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

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

Funding Updates:

- 12.01.22 - Scientists at UCSD Receive $4.8M to Pursue Gene Therapy for 'Incurable' Disease (today.ucsd)
  - CIRM grant will fund novel gene therapy aiming for one-time, lifelong treatment of Friedreich's ataxia, a progressive neuromuscular disorder; second CIRM grant will advance efforts to leverage UC San Diego research on another rare disease.
- 12.01.22 - Lundbeck-partnered preclinical biotech nabs Series A from AstraZeneca (Endpts)
  - Cambridge, MA biotech Rgenta Therapeutics has secured $52 million in a Series A to take oral small molecules into the clinic.The 20-person biotech, which is already allied with Lundbeck, looks to ink more pharma partnerships within its core areas of oncology and neuroscience rare diseases, as well as in other disease areas the startup thinks the RNA-targeting platform can go, CSO Travis Wager told Endpoints News ahead of the Tuesday morning news. The Series A comes from AstraZeneca's $1 billion fund with China International Capital Corporation Limited, or CICC Healthcare Investment Fund, as well as Korea Investment Partners, Delos Capital, Lilly Asia Venture, Vivo Capital, Matrix Partners China and others, like $20 million seed round leader Boehringer Ingelheim Venture Fund. Other backers include Kaitai Capital and Legend Star Fund.
- 12.07.22 - Vor Bio Prices Underwritten Offering of Common Stock and Concurrent Private Placement for Aggregate Proceeds of $115.8 Million (PR)
  - Vor Bio currently intends to use the net proceeds from the underwritten offering and concurrent private placement primarily to fund the continued clinical development of pipeline products and for working capital and general corporate purposes.
- 12.07.22 - ExPLoRNA Therapeutics Receives Funding to Advance its Novel mRNA Technology (PR)
  - Total funding received from the Bill & Melinda Gates Foundation is $813,578 and will be used for a 14-month project. The project aims to better understand the benefits of ExPLoRNA's cap analogs, especially in terms of reducing the mRNA dose needed for therapeutic effects in the settings of vaccination and monoclonal antibody production. The planned experiments include the use of the state-of-the-art, clinically validated lipid nanoparticle (LNP) formulations from Acuitas Therapeutics. Acuitas is known for its successful role in providing the LNP technology for one of the only two regulatory-approved mRNA-based COVID-19 vaccines.
- 12.08.22 - Cabaletta Bio Announces $35 Million Offering (PR)
  - The oversubscribed offering involved participation from new and existing investors, including Venrock Healthcare Capital Partners, Adage Capital Partners LP, Cormorant Asset Management and an undisclosed life sciences-focused investment fund, among others. Cabaletta intends to use the net proceeds from the offering, together with existing cash and cash equivalents, to fund the completion of preclinical development and the Investigational New Drug ("IND") application submission for CABA-201, the initial clinical development of CABA-201 in multiple indications and the ongoing clinical studies for its DSG3-CAART and MuSK-CAART product candidates, as well as for working capital and general corporate purposes.
- 12.08.22 - Autolus Therapeutics to Receive $70 Million in Milestone Payments from Blackstone Life Sciences (PR)
  - Development milestone of $35m achieved earlier than anticipated as a result of the positive interim analysis of Autolus' pivotal FELIX Phase 2 trial, announced in a separate press release today. Manufacturing milestone of $35m achieved as a result of completion of planned activities supporting the performance and qualification of the obecabtagene autoleucel (obe-cel) manufacturing process
- 12.08.22 - SonoThera™ Completes $60.75M Series A Funding for Ultrasound-Guided, Nonviral Gene Therapy Platform Development (PR)
  - Lead investor ARCH Venture Partners is joined by Illumina Ventures, Johnson & Johnson Innovation – JJDC, Inc., Vertex Ventures HC, Medical Excellence Capital and others in global biotechnology and pharmaceutical investment. Funding will support platform optimization, preclinical development, early manufacturing, and initial proof of concept in humans. SonoThera's technology platform and treatments aim to overcome the most significant delivery challenges in gene therapy. SonoThera's platform uses a microbubble-mediated biophysical process to non-invasively deliver nucleic acid payloads of diverse formats and sizes, selectively targeting a wide range of organs within the body.
- 12.12.22 - Gamida Cell Announces Closing of $25 Million Financing With Highbridge (PR)
  - The proceeds from the term loan, together with the net proceeds from Gamida Cell's $20 million public offering of ordinary shares announced on September 27, 2022 and its existing cash and cash equivalents and trading financial assets, are expected to (i) fund commercial readiness and initial launch activities to support launch of omidubicel, if approved; (ii) fund the continued development of its NK product pipeline, including clinical stage asset GDA-201; and (iii) be used for general corporate purposes, including general and administrative expenses and working capital.
- 12.13.22 - Flagship Pioneering Unveils Montai Health to Treat and Preempt Chronic Disease Afflicting Two Billion People Worldwide (PR)
  - Montai is building the world's first Anthromolecule™ Bioactivity Atlas founded on a privileged class of molecules with a long history of safe, chronic human consumption.Flagship has initially committed $50 million to support the development of Montai's platform and its initial pipeline of new medicines. The company also announced that Margo Georgiadis, MBA, Flagship Pioneering CEO-Partner, who previously served as the CEO of Ancestry and President of the Americas at Google, is leading Montai as CEO.
- 12.14.22 - Replay targets genetic brain disorders with new gene therapy company, Kaleibe, utilising big DNA HSV technology (Biospace)
  - Thirdproduct company from Replay expands use ofnext-generationhigh payload capacity HSV delivery vectordeveloped at the University of Pittsburgh by Kaleibe co-founder Professor Joe Glorioso into brain indications. Experts in neuroscience and neurology, Professors Richard Wade-Martins andHoward J Federoff, also co-founders of Kaleibe. Initial programs to target genetic Parkinson's disease and Friedreich's ataxia.

- 12.15.22 - Enlaza Therapeutics Launches with $61 Million Seed Financing to Advance the First Covalent Biologic Therapeutic Platform (PR)
  - The financing was led by Avalon Ventures and joined by Lightspeed Venture Partners, Frazier Life Sciences, and Samsara BioCapital. The financing will be used to further advance Enlaza's proprietary War-LockTM platform and build a pipeline of covalent biologics with an initial focus on developing novel, differentiated cancer therapeutics with targeted efficacy and low toxicity.
- 12.15.22 - Avidity Biosciences, Inc. Announces Pricing of Upsized Public Offering of Common Stock (PR)
  - Avidity intends to use the net proceeds from this offering, together with its existing cash, cash equivalents, and marketable securities: to fund the research and development of its development programs, to continue development work associated with advancing its AOC platform and for working capital and general corporate purposes. A new class of RNA therapeutics - Antibody Oligonucleotide Conjugates (AOCsTM). Avidity's proprietary AOCs.
- 12.19.22 - A2 Biotherapeutics raises $62M for solid tumor cell therapies (Endpts)
  - A2 Biotherapeutics has secured $62 million in new financing — and plans to send two new cell therapies into the clinic in the new year with it. InvestorsCasdin capital anhd Hartford Healthcare.
- 12.19.22 - Protillion Biosciences Closes $18M Series A Round (genomeweb)
  - The Burlingame, California-based company said it will use the funds to drive development of its technology for identifying and optimizing new protein therapeutics. The technology can characterize the binding affinity of around 1 million antibody variants in two-day automated experiments, according to the firm, providing antibody binding information with amino acid-level resolution at high throughput.
- 12.21.22 - Aspen Neuroscience Inks $40 Million Debt Deal with Silicon Valley Bank (PR)
  - The San Diego-based company has recently announced the achievement of key pre-clinical and corporate milestones, including raising $147.5 million in Series B financing to support the planned studies of the company's lead product candidate for Parkinson's Disease, ANPD001: a first-in-kind iPSC based autologous cell therapy. The company also recently announced the start of its trial ready patient screening cohort study for ANPD001.
- 12.21.22 - Quris reels in $9M in seed funding to support AI innovations in trials (Fiercebiotech)
  - Quris, a Boston- and Israel-based group focused on bio AI, reeled in another $9 million in seed funding, bringing its total to $37 million.The latest round of fundraising was led by SoftBank Vision Fund 2 and included GlenRock Capital, iAngels, Welltech Ventures and Richter Group, the company said in a Dec. 20 press release. The funds will be used to hire new employees and grow its bio-AI platform. Quris discovered hundreds of novel microRNA genes through analysis of the human genome. The company uses patients-on-a-chip technology to generate a proprietary data set that is automated, predictive and uses classification algorithms to identify which drug candidates will work safely in humans.

Manufacturing & CDMO Updates:

- 12.08.22 - The CDMO space does not have the capacity to meet fill-finish demand, says Lonza (BioprocessintI)
  - Pipeline expansions are placing pressure on fill-finish capabilities and have caused a surge of investment in infrastructure, says Peter Droc, head of drug product services at Lonza. Swiss contract development manufacturing organization (CDMO) Lonza has made a string of investments to bolster its fill-finish capabilities. In July 2022, the firm forked out $521 million to construct a commercial large-scale fill-finish facility at its site in Stein, Switzerland. And less than a year before this – at the same plant – it added additional drug product manufacturing capabilities to accelerate its service offerings in Basel and Visp. Additionally, in August 2021 Lonza installed an aseptic fill-finish manufacturing line at its mammalian plant located in Guangzhou, China.
- 12.13.22 - Fujifilm's Life Sciences Corporate Venture Capital Fund Invests in PhenoVista Biosciences (PR)
  - Investment will Accelerate Development of New High-content Screening Assays. PhenoVista Biosciences (PhenoVista), a contract research organization (CRO) and leading provider of both custom and off-the-shelf, imaging-based assay services based in San Diego, California, today announced a strategic investment by FUJIFILM Corporation, Tokyo. FUJIFILM Corporation's Life Sciences Corporate Venture Capital (LS-CVC) strategic investment fund is investing in PhenoVista to accelerate the development of new high-content screening (HCS) assay services using FUJIFILM iCell® differentiated induced pluripotent stem cells (iPSCs) and PhenoVista's cutting-edge imaging technologies. Launched in February 2022, LS-CVC is a newly established group within Fujifilm's Life Sciences Strategy Headquarters in Tokyo. Fujifilm is initially investing 7B¥ (about 50 million USD) to start the fund, targeting cutting-edge biotechnology primarily through partnerships with early-stage companies around the world.
- Catalent to Expand Its Biologics Analytical Services with New Facility in Durham, North Carolina (LifeSciKnowledgeHub)
  - Catalent plans to invest up to $40 million to fit out the 80,000 square-foot facility with state-of-the-art equipment and instrumentation, including automation and digitization capabilities. When complete it will provide comprehensive solutions that include bioassays, physico-chemical testing, and full product and process characterization, as well as process validation support, stability testing, in-process manufacturing and formulation analysis, and post-packaging identification. Catalent expects to complete the facility by mid-2023, which will support the hiring of over 200 scientists and technicians over the next five years.
- 12.20.22 - SCORPION BIOLOGICAL SERVICES BECOMES SCORPIUS BIOMANUFACTURING TO REFLECT EXPANSION (PR)
  - Scorpion Biological Services today announced it has changed its name to Scorpius BioManufacturing reflecting the company's recently expanded manufacturing capabilities and the grand opening of its new biomanufacturing facility in San Antonio in October. The new facility offers GCP, GLP and GMP biomanufacturing capabilities in both mammalian and microbial modalities. Suites and equipment trains are flexible, with bioreactor sizes ranging from 50 L up to 2,000 L, and best-in-class equipment installed across the facility to produce high-quality material for clients. Scorpius offers the capability to manufacture a wide range of products, including cell therapy, recombinant proteins from mammalian or microbial systems, and DNA vectors.
- 01.02.22 - Polysciences Announces New Bioprocessing Brand, Kyfora Bio (PR)
  - Building on Polysciences' extensive experience in developing and manufacturing monomers, polymers, microspheres, and specialty reagents for the medical device, diagnostic, and pharmaceutical industries, Kyfora Bio will focus on the synthesis of cationic polymers, lipids, and other materials used in nucleic acid delivery for cell and gene therapies.Based in Horsham, PA, Kyfora Bio will occupy a 45,000 square foot cGMP facility outfitted with administrative offices, R&D laboratories, manufacturing suites, and QC laboratories for chemical and biological performance testing. The new site is a short drive from Polysciences' 250,000 square foot Warrington, PA campus which serves as the company's headquarters and houses manufacturing, R&D, QA/QC, and product storage facilities.

Private Equity & VC Fund Updates:

- 12.06.22 - Avalon BioVentures Raises First Fund Dedicated to Early-Stage Biotech Investments (PR)
  - The company announced the closing of its first venture fund, ABV1, with $135 million in funding from new and existing institutional investors. Avalon BioVentures emerged from Avalon Ventures ("Avalon") shared life-science/high-tech investing funds as a life-science only fund and will continue to leverage Avalon's proven team and company accelerator (the "Accelerator") to create, fund, and lead companies developing breakthrough therapies to improve health.

Clinical and Commerical Milestones:

- 12.01.22 - SparingVision's lead asset SPVN06 clears IND application in the US for the treatment of retinitis pigmentosa (PR)
  - SparingVision set to advance into the clinic with breakthrough gene-independent approach targeting retinitis pigmentosa (RP), one of the leading causes of blindness globally. 33-patient Phase I/II clinical trial PRODYGY includes safety/tolerability, efficacy and quality of life endpoints. First safety data expected in 2023 with full read-out expected in 2025.
- 12.02.22 - Opus Genetics Receives FDA Clearance of IND Application for OPGx-001, a Gene Therapy Candidate Intended for the Treatment of Rare Inherited Retinal Disease LCA5 (PR)
  - OPGx-001 is Opus' first program to enter clinical evaluation and is designed to address vision loss due to mutations in the LCA5 gene, which causes one of the most severe forms of early-onset blinding disease Leber congenital amaurosis. Company anticipates initiating a Phase 1/2 clinical trial in early 2023 in the U.S.
- 12.06.22 - SQZ Biotechnologies Receives FDA Fast Track Designation for its eAPC Therapeutic Candidate for Treatment of HPV16+ Tumors and Presents Clinical Data for Multiple Programs at the European Society for Medical Oncology Immuno-Oncology Congress (PR)
  - Designation Represents Potential to Bring Important New Therapy to Patients Earlier. Stable Disease Observed in Two Out of Four Evaluable Patients in eAPC Phase 1/2 Trial Including a Pronounced Pharmacodynamic Response in a Patient with Prolonged Stable Disease. Interim Results from Ongoing SQZ® eAPC Phase 1/2 Trial Showed Favorable Safety Data and Investigational Therapy was Generally Well Tolerated. Median Drug Viability of Greater than 90 Percent for Both SQZ® eAPC and SQZ® APC Clinical Trials
- 12.06.22 - Sorrento Therapeutics has Received FDA Clearance to Initiate Clinical Trials with a Next Generation mRNA (STI-1557) Vaccine Against Omicron SARS-CoV-2 Virus (PR)
  - Modified mRNA sequence of the Spike protein prevents cleavage of the expressed protein, which may potentially result in a cleaner safety profile. If approved, this vaccine may provide an important alternative to the vaccines in the U.S. and enable improved access globally in countries like Mexico, Brazil, and China, where mRNA-based vaccines or vaccine boosters are not readily available. Sorrento's mRNA-based next generation vaccine platform can potentially be adapted rapidly to ever-emerging SARS-CoV-2 variants of concern and useful for other preventive and therapeutic applications to cancer, metabolic and autoimmune diseases.
- 12.06.22 - Editas Medicine Announces Positive Safety And Efficacy Data From The First Two Patients Treated In The RUBY Trial Of EDIT-301 For The Treatment Of Severe Sickle Cell Disease (PR)
  - EDIT-301 was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant. Both patients treated with EDIT-301 successfully engrafted and are free of vaso-occlusive events during the follow-up period. First patient treated has a total hemoglobin level of 16.4 g/dL and 45.4% fetal hemoglobin five months after treatment with EDIT-301
- 12.06.22 - BioCardia Announces FDA Approval of IND Application for Allogeneic NK1R+ Human Mesenchymal Stem Cells for Ischemic Heart Failure (PR)
  - BioCardia Announces FDA Approval of IND Application for Allogeneic NK1R+ Human Mesenchymal Stem Cells for Ischemic Heart Failure. Approval marks second clinical trial approved by FDA this year for Company's NK1R+ MSC platform. Allogeneic CardiALLO therapy for heart failure to complement autologous cell therapy currently enrolling in Phase III CardiAMP Heart Failure clinical trial
- 12.07.22 - NKARTA ANNOUNCES UPDATED CLINICAL DATA ON ANTI-CD19 ALLOGENEIC CAR-NK CELL THERAPY NKX019 FOR PATIENTS WITH RELAPSED OR REFRACTORY NON-HODGKIN LYMPHOMA (PR)
  - 7 of 10 patients treated with NKX019 monotherapy at 1 billion and 1.5 billion CAR NK cells per dose achieved complete response (70% CR rate).5 CRs achieved across all dose levels after a single cycle (3 weekly doses) of NKX019 monotherapy; 3 partial responses deepened to CR with additional cycles. Patients with CR observed across multiple NHL histologies, including LBCL
- 12.07.22 - First AML Patient Successfully Transplanted with Vor Bio's Investigational Trem-cel (VOR33) and Tolerated Mylotarg™ (PR)
  - Initial clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of tremtelectogene empogeditemcel or "trem-cel" (formerly VOR33) in patients with acute myeloid leukemia (AML). The data observed from the first treated patient support the potential of a trem-cel transplant to be successfully manufactured, to engraft normally, and to maintain blood counts following treatment with the CD33-targeted therapy Mylotarg. The clinical trial continues to enroll patients and additional data are expected in 2023.
- 12.07.22 - AVROBIO Announces New Positive Clinical Data and Outlines Clinical Development Plan Following Regulatory Discussions for its Gaucher Disease Gene Therapy (PR)
  - New compelling clinical data from first-ever Gaucher disease type 3 (GD3) patient and four Gaucher disease type 1 (GD1) patients dosed with investigational AVR-RD-02. Data from first pediatric GD3 patient, the more severe, progressive form of Gaucher disease, show biochemical correction and improvement in major refractory element of disease 15 months post gene therapy. Data from first four adult patients dosed in GD1 clinical trial show important reductions below baseline ERT levels in liver and spleen volume up to two years post gene therapy. Following positive feedback from FDA and MHRA, registrational, global Phase 2/3 clinical trial for GD3 planned for second half 2023. AVROBIO believes plato® gene therapy platform is late-stage ready with no major CMC changes anticipated
- 12.08.22 - REGENXBIO ANNOUNCES COMPLETION OF DOSING IN THE PHASE I/II TRIAL OF RGX-111 FOR THE TREATMENT OF SEVERE MPS I (PR)
  - RGX-111 is an investigational AAV Therapeutic for the treatment of severe MPS I that is part of REGENXBIO's clinical-stage pipeline of neurodegenerative disease programs. Expanded Cohort 2 enrollment is complete; eight patients have received RGX-111 in the trialCompany intends to manufacture commercial-scale cGMP material to support the continued development of RGX-111
- 12.09.22 - Kite and Arcellx Announce Strategic Collaboration to Co-develop and Co-commercialize Late-stage Clinical CART-ddBCMA in Multiple Myeloma (PR)
  - Global strategic collaboration to co-develop and co-commercialize Arcellx's lead late-stage product candidate, CART-ddBCMA, for the treatment of patients with relapsed or refractory multiple myeloma. Multiple myeloma is an incurable disease for most patients and the need remains for effective, safe, and broadly accessible therapies. Currently in Phase 2 clinical development, CART-ddBCMA is an investigational cell therapy product comprising autologous T cells that have been genetically modified to target multiple myeloma. CART-ddBCMA utilizes Arcellx's novel D-Domain binder. Kite and Arcellx will jointly advance the CART-ddBCMA asset.
- 12.12.22 - Orchard Therapeutics Announces Promising Early Neurocognitive Outcomes from Ongoing Proof-of-concept Study of OTL-201 in MPS-IIIA (PR)
  - All patients achieved sustained engraftment and supraphysiological SGSH enzyme levels with median 1.5 years follow-up. Four out of five patients demonstrated gain of cognitive skills in line with development in healthy children with one patient showing a marked improvement compared to disease natural history.
- 12.12.22 - Spark Therapeutics Announces Updated Phase 1/2 Study Results Supporting the Durability of Investigational Gene Therapy SPK-8011 in Patients With Hemophilia A (PR)
  - Multi-year data, with up to five years of follow up, show sustained expression of FVIII in more than 90% of participants, in addition to improved annual bleeding and infusion rates

- 12.12.22 - SwanBio Therapeutics Initiates First-in-Human Study of AAV Gene Therapy for Adrenomyeloneuropathy (PR)
  - initiation of its PROPEL clinical trial. The first-in-human trial will study the company's lead candidate, SBT101, an investigational AAV-based gene therapy intended to treat the progressive and debilitating neurodegenerative disease adrenomyeloneuropathy (AMN).
- 12.12.22 - Vertex, Moderna prepare to take their inhaled mRNA cystic fibrosis drug into the clinic (Endpts)
  - The two announced Monday morning that the first program from their collaboration would enter the clinic "in the coming weeks" after the FDA cleared its IND. Researchers will test the drug, known as VX-522, in adults with cystic fibrosis in a single-dose escalation study. Participants must also have "a CFTR genotype not [be] responsive to CFTR modulator therapy," Vertex added. Monday's news stems from an agreement Vertex and Moderna signed back in 2016, when Vertex only had two drug approvals and well before Moderna became a Covid-19 vaccine powerhouse — Endpoints News described the latter as "trendy" at the time. Moderna was promised $40 million upfront at the time and the pair extended the deal twice, in 2019 and 2020.
- 12.13.22 - Moderna vaccine succeeds in early-stage skin cancer study with Merck's Keytruda (Biopharmadive)
  - A Moderna personalized cancer vaccine combined with Merck & Co.'s immunotherapy Keytruda kept people with melanoma alive and disease free after surgery significantly longer than Keytruda alone in a mid-stage trial, the companies said Tuesday. It was the first major finding for one of Moderna's non-COVID vaccines, which was a focus for its investors before the pandemic began in 2020. The companies said they hope to get a Phase 3 trial underway in 2023 and will begin testing the combination in other types of cancer. In October, Merck paid $250 million to license the Moderna vaccine, for which it had originally signed an option in 2016. Combination therapies are one way that Merck could help stave off biosimilar competition to Keytruda when the drug's main patent expires in 2028.
- 12.14.22 - PASSAGE BIO ANNOUNCES POSITIVE INTERIM CLINICAL DATA FROM FIRST SIX PATIENTS WITH GM1 GANGLIOSIDOSIS IN IMAGINE-1 STUDY (PR)
  - new interim safety, biomarker, and clinical development results from cohorts 1-3 in the Imagine-1 clinical study. Imagine-1 is a Phase 1/2, global, open-label, dose-escalation study of the AAVhu68 gene therapy PBGM01 delivered by intra-cisterna magna (ICM) injection in four cohorts of pediatric subjects with early and late infantile GM1 Gangliosidosis (GM1). GM1 is a rare, fatal lysosomal storage disease in which mutations in the GLB1 gene result in very low activity of the enzyme beta-galactosidase (β-Gal). The interim data include six treated patients from the first three cohorts. Cohort 4 (early infantile, high dose) patients have been dosed and data is expected by mid-2023.
- 12.14.22 - Aligos Therapeutics Initiates Dosing with its Small Interfering RNA (siRNA) Drug Candidate, ALG-125755, in Subjects with Chronic Hepatitis B (PR)
  - Aligos Therapeutics, Inc. is a clinical stage biopharmaceutical company that was founded in 2018 with the mission to become a world leader in the treatment of viral infections and liver diseases. Aligos is focused on the discovery and development of targeted antiviral therapies for chronic hepatitis B (CHB) and coronaviruses as well as leveraging its expertise in liver diseases to create targeted therapeutics for nonalcoholic steatohepatitis (NASH).
- 12.16.22 - FDA Approves First Gene Therapy for the Treatment of High-Risk, Non-Muscle-Invasive Bladder Cancer (FDA)
  - Today, the U.S. Food and Drug Administration approved Adstiladrin (nadofaragene firadenovec-vncg), a non-replicating (cannot multiply in human cells) adenoviral vector based gene therapy indicated for the treatment of adult patients with high-risk Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle-invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors.
- 12.19.22 - Neurophth Receives IND Clearance from FDA for AAV-ND1 Gene Therapy of LHON (PR)
  - FDA clearance of its investigational new drug (IND) application on the in-vivo gene replacement therapy NFS-02 (rAAV2-ND1), for the treatment of Leber hereditary optic neuropathy (LHON) associated with ND1 mutation.
- 12.19.22 - Ferring grabs first gene therapy approval in bladder cancer (Pharmamanufacturing)
  - Ferring Pharmaceuticals announced that the U.S. FDA has approved its novel adenovirus vector-based gene therapy, Adstiladrin — marking the first gene therapy approval for bladder cancer. Specifically, Adstiladrin was approved for the treatment of adult patients with high-risk, Bacillus Calmette-Guérin-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors.
- 12.19.22 - FDA lifts hold on Bluebird's sickle cell gene therapy (Biopharmadive)
  - Bluebird bio can resume enrolling and treating children and adolescents with sickle cell disease after the Food and Drug Administration lifted a clinical hold on the biotechnology company's gene therapy for the blood disorder. The agency had imposed the partial study suspension in December 2021 after one trial participant developed a potentially concerning case of persistent anemia.
- 12.29.22 - Pfizer Announces Positive Top-Line Results from Phase 3 Study of Hemophilia B Gene Therapy Candidate (PR)
  - Fidanacogene elaparvovec was generally well-tolerated, with a safety profile consistent with Phase 1/2 results. Fourteen serious adverse events (SAEs) were reported in seven (16%) patients, with two assessed as related to treatment, a duodenal ulcer hemorrhage occurring in the setting of corticosteroid use, and an immune-mediated elevation of liver aminotransferase levels. No deaths, SAEs associated with infusion reactions, thrombotic events, or FIX inhibitors were reported.Fidanacogene elaparvovec is a novel, investigational vector that contains a bio-engineered adeno-associated virus (AAV) capsid (protein shell) and a high-activity human coagulation FIX gene.
- 12.29.22 - Gene Therapy for ART-SCID Shows "Amazing" Results in 10 Children (genengnews)
  - A new gene therapy has been shown to be safe and effective in 10 young children who were recently diagnosed with a rare immunodeficiency disorder. This disorder, Artemis-deficient severe combined immunodeficiency (ART-SCID), is typically treated by means of allogeneic hematopoietic-cell transplantation—that is, with a bone marrow transplant from a healthy donor, ideally a matched brother or sister. Because ART-SCID is poorly responsive to conventional treatment, scientists based at UC San Francisco (UCSF) pioneered a new therapeutic approach, one that introduces a functional gene for the Artemis protein, an endonuclease that helps repair double-strand DNA breaks and plays a crucial role in the development of B and T lymphocytes.The children in the trial—all under the age of 5—are living at home with their families, attending daycare and preschool, playing outside, and living normal lives, said Mort Cowan, MD, UCSF pediatrics professor, and the trial's lead investigator.

Collaboration Updates:

- 12.02.22 - Immunocore and Gadeta Announce Agreement to Develop First Gamma Delta TCR ImmTAC for Solid Tumors (PR)
  - Gamma delta TCRs offer potential to address large number of patients without HLA restrictions.Agreement combines Gadeta's gamma delta target and TCR identification expertise with Immunocore's TCR bispecific engineering, development and commercialization capabilities to develop gamma delta ImmTAC therapies.Immunocore has an option for an exclusive license to further research, develop and commercialize ImmTAC candidates from the collaboration.
- 12.05.22 - MaxCyte Signs Strategic Platform License with Curamys to Enable Cell & Gene Therapies for the Treatment of Rare Intractable Diseases (PR)
  - Under the terms of the agreement, Curamys obtains non-exclusive clinical and commercial rights to use MaxCyte's Flow Electroporation® technology and ExPERT™ platform. In return, MaxCyte is entitled to receive platform licensing fees and program-related revenue. Curamys, a South Korean biotechnology company that develops cell & gene therapy using cell fusion technology to treat rare intractable diseases, including Duchenne muscular dystrophy and amyotrophic lateral sclerosis, today announced the signing of a strategic platform license (SPL).

- 12.08.22 - Vertex and Entrada Therapeutics Establish Collaboration to Discover and Develop Endosomal Escape Vehicle (EEV) Therapeutics for Myotonic Dystrophy Type 1 (DM1) (PR)
  - Under the terms of the agreement, Entrada will receive an upfront payment of $224 million, as well as an equity investment of $26 million. Entrada is eligible to receive up to $485 million for the successful achievement of certain research, development, regulatory and commercial milestones, and tiered royalties on future net sales for any products that may result from this collaboration agreement. The agreement includes a four-year global research collaboration whereby Entrada will continue to advance and receive payments for certain research activities related to ENTR-701, as well as additional DM1-related research activities. Vertex will be responsible for global development, manufacturing and commercialization of ENTR-701 and any additional programs stemming from Entrada's DM1 research efforts.
- 12.08.22 - Actinium Pharmaceuticals, Inc. Announces Research Collaboration with Columbia University to Study Actimab-A in AML Patients Following Transplant of Engineered Hematopoietic Stem Cells Gene Edited to be CD33 Negative (PR)
  - collaboration builds on the groundbreaking research of Columbia University oncologist Dr. Siddhartha Mukherjee that uses gene-editing to remove the CD33 surface protein from hematopoietic stem cells. Actimab-A to be used post-transplant of these engineered stem cells to prevent relapse by selectively targeting residual CD33 positive leukemia cells while sparing the engineered stem cells. High rates of measurable residual disease negativity demonstrated by Actimab-A + CLAG-M validates merits of Actimab-A's use to prevent disease relapse post gene edited stem cell transplant
- 12.13.22 - Wave Life Sciences to receive $170M upfront in new partnership with GSK to develop a large number of candidates (Endpts)
  - The deal will have a four-year research term at the beginning. It will bring together GSK's experience in human genetics and its wider development and commercial abilities with Wave's drug discovery and development platform, PRISM. The deal also has two elements, first, a discovery collaboration, allowing GSK to potentially advance up to eight programs and Wave to advance up to three. GSK also receives the exclusive global license for WVE-006.
- 12.14.22 - Oxford Biomedica Solutions forms new partnerships with three biotechnology companies (Biospace)
  - Oxford Biomedica Solutions LLC, an AAV manufacturing and innovation company, announced today that it has signed agreements with three additional U.S. based, biotechnology companies. This places Oxford Biomedica Solutions ahead of the previously stated target of two new partners by the end of the calendar year.Under these additional agreements, Oxford Biomedica Solutions will provide its full platform offering to support the new partners' gene therapy programs, which cover a broad range of indications, including CNS, autoimmune, oncology, muscular, and rare metabolic disease. Oxford Biomedica Solutions' proven platform has demonstrated consistent bioreactor titers of E15 vg/L and achieved over 90% fully intact vector for multiple constructs.
- 12.15.22 - GenScript and Avectas aim to reduce the cost of non-viral cell engineering (PR)
  - Today, GenScript announced a partnership with Ireland-based Avectas that focuses on improving the pathway for delivery of modified DNA into a target cell. By combining Avectas' cell engineering technology and know-how with GenScript's expertise in synthetic long oligo production, the scientists aim to demonstrate a novel and efficient solution for cell therapy manufacturing — and to improve editing efficiency and cell viability over traditional delivery methods.The research teams will apply the Avectas SOLUPORE® technology to permeabilize the target cell membrane so that engineered cargoes can be delivered while retaining very high levels of cell viability and functionality. GenCRISPR™ synthetic sgRNA and Cas9 protein are then complexed into a ribonucleic protein that is co-delivered with GenExact™ ssDNA HDR templates into the cell nucleus.
- 12.15.22 - Evotec achieves further progress in neuroscience collaboration with Bristol Myers Squibb (PR)
  - The collaboration expands the portfolio by two additional drug discovery projects and has designated a target-based programme for further development, triggering payments in total of US$ 26 m to Evotec. The collaboration was initiated in December 2016 with the goal of identifying disease-modifying treatments for a broad range of neurodegenerative diseases. Currently approved drugs only offer short-term management of the patients' symptoms and there is a huge unmet medical need for treatments that slow down or reverse disease progression. The collaboration leverages Evotec's precision medicine technologies for modality-agnostic drug discovery and development. A first programme originating from the collaboration, EVT8683, was in-licensed by Bristol Myers Squibb in September 2021, following the successful filing of an IND application with the FDA.
- 12.15.22 - Genprex Strengthens Diabetes Gene Therapy Program with License of Additional Technology from University of Pittsburgh (PR)
  - Genprex, Inc. ("Genprex" or the "Company") (NASDAQ: GNPX), a clinical-stage gene therapy company focused on developing life-changing therapies for patients with cancer and diabetes, today announced it has entered into an exclusive license agreement (the Agreement) with the University of Pittsburgh, granting Genprex a worldwide, exclusive license to certain patent applications and related technology and a worldwide, non-exclusive license to use certain related know-how, all related to modulating autoimmunity in Type 1 diabetes by using gene therapy. The preclinical technology transforms macrophages enabling them to reduce autoimmune activity in Type 1 diabetes and could be complementary to the Company's existing diabetes technology.
- 12.15.22 - Ori Biotech and Inceptor Bio partner to accelerate solid tumor cell therapy access (PR)
  - Ori Biotech (Ori), a leader in cell and gene therapy (CGT) manufacturing technologies, and Inceptor Bio, a next-gen cell therapy biotechnology company, have announced a new partnership to utilize Ori Biotech's manufacturing platform technology and expertise to support initial process development through scale-up for manufacturing of Inceptor's novel CAR-M, CAR-T and CAR-NK programs. Inceptor Bio is the third partner to join Ori's LightSpeed Early Access Program (LEAP), granting pre-commercial access to Ori Biotech's fully automated CGT manufacturing platform and digital capabilities.
- 12.19.22 - Arrowhead Pharmaceuticals Announces $25 Million Milestone Payment from Amgen (PR)
  - This milestone was triggered by the first subject enrolled in Amgen's Phase 3 trial of olpasiran. Arrowhead is further eligible to receive up to an additional $535 million in aggregate development, regulatory, and sales milestone payments from Amgen and Royalty Pharma. Olpasiran is a small interfering RNA (siRNA) originally developed by Arrowhead using its proprietary Targeted RNAi Molecule, or TRiM, platform and licensed to Amgen in 2016. It is designed to lower levels of lipoprotein(a) (Lp(a)), a genetically determined risk factor for cardiovascular disease.
- 12.22.22 - Eli Lilly Expands RNA Editing Collaboration with ProQR Therapeutics (Biospace)
  - Lilly and Netherlands-based ProQR initially entered into a five-target collaboration in 2021. The partnership harnesses ProQR's proprietary Axiomer RNA editing platform to target disorders of the liver and nervous systems. The Axiomer platform enables the editing of single nucleotides within RNA in a highly-specific manner. This expanded partnership builds on the prior agreement by exploring further potential applications of Axiomer's novel RNA editing platform.

M&A Updates:

- 12.01.22 - Syncona Limited Portfolio Company Successfully Completes Tender Offer and Acquisition of Applied Genetic Technologies Corporation (PR)
  - As of November 30, 2022, the common stock of AGTC will no longer be listed for trading on the Nasdaq Global Stock Market.

- 12.01.22 - Rocket Pharmaceuticals Completes Acquisition of Renovacor (PR)
  - The acquisition provides Rocket with Renovacor's most advanced program, REN-001, an AAV-based gene therapy targeting BAG3-associated dilated cardiomyopathy (DCM), a severe form of heart failure. BAG3-DCM represents a significant unmet medical need in a patient population with rapidly progressive cardiac dysfunction in whom no treatments targeting the underlying mechanism of disease exist. Additionally, Rocket gains access to world-class scientific collaborators, a robust intellectual property portfolio and personnel with expertise in BAG3-DCM. These assets and capabilities altogether further extend Rocket's leadership position in AAV-based cardiac gene therapy and help advance the Company's goal of pursuing gene therapy cures for patients living with rare and devastating diseases.
- 12.01.22 - Lilly Completes Acquisition of Akouos Expanding Efforts to Help People with Genetic Diseases for $487M buyout expands its gene therapy scope to hearing loss (PR)
  - "Akouos brings more top-tier talent and an important pipeline to Lilly's Institute for Genetic Medicine that will further accelerate our work to advance genetic medicines for people living with difficult-to-treat diseases," said Andrew C. Adams, Ph.D., senior vice president of genetic medicine at Lilly and co-director of the Institute for Genetic Medicine. "We look forward to supporting and enabling the Akouos team to continue their ground-breaking work developing potential genetic medicines for inner ear conditions and to help fulfill the mission of making healthy hearing available to all."
- 12.05.22 - Solid Biosciences Announces Closing of Acquisition of AavantiBio and Concurrent $75 Million Private Placement (PR)
  - transactions create a precision genetic medicine company focused on neuromuscular and cardiac diseases, led by industry veteran Bo Cumbo -/ Company to leverage synergies and 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. Combined company has approximately $215 million in cash and investments, which is expected to fund operations into 2025 and support attainment of key milestones for lead gene therapy programs
- 12.06.22 - MilliporeSigma Gains Leading Perfusion Micro-Bioreactor with Erbi Biosystems Acquisition (PR)
  - Erbi's differentiated 2 ml micro-bioreactor platform technology, Breez™, enables rapid lab testing for bioprocessing scale up. Allows MilliporeSigma to offer full range of bioreactors, from 2ml to 2000L. Adds to company's expertise in monoclonal antibody (mAb) process development. Erbi Biosystems is revolutionizing cell therapy and bioprocessing process development with the Breez™, a fully automated 2 mL TruePerfusion™ bioreactor that reduces by at least 1/3 the skilled labor required. The platform offers a 90% footprint reduction using disposable, functionally closed single-use cassettes with proprietary microfluidic technology processes capable of performing activation, transduction, intensified growth, and diafiltration steps.
- 12.08.22 - Polyplus announces completion of acquisition of Xpress Biologics to expand plasmid DNA (PR)
  - Polyplus has announced the completion of an acquisition of Belgian Xpress Biologics to expand plasmid DNA technology. Xpress Biologics specializes in the production of plasmid DNA and protein using microbial expression systems. Polypus concentrates on the manufacturing of advanced therapeutic medicinal products from research to commercial grade. The acquisition will strategically expand the Polyplus plasmid DNA engineering technology and services portfolio. Polyplus said it will strengthen its position of focusing on improving gene-therapy viral vector upstream processes, economics and quality.
- 12.20.22 - Integrated DNA Technologies Acquires ArcherDX Next Generation Sequencing Research Assays from Invitae Corporation (PR)
  - Integrated DNA Technologies, Inc. (IDT) today announced it closed on the purchase of Next Generation Sequencing (NGS) research assays from Invitae Corporation (NYSE: NVTA) under the trademarked name Archer®. The integration of IDT's portfolio with the acquired NGS research assays—which have been foundational in researching novel cancer fusions—will empower labs with an all-in-one solution to uncover biomarkers and advance cancer discoveries. The transaction enables IDT to expand its existing operations, build upon the legacy Archer portfolio, and welcome more than 100 new associates globally.
- 12.20.22 - Kite to Acquire Tmunity Therapeutics to Pursue Next Generation CAR T-Cell Therapy Advancements in Cancer (PR)
  - Acquisition Complements Kite's Existing In-House Research Capabilities. Provides Kite with Rapid Manufacturing Processes, 'Armored' CAR T Technology Platform, and Access to Certain Future Innovations Through a Sponsored Research and License Agreement with the University of Pennsylvania Cell Therapy Researchers Who Founded Tmunity to Consult as Senior Scientific Advisors for Kite. The acquisition will provide Kite with pre-clinical and clinical programs, including an 'armored' CAR T technology platform, which potentially could be applied to a variety of CAR T's to enhance anti-tumor activity, as well as rapid manufacturing processes. Tmunity's prostate-specific membrane antigen (PSMA) and prostate stem cell antigen (PSCA) assets are not part of the Kite acquisition and will be spun-out by Tmunity as part of the transaction.
- 12.29.22 - Sesen Bio and Carisma Therapeutics Announce Substantial Increase to Expected Special Cash Dividend in Connection with Pending Merger (PR)
  - Sesen Bio Stockholders Expected to Receive Approximately $70 Million Special Cash Dividend, an Increase from up to $25 Million Previously Announced. Contingent Value Right to Include Proceeds of Vicineum and Other Preclinical Assets, in Addition to Previously Announced Proceeds of Roche Agreement. The merger and related financing are expected to close in the first quarter of 2023, subject to approval by Sesen Bio stockholders and other customary closing conditions.
- 12.30.22 - Prenetics Announces Acquisition of ACT Genomics, Aiming for the Multi-billion Market of Precision Oncology (InsidePrecisionMedicine)
  - Primera Therapeutics, the first launch from cell and gene therapy (CGT) accelerator Mayflower BioVentures, announced it has signed a collaborative agreement with Cellectis that will see the two companies will work to develop gene editing methods for mutations in mitochondrial DNA (mtDNA) as potential in vivo therapies to treat mitochondrial disease. Under the terms of the agreement, Primera has the exclusive option rights to license up to five therapeutic candidates developed as part of the Cellectis collaboration. Upon exercise of the option, Cellectis will be eligible to receive up to $750 million in development and sales milestones. Additionally, it would receive high single-digit royalty payments on the net sales of ensuing products.

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