The Berke Report

Clinical and Commercial Updates for the Second Week of April 2023

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- 04.15.23 - Merck's Keytruda-chemo reduces risk of death from biliary tract cancer by 17% (endpts)
  - The 1,069-patient trial in metastatic or unresectable biliary tract cancers showed that patients in the Keytruda-chemo arm had a median overall survival of 12.7 months, compared with 10.9 months in those taking a placebo plus chemo. After a median follow-up of 25.6 months, patients treated with Keytruda (also known as pembrolizumab) had a 17% lower risk of death than patients who received just chemotherapy. The results were statistically significant. Biliary tract cancers are rare malignancies arising in the gallbladder, bile ducts, and the ampulla of Vater, the space where the pancreas and liver meet before emptying into the small intestine. The biliary tract cancers study missed several of the secondary endpoints, however — objective response rates didn't differ between the arms, although patients in the Keytruda arm had a longer duration of response, or 9.7 months versus 6.9 months. And while patients treated with Keytruda had a 14% lower risk of disease progression or death at a median follow-up of 13.6 months, this difference didn't meet the study's pre-specified requirements for statistical significance. Median progression-free survival was 6.5 months for Keytruda and 5.6 months in the placebo arm.
- 04.13.23 - Aldeyra Therapeutics Completes Enrollment in Phase 3 INVIGORATE-2 Clinical Trial in Allergic Conjunctivitis (PR)
  - The randomized, double-masked, crossover, vehicle-controlled Phase 3 clinical trial enrolled 131 seasonal allergic conjunctivitis patients who were evaluated for 3.5 hours in an allergen chamber designed to simulate real-world pollen exposure. Consistent with pivotal trials of approved allergic conjunctivitis products, the primary endpoint of INVIGORATE-2 is patient-reported ocular itching. Top-line results from the trial are expected in the first half of 2023.
- 04.13.23 - Amylyx Pharmaceuticals Announces First Participant Dosed in Phase 2 Study of AMX0035 for the Treatment of Wolfram Syndrome (PR)
  - HELIOS is an exploratory open-label proof of biology study assessing the effect of AMX0035 safety and tolerability, and various measures of endocrinological, neurological and ophthalmologic function. Amylyx anticipates topline results from HELIOS in 2024.
- 04.12.23 - Verrica Pharmaceuticals Announces Dosing of the First Patient in Part 2 of Phase 2 Study Evaluating VP-315 for the Treatment of Basal Cell Carcinoma (PR)
  - Verricais is a dermatology therapeutics company developing medications for skin diseases requiring medical interventions, today announced that the first patient has been dosed in Part 2 of a Phase 2 study evaluating the Company's potentially first-in-class oncolytic peptide, VP-315, for the treatment of basal cell carcinoma. Part 2 of the Phase 2 trial is designed to further explore dosing regimens to identify the recommended dose for Part 3 of the study, which is expected to start in the first half of 2024.
- 04.11.23 - HotSpot Therapeutics Achieves First-In-Human Dosing with HST-1011, An Investigational Oral Small Molecule Allosteric Inhibitor of CBL-B (PR)
  - initiation of dosing of HST-1011 in its Phase 1/2 clinical trial in patients with advanced solid tumors. HST-1011 is an orally bioavailable, potent, selective small molecule allosteric inhibitor of casitas B-lineage lymphoma-B (CBL-B).Phase 1/2 study designed to evaluate the safety, tolerability, pharmacokinetics, harmacodynamics and preliminary clinical activity of HST-1011 dosed as monotherapy and in combination with Regeneron's anti-PD-1 therapy, Libtayo® (cemiplimab) in patients with advanced solid tumors
- 04.11.23 - Takeda Receives FDA Approval to Expand the Use of HYQVIA® to Treat Primary Immunodeficiency in Children (PR)
  - HYQVIA® [Immune Globulin Infusion 10% (Human) with Recombinant Human Hyaluronidase], the Only Once-a-Month – Every Three or Four Weeks – Subcutaneous Immunoglobulin (ScIG), is Now Approved for People Two Years of Age and Older with Primary Immunodeficiency. Approval Supported by Pivotal Phase 3 Study that Demonstrated Reliable Infection Protection in Children 2-16 Years Old.
- 04.11.23 - HI-Bio Announces Positive Phase 2 Data on Felzartamab for the Treatment of Primary Membranous Nephropathy (PR)
  - Felzartamab showed dose-dependent reduction in pathogenic antibody levels across two clinical studies. Proteinuria remission observed across patient groups, including those starting with high aPLA2R titers or refractory to prior immunosuppressive therapies. Company intends to advance felzartamab into late-stage development in Primary Membranous Nephropathy and other autoantibody-driven immune-mediated diseases. Primary Membranous Nephropathy is a rare, high burden, immune-mediated kidney disease with no approved therapies
- 04.10.23 - Sumitomo Pharma Oncology Receives Orphan Drug Designation for TP-1287, an Investigational Oral CDK9 Inhibitor for the Treatment of Ewing Sarcoma (PR)
  - TP-1287 is currently being evaluated in a Phase 1, first-in-human study of oral TP-1287 in patients with advanced metastatic or progressive solid tumors who are refractory to, or intolerant of, established therapy known to provide clinical benefit for their condition, which is being conducted in the United States.