PROJECT EVOLUTION

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

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

Funding Updates:

- 11.03.22 - Kura Oncology Announces Financing Transactions with Bristol Myers Squibb and Hercules Capital, Providing Access to up to $150 Million (PR)
  - $25 million equity investment from Bristol Myers Squibb (NYSE: BMY) and a term loan facility with access to up to $125 million from Hercules Capital, Inc. (NYSE: HTGC). If the term loan is fully drawn, proceeds from these two transactions together with existing cash are expected to fund Kura's current operating plan into 2026.
- 11.07.22 - LevitasBio Completes $35M Series C Financing (PR)
  - The funding allows LevitasBio to accelerate its commercial success through the development of next generation high-throughput instruments for sample processing and analysis. For the first time ever, researchers using LevitasBio's groundbreaking technology can examine, analyze and understand true biological signatures without altering, stressing, or damaging cells—all while preserving the original samples. This new round of funding was led by Novalis Lifesciences with the participation of Pavilion Capital—a unit of Temasek Holdings Pte, an industry-leading strategic investor, and was supported by existing investor Decheng Capital.
- 11.08.22 - Juvena Therapeutics Secures $41 Million to Accelerate the Discovery and Development of Biologics for Chronic and Age-Related Diseases (PR)
  - Juvena, a biotechnology company scaling a computational platform to map the therapeutic potential of secreted proteins, announced today that it has raised $41 million in an oversubscribed Series A, bringing its total funding to $50 million. The new funding will power the expansion of Juvena's drug discovery platform and advance the company's biologics pipeline targeting chronic and age-related diseases. The Series A was co-led by Mubadala Capital and Horizons Ventures, with participation from Bison Ventures, Manta Ray Ventures, IRONGREY, Alumni Ventures, Plum Alley, Jeff Dean (SVP, Google Research and Health), Transform VC, Karl Pfleger, BoxOne Ventures, Intersect VC, Compound, Felicis, and other investors.
- 11.16.22 - SeQure Dx Emerges From Stealth Mode to Fulfill the Promise of On-Target Gene Editing Therapies for Biopharma Partners, Physicians, and Patients (PR)
  - SeQure Dx, a cutting-edge gene editing diagnostics company, has announced its emergence from stealth mode with an aim to partner with biopharma companies developing gene editing therapeutics. The company is armed with technology discovered and developed by the Joung Lab at Massachusetts General Hospital and Harvard Medical School and funding from a $17.5M Series A raised in 2021. SeQure Dx's platform comprises the only scalable, population-based, and editing technology-agnostic in vitro off-target evaluation method complemented by orthogonal methods required for off-target risk validation.
- 11.21.22 - Replay reveals second gene therapy spinout, debuting new biotech Telaria (Biopharmadive)
  - Biotechnology company Replay has launched another spinout built around its gene therapy technology, revealing Monday a new startup focused on rare skin diseases. Called Telaria, the startup is the second of five planned spinouts from what Replay describes as a "hub-and-spoke" model for sharing technology across multiple companies working on different diseases. In October, Replay debuted Eudora, which will focus on diseases of the eye. Like Eudora, Telaria will use Replay's herpes simplex viral vector to develop gene therapies. But its focus will be on a genetic skin disorder called recessive dystrophic epidermolysis bullosa, which causes fragile skin and leads to severe blistering and open wounds. There is currently no approved treatment, other biotechs such as Krystal Biotech and Abeona are developing R&D in the area. According to a Replay spokesperson, Telaria expects to begin clinical testing within two and a half years.
- 11.21.22 - Tenaya Therapeutics Announces Closing of $75M Public Offering (Biospace)
- 11.17.22 - Rezo, Founded by Renowned UCSF Scientists, Launches with $78 Million Series A to Advance Pipeline of Precision Therapeutics Based on Groundbreaking Integrated Disease Network Mapping Platform (PR)
  - Rezo's Sequence to Systems to Drugs (SSD) platform integrates data from proteomics, genetics, structural biology, and chemical biology approaches using sophisticated computational methods to create comprehensive maps of molecular disease networks. The platform enables the rapid identification and study of disease-causing protein and genetic interactions, which would be difficult to discover using traditional, often siloed, scientific methods. Rezo's disease-specific maps are designed to pinpoint novel, druggable targets that can be attacked with a range of treatment modalities. The Series A financing was led by SR One, a16z Bio + Health, and Norwest Venture Partners, and also included SV Angel, Liquid 2 Ventures, and Hawktail. Rezo's platform is based on technology from the Quantitative Biosciences Institute (QBI) at the University of California, San Francisco (UCSF) developed by Nevan Krogan, Ph.D., director of QBI and a co-founder and chief executive officer of Rezo.
- 11.17.22 - $20 Million Gift to UC San Diego Funds Research to Reverse Glaucoma (today.ucsd)
  - Researchers at the University of California San Diego may soon be closer to finding ways to reverse the effects of glaucoma, thanks to $20 million in support from Hanna and Mark Gleiberman. The gift will establish the Hanna and Mark Gleiberman Center for Glaucoma Research at UC San Diego. The center will be housed within the Viterbi Family Vision Research Center at UC San Diego. The funds are designated to stimulate research on advanced glaucoma, with the ultimate goal of protecting and restoring the vision of those who suffer from the condition.As someone who was diagnosed with glaucoma, Mark Gleiberman understands the need and urgency to find a cure.

- 11.21.22 - FogPharma hauls in $178M series D, the company's 2nd consecutive 9-digit financing round (Fiercebiotech)
  - Coming off a $107 million series C round in March 2021, FogPharma spirits were high and aspirations for its "universal druggability" platform were through the roof. FogPharma has raised $178 million in a series D round backed in part by Arch Venture Partners, the company announced Monday. The latest proceeds will be used to continue advancing the company's preclinical pipeline now at least six assets deep. The company plans to enter phase 1 trials with its lead asset, FOG-001, a beta-catenin inhibitor, in mid-2023. In addition to Arch, the full team of investors signing onto the latest round includes Milky Way Investments and Fidelity Management & Research Company. Additionally, Altos Lab founder and chief scientist Rick Klausner, M.D., is joining the company's board.
- 11.24.22 - MIT researchers reveal DNA "Paste" tech behind latest gene editing startup (Endpts)
  - In a paper published Thursday in Nature Biotechnology, MIT fellows Omar Abudayyeh, Jonathan Gootenberg and colleagues detail a technology they call PASTE, which they say can potentially be used to insert long strands of DNA and treat genetic diseases caused by many different mutations, such as cystic fibrosis and Leber congenital amaurosis, a rare eye disorder that causes blindness. The technology has been licensed to Tome Biosciences — a biotech co-founded by the duo back in February of 2021 and backed by ARCH, Google's venture arm, a16z, Longwood Fund, Polaris Partners and Alexandria Venture, which joined after its Series A, according to a recent pitch deck obtained by Endpoints News. According to an April SEC filing, Sana Biotechnology also has a stake in the company. Abudayyeh and Gootenberg declined to comment on Tome. The Watertown, MA-based biotech is led by CEO Rahul Kakkar and has more than 80 full-time employees as of the third quarter of this year, according to the pitch deck slides.
- 11.28.22 - Strand Therapeutics Announces Series A1 Bringing Total Round to $97M (PR)
  - Strand Therapeutics, the programmable mRNA company developing curative therapies for cancer and other diseases, today announced it has added an additional $45M to its Series A financing round, bringing the total amount raised in the Series A to $97 million. New investor FPV led the round, with participation from Eli Lilly, Potentum Partners, and existing investors Playground Global, and a further unannounced syndicate.The funding will be used to advance Strand's first drug candidate, a programmable mRNA therapy for solid tumor immuno-oncology into Phase 1 clinical trials next year. The funds will also be used to further develop its systemic delivery mechanism, which is designed to deliver tumor microenvironment-modifying mRNA to tumor sites and immune cells. To support these efforts, Strand will expand its multidisciplinary team across biology, bioengineering, bioinformatics, manufacturing, automation, and various G&A functions.
- 11.29.22 - Cajal Neuroscience Launches with $96 Million Series A to Transform Target and Drug Discovery in Neurodegeneration (PR)
  - Inspired by the pioneering work of Santiago Ramón y Cajal, whose discoveries on the structural and functional organization of the brain became the foundation of modern neuroscience, Cajal seeks to transform the field of neurodegeneration by revealing the complex and dynamic mechanisms driving disease. Cajal's platform combines state-of-the-art approaches and technologies, including integrative human genetics and multi-omics, highly multiplexed functional genomics and industrialized whole brain imaging. Through this approach, Cajal is systematically validating the thousands of targets implicated in diseases such as Parkinson's and Alzheimer's and creating a comprehensive understanding of neurodegeneration that reveals how, where and when different mechanisms contribute to disease.
- 11.30.22 - Jim Wilson biotech iECURE gets fresh $65M to push pediatric liver disease gene therapy into the clinic (Endpts)
  - Jim Wilson-founded biotech iECURE has wrapped a $65M Series A extension round to get its lead candidate — a gene replacement therapy for a rare inherited liver disease known as ornithine transcarbamylase deficiency, or OTC — into the clinic. This round was co-led by Novo Holdings and LYFE Capital, followed by initial investors Versant and OrbiMed as well. In September 2021, iECURE raised a $50 million Series A led by the latter two. The new cash infusion will get iECURE through an initial in-human trial, which CEO Joe Truitt told Endpoints News iECURE hopes to read out in 2024. But before then, iECURE has a number of items it has to check off first. Truitt says the biotech is planning to double its staff, which currently stands at 12 full-time members, including hiring a CMO, to prepare for clinical trials. In addition, while it's completed the pre-IND work, iECURE has to file its IND with the FDA to start the trial, which Truitt projected would happen next year, with the trial starting in late 2023.
- 11.30.22 - Scientists Receive $4.8M to Pursue Gene Therapy for 'Incurable' Disease (PR)
  - Friedreich's ataxia (FA) is an inherited, degenerative neuromuscular disorder that initially impairs motor function, such as gait and coordination, but can lead to scoliosis, heart disease, vision loss and diabetes. Cognitive function is not affected. One in 50,000 Americans has FA. On November 29, 2022, the California Institute for Regenerative Medicine (CIRM) awarded Cherqui and colleagues a grant of $4.8 million to move this approach closer to clinical trials. The funding will be used to develop a therapy based on gene-edited hematopoietic stem and progenitor cells derived from FA patients, which would be re-infused as a one-time, lifelong treatment.

Manufacturing Updates:

- 11.03.22 - ElevateBio BaseCamp Unveils its LentiPeakTM Lentiviral Vector Platform (PR)
  - ElevateBio BaseCamp's proprietary LentiPeakTM lentiviral vector platform. LentiPeak is a serum-free, suspension-based, scalable production platform that has demonstrated high volumetric productivity of therapeutically relevant vector yields that meet regulatory guidelines. LentiPeak will enable efficient transition for cell and gene therapies from preclinical stage through clinical development and commercialization with accelerated timelines and reduced manufacturing costs.
- 11.07.22 - Resolution Therapeutics and CCRM sign collaboration agreement to develop and scale up manufacturing for induced pluripotent stem cell (iPSC)-derived macrophage cell therapies (PR)
  - "The project with Resolution is being executed by OmniaBio, leveraging the technical skills within the teams at CCRM and OmniaBio," explains Mitchel Sivilotti, CEO of OmniaBio Inc. "The launch of OmniaBio creates end-to-end support for companies like Resolution who need to move from process development to clinical-stage manufacturing and eventually commercial-scale manufacturing. OmniaBio has the expertise, infrastructure and a strong focus on cell and gene therapies to deliver impact on this project and eventually to patients."

- 11.07.22 - Charles River Announces Cell Therapy Manufacturing Capacity Expansion, Reinforcing Commitment to CDMO Clients (PR)
  - Expansion of its cell therapy contract development and manufacturing (CDMO) facility in Memphis, Tenn. The expanded space is suitable for clinical and commercial cell therapy manufacturing, with an additional nine (9) state-of-the-art processing suites, adding to an existing 16 cleanrooms.
- 11.10.22 - Cellipont Bioservices Welcomes Area Dignitaries to Celebrate Groundbreaking for New Commercial-Ready Cell Therapy Facility in The Woodlands, Texas (PR)
  - The new facility will be commissioned in phases, with the first phase scheduled to begin operation in the first half of 2023. Cellipont is currently headquartered in Poway, California, near San Diego. The company plans to make The Woodlands its headquarters location after completion.
- 11.16.22 - Thermo Fisher Scientific Introduces All-in-One AAV Production System for Scalable Gene Therapy Workflows and Commercial Applications (PR)
  - Thermo introduced the Gibco CTS AAV-MAX Helper-Free AAV Production System, a new all-in-one solution designed to help meet clinical and commercial demands for cost-effective and scalable development of adeno-associated virus (AAV)-based gene therapies. To date, it is the only product of its kind that is manufactured under cGMP conditions to enable large-scale applications. AAV is used in 82% of viral vector-based gene therapies in the pipeline, and scaled production is critical to accelerate the transition from research to commercialization to bring gene therapies to market faster and at lower costs
- 11.16.22 - CellVax Selects Theragent as Manufacturing Partner for Phase 2 Clinical Trial for Prostate Cancer Immunotherapy (PR)
  - Theragent and CellVax have started preparations for the manufacturing partnership, including project planning and analytical method tech transfer. Manufacturing services will commence in late 2022, for which Theragent has agreed to supply autologous cell-derived doses to support CellVax's early and pivotal trials in the U.S. FK-PC101 is a cell-based autologous cancer vaccine for prostate cancer patients who have a high risk of recurrence after prostatectomy.
- 11.22.22 - Fujifilm Invests $188M in New Cell Culture Media Manufacturing Facility (ContractPharma)
  - Fujifilm Corporation announced a $188 million investment to establish a cell culture media manufacturing facility in Research Triangle Park (RTP), NC. The new site will be operated by Fujifilm Irvine Scientific, Inc., a subsidiary of Fujifilm Corporation, and a leader in the development and manufacture of advanced cell culture solutions for life science research, bioproduction, cell therapy manufacturing, and medical applications. The new facility is planned to ensure that Fujifilm Irvine Scientific can meet increasing market demands for high quality cell culture media solutions. The state-of-the-art manufacturing facility will be over 250,000 square feet and located across 64 acres in RTP. The site will support cGMP manufacturing of animal component-free, dry powder, and liquid media, adding additional production capacity for Fujifilm Irvine Scientific of 800,000 kg/year for dry powder, 3,300,000 L/year for liquid, and 40,000 L/day of Water for Injection (WFI).
- 11.29.22 - Resilience purchases Ohio biomanufacturing site from AstraZeneca (Endpts)
  - The facility is based in the town of West Chester, OH, just north of Cincinnati. Resilience will produce "select" AstraZeneca medicines at the facility as part of the supply agreement. The financial terms of the agreement weren't disclosed, but the deal is expected to close early next year. By acquiring the West Chester plant, Resilience will get all the site's physical assets, retain its leadership and around 500 employees, and plan to invest in the workforce and the wider facility. The site itself is around 580,000 square feet and is equipped with many manufacturing capabilities, including aseptic filling, inspection, packaging, cold-chain operations, autoinjectors, and a virtual reality training center.The addition of the West Chester site now gives Resilience close to two million square feet of biomanufacturing space.
- 11.29.22 - Eikonoklastes Therapeutics and Forge Biologics Announce Viral Vector Contract Development and AAV Manufacturing Partnership (PR)
  - Forge will provide adeno-associated viral (AAV) process development and manufacturing services for ET-101. Eikonoklastes will utilize Forge's platform process including its proprietary HEK 293 suspension Ignition Cells™ and pEMBR™ adenovirus helper plasmid. All development and AAV manufacturing activities will occur at the Hearth, Forge's 200,000 square foot gene therapy facility in Columbus, Ohio.
- 11.30.22 - Wheeler Bio Licenses ATUM's Proprietary Leap-In Transposase Technology (Readmagazine)
  - Wheeler Bio, Inc., a contract development and manufacturing organization (CDMO) specializing in the development and manufacturing of mammalian-expressed protein therapeutics, today announced it has been granted a license to ATUM's proprietary Leap-In Transposase® and miCHO cell line development technology. The Leap-In Transposase® technology is leading the industry as an incredibly efficient gene delivery system utilizing transposon-based vectors to integrate intact DNA constructs into transcriptionally active loci of the host cell genome. When used in a cell line development program, the Leap-In Transposase® technology delivers highly productive, incredibly stable and robust cell lines – including at the stable pool stage.

Clinical and Commerical Milestones:

- .11.02.22 - Omega Therapeutics Receives Orphan Drug Designation for OTX-2002 for the Treatment of Hepatocellular Carcinoma (PR)
  - Omega Therapeutics, Inc., a clinical-stage biotechnology company pioneering the first systematic approach to use mRNA therapeutics as a new class of programmable epigenetic medicines, today announced that the U.S. FDA had granted Orphan Drug Designation for OTX-2002, OTX-2002 is a first-in-class Omega Epigenomic Controller™ in development for the treatment of hepatocellular carcinoma (HCC). OTX-2002 is an mRNA therapeutic delivered via lipid nanoparticles (LNPs) and is designed to downregulate MYC expression pre-transcriptionally through epigenetic modulation while potentially overcoming MYC autoregulation. for the treatment of HCC.
- 11.03.22 - Creative Medical Technology Holdings Announces FDA Clearance of Investigational New Drug (IND) Application for AlloStem™, a Novel Cell Therapy for the Treatment of Type 1 Diabetes (PR)
  - AlloStem™ leverages a unique approach to harnessing the power of Perinatal Tissue Derived Cells® (PRDC) to multi-potentialities, including self-renewal ability, low antigenicity, reduced toxicity, and large-scale clinical expansion. The primary objective of the study (CELZ-201) is to evaluate AlloStem™ in patients with newly diagnosed Type 1 Diabetes. Patient recruitment is expected to begin in Q1 2023 with trial commencement updates to follow.

- 11.07.22 - BioMarin Announces Incremental Progress on Biologics License Application (BLA) Review for Valoctocogene Roxaparvovec AAV Gene Therapy for Adults with Severe Hemophilia A Program (PR)
  - Today a progress update on its Biologics License Application (BLA) for valoctocogene roxaparvovec AAV gene therapy for adults with severe Hemophilia A that is currently under review by the U.S. Food and Drug Administration (FDA). As part of their review of the BLA, the FDA has scheduled its Pre-Licensure Inspection (PLI) of BioMarin's gene therapy manufacturing facility, located in Novato, CA. As anticipated, the FDA also has requested that the Company submit results from the upcoming three-year data analysis from the ongoing Phase 3 GENEr8-1 Study. While the FDA did not communicate a change to the current PDUFA target action date of March 31, 2023, the Agency stated that submission of these results may qualify as a Major Amendment, which would extend the action date by 3 months. FDA will evaluate the additional data prior to making this determination.
- 11.07.22 - GDA-501, NAM enabled NK Cell Therapy, Demonstrates Promising Antitumor Activity Against HER2+ Cancers (PR)
  - Preclinical data presented at Society of Immunotherapy of Cancer's 37th Annual Meeting shows proprietary NAM technology expands NK cells, enhances functionality, increases antitumor activity, and improves homing to targeted cancer cells. Data demonstrates that genetically modified NK cell GDA-501 enhances potency, persistence and cytotoxicity against cancer cells expressing HER2
- 11.10.22 - PACT Pharma Reports Data From First Clinical Study Using CRISPR to Substitute a Gene in Patients' Immune Cells to Treat Cancer (PR)
  - PACT Pharma, Inc., a privately held biopharmaceutical company developing transformational personalized neoantigen-specific and off-the-shelf T cell receptor (TCR)-T cell therapies for the eradication of solid tumors, today reported data from the first clinical study using CRISPR gene editing technology to substitute a gene in patients' immune cells to treat cancer. Results from the study, which was conducted with collaborators at nine academic centers using PACT's proprietary platforms, demonstrated early proof-of-concept that a patient's immune system can be reprogrammed to recognize their own cancer.
- 11.11.22 - Kyverna Therapeutics Announces FDA Clearance of IND for KYV-101, a Novel Fully Human CD19 CAR T-Cell Therapy to Treat Lupus Nephritis (PR)
  - KYV-101 is an autologous version of a novel, fully human clinical-stage anti-CD19 chimeric antigen receptor T-cell (CAR T) construct with properties well suited for use in B cell-driven autoimmune diseases such as lupus nephritis and other B-cell driven autoimmune diseases. In a 20-patient Phase 1/2 study in oncology, expected anti-lymphoma activity was associated with a significant reduction of cytokines released that translated into a strong reduction of cytokine-driven side effects such as the rate of immune effector cells-associated neurotoxicity syndrome (ICANS).
- 11.14.22 - Legend Biotech small cell lung cancer drug gets FDA ok for clinical development (Labiotech.eu)
  - LB2102 is designed to selectively target delta-like ligand 3 (DLL-3), which is highly restricted to various malignancies, including SCLC, large cell neuroendocrine carcinoma (LCNEC), certain other neuroendocrine tumors and some prostate cancers. DLL-3 has also been linked to tumor growth, migration and invasion. The phase 1, first-in-human, open-label clinical study is designed to evaluate the safety and preliminary efficacy of LB2102 in subjects with extensive stage SCLC and patients with LCNEC, as well as to determine the recommended dose for phase 2.
- 11.14.22 - Beam Therapeutics Enrolls First Patient in BEACON Clinical Trial of BEAM-101 Base Editing Therapy Candidate for the Treatment of Sickle Cell Disease (PR)
  - the first patient has been enrolled in the company's BEACON trial. BEACON is an open-label, single-arm, multicenter, Phase 1/2 clinical trial designed to evaluate the safety and efficacy of BEAM-101 in adult patients with severe sickle cell disease (SCD). BEAM-101 is a patient-specific, autologous hematopoietic stem cell (HSC) investigational therapy, which incorporates base edits that are designed to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin.
- 11.17.22 - Iveric Bio Announces FDA Has Granted Breakthrough Therapy Designation for Avacincaptad Pegol for Geographic Atrophy (PR)
  - FDA) has granted Breakthrough Therapy designation for avacincaptad pegol (ACP, also known as Zimura®), a novel investigational complement C5 inhibitor for the treatment of geographic atrophy (GA) secondary to Age-Related Macular Degeneration (AMD). To date, ACP is the first and only investigational therapy to receive Breakthrough Therapy designation status for this indication, which was granted based on the 12-month pre-specified primary endpoint data in the GATHER1 and GATHER2 pivotal clinical trials.
- 11.21.22 - Caribou Biosciences Announces FDA Clearance of IND Application for CB-011, an Allogeneic Anti-BCMA CAR-T Cell Therapy for the Treatment of Relapsed or Refractory Multiple Myeloma (PR)
  - IND application from the U.S. Food and Drug Administration (FDA) for CB-011, a genome-edited allogeneic anti-BCMA CAR-T cell therapy with immune cloaking. The CaMMouflage Phase 1 clinical trial, a multicenter, open-label study to evaluate the safety and efficacy of a single dose of CB-011 in adult patients with relapsed or refractory multiple myeloma (r/r MM), is expected to initiate patient enrollment for treatment at dose level 1 (50x106 CAR-T cells) in early 2023.
- 11.21.22 - Neurona Therapeutics Presents Encouraging Clinical Data from First-Ever Regenerative Human Cell Therapy Trial in Adults with Drug-Resistant Focal Epilepsy and Shows Supporting Manufacturing, Delivery, and Preclinical Data (PR)
  - In addition to the preclinical efficacy and NRTX-1001 characterization data supporting the clinical program, the presentation highlighted data from the first two adult patients with mesial temporal lobe epilepsy (MTLE) treated in Neurona's clinical trial. The first patient had a 9-year history of seizures and in the six months prior to the administration of NRTX-1001, the patient experienced an average of 32 seizures per month, despite being on several antiepileptic medications. The patient received a single administration of NRTX-1001, the treatment was well tolerated, and there have been no serious or severe adverse events associated with the treatment to date. The patient reported four seizures during the first three months since receiving NRTX-1001. The second patient treated in the trial also had drug-resistant seizures, with an average of 14 seizures per month in the six months before treatment. This individual received NRTX-1001, returned home as planned the day following treatment and, in the first week post-treatment, had not experienced any serious or severe adverse events, or seizures.

- 11.22.22 - uniQure announces FDA approval of first gene therapy for adults with hemophilia B (PR)
  - The product is approved for the treatment of adults with hemophilia B who currently use factor IX prophylaxis therapy or have current or historical life-threatening hemorrhage or have repeated, serious spontaneous bleeding episodes. CSL licensed the exclusive global rights to HEMGENIX from uniQure in May 2021 and is now solely responsible for the further development, registration, and commercialization of the therapy. Australian drugmaker CSL, which licensed Hemgenix from UniQure and will market the drug, set the treatment's list price at $3.5 million, making it the most expensive medicine in the U.S. on a single-use basis. (Biopharmadive)
- 11.23.22 - On a roll, Takeda's dengue vaccine shifts into FDA's fast review lane (Fiercepharma)
  - Three months after gaining its first worldwide approval, from Indonesia, for its dengue fever vaccine, Takeda is progressing toward a blessing from the FDA. The Japanese company's Qdenga (TAK-003) shot has received a priority review designation for its biologics license application. It will be evaluated for its ability to prevent dengue disease from any of the fever's four serotypes in individuals ages 4 through 60. Developing a dengue vaccine has been a decade-long pursuit for Takeda, which projects peak revenue potential to come in at between $700 million and $1.6 billion. Dengue has appeared in more than 125 countries and is one of the primary causes of hospitalization in children in many parts of the world. Sanofi was first with a vaccine for dengue. But its launch of Dengvaxia in the Philippines was derailed in 2017 when the company revealed that the shot could cause more serious dengue infection if given to patients who had never contracted the virus.
- 11.28.22 - LINEAGE ANNOUNCES LAUNCH OF PHASE 2A STUDY BY GENENTECH OF RG6501 (OPREGEN®) IN PATIENTS WITH GEOGRAPHIC ATROPHY SECONDARY TO AGE-RELATED MACULAR DEGENERATION (PR)
  - Announced today that its partner Genentech, a member of the Roche Group, has launched a Phase 2a, multicenter, open-label, single-arm clinical study of RG6501 (OpRegen), a retinal pigment epithelial cell therapy. The study is intended to optimize subretinal surgical delivery and evaluate the safety and activity of OpRegen in approximately 30, and up to 60 patients with geographic atrophy (GA) secondary to age-related macular degeneration. The primary objectives of the study are to evaluate (i) the proportion of patients with subretinal surgical delivery of OpRegen to target regions under the retina, and (ii) to evaluate the safety of subretinal surgical delivery of OpRegen as measured by the incidence and severity of procedure-related adverse events at 3 months following surgery. A key secondary objective is to evaluate the proportion of patients with qualitative improvement in retinal structure, as determined by Optical Coherence Tomography (SD-OCT) imaging, within 3 months following surgery. RG6501 (OpRegen) is currently being developed under an exclusive worldwide collaboration between Lineage, Roche and Genentech.
- 11.28.22 - Tenaya Therapeutics Receives Orphan Drug Designation from the U.S. Food and Drug Administration for its Gene Therapy for Genetic Arrhythmogenic Right Ventricular Cardiomyopathy (PR)
  - TN-401 is an adeno-associated virus (AAV)-based gene therapy being developed for the treatment of genetic ARVC caused by Plakophilin-2 (PKP2) gene mutations. Mutations of the PKP2 gene can cause severe disease, including enlargement of the right ventricle in affected individuals, cardiac dysfunction, significant arrhythmia and sudden cardiac death in adults and children.
- 11.28.22 - Sarepta announced that the U.S. Food and Drug Administration accepted for filing and priority review the BLA for SRP-9001. (PR)
  - Investigational gene therapy for the treatment of ambulant individuals living with Duchenne muscular dystrophy. SRP-9001 is being developed in partnership with Roche. SRP-9001 would be the first gene therapy for Duchenne, a one-time treatment designed to treat the underlying cause of DMD by delivering a functional shortened dystrophin to muscle. Regulatory action date of May 29, 2023
- 11.28.22 - Axsome's Positive Alzheimer's Agitation Data Propel FDA Talks (Biospace)
  - A Phase III trial of Axsome Therapeutics' oral N-methyl D-aspartate (NMDA) receptor antagonist hit both primary and secondary endpoints in patients with Alzheimer's disease agitation, a condition for which there is currently no approved treatment. In the ACCORD study, AXS-05 showed "substantially and statistically significantly" delayed time to relapse and prevented relapse of agitation in patients with Alzheimer's disease, Axsome announced Monday.
- 11.29.22 - Allogene Therapeutics R&D Showcase Features Hematologic and Solid Tumor Advances Across its AlloCAR T™ Platform (PR)
  - R&D Showcase providing an extensive overview of pipeline advances from three clinical stage AlloCAR T programs. The event also included panel discussions with leading experts on the potential for these programs to substantially improve patient care and access if approved. Clinical updates on the Company's hematologic franchises focused on investigational products targeting CD19 and BCMA for the treatment of large B cell lymphoma (LBCL) and multiple myeloma (MM), respectively. The solid tumor presentation provided the first look at initial clinical data on ALLO-316, an AlloCAR T product candidate targeting CD70 for the treatment of clear cell renal cell carcinoma (RCC). The Company also unveiled its Dagger™ technology, the Company's next generation allogeneic platform technology designed to prevent immune rejection and enable a window of persistence during which AlloCAR T cells can expand and actively target and destroy cancer cells. Dagger has the potential to enable a pipeline of innovative product candidates.
- 11.30.22 - Sensorion receives Orphan Drug Designation for OTOF-GT for the treatment of otoferlin gene-mediated hearing loss from the US Food and Drug Administration (PR)
  - FDA has granted Orphan Drug Designation (ODD) to OTOF-GT, Sensorion's lead gene therapy program, intended for the treatment of otoferlin gene mediated hearing loss. Sensorion is on track to file a Clinical Trial Application for OTOF-GT in the first half of 2023. Sensorion's OTOF-GT dual vector AAV gene therapy development program aims to restore hearing in people living with otoferlin deficiency. Patients with mutations in OTOF suffer from severe to profound sensorineural prelingual non syndromic hearing loss. Otoferlin deficiency is responsible for up to 8% of all cases of congenital hearing loss, with around 20,000 people affected in the US and Europe. Three months after gaining its first worldwide approval, from Indonesia, for its dengue fever vaccine, Takeda is progressing toward a blessing from the FDA. The Japanese company's Qdenga (TAK-003) shot has received a priority review designation for its biologics license application. It will be evaluated for its ability to prevent dengue disease from any of the fever's four serotypes in individuals ages 4 through 60. Developing a dengue vaccine has been a decade-long pursuit for Takeda, which projects peak revenue potential to come in at between $700 million and $1.6 billion. Dengue has appeared in more than 125 countries and is one of the primary causes of hospitalization in children in many parts of the world. Sanofi was first with a vaccine for dengue. But its launch of Dengvaxia in the Philippines was derailed in 2017 when the company revealed that the shot could cause more serious dengue infection if given to patients who had never contracted the virus.
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Collaborations Updates:

- 11.01.22 - Sherlock Biosciences and Tolo Biotech Expand Collaboration to Advance Development and Adoption of CRISPR-based Diagnostics (PR)
  - Sherlock Biosciences, a company engineering biology to bring next-generation diagnostics to the point-of-need, today announced it has signed a licensing agreement with Shanghai-based Tolo Biotech. The agreement grants co-exclusive rights to Cas12 and Cas13 CRISPR diagnostic methods in markets outside of the U.S. and Greater China. As the only two companies with rights to the Cas12 and Cas13 method patents in the diagnostics market, this agreement furthers Sherlock and Tolo's collaboration and provides the partners with rights to the most comprehensive portfolio of diagnostic CRISPR patents.
- 11.04.22 - Affimed and Artiva Biotherapeutics Announce Partnership to Advance Combination Therapy of Innate Cell Engager (ICE®) AFM13 and Off-the-Shelf Allogeneic NK Cell Therapy AB-101 (PR)
  - a new strategic partnership to jointly develop, manufacture, and commercialize a combination therapy comprised of Affimed's Innate Cell Engager (ICE®) AFM13 and Artiva's cord blood-derived, cryopreserved off-the-shelf allogeneic NK cell product candidate, AB-101. Affimed submitted a pre-IND meeting request for the AFM13 and AB-101 co-administered combination therapy to the FDA requesting feedback on the clinical trial design in relapsed/refractory (r/r) Hodgkin lymphoma (HL) with an exploratory arm evaluating the combination in r/r CD30-positive peripheral T-cell lymphoma (PTCL) and potential path to registration. FDA responded to this request and guided to providing feedback by Q1 2023.
- 11.04.22 - NKGen Biotech Announces Collaboration with the Parkinson's Foundation to Bring Its Novel Natural Killer Cell Therapy to the Clinic for Advanced Parkinson's Disease (PR)
  - The collaboration with the Parkinson's Foundation will focus on ways to help accelerate NKGen's clinical program using its novel autologous NK cell therapy (SNK01) for the treatment of advanced Parkinson's disease (PD) through its network of clinical partners and donors.
- 11.07.22 - Fate Therapeutics Announces Exercise by ONO Pharmaceutical of Option to HER2-targeted CAR T-Cell Product Candidate for Solid Tumors (PR)
  - Under the terms of the Collaboration and Option Agreement, Fate will receive a milestone payment in connection with ONO's exercise of its option to FT825/ONO-8250. The parties will jointly develop and commercialize FT825/ONO-8250 in the U.S. and Europe, and ONO maintains exclusive development and commercialization rights for FT825/ONO-8250 in the rest of the world. Fate is eligible to receive clinical, regulatory and commercial milestone payments as well as tiered royalties on net sales outside of the United States and Europe by ONO. The parties recently expanded their collaboration to initiate preclinical development of an additional program targeting a second solid tumor antigen.
- 11.07.22 - Tessa Therapeutics, Baylor College of Medicine Execute Agreement For Global Commercial Rights to 'Off-the-Shelf' CAR-T Platform (PR)
  - exclusive agreement with Baylor College of Medicine for worldwide commercial rights to the allogeneic Epstein-Barr virus specific T-cell (EBVST) technology platform developed jointly by Tessa's Scientific Co-Founder, Malcolm Brenner, M.D., Ph.D., and his colleagues Cliona Rooney Ph.D. and Helen Heslop M.D., D.Sc. at Baylor College of Medicine. Tessa is currently advancing a pipeline of products that utilize CD30.CAR-modified EBVSTs, including its lead allogeneic cell therapy, TT11X, which is being co-developed for the treatment of relapsed or refractory CD30-positive lymphomas. CD30.ALLO Virus specific T-cells (VSTs) are highly specialized T cells that can recognize CD30+ tumors.
- 11.07.22 - Cyagen and Neurophth Enter Global Strategic Collaboration to Develop AI-Designed AAV Gene Therapy Vectors for Ophthalmic Disorders (Biospace)
  - Under the terms of the agreement, Cyagen will apply its proprietary artificial intelligence (AI)-powered high-throughput platform to discover novel AAV vectors with optimized tissue targeting capability, tissue specificity, and productivity. Cyagen and Neurophth will both be responsible for evaluating the functional properties of the novel AAV vectors in rodent and NHP models, and Neurophth will be responsible for conducting clinical trials and commercialization for gene therapy products developed using Cyagen's novel AAV capsids. Cyagen could receive research phase and clinical phase milestone payments, as well as sales royalties that may exceed $140 million.
- 11.10.22 - Applied Molecular Transport Announces FDA Orphan Drug Designation Granted to AMT-101 for Treatment of Pouchitis (PR)
  - U.S. Food and Drug Administration has granted Orphan Drug Designation for AMT-101 in patients with pouchitis, an indication with significant unmet medical need and no current FDA-approved products. AMT-101 is an investigational, once-daily, GI-selective, oral fusion of IL-10 and AMT's proprietary carrier molecule, which is also in development for the treatment of ulcerative colitis (UC) and rheumatoid arthritis (RA).
- 11.10.22 - Danaher Launches Beacon Initiative and Its First Partnership Focused on Gene Therapy Innovation (PR)
  - Danaher Corporation (NYSE: DHR), a global science and technology innovator, today announced that it has entered a strategic partnership with Duke University to form its first Danaher Beacon for Gene Therapy Innovation. Danaher Beacons is a new initiative designed to access breakthrough science to create technologies and applications that will improve human health. The program invests in product innovation to advance external R&D strategies with a focus on genomic medicines, precision diagnostics, next generation biomanufacturing, human systems, and data sciences.
- 11.14.22 - Ionis partners with Metagenomi to add gene editing to its broad technology platform (PR)
  - The collaboration will leverage Ionis' extensive expertise in RNA-targeted therapeutics and Metagenomi's versatile next-generation gene editing systems to pursue a mix of validated and novel genetic targets that have the potential to expand therapeutic options for patients. The companies will jointly conduct research initially to deliver investigational medicines for up to four genetic targets. Ionis has the right to add four more targets upon achievement of pre-determined development milestones.
- 11.15.22 - ElevateBio and Affini-T Therapeutics Announce Partnership to Advance Affini-T's T Cell Therapy Programs Targeting Core Oncogenic Drivers (PR)
  - Affini-T will leverage ElevateBio BaseCamp's LentiPeakTM lentiviral vector technology platform and cell product manufacturing capabilities to progress its investigational T cell therapies targeting core oncogenic drivers into clinical development –
- 11.18.22 - Applied Cells Inc. and GenScript Enter Strategic Collaboration to Deliver Combined Solutions for Cell Therapy Development (PR)
  - The strategic collaboration to deliver combined cell isolation solutions for cell therapy drug development worldwide. Under this collaboration, GenScript will develop and supply its proprietary research and cGMP grade CytoSinct™ reagents for use in developing CAR-T and other Cell Therapy products on the Applied Cells MARS® Platform.

- 11.21.22 - Umoja Biopharma and IASO Biotherapeutics Announce Research Collaboration to Bring Off-the-Shelf Therapies to Patients with Hematological Malignancies (PR)
  - Umoja's iPSC-based allogeneic cell therapy platform uses its synthetic receptor enabled differentiation (ShRED) manufacturing process to direct differentiation and expansion of iCILs, a novel class of innate lymphocyte, with potent anti-tumor activity. ShRED generated-iCILs retain functionality in feeder-free culture after 100 days and, as ShRED does not require feeder-cells to induce effector cell expansion, these cells retain their proliferative capacity without the need for multiple complex raw materials.IASO's strong capability in screening potentially best-in-class CARs utilizing its proprietary fully-human antibody discovery platform (IMARS), a high-throughput CAR screening platform, and executing clinical trials rapidly, as well as its fully in-house GMP facility for plasmid, virus vector, and CAR-T cell manufacturing with over 90% success rate aims to bring its innovative therapies to a broader population globally.
- 11.21.22 - MassBio and Beacon Capital Partners are launching a life sciences workforce training center to meet growing demand (PR)
  - Massachusetts Biotechnology Council (MassBio®) and Beacon Capital Partners announced a partnership to launch a nearly 4,000 square foot workforce training center at Southline Boston, the redevelopment project of the former Boston Globe building in Dorchester. Expected to open in Q4 of 2023, the MassBio Training Center will launch with three distinct fast-track certificate training programs that are purpose-built to meet both the needs of the life sciences industry and the unique needs of prospective learners. The certificate programs are being designed to provide entry into life sciences careers such as technicians and lab assistants for individuals with a high school degree or its equivalent. The Training Center and its unique model are a response to the current disconnect between the existing workforce demand—there are thousands of job openings in the life sciences industry in Massachusetts—with the existing educational pathways in the state's training landscape, as identified in MassBio's recent workforce report.

M&A Updates:

- 11.16.22 - Kriya Acquires Redpin Therapeutics, Adding Neurology Pipeline to Gene Therapy Portfolio (PR)
  - Redpin Therapeutics, Inc., a privately held biotechnology company developing regulatable gene therapies for intractable nervous system diseases. The acquisition serves as the foundation for Kriya's neurology therapeutic area portfolio, with two lead gene therapy programs focused on epilepsy and trigeminal neuralgia (TN).
- 11.23.22 - Merck & Co. to acquire Imago BioSciences (c&en)
  - Merck's acquisition of Imago strengthens the larger firm's presence in hematology, says Hugh Y. Rienhoff Jr., Imago's founder and CEO. Last year, Merck bought Acceleron Pharma for $11.5 billion to obtain the fusion-protein drug sotatercept, which recently aced its Phase 3 trials to treat pulmonary arterial hypertension. Its lead asset is bomedemstat, an inhibitor of lysine-specific demethylase 1 (LSD1). LSD1 is an epigenetic protein that regulates the maturation of bone marrow stem cells and their subsequent differentiation. By inhibiting LSD1, bomedemstat restricts the blood cell count and potentially reduces the population of malignant stem cells behind myeloproliferative
- 11.29.22 - AstraZeneca Deepens Cancer Cell Therapy Portfolio with $320M Neogene Buyout (Biospace)
  - AstraZeneca and Neogene Therapeutics have entered into a definitive acquisition agreement in which AstraZeneca will buy all outstanding equity in the smaller company for $320 million. AstraZeneca will make an upfront initial payment of $200 million once the buyout is closed. The remaining $120 million will be paid according to certain milestone Once the acquisition is completed, Neogene will operate as a wholly-owned subsidiary of AstraZeneca, with footprints in Amsterdam and Santa Monica, California. The companies expect the deal to close early next year. Unlike most cellular approaches to cancer, which prime the immune system against a surface target, T-cell receptor therapies can hone in on targets inside tumor cells, including genetic mutations that can drive oncogenesis.

CRO Updates:

- 11.28.22 - Charles River Laboratories Opens Contract Vivarium Space in Chicago, Enhancing Access to AAALAC-Accredited, Turnkey Research Program (PR)
  - CRADL Chicago is located in Fulton Labs, in the heart of Chicago's Fulton Market, which is quickly emerging as a hub of the local life sciences community. Fulton Labs also includes premier wet and dry laboratory and office space, offered by Portal Innovations. By offering on-demand facilities, both emerging and established biopharmaceutical companies and research institutions can quickly start new projects, accelerating the early stage of research. This allows scientists to focus on research, while leaving the animal husbandry and daily vivarium management to a trusted partner, ith the acquisition of Explora BioLabs in April 2022, the CRADL-Explora Vivarium Network was established as a premier partner for clients to launch or expand their drug research programs, with the added benefit of access to Charles River's complete portfolio of integrated drug discovery and non-clinical development resources. The CRADL-Explora Vivarium Network operates 28 vivarium facilities.
- 11.14.22 - Curavit Raises $5 Million in Series A Funding to Accelerate Growth in Digital Therapeutics Clinical Research (PR)
  - a virtual contract research organization (VCRO) that specializes in decentralized clinical trials (DCTs), today announced it raised $5 million in Series A funding to accelerate its growing portfolio of research in the global digital therapeutics (DTx) market (projected to grow at a CAGR of 31.4% through 2026). The new funding will be used to expand the company's DCT capabilities, partnerships, and market adoption of Curavit's virtual CRO services and platform for prescription and non-prescription digital therapeutics trials. The funding was led by early-stage technology investor Osage Venture Partners with additional investment from Royal Street Ventures and Narrow Gauge Ventures. It adds to early investments from Curavit founders and individual investors, including industry veterans Clark Golestani, former president of emerging businesses and global CIO of Merck, and former president and co-founder of Veeva Systems, Matt Wallach.
- 11.29.22 - Mispro to Open Contract Vivarium in Boston's Seaport (PR)
  - Mispro's Seaport location will offer 10k sq ft of state-of-the-art vivarium research space where biosciences companies of all sizes and therapeutic indications can conduct preclinical in vivo drug development studies with the support of Mispro's husbandry, technical, and regulatory compliance oversight services. Mispro's contract vivarium (CV) facilities offer a choice of private or shared room configurations, all fully equipped and research ready for preclinical rodent studies. Private suites are also available, allowing companies to customize their in vivo lab space and accommodate larger teams.
- 11.30.22 - CRO startup Vial scores $67M Series B led by General Catalyst (endpts)
  - Vial, a CRO specializing in offering clinical trial services to biotech companies, raised $67 million in a new round of funding, bringing its total money raised to $100 million. Vial is not alone in trying to change the way clinical trials are done. Companies like 4G Clinical, Huma, Clinsource and Reify Health have taken steps to cut down the time and effort it takes to run studies by using cloud-based trial services and new technologies that promise to streamline clinical trials. In some cases, they've taken on additional challenges, such as decentralizing trials and increasing diversity.