PROJECT EVOLUTION

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Project Evolution's Advanced Therapies Recap

For Inquiries or Questions, Please Contact
Bryan Kennedy
BK@Projectevolution.org

In a Fast-Moving Industry, Stay Up-to-Date on the Happenings. Here is a Glimpse of Our Favorite Stories from the First Half Jan 2023.

Funding Updates:

- 01.10.23 - CF Foundation Invests Up to $3.5M in Gensaic for Novel Gene Delivery Method (PR)
  - Under the terms of the 1st tranche, Gensaic will harness its proprietary PDP platform to achieve pre-clinical milestones upon which the 2nd tranche will be transacted. Gensaic will retain all rights to the continued development of a lead program.
- 01.09.23 - SANGUINE BIOSCIENCES PARTNERS WITH BROADOAK CAPITAL PARTNERS TO ACCELERATE LAUNCH OF NEW PRODUCTS AND SERVICES IN SUPPORT OF CELL & GENE THERAPY MARKETS (PR)
  - Investment will support ongoing partnerships with apheresis clinics, expansion of laboratory services, enhancement of patient data aggregation, and amplification of Sanguine's network of 60,000+ research-ready patient donor network.
- 01.09.23 - PBS Biotech Raises $22M to Expand Single-Use Manufacturing Products and Services for Cell Therapy Clients (PR)
  - PBS Biotech ("PBS"), an innovative, single-use bioreactor manufacturer and process development services provider, has completed a $22 million financing led by Avego Management, LLC ("Avego") with additional investment from existing investor BroadOak Capital Partners ("BroadOak"). This financing will be used to improve the company's products portfolio, expand its process development services capability, and increase customer support for global cell therapy clients.
- 01.06.23 - Synthekine Secures $100 Million Series C Financing to Advance Pipeline of Engineered Cytokine Therapeutics (PR)
  - Proceeds of the financing will be used to advance Synthekine's differentiated therapeutic pipeline, led by its alpha/beta-biased IL-2 partial agonist, STK-012, which is currently in a Phase 1 clinical trial. In addition, it will support upcoming clinical studies of its orthogonal IL-2 and CD19 CAR-T combination therapy, STK-009 + SYNCAR-001, which the company anticipates to begin early this year. Funds will also be used to progress the company's IL-12 partial agonist program, which is currently in IND-enabling studies. IL-12 is a potent cytokine with potential for the treatment of cancer, but administration of unmodified forms of the cytokine have been limited by a narrow therapeutic window, including life-threatening toxicities in patients. Preclinical data for Synthekine's IL-12 partial agonist program demonstrates potent anti-tumor efficacy in mouse models while avoiding induction of systemic toxicity. In addition, funds will also be used to advance toward clinical investigation its orthogonal IL-2 system with a GPC3 CAR-T therapy. Preclinical data for this program, the company's first cell therapy program for solid tumors, were presented at AACR 2022.
- 01.06.23 - Battelle, AmplifyBio, Andelyn Biosciences Win Research Contract for National Institute of Neurological Disorders and Stroke (PR)
  - The team of Battelle, AmplifyBio and Andelyn Biosciences has won a seat on an eight-year, $149 million indefinite delivery, indefinite quantity (IDIQ) contract vehicle for the National Institute of Neurological Disorders and Stroke. As one of the prime contractors on the multi-award IDIQ, the team will provide manufacturing and nonclinical support for translational development of therapeutic biotechnology products in the National Institutes of Health's drug discovery and development program that addresses neurological conditions.
- 01.05.23 - Perceive Biotherapeutics Attracts $78M Series B Financing to Advance Diversified Pipeline (PR)
  - the closing of a $78M Series B funding round led by Johnson & Johnson Innovation – JJDC, Inc.(JJDC), joining existing Series A investor Deerfield Management and new investors Braidwell LP, the Retinal Degeneration Fund, and Catalio Capital Management, LP. Perceive Biotherapeutics is focused on the discovery and development of transformative gene therapies and other therapeutics for ocular diseases with high unmet need. By leveraging deep genetic understandings, Perceive Bio researchers have elucidated key protective biological targets. These targets uniquely position the Company to accelerate the development of best-in-class therapies for programs that address over 50% of currently untreatable retinal blindness. Perceive Bio is advancing therapeutic programs in geographic atrophy / age-related macular degeneration, glaucoma, and additional undisclosed disease areas.

Manufacturing & CDMO Updates:

- 01.16.23 - Charles River Launches CliniPrime Suite of GMP-Compliant Cellular Products (PR)
  - The first CliniPrime product, the CliniPrime Fresh Leukopak, fulfills the industry need for ready access to cGMP-enabled, enriched leukocyte cellular starting materials. CliniPrime leverages Charles River's established production processes to provide advanced therapy programs a high-quality product offering to support both clinical trial development and commercialization, while reducing client resource investment and risk. With the CliniPrime portfolio Charles River now offers two options for GMP-compliant cellular starting material. CliniPrime products provide a standardized production process while GMPrime™ products enable clients to customize the production process of cellular starting material to meet specific needs for their program. Those two options for GMP-compliant starting material are also complemented by the HemaPrime portfolio of research use only (RUO) cellular products that include fresh leukopaks and other cellular products.

- 01.12.23 - Boyd Street Ventures Invests in Biomanufacturing Pioneer Wheeler Bio (kron4)
  - Wheeler Bio is a biomanufacturing pioneer founded by a team of industry experts and strategic investors who believe a different CDMO (Contract Development and Manufacturing Organization) model is needed to help innovators progress faster with new therapeutic candidates. Their novel hub-and-spoke concept, centered in Oklahoma City, was created to revolutionize the speed of drug development. A centerpiece of Wheeler's offerings is Portable CMC™, a machine learning built platform for early clinical phase mAbs coupled with a disruptive service offering which accelerate time to first-in-human studies (clinical phase 1). With Portable CMC™, Wheeler Bio simplifies the connection between early drug discovery and clinical manufacturing.
- 01.12.23 - Samsung Biologics jumping into ADC, gene therapy (Koreabiomed)
  - Samsung Biologics unveiled its aspiration to become a top-tier global biopharmaceutical company by 2030 during the 2023 J.P. Morgan Healthcare Conference's Main Track on Wednesday. John Rim, CEO of the biotech unit of Samsung Group, revealed a detailed plan for entering new business areas -- antibody drug conjugates (ADCs) and cell and gene therapy (CGT). Samsung Biologics will build facilities for ADCs this year, with the aim of the first ADC production in the first quarter of 2024. Notably, through the Life Science Fund, which is a 150 billion won ($120 million) fund formed by Samsung Biologics and Samsung C&T, the company will be investing in a third company, which specializes in ADC, within the first quarter of this year. The Life Science Fund had previously invested in Jaguar Gene Therapy, a U.S. gene therapy development company, for 20 billion won, and Senda Biosciences, a U.S. biotechnology company, for 19 billion won in 2022.
- 01.11.23 - Contract manufacturer nets capital investment to expand its physical space and install a new fill-finish line (endpts)
  - , Jon Lenihan, the SVP of commercial at Argonaut, said that while the company won't disclose the size of the investment. the funds will be going toward installing a new fill-finish line and acquiring more space for the company. Lenihan stated that the company is already moving forward with this and has taken steps to purchase the equipment. For boosting the size of its footprint, Argonaut will either acquire or lease a building next to or near its current 100,000-square-foot facility in Carlsbad, CA, and plans to tack on another 25,000 to 30,000 square-feet of space in total. Lenihan noted that this can help the company have more room to install a third fill-finish line in the future.The California-based CMO Argonaut Manufacturing Services has roped in some cash to start the year in an effort to make a significant expansion to its business. "Our focus area is those types of projects that are kind of orphan or ultra-orphan type, commercialization size products, 15-to-20,000-unit batches. This new line will double that size and scale," Lenihan said.
- 01.11.23 - Lotte Biologics pledges $3b to beef up production at home (Koreahearld)
  - Lotte Biologics will invest around $3 billion by 2030 to build new CDMO plants in South Korea, Lotte Biologics CEO Richard Lee announced during the company's presentation at the 41st JPMorgan Healthcare Conference held in San Francisco, Tuesday.The investment will go toward building three plants, with a combined manufacturing capacity of 360,000 liters, 120,000 each, according to Lee. About $1 billion will be needed per plant, Lee estimated. The company will start to build its first plant in the second half this year, finish construction by 2025, adopt good manufacturing practices in 2026 and start commercial operation in 2027.It aims to have all three plants up and running by 2034.
- 01.10.23 - Forge Biologics and Oculogenex Announce AAV Contract Development and Manufacturing Partnership (PR)
  - Forge to provide manufacturing and development services for Oculogenex's novel AAV gene therapy to improve vision in patients with macular degeneration. Oculogenex's investigational approach is a novel ocular regenerative AAV gene therapy that is delivered as a one-time injection into the eye. Preclinical data demonstrate that this approach makes retinal cells more resistant to stress, helps cells repair damage, and increases the cells' lifespans. The approach has the potential to activate dormant stem cells in the retina to replace lost tissue. Post-administration benefits have been demonstrated in preclinical research, and the company is completing additional preclinical research to prepare for entry into Phase I clinical trials.
- 01.10.23 - Resilience Announces Equity Investment from Mubadala and Funding of New Biopharma Manufacturing Facility in the United Arab Emirates (PR)
  - Under the agreement, Mubadala will establish the new manufacturing facility, which will be operated by Resilience, to manufacture certain biopharmaceutical-related products in the UAE. The facility will include a range of therapeutics for complex diseases such as cancer, infectious diseases, and inflammatory and autoimmune disorders, as well as vaccines. As part of the collaboration, Resilience has agreed to provide manufacturing, technology, and operational expertise for the Abu Dhabi-based facility and integrate the site as a node within its global network. The facility would be the first Good Manufacturing Practice (GMP) biopharma facility in the region based in Abu Dhabi to manufacture essential life sciences products for advanced therapies.
- 01.10.23 - Kite Pharma Expands Cell Therapy Operations in Maryland.
  - announced an expansion to our cell therapy operations in Frederick, Maryland with a new, centralized raw materials warehouse that will serve Kite's global manufacturing network, bringing an additional 100 jobs to the area and deepening the company's investment in the local life sciences community.
- 01.10.23 - Forecyte Bio opened its Maryland GMP Site for plasmid DNA, Gene and Cell Therapies.
  - Forecyte Bio Limited is a CDMO company founded in 2021 to service the rapidly growing Cell and Gene Therapy (CGT) industry. Built on decades of experience and expertise from its core technical experts on CGT CMC processes, Forecyte Bio offers an integrated service package to accelerate CGT projects from early concept to IND filing and eventual commercial licensure and manufacturing. Our mission is to accelerate patient access to cell and gene therapy with quality, speed, and agility.
- 01.09.23 - Updated: Agilent throws down $725M to double manufacturing capacity for nucleic acids to meet API demand (endpts)
  - Agilent Technologies, which spun out of American tech giant Hewlett Packard back in 1999, put the word out on Monday, saying that the $725 million will be used to double the company's current manufacturing capacity for therapeutic nucleic acids. These nucleic acids, also known as therapeutic oligonucleotides, are DNA or RNA molecules that act as the API for certain drugs that target indications such as cancer or rare diseases. In order for the company to double its capacity, Agilent is expanding its manufacturing facility in the town of Frederick, Colorado, which opened back in 2019. An Agilent spokesperson told Endpoints News via email that the expansion will add 198,000 square feet to the facility and double the facility's dedicated manufacturing space. A spokesperson added that the expansion will also generate 160 new jobs in the state. A press release from Colorado Governor Jared Polis' office on Monday said that the state's Economic Development Commission approved up to $1,787,500 in a performance-based incentive over a 5-year period, at $6,500 per net new job. That incentive is contingent upon Agilent meeting new job creation and salary requirements, as well as local match incentives.

- 01.05.23 - WuXi inks $1.5B GSK pact, a big biobuck bet but a blip in billions lost from US unverified list (Endpts)
  - Only $40 million of the GSK deal will initially go to the Shanghai-based contract research and manufacturing giant, but with a biobuck bet above $1 billion, the Big Pharma sees lots of potential. The pact is a boon to WuXi, which suffered a loss of billions in market value after being placed on the US Department of Commerce's unverified list last February. WuXi was removed from the list last month. Kicking off the deal is one preclinical bispecific antibody that targets a tumor-associated antigen on bad cells and CD3 expression on T cells, WuXi announced Thursday. CD3 is one branch of Amgen's Blincyto bispecific, and it's one part of the focus of Roche and Genentech's Lunsumio. FDA cleared the bispecific as a third-line treatment for adults with relapsed or refractory follicular lymphoma last month.
- 01.05.23 - Sarepta and Catalent Expand Strategic Manufacturing Partnership with Commercial Supply Agreement for Duchenne Muscular Dystrophy Gene Therapy Candidate (PR)
  - Commercial supply agreement for Catalent to manufacture delandistrogene moxeparvovec (SRP-9001), Sarepta's most advanced gene therapy candidate for the treatment of Duchenne muscular dystrophy (DMD). The agreement also structures how Catalent may support multiple gene therapy candidates in Sarepta's pipeline for limb-girdle muscular dystrophy (LGMD). In November 2022, Sarepta announced that the U.S. Food and Drug Administration (FDA) had accepted its biologics license application (BLA) seeking accelerated approval of delandistrogene moxeparvovec. Under the terms of this expanded agreement, Catalent will be Sarepta's primary commercial manufacturing partner for this therapy.

Clinical and Commerical Milestones:

- 01.06.23 - Cellenkos Announces First Patient Dosed with CK0804 Cell Therapy in LIMBER-TREG108 Clinical Trial (PR)
  - First patient dosed with CK0804 Treg cells as an add on therapy to ruxolitinib in patients with myelofibrosis who experience a suboptimal response to ruxolitinib. On-going Phase 1b study evaluating safety of CK0804
- 01.06.23 - Celularity's Placental-Derived Allogeneic Cell Therapy Provides Clinically Meaningful Benefit and Durable Biological Effect in Patients with Moderate to Severe Crohn's Disease in Phase 1, Phase 1b/2a and Phase 1b Studies (PR)
  - Legacy studies employing a single treatment course of two infusions seven days apart demonstrated greatest benefit in three early studies. Data support further investigation of novel genetically modified cell therapy in Crohn's disease
- 01.05.23 - Orchard Therapeutics Announces U.S. FDA Clearance of IND Application for OTL-203 in MPS-IH (PR)
  - The study is a multi-center, randomized, active controlled clinical trial designed to evaluate the efficacy and safety of OTL-203 in patients with MPS-IH compared to standard of care with allogeneic hematopoietic stem cell transplant (HSCT). A total of 40 patients with a confirmed diagnosis of MPS-IH who meet the study inclusion criteria will be randomized 1:1 to receive either OTL-203 or allogeneic HSCT. The study is powered to demonstrate superiority of OTL-203 over HSCT.

Collaborations

- 01.11.23 - Myrtelle and rAAVen Therapeutics to Develop Novel Gene Therapy Vectors (PR)
  - The partnership will leverage rAAVen's expertise in modifying established viral vectors for the design and production of new viral vectors as potential precision treatments for a broad spectrum of diseases in combination with Myrtelle's expertise in CNS gene therapy research and development. rAAVen will utilize its unique platform for AAV development that combines state-of-the-art methodologies within cloning, viral vector production and next generation sequencing. Myrtelle will test the vectors in a range of myelin-based disorders and, if successful, pursue further development. Myrtelle will own the vector compositions and retain exclusive worldwide rights to commercialize the resulting gene therapies. In return, rAAVen will receive milestone and sales-based royalty payments.
- 01.11.23 - Arbor Biotechnologies Announces Expanded Strategic Partnership with Vertex, Now Extending to Precision Gene Editing Using Reverse Transcriptase (PR)
  - Under the terms of the agreement, Arbor is eligible to receive payments based upon the successful achievement of specified research, development, regulatory and commercial milestones. In addition, Vertex will pay tiered royalties on future net sales of any products that may result from this collaboration.This marks a significant expansion of the relationship between the two companies, who first partnered on gene-editing therapies in 2018 and entered into a new relationship in 2021 around next-generation cell therapy approaches in diabetes and hemoglobinopathies, among other diseases.
- 01.10.23 - AbbVie and Anima Biotech Announce Collaboration for the Discovery and Development of mRNA Biology Modulators (PR)
  - Under the terms of the agreement, Anima will receive an upfront payment of $42 million and may be eligible to receive up to $540 million in option fees and research and development milestones in the aggregate across the three targets, with potential for further commercial milestones as well as tiered royalties on net sales. AbbVie has an option to expand the collaboration with up to three additional targets under the same terms as the initial collaboration, which may increase the potential value of the collaboration.
- 01.09.23 - Selecta Biosciences and Astellas Announce Exclusive Licensing and Development Agreement for Xork IgG Protease (PR)
  - an exclusive licensing and development agreement for IdeXork (Xork). Xork is being studied as a potential next generation immunoglobulin G (IgG) protease that will be developed by Astellas for use with AT845, an investigational, adeno-associated virus (AAV)-based treatment for Late-Onset Pompe disease (LOPD) in adults. Under the terms of the agreement, Selecta will receive a $10M upfront payment and is eligible to receive up to $340M for certain additional development and commercial milestones plus royalties on any potential commercial sales where Xork is used as a pre-treatment for AT845. Selecta is responsible for the development and manufacturing of Xork and will maintain the rights for the development of additional indications beyond Pompe disease. Astellas would have the sole and exclusive right to commercialize Xork for use in Pompe disease with an Astellas gene therapy investigational or authorized product, with a current focus on AT845.

- 01.09.23 - Boehringer Ingelheim and 3T Biosciences Join Forces to Develop Next-Generation Cancer Immunotherapies (PR)
  - Under the agreement, Boehringer Ingelheim will provide patient-derived TCR data to fuel 3T's target discovery efforts to identify antigens using its 3T TRACE discovery platform. 3T will receive an upfront payment and research and development support, and is eligible for discovery, preclinical, clinical, regulatory, and commercial milestones totaling $268 million in addition to royalties on future Boehringer Ingelheim product sales. Boehringer Ingelheim is eligible to receive royalties on future product sales by 3T Biosciences arising from the agreement.
- 01.09.23 - Neurocrine Biosciences and Voyager Therapeutics Enter Strategic Collaboration for Development and Commercialization of Voyager's GBA1 Program and Other Next-Generation Gene Therapies for Neurological Diseases (PR)
  - Voyager to receive up-front consideration of $175 million including a $39 million equity investment, up to $1.5 billion in potential development milestones, additional potential commercial milestones, tiered royalties on net sales, program funding, and an option to elect 50/50 cost- and profit-sharing in the U.S. for the GBA1 program following Phase 1 readou. Neurocrine to receive worldwide rights to Voyager's GBA1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases and three gene therapy programs directed to rare CNS targets, each enabled by Voyager's next-generation TRACERTM capsids, as well as additional equity in Voyager. Jude Onyia, Ph.D., Chief Scientific Officer at Neurocrine Biosciences, will join Voyager's Board of Directors.
- 01.09.23 - Autolus Therapeutics Announces Collaboration with Cabaletta Bio for Use of Autolus' Safety Switch System in Cell Therapies for Autoimmune Disease (PR)
  - Under the terms of the agreement, Autolus will receive an upfront payment for non-exclusive access to the RQR8 safety switch for use in Cabaletta's CD19-CAR T cell therapy program for the treatment of autoimmune disease, with the potential for near term option exercise fees and development and regulatory milestone payments. In addition, Autolus is entitled to receive royalties on net sales of all Cabaletta cell therapy products that incorporate the RQR8 safety switch.

Investment Funds

- 01.12.23 - Sanofi tops off venture arm, bringing fund to $750M (Biopharmadive)
  - French drugmaker Sanofi is planning a multi-year capital infusion that will bring the total funding in its Sanofi Ventures evergreen fund to more than $750 million. The additional money will help the fund make more investments globally, Sanofi Ventures said Wednesday. The evergreen fund finances a variety of companies and also helps hunt for business development and acquisition opportunities for Paris-based Sanofi. To date, Sanofi Ventures has focused mostly on biotechnology investments, with the remaining 20% of its funding going toward digital health companies. The ventures group closed 10 investments last year.