The Berke Report

Clinical and Commercial Updates for Week One of May 2023

Contact Carl Berke For More Information Carl@Berkesearch.com

- 05.05.23 - FibroGen Announces Results for MATTERHORN, a Phase 3 Clinical Study of Roxadustat for the Treatment of Anemia in Patients with Myelodysplastic Syndromes (PR)
  - A total of one-hundred forty (140) patients were enrolled in MATTERHORN, a Phase 3, double-blind placebo-controlled study investigating the safety and efficacy of roxadustat for treatment of anemia in patients with lower risk transfusion-dependent myelodysplastic syndromes. The primary endpoint of the study is transfusion independence for ≥ 56 consecutive days during the first 28 weeks of treatment, and patients are followed for up to 52 weeks. The MATTERHORN study is sponsored and conducted by FibroGen and is part of FibroGen's co-development collaborations with AstraZeneca and with Astellas Pharma Inc.
- 05.03.23 - Vertex Announces U.S. FDA Approval for KALYDECO® (ivacaftor) to Treat Eligible Infants With CF Ages 1 Month and Older (PR)
  - The approval was supported by a cohort in the Phase 3, 24-week, open-label study to evaluate the safety, pharmacokinetics and pharmacodynamics of ivacaftor in subjects with CF who are less than 24 months of age and have an ivacaftor-responsive CFTR mutation. This cohort demonstrated a safety profile similar to that observed in older children and adults.
- 05.03.23 - PharmaEssentia Initiates Phase 3b Trial of Ropeginterferon alfa-2b-njft Investigatng New Dosing Regimen for Patients With Polycythemia Vera (PR)
  - Ropeginterferon alfa-2b-njft (marketed as BESREMi®) was approved by the U.S. Food and Drug Administration in November 2021 as a treatment for adults with PV.1 PV is a rare, chronic and life-threatening blood cancer caused by a mutation in hematopoietic stem cells in the bone marrow, resulting in the overproduction of red blood cells, white blood cells and platelets. Individuals with PV are at risk for serious health problems, including blood clots, stroke and heart attack.2,3 Without proper management, this debilitating cancer can progress into myelofibrosis and other malignancies, including acute myeloid leukemia.4
- 05.03.23 - Bristol Myers Squibb Receives European Commission Approval for CAR T Cell Therapy Breyanzi (lisocabtagene maraleucel) for Relapsed or Refractory Large B-cell Lymphoma After One Prior Therapy (PR)
  - Approval of Breyanzi based on the pivotal Phase 3 TRANSFORM trial, in which Breyanzi significantly improved event-free survival compared to standard of care with a manageable and well-established safety profile. The approval is based on results from the pivotal Phase 3 TRANSFORM trial in which Breyanzi demonstrated statistically significant and clinically meaningful improvements in the study's primary endpoint of event-free survival (EFS), and key secondary endpoints of complete responses (CR) and progression-free survival (PFS) compared to standard therapy (consisting of salvage immunochemotherapy followed by high-dose chemotherapy and hematopoietic stem cell transplant [HSCT]), along with a manageable and well-established safety profile.
- 05.02.23 - Amolyt Pharma Announces Phase 3 Clinical Trial of Eneboparatide for the Treatment of Hypoparathyroidism following Positive End of Phase 2 Meeting with FDA (PR)
  - The Calypso trial is expected to be the largest Phase 3 trial to date conducted in hypoparathyroidism with topline data expected by end of 2024. In addition to serum calcium control and elimination of standard of care, the study will also assess normalization of urinary calcium as a key secondary efficacy endpoint. The effect of eneboparatide on bone quantity and quality will also be evaluated. Calypso design builds on the findings from the Phase 2a trial in relation to control of serum calcium, discontinuation of oral supplements, normalization of hypercalciuria and induction of a balanced bone turnover.
- 05.02.23 - Eloxx Pharmaceuticals Announces FDA Clearance to Begin Single Ascending Dose Study of ZKN-013 (PR)
  - ZKN-013 is in development for potential treatment of recessive dystrophic and junctional epidermolysis bullosa (RDEB and JEB) and Familial Adenomatous Polyposis (FAP)
- 05.02.23 - Scorpion Therapeutics Announces First Patient Dosed in Phase 1/2 Clinical Trial of STX-478, Its Mutant-Selective PI3K**α** Inhibitor for the Treatment of Breast Cancer and Other Solid Tumors (PR)
  - STX-478 has a potential best-in-class profile and is Scorpion's first program to enter clinical development. Initial Phase 1 safety, pharmacokinetic and pharmacodynamic data and preliminary efficacy assessment expected in 2024 -
- 05.01.23 - Travere Therapeutics Announces Topline Results from Two-Year Primary Efficacy Endpoint in Pivotal Phase 3 DUPLEX Study of Sparsentan in Focal Segmental Glomerulosclerosis (PR)
  - The DUPLEX Study did not achieve the primary efficacy eGFR slope endpoint over 108 weeks of treatment. Secondary and topline exploratory endpoints trended favorably for sparsentan. Treatment with sparsentan resulted in a reduction of proteinuria that was sustained through 108 weeks of treatment. Sparsentan was well-tolerated with a consistent safety profile across all clinical trials conducted to date and comparable to irbesartan
- 05.01.23 - Erasca Granted FDA Fast Track Designation for CNS-Penetrant EGFR Inhibitor ERAS-801 in Patients with Glioblastoma (PR)
  - ERAS-801 has demonstrated broad preclinical activity against oncogenic EGFR variants and wildtype alterations. ERAS-801 is an orally bioavailable, small molecule EGFR inhibitor that exhibited substantial central nervous system (CNS) penetration in animal studies.

- 05.01.23 - U.S. FDA Accepts for Priority Review Supplemental Biologics License Application and EMA Validates Application for Reblozyl® (luspatercept-aamt) as First-Line Treatment of Anemia in Adults with Lower-Risk Myelodysplastic Syndromes (PR)
    - Applications based on results from Phase 3 COMMANDS study in which first-in-class Reblozyl demonstrated a highly statistically significant and clinically meaningful improvement compared to an erythropoiesis-stimulating agent in patients with very low/low/intermediate-risk MDS. U.S. FDA has assigned a target action date of August 28, 2023
- 05.01.23 - Biomea Fusion Announces FDA Clearance of Investigational New Drug (IND) Application for Covalent FLT3 Inhibitor BMF-500 in Relapsed or Refractory Acute Leukemia (PR)
    - BMF-500, a novel 3rd generation covalent inhibitor of fms-like tyrosine kinase 3 (FLT3), is the second investigational compound, discovered and developed by Biomea's FUSION™ System, to advance to the clinic.
- 05.01.23 - Bristol Myers' CAR-T Breyanzi hits endpoints in two lymphoma studies (endpts)
    - The company said Monday that the therapy met the primary endpoint of overall response rate and a secondary endpoint of complete response rate in both a Phase I and a separate Phase II trial. Bristol Myers noted that no new safety signals were reported. The Phase I study, a pivotal trial called TRANSCEND NHL 001, is an open-label and single-arm trial investigating Breyanzi in patients with five different types of non-Hodgkin lymphomas. The company said overall response rate was met in the subset of patients with mantle cell lymphoma. Earlier this year, Eli Lilly's BTK inhibitor Jaypirca received FDA approval for patients with relapsed or refractory mantle cell lymphoma.