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# The Berke Report

## First Half of Feb 2023 Clinical & Commercial News

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- 02.16.23 - FDA gives green light to Chiesi's Lamzede (pharmaphorum)
  - US regulators have approved Chiesi's Lamzede (velmanase alfa-tycv) as the first enzyme replacement therapy for the treatment of non-central nervous system manifestations of alpha-mannosidosis (AM) in adult and paediatric patients. Lamzede (velmanase alfa-tycv) is a recombinant form of human alpha-mannosidase designed to replace or support the function of the natural enzyme and prevent the build-up of mannose–rich oligosaccharides in various tissues in the body.
- 02.16.23 - Inozyme Pharma Reports Positive Topline Data from Ongoing Phase 1/2 Trials of INZ-701 (PR)
  - Rapid, significant, and sustained increase in plasma pyrophosphate (PPi) observed and encouraging patient reported outcome data in all dose cohorts in ENPP1 Deficiency trial. Rapid and significant increase in PPi observed in all dose cohorts with sustained increase observed in highest dose cohort in ABCC6 Deficiency.(PXE) trial - INZ-701 was generally well-tolerated and exhibited a favorable safety profile in both trials.
- 02.15.23 - Coherus and Junshi Biosciences Announce Positive Final Overall Survival Results of JUPITER-02, a Phase 3 Clinical Trial Evaluating Toripalimab as Treatment for Recurrent or Metastatic Nasopharyngeal Carcinoma (PR)
  - The FDA has granted Breakthrough Therapy designations and priority review for the toripalimab Biologics License Application ("BLA") for use in combination with gemcitabine and cisplatin as first-line treatment for patients with advanced recurrent or metastatic NPC and for toripalimab monotherapy for the second-line or later treatment of recurrent or metastatic NPC after platinum-containing chemotherapy. Recurrent or metastatic NPC is an aggressive head and neck tumor which has no FDA-approved treatment options.
- 02.14.23 - Kinnate Biopharma Inc. Receives Fast Track Designation from the U.S. Food and Drug Administration for KIN-3248, an Investigational Pan-FGFR Inhibitor (PR)
  - Fast Track designation for Kinnate's investigational pan-FGFR inhibitor, KIN-3248, for the treatment of patients with unresectable, locally advanced or metastatic cholangiocarcinoma (CCA) harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other alterations, who have received at least one prior systemic therapy. Cholangiocarcinoma, also known as bile duct cancer, is a rare condition, often diagnosed when it is advanced. Research has shown that FGFR is an actionable alteration in patients with CCA. FGFRs are tyrosine kinases that play a crucial role in cell proliferation, differentiation, migration and survival. FGFR2 gene fusions or other alterations are identified in approximately 16% of intrahepatic cholangiocarcinoma (ICC) tumors.
- 02.13.23 - Exelixis and Sairopa Announce US FDA Clears Investigational New Drug Application for ADU-1805 in Patients with Advanced Solid Tumors (PR)
  - Under the terms of the clinical development and option agreement announced in November 2022, Exelixis has the option to obtain an exclusive, worldwide license to develop and commercialize ADU-1805 and other anti-SIRPα antibodies upon review of data from prespecified phase 1 clinical studies of ADU-1805 to be completed by Sairopa during the option period. This IND clearance triggers a $35 million milestone payment to Sairopa which will be paid in the first quarter of 2023.
- 02.13.23 - Opdivo® (nivolumab) in Combination with CABOMETYX® (cabozantinib) Shows Durable Survival with Over Three Years of Follow-Up in the CheckMate -9ER Trial in First-Line Advanced Renal Cell Carcinoma (PR)
  - Data to be featured at ASCO GU 2023 demonstrate continued overall survival, progression-free survival and objective response rate benefits with Opdivo in combination with CABOMETYX compared to sunitinib, regardless of IMDC risk score. These three-year data – with a median follow-up of 44 months – from CheckMate -9ER represent the longest reported follow-up in any Phase 3 trial with an immunotherapy-tyrosine kinase inhibitor regimen in this population. In an exploratory biomarker analysis, median progression-free survival and overall survival were improved with the combination of Opdivo and CABOMETYX regardless of PD-L1 status
- 02.10.23 - Incyte Announces 52-Week Results from Phase 2 Study Evaluating Povorcitinib (INCB54707) in Patients with Hidradenitis Suppurativa (PR)
  - Results from open-label extension period of the Phase 2 trial demonstrate that longer-term treatment with povorcitinib 75 mg resulted in sustained and durable efficacy across all treatment arms. Data featured as an oral presentation at the European Hidradenitis Suppurativa Foundation conference. Hidradenitis suppurativa (HS) is a chronic and debilitating inflammatory skin condition characterized by painful nodules and abscesses that can lead to irreversible tissue destruction and scarring
- 02.10.23 - Mineralys, amid signs of thawing IPO market, upsizes offering to raise $192M for race with AstraZeneca (fiercebiotech)
  - In the first half of 2023, Mineralys plans to start a phase 2 clinical trial to evaluate its lead candidate, the aldosterone synthase inhibitor lorundrostat, as an add-on drug in patients with uncontrolled or resistant hypertension (uHTN/rHTN). It also expects to kick off another phase 2 trial of lorundrostat for the treatment of uHTN and rHTN in a chronic kidney disease population around the midpoint of the year.

- 02.09.23 - Kura Oncology Announces First Patients Dosed in Phase 2 Registration-Directed Trial of Ziftomenib in NPM1-Mutant Acute Myeloid Leukemia (PR)
  - The primary endpoint in the Phase 2 registration-directed trial in patients with NPM1-mutant relapsed or refractory AML, is CR or complete response with hematologic recovery (CRh), and key secondary endpoints include clinical benefit as well as safety and tolerability. In addition to continued evaluation of ziftomenib as a monotherapy in NPM1-mutant AML, Kura plans to initiate the KOMET-007 and KOMET-008 trials later this year to evaluate ziftomenib in combination with current standards of care in earlier lines of therapy and across multiple patient populations, including NPM1-mutant and KMT2A-rearranged AML.
- 02.09.23 - New Phase III Data Show Genentech's Vabysmo Rapidly Improved Vision and Reduced Retinal Fluid in People With Retinal Vein Occlusion (RVO) (PR)
  - Vabysmo met its primary endpoint in two clinical trials, BALATON and COMINO, showing non-inferior visual acuity gains compared to aflibercept. More Vabysmo patients showed an absence of blood vessel leakage in the retina compared to aflibercept in a pre-specified exploratory endpoint. If approved, RVO would be the third indication for Vabysmo in addition to wet, or neovascular, age-related macular degeneration and diabetic macular edema
- 02.06.23 - 'Smooth and very much on track': Eisai reports first shipments of Leqembi
  - Eisai and Biogen began shipping out doses of their newly approved Alzheimer's drug Leqembi ahead of schedule last month, according to Eisai's US chairman and CEO Ivan Cheung. Leqembi won an accelerated approval back in January for use in patients with mild cognitive impairment from Alzheimer's who have confirmed presence of amyloid beta pathology prior to treatment. It's a second chance in Alzheimer's for Eisai and Biogen, whose controversial predecessor Aduhelm suffered a commercial flop following its accelerated approval in 2021. The first batch of Leqembi vials arrived "ahead of schedule" on Jan. 17, and the first sales were recorded the following day. The first prescription was written on Jan. 23, and the first infusion occurred last week, according to Cheung. Prescriptions so far have been limited to patients who are participating in the company's assistance program, or paying out-of-pocket, he said. Leqembi is priced at $26,500 per year.
- 02.06.23 - Inmagene receives IND clearance for IMG-008, a long-acting IL-36R mAb (PR)
  - IMG-008, a humanized monoclonal antibody (mAb) that specifically targets human interleukin 36 receptor (IL-36R), potently blocks the IL-36 signaling and reduces cytokine releases and inflammatory responses. In a pre-clinical study in monkeys, IMG-008 has demonstrated over 4 times longer half-life and over 2 times higher exposure than spesolimab analog. IMG-008 may potentially provide a treatment option for multiple inflammatory diseases, such as generalized pustular psoriasis (GPP) and hidradenitis suppurativa (HS). Patients with the target diseases often suffer from high levels of inflammation, repeated flares, and comorbidities associated with obesity.
- 02.06.23 - Boarding priority review track, Biogen and Sage get August decision date for depression drug approval (fiercebiotech)
  - Add Aug. 5 to your diaries. That is the FDA's decision date for the approval of Biogen and Sage Therapeutics' zuranolone, which the agency has accepted for priority review as an oral treatment of major depressive disorder (MDD) and postpartum depression (PPD). Biogen and Sage began a rolling submission for zuranolone in MDD in May. At that time, the partners planned to submit an associated filing in PPD in the first half of 2023. In the end, the team wrapped up submissions in both indications late last year—and has been rewarded with a FDA decision date that keeps alive its hopes of launching zuranolone in the second half of 2023.
- 02.06.23 - Endogena Therapeutics Receives US FDA Fast Track Designation for EA-2353 for the Treatment of Retinitis Pigmentosa (PR)
  - EA-2353 takes a novel, small-molecule approach and selectively activates endogenous retinal stem and progenitor cells, which differentiate into photoreceptors and can potentially preserve or restore visual function. This gene-independent treatment approach has significant advantages in RP, which has multiple genetic causes. EA-2353 was granted orphan drug designation by the US FDA in May 2021.
- 02.06.23 - Biogen and Sage Therapeutics Announce FDA Accepts Filing of New Drug Application and Grants Priority Review of Zuranolone in the Treatment of Major Depressive Disorder and Postpartum Depression (PR)
  - Zuranolone is being evaluated as a potential 14-day, rapid-acting, once-daily, oral medication to treat major depressive disorder (MDD) and postpartum depression (PPD). Depression is a public health issue with significant unmet medical need. he application has been granted priority review and the FDA has assigned a Prescription Drug User Fee Act (PDUFA) action date of August 5, 2023.
- 02.03.23 - U.S. FDA Approves Gilead's Trodelvy® in Pre-treated HR+/HER2- Metastatic Breast Cancer (PR)
  - The approval is based on statistically significant and clinically meaningful progression-free survival and overall survival data from the Phase 3 TROPiCS-02 study. Trodelvy is now also recommended as a Category 1, preferred treatment for metastatic HR+/HER2- breast cancer by the National Comprehensive Cancer Network® (NCCN®) as defined in the Clinical Practice Guidelines in Oncology (NCCN Guidelines®). The European Medicines Agency has also validated a Type II Variation Marketing Authorization Application for Trodelvy in HR+/HER2- metastatic breast cancer.
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- 02.02.23 - INOVIO Reports Positive Phase 1b Results for INO-4201 as an Ebola Booster for rVSV-ZEBOV (Ervebo®) (PR)
  - INO-4201 was evaluated in a 46-participant randomized, placebo-controlled Phase 1b trial (NCT04906629) to assess its safety, tolerability, and immunogenicity in healthy adult participants who previously received a single injection of Ervebo, a vaccine approved by the U.S. Food and Drug Administration for the prevention of disease caused by Zaire ebolavirus in individuals 18 years of age and older. The participants were dosed with 1 mg of INO-4201 injected intradermally followed by electroporation using our investigational proprietary smart device, CELLECTRA®. The trial was designed to test whether INO-4201 can be used as a booster in healthy participants previously vaccinated with Ervebo. The trial was spearheaded by Global Urgent and Advanced Research and Development (GuardRX), sponsored by Geneva University Hospitals, and funded by the U.S. Defense Advanced Research Projects Agency (DARPA).
- 02.02.23 - RefleXion Receives FDA Clearance for SCINTIX Biology-Guided Radiotherapy; Cutting-edge Treatment Applicable for Early and Late-stage Cancers (PR)
  - SCINTIX is the first and only radiotherapy that allows each cancer's unique biology to autonomously determine where and how much radiation to deliver, second-by-second, during actual treatment delivery. This expands the RefleXion® X1 into the only dual-treatment modality platform that can treat patients with indicated solid tumors of any stage. The SCINTIX biologic modality tracks tumor motion from all types of movement, including expected motion from internal processes such as breathing and digestion or unexpected movement by a patient. The X1 also has a state-of-the-art anatomic modality previously cleared by the FDA for solid tumors located anywhere in the body.
- 02.01.23 - First U.S. Patient Dosed in Aulos Bioscience's Phase 1/2 Clinical Trial of AU-007, a Novel IL-2 Therapeutic for the Treatment of Solid Tumors (PR)
  - Aulos Bioscience, an immuno-oncology company working to revolutionize cancer care through the development of potentially best-in-class IL-2 therapeutics, today announced that the first patient has been dosed in the United States in its Phase 1/2 clinical trial evaluating AU-007 for the treatment of solid tumors. AU-007 is a human monoclonal antibody computationally designed by Biolojic Design to harness the power of interleukin-2 (IL-2) and eradicate solid tumors