PROJECT EVOLUTION

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FIRST HALF MARCH 2023

ADVANCED THERAPIES CLINICAL & COMMERCIAL NEWS

REACH OUT TO BRYAN KENNEDY AT BK@PROJECTEVOLUTION.ORG WITH ANY QUESTIONS

- 03.15.23 - ReCode Therapeutics Announces First Participants Dosed in a Phase 1 Healthy Volunteer Clinical Trial of Novel Disease-Modifying Genetic Medicine, RCT1100 for the Treatment of Primary Ciliary Dyskinesia (PR)
  - Phase 1 trial to evaluate safety and tolerability of a single ascending dose of RCT1100, an inhaled mRNA-based genetic medicine. – First clinical-stage candidate from ReCode's proprietary SORT LNP delivery platform designed to restore ciliary function in the lungs of people with PCD caused by pathogenic mutations in the DNAI1 gene; IND submission planned for 2H23.
- 03.14.23 - TScan Therapeutics Announces First Patient Dosed in Phase 1 Umbrella Clinical Trial Evaluating TSC-100 and TSC-101 for the Treatment of Hematologic Malignancies (PR)
  - Patient treated with TSC-101, the first clinical cell therapy product targeting minor histocompatibility antigen HA-2 to treat leukemia and prevent relapse following hematopoietic cell transplantation. The Phase 1 umbrella trial is a multi-arm, i3+3 study evaluating TSC-100, TSC-101, and standard of care HCT alone (control arm) in patients with AML, ALL or MDS. Treatment assignment is based on HLA and antigen expression, and the endpoints will include safety of repeated doses and efficacy of the TCR-T as compared to the control arm. Exploratory endpoints include cellular kinetics, minimal residual disease rates, percentage of donor chimerism, and persistence of TSC-100 and TSC-101.
- 03.09.23 - Vertex Announces FDA Clearance of Investigational New Drug Application for VX-264, a Novel Encapsulated Cell Therapy for the Treatment of Type 1 Diabetes (PR)
  - Vertex plans to initiate a Phase 1/2 clinical trial in the first half of 2023 to study the safety, tolerability and efficacy of VX-264 in patients with T1D. The company previously received approval from Health Canada on the Clinical Trial Application (CTA) for VX-264, and the Phase 1/2 trial is ongoing in Canada.
- 03.08.23 - Estrella Biopharma Announces FDA Clearance of IND Application for Phase I/II Clinical Trial (Starlight-1) of EB103, a CD19-Targeted ARTEMIS® T Cell Therapy, to Patients with B-Cell Lymphomas (PR)
  - The Starlight-1 Phase I/II clinical trial is designed to assess the safety, tolerability, recommended Phase II dose (RP2D), and preliminary anti-cancer activity of EB103 for the treatment of R/R B-cell NHL patients. The study is expected to enroll patients initially at UC Davis Health.
- 03.08.23 - Sernova Announces Initial Islet Transplantation in First Two Patients Enrolled in Second Cohort of its U.S. Phase 1/2 Clinical Trial for Treatment of Type 1 Diabetes (PR)
  - Announced today that the first two patients in the second cohort of its active U.S. Phase 1/2 clinical trial for the treatment of type 1 diabetes ("T1D") and hypoglycemia unawareness (the "T1D Study") received their first islet transplant into the higher capacity 10-channel Cell Pouch™. These patients will be monitored for safety and efficacy for three months after which a second dose of islets is anticipated to be transplanted in accordance with the protocol. Additionally, a third enrolled patient has now been implanted with the higher capacity Cell Pouch and awaits islet transplant in the coming weeks.
- 03.07.23 - FDA Accepts Mesoblast's Resubmission of the Biologic License Application for Remestemcel-L In Children with Steroid-Refractory Acute Graft Versus Host Disease as a Complete Response and Sets Goal Date of August 2, 2023 (PR)
  - Mesoblast's lead product candidate, Remestemcel-L, is an investigational therapy comprising culture expanded mesenchymal stromal cells derived from the bone marrow of an unrelated donor. It is administered to patients in a series of intravenous infusions. Remestemcel-L is believed to have immunomodulatory properties to counteract the inflammatory processes that are implicated in SR-aGVHD by down-regulating the production of pro-inflammatory cytokines, increasing production of anti-inflammatory cytokines, and enabling recruitment of naturally occurring anti-inflammatory cells to involved tissues. The BLA resubmission also contains results of a 4-year survival study performed by the Center for International Blood and Marrow Transplant Research (CIBMTR) on 51 evaluable patients with SR-aGVHD who were enrolled in the Phase 3 trial. The results demonstrated durability of the early day 180 survival benefits, with 63% survival at 1 year and 51% at 2 years in a group of children with predominantly grade C/D disease (89%) and with expected 2 year survival of just 25-38% using best available therapy.1,8-9
- 03.07.23 - BioMarin, as expected, sees delay to FDA review of hemophilia gene therapy (biopharmadive)
  - The Food and Drug Administration will take another three months to complete its second review of BioMarin's gene therapy for hemophilia A, now promising an answer by June 30. BioMarin had prepared investors last year for the possibility of an extension beyond the original target of March 31 after it announced plans to give the FDA three years' worth of patient follow-up data from its principal clinical trial. It got those results in January and shared them with regulators. The Phase 3 study is the "longest and largest" yet for a gene therapy for hemophilia and strengthens the company's approval bid, BioMarin said Monday. Still, the FDA deemed the submission a "major amendment" to the application, allowing the agency to extend the review time. The company appeared on track to bring a long-lasting therapy to patients who suffer from the genetic bleeding disease back in 2019, when it first sought approval for Roctavian. The next year, the FDA surprised the gene therapy world by rejecting it, saying the agency needed more evidence that the one-time treatment would provide lasting benefit

About Us:

PROJECT EVOLUTION

Project Evolution is an innovative, end-to-end, HUMAN CAPITAL platform that enables leading Life Scientific organizations to identify and secure the talent required to drive innovation. Our collaborative platform is powered by the industry-leading capabilities of Berke Executive Search and GTS Scientific. The highly-successful Joint Venture bridge Executive and Contingent placement and enable organizations of all sizes the ability to secure highly specialized talent quickly and efficiently.

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