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

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

Funding Updates:

- 09.07.22 - Watch out Moderna: John Maraganore, ARCH and Beam are hatching a platform play 'to lead the future of RNA therapies' (Endpts)
  - John Maraganore is coming on board as a co-founder of Orbital Therapeutics. The Alnylam founder and former CEO told Endpoints News in an email that he started getting involved soon after becoming a venture partner at ARCH last year. Alongside Giuseppe Ciaramella, who was a driving force at Moderna from 2014, as CSO of the infectious disease division, he helped build the initial mRNA vaccine pipeline and steered Moderna's first vaccine program toward an IND. ARCH's Kristina Burow and Carol Suh are also playing a part in putting the company together, while Ciaramella brought together a quartet of star researchers to be his scientific co-founders: Stanford's Howard Chang and Ravi Majeti; Drew Weissman at the University of Pennsylvania; and Gene Yeo out at UC-San Diego.
- 09.08.22 - Innervace Secures Up to $40 Million to Advance Novel Regenerative Medicine Platform for Neurological Disorders (PR)
  - Series A financing round, led by Deerfield Management, to accelerate a new cell therapy modality for the treatment of neurological disorders, including Parkinson's Disease. Additionally, this round of funding enables licensing of a cell source with a specific (A9) dopaminergic phenotype from a research collaboration between UC San Diego and Deerfield Management. Using these cells and a biofabricated scaffold pathway reconstruction strategy differentiates Innervace's approach from current cell therapy approaches for Parkinson's.
- 09.12.22 - TScan Therapeutics Secures Convertible Debt Facility for up to $60 Million with K2 HealthVentures (PR)
  - Financing supports management's focus on clinical execution and advancing its ImmunoBank. Initial $30 million tranche extends cash runway into the second quarter of 2024.
- 09.14.22 - SparingVision Raises €75 Million Series B to Continue Building World-Leading Portfolio of Genomic Medicines for Ocular Diseases (PR)
  - The financing will be used to fund the first-in-human trials of the Company's two lead gene-independent assets, SPVN06 and SPVN20, as well as the development of genome editing assets through its collaboration with Intellia Therapeutics.The financing extends SparingVision's cash runway to the second half of 2025.
- 09.20.22 - ImmunoScape Raises $14M to Facilitate the Discovery and Characterization of Cancer-Specific T-cell Receptors (PR)
  - ImmunoScape's Deep Immunomics and machine learning platforms comprise a highly effective method for the discovery of T-cell receptor (TCR) candidates, targeting novel antigens such as cancer-specific shared splice variants and human endogenous retroviral elements. The company is poised to take a set of its proprietary TCRs into the clinic as TCR-T-cell therapies for the treatment of solid tumors. Anzu Partners led the $14M financing round with participation from Amgen Ventures and EDBI.
- 09.29.22 - iXCells Biotechnologies Secures Strategic Growth Investment from Great Point Partners (PR)
  - XCells Biotechnologies is a high growth provider of cell-based products and discovery services to the academic, biotech and pharmaceutical communities worldwide, with special focus on primary and induced pluripotent stem cell ("iPSC") derived cellular models, today announced receiving a growth investment from Connecticut-based Great Point Partners.
- 09.30.22 - EnPlusOne Biosciences Launches Enzymatic RNA Synthesis Platform with $12 Million in Seed Financing (PR)
  - RNA synthesis platform to target synthesis lengths greater than 100-nt, large-scale GMP manufacturing, and novel therapeutic modifications in collaboration with industry partners. Advanced-stage technology with over six years of development at the Wyss Institute at Harvard University, co-founded by Dr. George Church of Harvard Medical School, and the first spin-out from the Northpond Labs Alliance. Funding was led by Northpond Ventures with participation by Breakout Ventures and Coatue

Manufacturing Updates:

- 09.06.22 - Lonza, Touchlight partner on 'doggybone DNA' development (Fiercepharma)
  - CDMO giant Lonza inked a deal with Touchlight to expand production and distribution of the biotech's so-called doggybone DNA platform. As part of the collaboration, Lonza gets access to Touchlight's technology, which is a minimal, lineal, covalently closed structure that eliminates bacterial sequences and should accelerate speed and scale in manufacturing, the company said. In return, Touchlight will be able to expand the channels customers can use to access its doggybone DNA (dbDNA) platform. Financial terms of the agreement weren't disclosed.
- 09.08.22 - Orca Bio, hunting cell therapy's production white whale, tees up new commercial plant (Fiercepharma)
  - Orca, focused on development of precision cell therapies for cancer, genetic blood disorders and autoimmune diseases, has drafted plans to build a new, 100,000-square-foot commercial facility in Sacramento, California. The plant will be used to tackle late-stage development of Orca's pod of cell therapy prospects including Orca-T, the company's most advanced allogeneic candidate, which is in phase 3 testing in a trio of blood cancers. The plant will ultimately have the capacity to crank out roughly 3,000 cell therapy products per year, plus it will be equipped to scale to meet future demand.
- 09.20.22 - BioIVT Launches GMP-grade VivoSTART Leukopaks for Cell and Gene Therapy Manufacturing (Biopharmadive)
  - BioIVT, a leading provider of biospecimens, research models and services for drug and diagnostic development, today announced the launch of its GMP grade VivoSTART™ leukopaks. Leukopaks contain concentrated white blood cells and are used to research and manufacture cell and gene therapies. GMP-compliant VivoSTART leukopaks will serve as the ideal starting material for allogeneic ex vivo cell therapies and other advanced therapeutic applications.
- 09.20.22 - Center for Breakthrough Medicines (CBM) and jCyte Announce Strategic Partnership to Manufacture jCyte's Innovative Cell Therapy for Treatment of Retinal Degenerative Diseases (PR)
  - multi-year manufacturing agreement where CBM will provide Phase 3 clinical trial supplies and commercial drug product following Biologics License Application (BLA) approval of jCyte's proprietary jCell platform technology. CBM will also provide all BLA-enabling Chemistry, Manufacturing and Controls (CMC) work to support the program through regulatory approval and commercialization.
- 09.27.22 - Forge Biologics Fuels Gene Therapy Manufacturing Engine with Launch of Plasmid DNA Manufacturing Services to Support AAV Clients (PR)
  - Enables gene therapy clients to accelerate AAV manufacturing with seamless incorporation into the Company's HEK 293 platform suspension process with access to end-to-end capabilities. New offerings of Research-Grade and GMP-Pathway expedite Phase 1/2 clinical trial timelines, with GMP-Grade available in 2023.
- 09.28.22 - Vor Bio Initiates In-house Clinical Manufacturing at Cambridge, MA Headquarters (PR)
  - The company announced the opening of its new in-house clinical manufacturing facility in Cambridge, Mass. co-located in the Company's current headquarters. The new facility is designed to support Vor Bio's development of potentially transformative engineered hematopoietic stem cells (eHSCs) and CAR-T cell therapeutic candidates for patients with blood cancers. The Vor Bio facility will enable end-to-end oversight of drug product for planned clinical trials, initially manufacturing clinical supply to support the IND for VCAR33allo, which is on-track for submission in the first half of 2023

Fund Raising Updates:

- 09.12.22 - Pretzel Therapeutics Launches With $72.5 Million Series A Financing to Pioneer Mitochondrial Therapies (PR)
  - Pretzel founded by leading academic experts in mitochondrial biology and backed by world-class investor syndicate - as led by ARCH Venture Partners and Mubadala Capital with participating investors HealthCap, Cambridge Innovation Capital, Cambridge Enterprise, Angelini Ventures, GV, Invus, Eir Ventures, GU Ventures, and Karolinska Institutet Holding. Platform technologies in gene correction, genome expression modulation, and mitochondrial quality control to enable wide variety of therapeutic approaches.
- 09.12.22 - Forge Biologics Announces $90 Million Series C Financing to Expand Client Offerings and Add Services to Enhance End-to-End Gene Therapy Manufacturing Platforms (PR)
  - Total capital raised of $330 million since the Company's launch in 2020. Financing expands planned usage of Company's 20 cGMP suites containing multiple 50L, 500L, 1,000L and 5000L bioreactors for research-to-commercial gene therapy manufacturing. The current round was co-led by Drive Capital and Aisling Capital with an additional undisclosed strategic investor.
- 09.14.22 - Capstan Therapeutics Launches with $165 Million to Deliver on the Clinical Promise of Precise In Vivo Cell Engineering (PR)
  - Capstan's foundation is built on research developed by world-renowned mRNA and cell therapy scientists and clinicians at the University of Pennsylvania. Therapy development focused on cell type-specific engineering across oncology, autoimmune disease, blood disorders, and fibrosis. $165 million seed and Series A financings include significant investment from leading biopharmaceutical companies including Pfizer Ventures, Leaps by Bayer, Novartis Venture Fund, Eli Lilly and Company and Bristol Myers Squibb, as well as top life science investors including OrbiMed, RA Capital, Vida Ventures, Polaris Partners, and Alexandria Venture Investments. Laura Shawver, Ph.D., former CEO of Silverback Therapeutics and Synthorx, appointed as President and Chief Executive Officer.

- 09.15.22 - Celularity Enters Into $150 Million Pre-Paid Advance Agreement with Yorkville (PR)
  - Celularity intends to use the proceeds for working capital and other general corporate purposes. General corporate purposes may include research and development and clinical development costs to support the development of its cellular therapy candidates and the expansion of our research and development programs, as well as costs associated with its commercial biomaterials businesses; working capital; capital expenditures; and other general corporate purposes.
- 09.20.22 - ImmunoScape Raises $14M to Facilitate the Discovery and Characterization of Cancer-Specific T-cell Receptors (PR)
  - Anzu Partners led the $14M financing round with participation from Amgen Ventures and EDBI. ImmunoScape's Deep Immunomics and machine learning platforms comprise a highly effective method for the discovery of T-cell receptor (TCR) candidates, targeting novel antigens such as cancer-specific shared splice variants and human endogenous retroviral elements. The company is poised to take a set of its proprietary TCRs into the clinic as TCR-T-cell therapies for the treatment of solid tumors.
- 09.22.22 - Bayer's venture arm ramps up, seeking to invest $1.3 billion (STAT)
  - Over the last six years, a venture capital team run out of pharmaceutical giant Bayer invested roughly $1.5 billion into small biotech startups. Now, it's ramping up. The firm plans to invest a nearly identical pot of money — this time, $1.3 billion — in half the time.Bayer launched its venture arm, Leaps by Bayer, in 2015.. But more importantly, its mandate is to find biotech and agriculture startups that might one day make for interesting R&D partners, or acquisition targets.Leaps by Bayer has invested in more than 50 companies so far.
- 09.26.22 - They're going for it: David Liu's preclinical biotech shoots for a (maybe $200M) IPO (Endpts)
  - Can a preclinical biotech with grand ambitions, a star scientific founder and enthusiastic backers with deep pockets fly a big IPO against the gale force headwinds we've seen this year? In this economy? The people at Prime Medicine, which bills itself as a CRISPR 3.0 play, aim to find out if they can buck the trend — which has relented enough to allow for one upsized biotech IPO to get through — and possibly help pry open a window that was slammed shut at the beginning of the year.
- 09.27.22 - George Church's woolly mammoth revival biotech Colossal spins out computational bio outfit (Endpts)
  - Colossal Biosciences has a somewhat lofty goal — revive the woolly mammoth. When the biotech launched in 2021, its CEO Ben Lamm compared its mission to the Apollo program in an interview with CNN, equating its aspirations to space exploration but noting that it could make other breakthroughs along the way, as Apollo did with GPS. Now Colossal is sharing its version of the GPS, an AI platform that is meant to make managing massive datasets — commonplace in biotech today — more user-friendly and less code-heavy. Colossal, which was co-founded by famed geneticist George Church, has spun that project out into a new company dubbed Form Bio. To start out, Form is getting $30 million from Jazz Venture Partners in a Series A. Kent Wakeford, Colossal's COO, will be transitioning to become Form's co-CEO alongside Andrew Busey. Claire Aldridge, one of Colossal's advisors, will be doubling as the CSO. And Adam Milne will be stepping in as COO at Colossal.
- 09.28.22 - Gamida Cell Announces Entry into Commitment Letter with Highbridge for $25 Million Financing (PR)
  - Gamida Cell is pioneering a diverse immunotherapy pipeline of potentially curative cell therapy candidates for patients with solid tumor and blood cancers and other serious blood diseases. Omidubicel is an advanced cell therapy candidate developed as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplant for patients with blood cancers. Omidubicel demonstrated a statistically significant reduction in time to neutrophil engraftment in comparison to standard umbilical cord blood in an international, multi-center, randomized Phase 3 study (NCT0273029) in patients with hematologic malignancies undergoing allogeneic bone marrow transplant.

M&A Updates:

- 09.20.22 - Rocket Pharmaceuticals to Acquire Renovacor, Extending Leadership in AAV-based Cardiac Gene Therapy (PR)
  - The acquisition further strengthens Rocket's leadership in AAV-based cardiac gene therapy and expands Company's near-term clinical assets for the treatment of heart conditions. Creates strong synergies by combining key assets, personnel, capabilities and IP, as well as access to world-leading scientific and clinical collaborators. Rocket believes compelling preclinical data generated by Renovacor validates mechanism of action of AAV-based transgene replacement strategy for BAG-3 dilated cardiomyopathy. Expected to add approximately $38M in projected cash at closing; combined with recent $26M ATM sale, extends cash runway into 2Q'24
- 09.21.22 - Sesen Bio and Carisma Therapeutics Announce Merger Agreement (PR)
  - Transaction to create a well-funded, clinical-stage biotechnology company advancing engineered macrophages for the treatment of cancer and other serious disorders. Combined company is expected to have approximately $180 million of cash, cash equivalents and marketable securities at close, including $30 million from concurrent financing by Carisma, which is expected to fund the combined company through 2024. Cash runway of a combined company expected to enable multiple clinical readouts across Carisma programs

- 09.28.22 - iuvo BioScience to be Acquired by Ampersand Capital Partners (PR)
  - iuvo BioScience, a specialty CRO providing laboratory services, preclinical and clinical development services, and scientific consulting services, today announced it has signed a definitive agreement to be acquired by Ampersand Capital Partners, a private equity firm specializing in growth equity investments in the life science and healthcare sectors. Ampersand's investment will support the expansion of iuvo's integrated service portfolio and laboratory capacity, and fund an active inorganic growth strategy. As part of the transaction, iuvo also announced the appointment of Daniel Spasic as Chairman of the company's Board of Directors. Daniel is a pharmaceutical services executive with over 25 years of experience in the CRO industry. HQ in Rochchester, NY.
- 09.30.22 - Solid Biosciences Announces Acquisition of AavantiBio and Concurrent $75 Million Private Placement (PR)
  - Transactions to create a precision genetic medicine company focused on neuromuscular and cardiac rare diseases, led by industry veteran and current AavantiBio CEO, Bo Cumbo. Strong synergies expected by combining key assets, including product candidates for Duchenne muscular dystrophy, Friedreich's ataxia, BAG3 mediated dilated cardiomyopathy and other undisclosed cardiac diseases, novel capsid libraries, and personnel. The new combined company is expected to have approximately $215 million in cash and investments, which is expected to fund the combined company into 2025 and support attainment of key milestones for lead gene therapy programs

Clinical & Approval Updates:

- 09.09.22 - #ESMO22: Adaptimmune touts new Phl data for its cell therapy treatment (Endpts)
  - As the cell therapy biotech Adaptimmune has been eager to present data to progress with the FDA, the company hopes that its latest showing at ESMO will begin to impress regulators and investors. On Friday, the UK biotech presented the newest Phase I SURPASS trial data for its MAGE-A4 cell therapy program. The trial had 44 patients who received a single dose of the therapy with 43 able to be evaluated. Additionally, 25 patients had late-stage ovarian, urothelial and head and neck cancer. Here, the biotech saw 11 patients respond, good for a 44% objective response rate.
- 09.13.22 - Akouos Receives FDA Clearance of its IND Application for AK-OTOF, a Gene Therapy Intended for the Treatment of OTOF-mediated Hearing Loss (PR)
  - The IND for AK-OTOF is the first to receive FDA clearance for a genetic form of hearing loss and the first for an AAV vector therapy with the potential to treat an inner ear condition. Akouos plans to initiate a pediatric Phase 1/2 clinical trial, including children as young as two years of age in the dose-escalation phase (Part A), to evaluate AK-OTOF for the treatment of OTOF-mediated hearing loss. Based on auditory brainstem response data from nonclinical studies, a one-time administration of AK-OTOF has the potential to deliver durable restoration of auditory function
- 09.16.22 - bluebird bio Receives FDA Accelerated Approval for SKYSONA® Gene Therapy for Early, Active Cerebral Adrenoleukodystrophy (CALD) (PR)
  - SKYSONA is the first FDA approved therapy shown to slow the progression of neurologic dysfunction in boys with this devastating and fatal neurodegenerative disease. FDA) has granted Accelerated Approval of SKYSONA® (elivaldogene autotemcel), also known as eli-cel, to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD). The Company also confirmed that the previous clinical hold on the eli-cel clinical development program has been lifted.
- 09.20.22 - Verismo Therapeutics Announces FDA Clearance of IND Application for SynKIR-110™, a KIR-CAR T Cell Immunotherapy Candidate (PR)
  - a Penn-spinout behind the novel KIR-CAR platform technology, today announced that is has received clearance of its Investigational New Drug (IND) application from the U.S. Food and Drug Administration (FDA) to initiate a first-in-human Phase 1 clinical trial of SynKIR-110. The Phase 1 trial, STAR-101 (SynKIR T cell Advanced Research), will assess safety, tolerability, and preliminary efficacy of SynKIR-110 in patients with mesothelin expressing ovarian cancer, cholangiocarcinoma and mesothelioma.
- 09.20.22 - Artiva Biotherapeutics Announces FDA Allowance of IND for AB-201, a HER2-Targeted CAR-NK for the Treatment of Solid Tumors (PR)
  - (IND) application for AB-201. AB-201 is an allogeneic HER2-targeted chimeric antigen receptor NK (CAR-NK) cell therapy for the treatment of solid tumors in the outpatient setting with the option for repeat dosing. Starting in the first half of 2023, Artiva plans to conduct a clinical study of AB-201 in HER2-expressing cancer patients at multiple clinical sites in the U.S.
- 09.21.22 - Cellenkos Receives FDA Clearance of IND Application for CK0803 for the Treatment of Amyotrophic Lateral Sclerosis (ALS) (PR)
  - The company is developing transformative cellular therapeutics for treatment of inflammatory disorders and autoimmune diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application to initiate a Phase 1 safety study followed by a Phase 1b randomized, double blind, placebo control trial of CK0803, neurotrophic allogeneic Treg cells, in patients with amyotrophic lateral sclerosis (ALS)

- 09.23.22 - Pfizer, Sangamo set to resume gene therapy study after safety delay (biopharmadive)
  - Pfizer and Sangamo Therapeutics will soon resume a Phase 3 trial of their gene therapy for the blood disorder hemophilia A, nearly a year after safety concerns forced its suspension.The study was originally paused last fall due to safety concerns over blood clotting. The gene therapy, dubbed giroctocogene fitelparvovec, is meant to treat people with severe hemophilia A, who either lack or have low levels of a needed blood clotting protein.
- 09.28.22 - Verismo Therapeutics Receives U.S. FDA Orphan Drug Designation for SynKIR-110™, a First-in-Class KIR-CAR T Cell Immunotherapy Candidate, for the Treatment of Mesothelioma (PR)
  - a clinical-stage CAR-T company and Penn spinout, and pioneer of the novel KIR-CAR platform technology, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to SynKIR-110™ for the treatment of patients with mesothelin-expressing mesotheliomas.
- 09.28.22 - CRISPR Therapeutics Announces FDA RMAT Designation Granted to CTX130™ for the Treatment of Cutaneous T-Cell Lymphomas (CTCL) (PR)
  - "The RMAT designation is an important milestone for the CTX130 program that recognizes the transformative potential of our cell therapy in patients with T-cell lymphomas based upon encouraging clinical data to date," said Phuong Khanh (P.K.) Morrow, M.D., FACP, Chief Medical Officer of CRISPR Therapeutics. "We continue to work with a sense of urgency to bring our broad portfolio of allogeneic cell therapies to patients in need."
- 09.29.22 - Sarepta Therapeutics Submits Biologics License Application for SRP-9001 for the Treatment of Ambulant Patients with Duchenne Muscular Dystrophy (PR)
  - The BLA is submitted for accelerated approval based on the expression of SRP-9001 dystrophin protein, an internally shortened and functional version of dystrophin, as a surrogate endpoint reasonably likely to predict clinical benefit. Among other things, the BLA is based on positive pre-clinical, biomarker and clinical functional results. In clinical trials, SRP-9001 demonstrated positive results at multiple time points, including one-, two- and four-years after treatment, in addition to a consistent safety profile. The submitted BLA for SRP-9001 includes efficacy and safety data from Studies SRP-9001-101, SRP-9001-102, SRP-9001-103 (also known as ENDEAVOR).