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Project Evolution's U.S. Advanced Therapies May 2022 Recap

In a Fast-Moving Industry, Stay Up-to-Date on the Happenings. Here is a Glimpse of Our Favorite Stories from May 2022.

Funding Updates:

- 05.01.22 - Kelonia Therapeutics Launches with $50M Series A Financing to Pioneer Precision Targeted Genetic Medicines (Press Release)
  - The company will use the funding to develop an "off-the-shelf" chimeric antigen receptor (CAR) to treat hematologic cancer that may enable the unrivaled clinical benefit of CAR T without the typical toxicities and with the ease of access of conventional medicines. Additionally, the company will advance other programs for oncology and non-oncology indications and further expand its gene delivery platform and capabilities.
- 05.09.22 - Aspen Neuroscience a leading company developing a personalized cell replacement using a patient's own cells raises $147.5M via Series B financing. (Endpts)
  - Funding will be used to startup and advance its second preclinical Parkinson's bet (focused on GBA), build out its platform to include two more programs, and expand the management team, including a chief technology officer and general counsel. Financing will also bankroll the startup's manufacturing operations, which are all in-house at a facility near the biotech's San Diego headquarters. The round was led by GV, LYFE Capital, and Revelation Partners.
- 05.18.22 - SwanBio Therapeutics Announces $56M Series B Financing to Advance Novel Gene Therapies for Neurological Conditions (Press Release)
  - SwanBio Therapeutics, a gene therapy company advancing AAV-based therapies for the treatment of devastating, inherited neurological conditions, today announced the completion of a $56 million Series B financing round, led by founding investors Syncona Limited and Mass General Brigham Ventures. The new funding brings SwanBio's total financing raised to date to $133 million,
- 05.19.22 - Inceptor Bio to use $37M Series A to nudge CAR-T toward the clinic, and establish a manufacturing base (Fiercebiotech)
  - Inceptor Bio will use proceeds from a $37M Series A round to push its lead CAR-T program into phase 1 trials in 2023 and set its 29,000 SF manufacturing facility in Gainesville, FL, which the company acquired from Arranta Bio in October 2021.
- 05.16.22 - Kriya Announces $270M Series C Financing to Advance Fully Integrated Gene Therapy Engine (Biospace)
  - The financing will be used to support the advancement of the company's fully integrated gene therapy pipeline and expand its engineering, manufacturing, and computational platforms. In addition to the expansion of its pipeline and technology platform, Kriya also opened the doors to its new scalable GMP manufacturing space in North Carolina that will bolster the manufacturing of its gene therapies for oncology, diabetes, severe obesity, ophthalmology, and other indications.
- 05.20.22 - Castle Creek Biosciences Raises an oversubscribed and upsized preferred stock financing of $112.8M to Advance Novel Gene Therapies and Expand Pipeline (Biospace)
  - The financing is expected to provide sufficient capital for the company's completion of a Phase 3 study and issuance of topline results of its lead ex vivo product candidate for recessive dystrophic epidermolysis bullosa (RDEB), a progressive, painful, and debilitating rare genetic skin disorder, and positions Castle Creek to advance it's in vivo work to submit an IND application to the U.S. FDA for hereditary tyrosinemia type 1 (HT1), its first indication using the in vivo gene therapy technology. Castle Creek's dual technology platform of ex vivo and in vivo technologies is the foundation for developing novel gene therapies for a broad range of genetic diseases with limited or no treatment options.
- 05.25.22 - Flagship unveils ProFound Therapeutics with $75M to explore trove of new proteins (Fierce Biotech)
  - The new company, unveiled Thursday, comes equipped with $75M to dive headfirst into the new repository, which was uncovered by honing in on potential proteins involved in translation. The trove of new proteins effectively more than doubles the known repository and creates a new frontier to be explored for potential therapeutic targets. The unearthing is the result of a new approach taken by ProFound and its leaders to reconsider what exactly defines a protein-coding gene and how a protein is coded in the genome.

Manufacturing Updates:

- 05.09.22 - Matica Bio opens CGT plant, with a little help from G-CON and Sartorius (Bioprocess International)
  - CDMO Matica Biotechnology opened the 45,000 sf facility this week to provide viral vectors and cell-based products to cell and gene therapy (CGT) developers. The facility is fully financed by Matica's parent company – though the size of the investment as not been revealed – and built out from an existing shell using modular cleanrooms from G-CON. The plant will benefit from PAT technologies, automation software, and single-use platforms supplied by bioprocess vendor Sartorius, after the two firms teamed up in October 2021.
- 05.19.22 - AGC Biologics Invests in Viral Vector Suspension at New U.S. Campus (Press Release)
  - The company is adding viral vector suspension technology and capacity for the development and manufacturing of gene therapies at its commercial-grade campus in Longmont, Col., USA. These new capabilities, which begin coming online in the third quarter of 2022, complement the campus' adherent viral vector and cell therapy offerings – enabling AGC Biologics to provide an in-depth variety of end-to-end cell and gene therapy services at this site.
- 05.19.22 - Catalent introduces new AAV gene therapy tech in aim to speed up manufacturing process (Endpts)
  - The UpTempo Virtuoso platform can streamline the AAV manufacturing process to cut back on the time it takes to go from clinic to in-human clinical trials. It includes protocols for cell culture, transfection, and downstream purification, and can simplify the critical supply chain. It's designed to produce products for the clinic in just nine months, Catalent says, and customers will have access to Catalent's plasmid DNA offerings, too.
- 05.19.22 - Be Bio and Resilience Announce Strategic Collaboration to Manufacture Engineered B Cells, a New Class of Cellular Medicines (Press Release)
  - Resilience to Manufacture and Supply Good Manufacturing Practices (GMP)-Grade Viral Vector and Drug Product for Be Bio's Initial Rare Disease Clinical Programs. The pact marks the latest deal for a fast moving biomanufacturing startup, which has relationships in place with Takeda, Moderna, bluebird and Harvard.
- 05.25.22 - Lilly plans to invest $2.1 billion in new manufacturing sites in Indiana (Press Release)
  - The company plans to expand its manufacturing footprint in Indiana by investing $2.1 billion in two new manufacturing sites at Indiana's LEAP Lebanon Innovation and Research District in Boone County. These new facilities will expand Lilly's manufacturing network for active ingredients and new therapeutic modalities, such as genetic medicines, and represent the company's dedication to strengthening its portfolio of potentially life-changing treatments. The proposed project is expected to create up to 500 new Lilly roles with an additional four indirect jobs for every Lilly position created, based on industry data. An estimated 1,500 construction jobs will be required while the facilities are being built.
- 05.27.22 - iECURE Enters Agreement with Center for Breakthrough Medicines (Contract Pharma)
  - Collaboration wherein CBM will produce and supply good manufacturing practices (GMP)-grade adeno-associated virus (AAV) for use in iECURE's future clinical studies for its gene-editing platform focused on mutation-agnostic in vivo gene insertion, or knock-in, editing for the treatment of liver disorders with significant unmet need,

Collaboration Updates:

- 05.02.22 - Scribe Therapeutics Expands Collaboration With Biogen to Second Target (Press Release)
  - CRISPR molecular engineering company co-founded by Nobel Laureate Jennifer Doudna continues to accelerate the development of best-in-class in vivo CRISPR-based medicines through the expansion of its ongoing collaboration with Biogen.
- 05.02.22 - Gilead and Dragonfly Announce Strategic Research Collaboration to Develop Natural Killer Cell Engagers in Oncology and Inflammation (Press Release)
  - Under the agreement, Gilead will receive an exclusive, worldwide license from Dragonfly for the 5T4-targeting investigational immunotherapy program, DF7001. The agreement also grants Gilead options, after the completion of certain preclinical activities, to license exclusive, worldwide rights to develop and commercialize additional NK cell engager programs using the Dragonfly Tri-specific NK Engager (TriNKET™) platform. TriNKETs are activators of the innate and adaptive immune systems, recruiting NK and cytotoxic T cells into the tumor microenvironment.
- 05.09.22 - Twist Bioscience Enters into Research, Exclusive Option and License Agreement with Astellas for Antibodies to Reduce Tumor Microenvironment-Mediated Immunosuppression (Biospace)
  - Under the terms of the agreement, the companies will jointly conduct research activities to identify and optimize proprietary Twist antagonist antibodies, targeting an undisclosed checkpoint inhibitor pathway in the tumor microenvironment (TME), as potential therapeutic development candidates. Under the terms of the agreement, Twist will receive an upfront payment from Astellas as well as an additional payment upon the exercise of the licensing option. Twist will receive payments connected to success-based clinical milestones as well as royalty payments on product sales for each licensed product. Astellas will be responsible for the development, manufacturing and commercialization of any licensed products.

- 05.10.22 - Avantor Collaborates with Cytovance Biologics (Genengnews)
  - Avantor reports that it will collaborate with Cytovance Biologics to accelerate plasmid DNA development for biopharma customers. The collaboration is expected to advance plasmid optimization and sourcing services for new and existing viral vector and mRNA-based vaccine and therapeutic customers.
- 05.17.22 - Ultragenyx acquires rights to AAV Gene Therapy ABO-102 for Sanfilippo Syndrome Type A (MPS IIIA) from Abeona Therapeutics (Press Release)
  - Under the terms of the agreement, Ultragenyx will assume responsibility for the ABO-102 program and in return Abeona is eligible to receive tiered royalties of up to 10% on net sales and commercial milestone payments following regulatory approval.

Clinical & Approval Updates:

- 05.09.22 - Achilles Therapeutics Doses First Patient with Higher-dose cNeT in Phase I/IIa CHIRON Trial in Advanced NSCLC and Initiates Enrollment in Cohort B of the THETIS Trial (cNeT + PD-1 checkpoint inhibitor) in Metastatic Malignant Melanoma (Press Release)
  - THETIS Cohort B enrollment follows positive Independent Data Safety Monitoring Committee review. Monotherapy data from higher-dose cohorts in both CHIRON and THETIS and combination data from THETIS Cohort B expected in 2H 2022.
- 05.12.22 - Caribou Biosciences Announces Positive Initial Data for CB-010 Anti-CD19 Allogeneic CAR-T Cell Therapy (Press Release)
  - The company announced initial results demonstrating a 100% overall response rate (ORR) and 80% complete response rate (CR) in cohort 1 (n=5 evaluable) from its ANTLER Phase 1 trial for CB-010 in patients with relapsed or refractory B cell non-Hodgkin lymphoma (r/r B-NHL).
- 05.24.22 - FDA Accepts CSL Behring's BLA for Etranacogene Dezaparvovec for Priority Review (Press Release)
  - If approved, etranacogene dezaparvovec would be the first gene therapy option for people living with hemophilia B. This milestone underscores CSL Behring's promise to develop and deliver a truly unique portfolio of patient-focused therapies for people with rare and serious medical conditions
- 05.10.22 - Arcellx Announces Dosing Of First Patient In Its Phase 1 Clinical Trial Evaluating ACLX-001 (Press Release)
  - ACLX-001 is the first therapeutic in the dosable and controllable ARC-SparX Platform, for the treatment of patients with relapsed or Refractory Multiple Myeloma. ARC-SparX is a novel CAR-T cell therapy treatment designed to allow for controllability and adaptability to potentially reduce toxicities associated with serious dose-limiting adverse events and overcome tumor heterogeneity
- 05.20.22 - Touting 100% OS rate in pivotal rare disease trial, Rocket Pharma ready to head to FDA
  - Reporting topline results from a Phase II pivotal trial involving patients with severe leukocyte adhesion deficiency-I (LAD-1), Rocket said among nine participants, the overall survival at one year is 100%.
- 05.28.22 - FDA approves Novartis Kymriah® CAR-T cell therapy for adult patients with relapsed or refractory follicular lymphoma (Press Release)
  - The approval is based on data from the Phase II ELARA trial, a single-arm, open-label trial, in which 90 patients were evaluated for efficacy with a median follow-up of approximately 17 months. Eighty-six percent of patients treated with Kymriah achieved a response including 68% who experienced a complete response. In accordance with the Accelerated Approval Program, continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s). Kymriah is now FDA approved in three indications and remains the only CAR-T cell therapy approved in both adult and pediatric settings.

Research Institute Updates:

- 05.12.22 - University Of Colorado To Invest $200 Million In New Regenerative Medicine Institute (Forbes)
  - The University of Colorado Anschutz Medical Campus has announced that it will make a $200 million investment over the next five years to create the Gates Institute, a research and treatment center that will focus on the development of new regenerative, cellular and gene therapies for a variety of serious illnesses. The new funding will consist of $20 million per year for five years invested by both the Anschutz campus itself and the Gates Frontiers Fund, a private family foundation established in 1946 that focuses its philanthropy on the fields of education, natural resources, and community development in Colorado.
- 05.31.22 - Icahn School of Medicine, RPI launch new institute focused on engineering and precision medicine (Endpts)
  - The Troy, NY-based Rensselaer Polytechnic Institute (RPI) and the Icahn School of Medicine at Mount Sinai have banded together to create the Center for Engineering and Precision Medicine (CEPM) in New York City. According to RPI, the PhD program for the center will accommodate 30 to 40 students, with the center aiming to bring 100 or more Rensselaer undergraduates on a steady basis to New York City as research students, for capstone design or internships.