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

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

Funding Updates:

- 07.12.22 - Epic Bio, Founded by CRISPR Pioneer, Launches to Revolutionize Genetic Medicine with Epigenetic Engineering with $55 million Series A Financing (Press Release)
  - Epic has developed the GEMS (Gene Expression Modulation System) platform to precisely modify gene expression. GEMS includes the largest known library of novel modulators combined with advanced functional and computational genomics capabilities to rapidly design guide RNAs that are highly specific to the targeted genes. Epic holds exclusive license to smallest known Cas protein, enabling in vivo delivery via AAV vector to wide range of target organs. Lead program in facioscapulohumeral muscular dystrophy (FSHD) on track for clinical initiation in 2023 –
- 07.13.22 - Xilis Announces Series A Extension, Bringing Total Round to Over US$89 Million (Press Release)
  - Xilis us developing its MicroOrganoSphere™ (MOS™) technology to guide precision therapy for cancer patients and accelerate drug discovery and development, has closed an extension of over US$19 million to its Series A financing round, bringing the total amount raised to over US$89 million. New investor FPV Ventures led the extension, with participation from fellow new investor Alexandria Venture Investments and existing investors EQT Life Sciences, Mubadala Capital Management, Pear Ventures, GV (formerly Google Ventures), the Duke Angel Network, Catalio Capital Management, Two Sigma Ventures, Felicis Ventures, Alix Ventures and other strategic partners.
- 07.19.22 - Frontera Therapeutics Completes $160 Million Series B Financing to Fund Clinical Development and Manufacturing Capabilities (Press Release)
  - The company announced the successful completion of a $160 million Series B funding round. The Series B investors include Boyu Capital, Sequoia China, OrbiMed, Creacion Ventures, and other investors. In addition, Frontera announced that the U.S. Food and Drug Administration (FDA) has accepted the company's Investigational New Drug (IND) application to initiate a first-in-human clinical trial of FT-001, a gene therapy candidate for the treatment of patients with a rare genetic retinal disease that leads to severe vision loss.
- 07.20.22 - Auron Therapeutics Announces $48 Million Series A Financing to drive novel oncology pipeline leveraging a machine learning, multi-omics based platform (Press Release)
  - The biopharmaceutical company focused on developing therapies that target dysregulated differentiation and cellular plasticity for treating cancer, today announced the completion of its $48 million Series A financing round. Proceeds will be used to advance the lead program toward clinical development and drive additional programs into drug discovery. Funds will also be used to expand the proprietary, machine learning-based computational platform, AURigin, that is used to identify novel drug targets, and add personnel to support and accelerate research and development.
- 07.20.22 - CAMP4 Secures $100 Million Series B Financing to Accelerate Expansion of its Novel Regulatory RNA-Targeting Platform and Advance Lead Programs into the Clinic (Press Release)
  - Proceeds to Support Clinical Development of Lead CNS & Liver Programs; IND for Dravet Syndrome is Anticipated by Mid-2023. New Capital Will Also Fuel Expansion of CAMP4's regRNA Actuating Platform™, Harnessing the Power of Regulatory RNA to Upregulate Gene Expression. Funding Round Led by Enavate Sciences, a Portfolio Company of Patient Square Capital; Enavate CEO Jim Boylan Joins CAMP4's Board of Directors
- 07.21.22 - Vertex to tackle liver disease with Verve, putting up $60M to partner on gene editing program (Fiercebiotech)
  - Vertex Pharmaceuticals is getting deeper into in vivo gene editing. Months after partnering with Mammoth Biosciences, the storied biotech is back with another deal, ponying up $60 million to work with Verve Therapeutics on a single undisclosed liver disease.
- 07.27.22 - Gilead again backs AlloVir's T cell therapy in $127M direct offering (Endpts)
  - Already stocked with $172 million at the end of June, the Massachusetts biotech wants the additional money to bankroll its three ongoing Phase III trials of its allogeneic T cell therapy posoleucel. The new funds will also support global regulatory filings for the therapy, the company said Wednesday. The off-the-shelf T cell therapy is being explored in kids and adults with weakened immune systems as a treatment and prevention tool for infections caused by six different viruses: adenovirus, BK virus, cytomegalovirus, Epstein-Barr virus, human herpesvirus 6 and JC virus. ElevateBio and Gilead are among a consortium of existing investors taking part in this week's direct offering, which also includes Series B backers F2 Ventures, Invus, Redmile Group and EcoR1 Capital. GMT Capital also joined for the direct offering.
- 07.28.22 - Vicinitas Therapeutics Launches With $65 Million in Series A Financing to Advance Precision Medicines to Stabilize Key Proteins to Treat Disease (Press Release)
  - Vicinitas Therapeutics, a biotechnology company advancing a proprietary targeted protein stabilization platform to develop novel therapeutics in cancer and genetic disorders, today launched with $65 million in Series A financing. The financing was co-led by a16z and Deerfield Management, with participation from Droia Ventures, GV, The Mark Foundation for Cancer Research and the Berkeley Catalyst Fund. Vicinitas Therapeutics is a spin-out company that resulted from the Deubiquitinase Targeting Chimera (DUBTAC) platform, which was developed through an academic-industry research collaboration between the Novartis Institutes for BioMedical Research and researchers at the University of California, Berkeley.

Manufacturing Updates:

- 07.01.22 - Cytiva and Bayer to collaborate on allogeneic cell therapy manufacturing platform (Press Release)
  - Both parties will provide personnel, resources, and facilities to work on the joint development program. Bayer's road map for allogeneic cell therapy manufacturing technology will be leveraged, and its development product portfolio will be among the product candidates used to support proof of concept testing of the technologies. Cytiva will utilize its equipment and consumable manufacturing expertise and technology roadmap to design the new platforms, and when complete, will make them commercially available.
- 07.07.22 - Kiromic BioPharma Achieves Milestone with Timely Completion of Expanded cGMP Manufacturing Facility to Support Cell Therapy Oncology Pipeline (Press Release)
  - The expanded facility located at Kiromic's headquarters is one of the conditions required for the Company to begin the activation of its cell therapy clinical trial for the DeltacelTM product candidate by the end of this year. The completion also addresses a key component in the clinical hold communication the Company received from the U.S. Food and Drug Administration (FDA) in June 2021. The expanded 34,000-square-foot facility includes flexible cellular therapy and viral vector suites, a dedicated cGMP microbiology lab, a dedicated cGMP quality control (QC) lab, a research and development laboratory, and an FDA Code of Federal Regulations (CFR-9) compliant vivarium.
- 07.21.22 - Oxford Biomedica expands Agreement with Juno Therapeutics, a Bristol Myers Squibb company; adding two new viral vector programmes for Bristol Myers Squibb CAR-T therapies (Press Release)
  - Oxford Biomedica plc announces that it has amended and expanded the License and Clinical Supply Agreement ("LSA") with Juno Therapeutics,, a wholly owned subsidiary of Bristol Myers Squibb Company. The amendment relates to the initiation of two new viral vector programmes for Bristol Myers Squibb CAR-T therapies.The LSA, announced in March 2020, granted Juno a non-exclusive license to Oxford Biomedica's LentiVector® platform to be used for the manufacture of viral vectors for CAR-T and TCR-T programs in oncology and other indications. Under the terms of the amendment, Oxford Biomedica will receive an undisclosed target nomination fee, as well as potential payments upon the achievement of certain milestones.
- 07.21.22 - Cytiva acquires a site in Michigan for resin manufacturing (Endpts)
  - The company has acquired a facility in Muskegon, Michigan. The new resins manufacturing site is part of Cytiva and Pall Corporation's $1.5 billion capacity expansion investment. Cytiva plans to transform the site into a 168,000-square-foot biomanufacturing center consisting of multiple buildings. Cytiva is now expanding beyond Sweden to manufacture these resins. Resins are critical in purifying and analyzing biomolecules so pharmaceutical and life sciences companies can make medicines. The facility will also bring in an estimated 200 employees when completed, but the financial details of the project were not disclosed.
- 07.25.22 - Pfizer forks out $470m to expand Pearl River vaccine plant (BioprecessIntl)
  - Bloomberg reported that Pfizer will invest $470 million to construct a building and renovate existing facilities located on its campus in Pearl River, which is 25 miles northwest of New York. A spokesperson confirmed this to BioProcess Insider, adding the plant will focus on the firm's messenger RNA (mRNA) portfolio. Pfizer anticipates completing the Pearl River expansion by 2026, which will add 260,000 square feet of operations, including 55,000 square feet of laboratory space.
- 07.28.22 - After netting $200M, Synthego to break ground on its manufacturing facility (Endpts)
  - The company has broken ground on a 20,000-square-foot manufacturing facility in the San Francisco Bay Area, expanding its GMP capacity by 30 times, Synthego said in an email to Endpoints News. The facility is also going to manufacture materials for translational and clinical research development for cell and gene therapies as well. The new facility is expected to be built and start operations within the year. According to the company, a significant amount of capacity has already been prioritized for its customers but it has not specified how many customers the company has.

Clinical & Approval Updates:

- 07.06.22 - Sarepta Therapeutics' Investigational Gene Therapy SRP-9001 for Duchenne Muscular Dystrophy Demonstrates Significant Functional Improvements Across Multiple Studies (Press Release)
  - Sarepta and its partner Roche present new results and analyses at the International Congress on Neuromuscular Diseases (ICNMD), which demonstrate that SRP-9001 shows consistent, statistically significant functional benefits in individuals with Duchenne versus a propensity-weighted external control that continue to positively diverge from natural history disease course
- 07.11.22 - SCG Cell Therapy Announces U.S FDA Clearance Of Investigational New Drug Application For SCG101, SCG's Novel TCR-T Cell Therapy For Hepatitis B-Related Liver Cancers (Press Release)
  - SCG101 is an autologous TCR T cell therapy that can recognize HBV-derived T cell epitope presented on the cell surface by specific major histocompatibility complex (MHC) class I molecules. With the specific HLA typing, SCG101 can redirect T cells specifically against the HBV antigen not only to target and eliminate HBsAg-positive HCC cells but also to eradicate HBV cccDNA (covalently closed circular DNA). This announcement follows SCG's previous IND approvals from China National Medical Products Administration (NMPA) in March 2022 and Singapore Health Sciences Authority (HSA) in May 2022. This further establishes SCG101 as the first TCR-T cell therapy product approved for clinical trial across the U.S, China and Singapore.
- 07.12.22 - Verve Therapeutics Doses First Human with an Investigational In Vivo Base Editing Medicine, VERVE-101, as a Potential Treatment for Heterozygous Familial Hypercholesterolemia (Press Release)
  - VERVE-101 is a novel, investigational gene editing medicine developed by Verve and designed to be a single-course treatment that permanently turns off the PCSK9 gene in the liver to reduce disease-driving low-density lipoprotein cholesterol (LDL-C). heart-1 is a global Phase 1b clinical trial that will evaluate VERVE-101 as a treatment for patients with heterozygous familial hypercholesterolemia (HeFH), a prevalent and potentially life-threatening subtype of atherosclerotic cardiovascular disease (ASCVD).

- 07.14.22 - NexImmune Announces IND Clearance by the US FDA for NEXI-003 for the Treatment of HPV-Related Cancers (Press Release)
  - The company is developing a novel approach to immunotherapy designed to orchestrate a targeted immune response by directing the function of antigen-specific T cells, has received IND clearance for the Company's first cellular therapy product candidate addressing solid tumors. NEXI-003, an autologous antigen-specific T cell product (CD3+/CD4-), is being developed for patients with relapsed or refractory human papillomavirus (HPV)-related cancers.
- 07.28.22 - ImmunityBio Announces FDA Acceptance of Biologics License Application for N-803 in BCG-Unresponsive Non-Muscle-Invasive Bladder Cancer Carcinoma In Situ (Press Release)
  - The FDA accepted for review a Biologics License Application (BLA) for its antibody cytokine fusion protein as a treatment for patients with BCG-unresponsive non-muscle-invasive bladder cancer carcinoma in situ (CIS) with or without Ta or T1 disease. ImmunityBio, a leading clinical-stage immunotherapy company, filed the BLA based on positive results from a series of studies of the investigational treatment, including the ongoing QUILT 3.032 trial. The Prescription Drug User Fee Act (PDUFA) target action date is May 23, 2023.
- 07.29.22 - Sarepta Therapeutics Announces Intent to Submit an Accelerated Approval Biologics License Application for its Gene Therapy SRP-9001 to Treat Duchenne Muscular Dystrophy (Press Release)
  - SRP-9001 was granted Fast Track designation in July 2020, an FDA process designed to facilitate the development and expedited review of drugs that treat serious conditions and fill unmet medical needs. In addition to Fast Track, SRP-9001 has also been granted Rare Pediatric Disease (RPD) designation in the United States, and Orphan Drug status in the United States, the European Union, Switzerland and Japan.
- 08.01.22 - Krystal Biotech Receives FDA Acceptance of KB407 IND Application for Cystic Fibrosis Clinical Trial (Press Release)
  - KB407 is a modified HSV-1 vector carrying two copies of the cystic fibrosis transmembrane conductance regulator (CFTR) gene to the respiratory cells in the lungs. By inducing expression of full length, normal CFTR protein in the lung, treatment with KB407 has potential to restore ion and water flow into and out of lung cells to correct the lung manifestations of the disease in patients regardless of their underlying genetic mutation.
- 08.01.22 - Gamida Cell Announces FDA Acceptance of Biologics License Application for Omidubicel with Priority Review (Press Release)
  - (FDA) has accepted for filing the Company's Biologics License Application (BLA) for omidubicel for the treatment of patients with blood cancers in need of an allogenic hematopoietic stem cell transplant. Omidubicel is a first-in-class, advanced NAM-enabled stem cell therapy candidate with breakthrough and orphan drug designations. PDUFA target action date is January 30, 2023.

- 06.13.22 - Kairos Ventures Raises $58M Fund for Early-Stage Life Sciences Startups (DOT.LA)
  - a Beverly Hills-based venture capital was founded in 2015, the venture capital firm is known for backing physical and life sciences startups in their infancy, often as soon as they leave the university research labs in which they were incubated. Kairos initially focused on research coming out of Caltech, Chief Research and Development Officer Alex Andrianopoulos told dot.LA last year, but has since widened its scope to around a dozen other universities and has invested in more than 55 companies. Kairos also provides no-strings-attached grants, typically ranging between $100,000 to $200,000, to academic researchers, founder and CEO Jim Demetraides told the Wall Street Journal. That approach "builds relationships" between Kairos and those university-stage, would-be startups once they are ready for their first rounds of venture money, Demetraides said, with the venture firm having funded around 120 research programs to date, he told the Journal.Kairos previously raised $25 million for its first fund and $85 million for its second fund, according to PitchBook Data.
- 06.15.22 - Third Rock Ventures Raises $1.1 Billion Fund VI (Press Release)
  - Third Rock Ventures today announced the closing of Third Rock Ventures Fund VI, raising $1.1 billion. Fund VI will continue Third Rock's core strategy to discover, launch and build new groundbreaking companies focused on improving the lives of patients. Third Rock employs a hands-on approach to partnering with founders to build companies and provides early and rigorous integration of science, medicine, business and strategy to build successful companies. To date, Third Rock has raised $3.8 billion and its funds have invested in 60 companies. Importantly, companies across the portfolio have brought 18 products to the market across multiple therapeutic modalities and a broad range of disease indications including cancer, cardiovascular disease, depression, sickle cell disease, and rare genetic diseases among others. Fund VI is able to provide both initial Series A as well as follow-on capital to support companies throughout their lifecycle. Furthermore, Third Rock intends to allocate capital from Fund VI to invest in groundbreaking new companies from in the broader biotech ecosystem that are strongly aligned with Third Rock's mission and strategy.
- 06.29.22 - As bear market continues to beat down biotech, ARCH closes a $3B early-stage fund (Endpts)
  - ARCH Venture Partners closed its 12th venture fund early Wednesday morning, the firm said, bringing in almost $3 billion to invest in early-stage biotechs. The move comes about a year and a half after ARCH announced its previous fund, for almost $2 billion back in January 2021. In a statement, ARCH managing director and co-founder Bob Nelsen appeared to brush off concerns about the broader market troubles, alluding to the downturn that's seen several biotechs downsize and the XBI fall back to almost pre-pandemic levels. The firm hasn't finalized plans regarding the number of companies in which it plans to invest, but Nelsen said a "good guess" would be around 20 to 25 "core" companies with a bunch of smaller seed rounds as small as $50,000 included.
- 06.30.22 - Telegraph Hill Partners Raises $525M Fifth Fund for New Life Science and Healthcare Investments (Press Release)
  - THP V, a new $525 million investment fund. The fund will make investments in innovative companies that are commercializing advanced technologies in areas including life science tools, reagents, specialty chemistries, medical technology, medical devices, diagnostics, healthcare IT, healthcare services, agriculture and animal health.