03.23 - Tango Therapeutics Announces FDA Clearance of Investigational New Drug Application for TNG260, a First-in-Class CoREST Inhibitor for the Treatment of STK11-Mutant Cancers (PR)
  - The CoREST complex has been shown to play a major role in regulating the expression of immunomodulatory proteins in STK11-mutant cancers. In syngeneic models with an STK11 mutation and an intact immune system, the combination of TNG260 with an anti-PD-1 antibody resulted in sustained complete tumor regressions and the induction of immune memory that prevented re-implantation of the same tumor xenograft.
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