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

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

Funding Updates:

- 08.04.22 - David Hallal's ElevateBio launches new company to 'disrupt' off-the-shelf cell therapy, but provides few other details (Endpts)
  - The new company comes out of a partnership with Boston Children's Hospital and research from George Daley, the dean of Harvard Medical School. The triumvirate claims to have found a way to design better off-the-shelf cell therapies using new methods discovered in Daley's Boston Children's Hospital lab (Harvard is not involved in the collaboration). But that's all they're saying right now. There's no information — yet — on how much money the company has at launch, how long that cash will last or how it plans to focus its efforts, with respect to tumor type. Hallal and Daley are even keeping the company's name a secret for now, but promise to provide more details "in the coming months," Hallal tells Endpoints News.
- 08.15.22 - Remedium Bio announces successful $2.3M Expanded Seed raise (PR)
  - The company closed more than $2.3M in its expanded seed round financing. Funding from the raise is being used to study Remedium's lead product, a single-injection gene therapy potentially capable of reversing cartilage loss, in a collaboration with Tufts University School of Medicine scientists researching rheumatic disorders. The financing was led by Sherwood Ventures and included participation from, LongevityTech.Fund, Primo Medical Group, Angel Star Ventures, Apis Health Angels, MicroVentures, and Guindy Alumni Angels.
- 08.16.22 - Flagship's Senda Biosciences Announces Close of $123 Million Series C Financing (PR)
  - The proceeds of the financing will be used to drive further development of Senda's proprietary programmable medicines platform and advance its first programs into clinical testing. Senda's platform deconstructs the chemical-addressing codes of natural nanoparticles from species across all kingdoms of life that have evolved to precisely shuttle biomolecules into human cells. By cataloging these chemical-addressing codes into a large, diverse atlas, Senda is programming nanoparticles with key bioproperties, including specific cell and tissue targeting and repeatable dosing. Combining the atlas with an mRNA engine, Senda's platform fully unlocks the potential to generate a new class of comprehensively programmed medicines. The unique properties of these medicines create new frontiers for therapeutics and vaccines with further possible applications in the gene-editing and protein-based therapy landscapes.
- 08.17.22 - Orna Therapeutics Raises $221 Million Series B Financing to Advance Circular RNA Platform and Accelerate Programs to the Clinic (Pharmatechfocus)
  - Orna Therapeutics, a biotechnology company pioneering a new class of fully engineered circular RNA therapies (oRNA), today announced the initial closing of its $221 million Series B financing. At signing, the company received approximately $121 million and expects to receive the remaining $100 million subject to customary closing conditions (including regulatory approval under the Hart-Scott-Rodino (HSR) Act). Merck participated as a new investor in the financing alongside commitments from founding investors MPM Capital and BioImpact Capital, an affiliate of MPM, among others. Orna was created in 2019 by MPM Capital and BioImpact Capital, with funding from the UBS Oncology Impact Fund. With the proceeds of the Series B financing, Orna anticipates advancing this program into clinical trials in 2024.
- 08.25.22 - Stanford spinout 3T Bio snags $40M for immunotherapies targeted to solid tumors (MedCityNews)
  - Artificial intelligence and machine learning are key to 3T Biosciences' approach to identifying novel drug targets and engineering better, safer cancer immunotherapies. The Stanford spinout is now out of stealth with a $40 million Series A financing and a plan to reach the clinic within two years. The cancer immunotherapies now available have been designed to address specific targets, but these antibody drugs and engineered T cell therapies still have not solved the targeting problem, said Stefan Scherer, CEO of biotech startup 3T Biosciences. Those therapies address targets on the surface of a tumor cells, and the easy targets have already been found. To identify novel targets that are unique to a tumor, Scherer contends that the search must go inside those cells.

Manufacturing Updates:

- 08.05.22 - Lykan Bioscience Has Merged with RoslinCT (PR)
  - Lykan Bioscience is an innovative contract development and manufacturing organization (CDMO) focused on cell-based therapies. With decades of biopharmaceutical industry experience, the company offers a full range of development and manufacturing services. The state-of-the-art, purpose-built facility offering eight independent manufacturing suites is uniquely designed to fully integrate cGMP principles and advanced software solutions to enable real-time testing and release of product. Located in Hopkinton, Massachusetts, 25 miles southwest of downtown Boston, WindRose is an equity investor in companies that operate within the services sectors of the healthcare industry. based in New York City and invests in companies throughout the United States. RoslinCT is a leading UK cell therapy contract development and manufacturing organization (CDMO) focused on providing services for companies developing cell-based therapeutic products. Originally founded in 2006 as a spin-out from the Roslin Institute, it has built on the broad range of scientific expertise available in the field of cell biology. Based at the Edinburgh BioQuarter, RoslinCT operates fully licensed GMP manufacturing facilities and has a proven track record in the delivery of cell-based products.
- 08.08.22 - The Navy Yard Welcomes Vinta Bio (PR)
  - One of Philadelphia's top Life Science campuses welcomed Vinta Bio as their newest Life Science tenant. Vinta Bio, Inc. provides CDMO (Contract Development and Manufacturing Organization) services in producing GMP-grade and research-grade viral vectors, such as but not limited to adeno-associated (AAV) and lenti viral vectors, as the carrying agents for a number of disease targets for its customers such as biotech and pharmaceutical companies, and academic research institutes and laboratories. The founders and management are at the top of the industry, who are the only ones having manufactured the early AAV vectors of the only two FDA-approved AAV gene therapy products, Luxturna and Zolgensma, and played critical roles in CMC filing for Luxturna. Vinta Bio has built a highly efficient and highly productive master cell bank, and just moved into a new state-of-the-art 22,000 sqf GMP manufacturing facility in Navy Yard in Philadelphia. The facility has 2 independent production rooms, 2 additional reserved production rooms, and a dedicated fill-and-finish room. It has on-site process development and analytical capabilities. Future expansion is planned in 2023, with additional cell therapy manufacturing as well as Phase III and commercial production. Vinta Bio currently has 20 employees and is forecasted to triple in size within the next 12 months
- 08.09.22 - Charles River Laboratories is First CDMO in North America to Receive EMA Approval to Commercially Produce an Allogeneic Cell Therapy Drug Product (PR)
  - The approval follows an inspection by the cell and gene therapy experts from the Italian inspectorate, Agenzia Italiana del Farmaco (AIFA), performed on the EMA's behalf. The GMP certification of Charles River's Memphis contract development and manufacturing (CDMO) facility complements an existing GMP license for Investigational Medicinal Product (IMP) production. The Memphis site can manufacture and ship drug products intended for European Union distribution. The approval recognizes Charles River's industry-leading expertise, multidisciplinary team, regulatory know-how, and quality standards.
- 08.17.22 - VGXI Announces Grand Opening Celebration for New Headquarters and Manufacturing Facility (PR)
  - VGXI, Inc., an industry-leading contract developer and manufacturer (CDMO) of nucleic acid biopharmaceuticals including gene therapies, DNA vaccines, and RNA medicines, announces the official Grand Opening celebration for its new headquarters and manufacturing facility located at Deison Technology Park in Conroe, TX. The ribbon-cutting ceremony for the 120,000 square-foot building is scheduled for October 7, 2022. The new VGXI headquarters features 4 distinct manufacturing trains with state-of-the-art production equipment, flexible fermentation capacity in excess of 3000L, and expanded GMP fill/finish capabilities. Dedicated areas are also available for mRNA manufacturing and small-scale, rapid turnaround services for personalized therapies. Qualification activities are fully underway in preparation for GMP production. To ensure a seamless transition and provide additional overflow capacity, VGXI's existing facility in The Woodlands will remain fully operational.
- 08.18.22 - Almac commits to $65M expansion for its Pennsylvania HQ amid wider investments (Endpts)
  - A Northern Irish contract manufacturer is making some big moves in the Keystone State. The Almac Group, a global contracting and manufacturing company for APIs and pharmaceuticals, is expanding its North American headquarters in the town of Souderton, PA, northwest of Philadelphia. The company is looking to invest $65 million into the facility with the plan to increase the facility's clinical capacity by 60%. Almac is also looking to add additional cold and ultra-low storage as well as just-in-time processing capabilities which Almac predicts will be to support the industry trend toward biologics and advanced therapeutic medicinal products (ATMPs). The Souderton expansion is expected to break ground sometime in early 2023, and once complete it will bring the company's North American headquarters to a total of 340,000 square feet.
- 08.23.22 - ElevateBio Partners with the California Institute for Regenerative Medicine to Accelerate the Development of Regenerative Medicines (PR)
  - ElevateBio to enable access to multiple induced pluripotent stem cell (iPSC) lines suitable for research through clinical development and commercialization. ElevateBio to offer end-to-end development and GMP manufacturing capabilities to bring concepts to commercialization for regenerative medicines.

- 08.24.22 - Thermo Fisher opens yet another production site, this one in Massachusetts for viral vectors (FiercePharma)
  - Located in Plainville, MA, the 300,000 square-foot-plant, located 40 miles south of the company's headquarters in Waltham, will employ 300 people and manufacture viral vectors, which are critical components in the development of gene therapies.This is Thermo Fisher's sixth viral vector plant between the United States and Europe. The site has a sustainable construction design, the company said, with flexible lab and production suites adjacent to warehousing and offices. Thermo Fisher revealed plans to build the site in May 2020, estimating its cost at $180 million and saying it would represent a twofold expansion of its viral vector capacity.
- 08.24.22 - Sonoma Biotherapeutics Enters Long-Term Lease Agreement to Establish a Treg Cell Therapy R&D and Manufacturing Center (PR)
  - Sonoma announced that it has entered into a lease agreement to develop an approximately 83,000 square-foot Research and Development (R&D) and Manufacturing Center to expand its operations in Seattle and complement its existing R&D enterprise in South San Francisco. The state-of-the-art Center will support the R&D and manufacturing of Sonoma Bio's pipeline of gene-modified Treg therapies for autoimmune and inflammatory diseases at scale.
- 08.29.22 - Applied StemCell Announces the Expansion of its cGMP Manufacturing Facility to Support Cell and Gene Therapy (PR)
  - Applied StemCell, Inc. (ASC), a leading cell and gene therapy CRO/CDMO focused on supporting the research community and biotechnology industry for their needs in developing and manufacturing cell and gene products, today announced the expansion of its Current Good Manufacturing (cGMP) facility. ASC has successfully carried out cell banking and product manufacturing projects in its current cGMP suite and is now set on building 4 additional cGMP cleanrooms, cryo-storage space, and a process development and QC/QA space. The expansion of the facility will increase its cell banking and cell product manufacturing capacity and allow ASC's team of experts to work simultaneously on multiple manufacturing projects such as iPSC generation, gene editing, differentiation, and cell bank manufacturing for safe and efficacious therapeutic products. Construction will begin within the next month, and the company has already begun the staff hiring process. ASC hopes to have the expansion completed and a team built that will be ready to take on as much as 4 times more new projects early next year.
- 08.31.22 - WuXi Biologics Announces GMP Release of Its First North American Biomanufacturing Facility in Cranbury, New Jersey (PR)
  - The company announced the release of its GMP phase I drug substance clinical manufacturing facility, MFG18, in Cranbury, New Jersey. The site's clinical manufacturing operations have an initial capacity of 4,000L and will grow to 6,000L, utilizing only single-use technology, adding to the Cranbury site's full process development capability. It is WuXi Bio's first GMP manufacturing facility in North America and was established in response to the growing need of customers worldwide.

M&A and Collaborations:

- 08.02.22 - Polyplus announces the availability of Transgene Plasmid Engineering Services for viral vector manufacturing. (PR)
  - Polyplus, leading upstream solutions provider for advanced biologic and cell and gene therapy production from research to commercial grade, today announces the availability of Transgene Plasmid Engineering Services for viral vector manufacturing. The expansion of plasmid services can be used stand alone, or as a complement to the industry standard PEIpro® and FectoVIR-AAV® reagents and kits for next generation viral vector and gene therapy manufacturing. Following the early 2022 acquisition of e-Zyvec, integration activities have focused on providing a deeper level of solutions support to customers. Using the proprietary e-Zyvec technology, plasmids are assembled de novo, from a library of DNA bricks, removing the use of standard backbones that contain undesired DNA sequences. Plasmids can be engineered from scratch in a record short time window of only two weeks using the Polyplus online plasmid engineering tool. These engineered plasmids can then be chosen for the delivery of the gene of interests used in gene therapies.
- 08.05.22 - Gilead Sciences to Acquire MiroBio for $405M (ContractPharma)
  - The acquisition adds MiroBio's discovery platform and portfolio of immune inhibitory receptor agonists. MiroBio's lead investigational antibody, MB272, is a selective agonist of immune inhibitory receptor B- and T-Lymphocyte Attenuator (BTLA) in Phase 1 clinical trials. MB272 targets T, B and dendritic cells to inhibit or blunt activation and suppress an inflammatory immune response. MiroBio's I-ReSToRE platform (REceptor Selection and Targeting to Reinstate immune Equilibrium) has the potential to be used to develop best-in-class agonist antibodies targeting immune inhibitory receptors, a novel approach to the treatment of inflammatory diseases. The I-ReSToRE platform supports identification and development of therapeutics that use inhibitory signaling networks with the goal of restoring immune homeostasis. Gilead anticipates advancing additional agonists derived from MiroBio's I-ReSToRE platform, including a PD-1 agonist, MB151, and other undisclosed early-stage programs, over the next several years.

- 08.08.22 - Sarepta licenses next-gen AAV tech from Broad Institute in deal covering five indications — including Duchenne (Endpts)
  - The biotech will license a new group of adeno-associated viruses from the institute for an undisclosed upfront payment and milestone promises, the pair announced Monday morning. Under the agreement, Sarepta will have the rights to five neuromuscular and cardiac indications, including Duchenne muscular dystrophy, where it's already well-versed. According to Sarepta, the deal covers the MyoAAV program that aims to deliver more efficient gene therapies using modified capsids. Endpoints News has also reached out to the Broad Institute and will update this story accordingly.
- 08.10.22 - In bid for new T cell therapies, Bristol Myers inks deal with Treg player GentiBio (Endpts)
  - A myriad of rare T cell types has popped on the radar in recent years as companies search for the next generation of T cell therapies. Among those are regulatory T cells, or Tregs, which were at the center of a number of big raises last year. Now one of those Treg players, GentiBio — which reeled in a $157 million Series A last year — has inked a deal with Bristol Myers Squibb, as the Big Pharma looks to add to its selection of T cell therapies, which currently includes two CAR-Ts. The two companies revealed few financial details of the deal, saying only that it could be worth up to $1.9 billion in downstream milestones. Bristol Myers will be leveraging GentiBio's Treg platform for up to three potential programs in inflammatory bowel diseases.
- 08.11.22 - WuXi ATU Announces Licensing Agreement with Janssen for TESSA™ Technology (PR)
  - WuXi Advanced Therapies (WuXi ATU) and Janssen for the licensing of the Tetracycline-Enabled Self-Silencing Adenovirus (TESSA™) technology – a high-performance system for adeno-associated viral vector (AAV) manufacture and a proprietary clonal suspension HEK293 cell line. Under the agreement, Janssen will use the TESSA™ platform for the in-house development, large-scale manufacturing and commercialization of AAV vectors. The technology will enable Janssen to produce higher quality AAV particles more efficiently with a focus on broader therapeutic indications of high value across multiple therapeutic areas – developing and delivering life-changing cell and gene therapies faster for patients in need.
- 08.15.22 - Resilience Announces Collaboration to Manufacture and Deliver Biotherapeutics for Rare and Complex Conditions (PR)
  - Resilience will collaborate with Mayo's Center for Regenerative Medicine in Two Discovery Square, located within the research corridor of the Destination Medical Center economic development initiative in Rochester, Minnesota. Resilience and Mayo will build embedded process and analytical development labs, as well as quality control labs. This "embedded" approach allows for closer proximity and collaboration to jointly develop and progress cell therapies and other advanced modalities into clinical care. The collaboration also aims to attract third-party biotech companies interested in sponsoring clinical trials for new therapeutics, as well as collaborating on their process and analytical development. In addition, the collaboration seeks to advance biologic discoveries toward early stage clinical trials.
- 08.16.22 - RoosterBio and AGC Biologics Announce Collaboration to Accelerate Manufacturing of Cell and Exosome Therapies (PR)
  - RoosterBio will utilize its extensive portfolio of cell and media products to develop robust, scalable processes for hMSC and exosome therapies. These capabilities include genetic engineering of cells and exosomes to express therapeutic targets, upstream processing in both 2D flask and 3D bioreactor systems, downstream purification to achieve desired purity and potency, and comprehensive analytical characterization of the resulting formulated cell or exosome therapy. The partnership creates an end-to-end solution for the development and production of hMSC and exosome therapeutics leveraging RoosterBio's well-established cell and media products and process development services, coupled with AGC Biologics' global cell and gene therapy manufacturing capabilities.
- 08.16.22 - Merck pays startup Orna $150M as 'circular RNA' attracts industry interest (Biopharmadive)
  - Merck & Co. is delving deeper into RNA drugmaking, announcing on Tuesday a lucrative deal with biotechnology startup Orna Therapeutics that it hopes could lead to multiple new drugs and vaccines. Through the deal, Merck will pay Orna $150 million upfront and invest another $100 million in a $221 million Series B round the Cambridge, Mass.-based biotech revealed separately on Tuesday. Orna could receive up to $3.5 billion in downstream payments, as well as royalties on any approved products that come from the deal. The two will work together to develop and commercialize "multiple" programs, including vaccines and therapeutics for infectious diseases and cancer. The alliance marks Merck's latest sign of interest in RNA drugmaking. The company has dabbled in RNA search over the years, but hasn't yet broken through with an approved product.
- 08.23.22 - Resilience Becomes Biomanufacturing Resource Partner for California Institute for Regenerative Medicine (PR)
  - Resilience, a technology-focused biomanufacturing company dedicated to broadening access to complex medicines, was named by the California Institute for Regenerative Medicine (CIRM) as one of the initial Industry Resource Partners within its Industry Alliance Program (IAP). As a GMP manufacturing partner, Resilience will launch an offering to support translational and clinical phase grant applications (TRAN, CLIN1, and CLIN2 grants) for cell therapy programs, with the potential to expand to other modalities including gene therapies and biologics in the future. R=

- 08.30.22 - Bayer's BlueRock inks cardiovascular cell therapy delivery deal (Fiercepharma)
  - Bayer's BlueRock Therapeutics has partnered with BioCardia on the delivery of cell therapies, paying a "sizable" upfront fee to access minimally invasive technology that could get its heart failure candidates to their targets. BlueRock is built on a platform for programming mature, differentiated cells back to induced pluripotent stem cells (iPSCs). By then re-differentiating the iPSCs, the biotech aims to replace cells damaged or lost to disease. BlueRock is applying the platform to a clutch of therapeutic areas, including cardiology. The cardiology program is seeking to replace the cells lost after a cardiac event to restore heart function.
- 08.31.22 - Mayo Clinic, Hibiscus BioVentures and Innoforce Announce Mayflower Cell and Gene Therapy Accelerator (Biospace)
  - Mayo Clinic, Hibiscus BioVentures (Hibiscus) and Innoforce announce the launch of Mayflower BioVentures, a cell and gene therapy accelerator dedicated to identifying and forming companies around technologies that address unmet patient needs. This academic and industry relationship will establish independent cell and gene therapy companies to advance the development of Mayo Clinic technologies through preclinical and early feasibility studies. With a shared interest in identifying treatments and preventing disease, the aim of Mayflower is to advance new cures for serious and complex conditions through cell and gene therapies.

Clinical Updates:

- 08.11.22 - Graphite Bio Doses First Patient with Investigational Gene Editing Therapy GPH101 for Sickle Cell Disease (PR)
  - The company announced that the first patient has been dosed with GPH101, now called nulabeglogene autogedtemcel (nula-cel), in the company's Phase 1/2 CEDAR trial in people with sickle cell disease (SCD). Nula-cel is an investigational gene editing therapy designed to directly correct the genetic mutation that causes SCD and definitively cure the disease. GPH101, now called nulabeglogene autogedtemcel (nula-cel), designed to directly correct the genetic mutation that causes sickle cell disease. Initial proof-of-concept data from Phase 1/2 CEDAR trial anticipated in mid-2023
- 08.17.22 - Bluebird bio Announces FDA Approval of ZYNTEGLO®, the First Gene Therapy for People with Beta-Thalassemia Who Require Regular Red Blood Cell Transfusions (PR)
  - The FDA has approved ZYNTEGLO® (betibeglogene autotemcel), also known as beti-cel, a one-time gene therapy custom-designed to treat the underlying genetic cause of beta-thalassemia in adult and pediatric patients who require regular red blood cell (RBC) transfusions.The approval of ZYNTEGLO is the culmination of nearly 10 years of clinical research of gene therapy in patients with transfusion-dependent beta-thalassemia. ZYNTEGLO works by adding functional copies of a modified form of the beta-globin gene (βA-T87Q-globin gene) into a patient's own hematopoietic (blood) stem cells (HSCs) to allow them to make normal to near normal levels of total hemoglobin without regular RBC transfusions. The functional beta-globin gene is added into a patient's cells outside of the body (ex-vivo), and then infused into the patient. Though ZYNTEGLO is designed to be administered to the patient once, the treatment process is comprised of several steps that may take place over the course of several months.
- 08.23.22 - Immatics Announces First Cancer Patient Treated with Second-Generation ACTengine® TCR-T Candidate IMA203CD8 Targeting PRAME (PR)
  - IMA203CD8 is a 2nd-generation product candidate co-expressing Immatics' proprietary CD8αβ co-receptor engaging functional CD4 and CD8 T cells directed against PRAM. Preclinical data with IMA203CD8 showed enhanced potency and prolonged anti-tumor activity mediated by activated TCR-engineered CD4 T cells. The IMA203CD8 Phase 1b expansion study is the third cohort of Immatics' multi-cohort strategy to achieve durable high response rates with TCR-T cells targeting PRAME-positive, hard-to-treat solid tumors. The First three patients to be treated at dose level 3 with the intention to advance directly to the recommended Phase 2 dose
- 08.23.22 - IECURE Receives Fda Rare Pediatric Disease Designation For Gtp-506, An Investigational Gene Editing Product Candidate For The Treatment Of Ornithine Transcarbamylase (Otc) Deficiency (PR)
  - The FDA decision recognizes OTC deficiency as a "rare disease or condition". GTP-506, a potential single-dose gene editing therapy designed to restore metabolic function in patients suffering with OTC deficiency, a rare urea cycle disorder. iECURE on-track to submit an Investigational New Drug (IND) application for GTP-506 in mid-2023. iECURE's approach to gene editing for its initial programs, including OTC deficiency, relies on the delivery of twin adeno-associated virus (AAV) capsids carrying different payloads. GTP-506 comprises two vectors, an ARCUS® nuclease vector (GTP-506A) targeting gene editing in the well-characterized PCSK9 gene locus and a therapeutic donor vector (GTP-506D) that inserts the OTC gene to provide the desired genetic correction.The cut in the PCSK9 site serves as the insertion site for the therapeutic gene, providing a potential path to permanent expression of a healthy gene.The company is collaborating with the University of Pennsylvania's Gene Therapy Program, or GTP, led by James M. Wilson, M.D., Ph.D., to utilize GTP's world-class translational expertise and infrastructure, which has helped generate our initial pipeline of potential product candidates.

- 08.24.22 - Ovid turns to gene therapy startup to restock drug pipeline (BiopharmaDive)
  - Ovid Therapeutics has struck a deal with young biotechnology company Gensaic, hoping the startup's method of delivering genetic medicines can yield new brain drugs. Under the deal, the partners will develop up to three gene-based treatments for neurological conditions Ovid is targeting. The New York biotech will get rights to license any gene therapies that emerge from the deal, so long as the two can agree on terms. Ovid also invested $5 million in the startup and committed to participate in future financing rounds. The deal is the latest step in a rebuilding plan for Ovid, a biotech former Teva and Bristol Myers Squibb executive Jeremy Levin formed seven years ago.
- 08.25.22 - Century Therapeutics Receives Study May Proceed Notification from FDA for CNTY-101, the First Allogeneic Cell Therapy Product Candidate Engineered to Overcome Three Major Pathways of Host vs Graft Rejection (PR)
  - The company announced today that the company has been notified by the U.S. Food and Drug Administration (FDA) that the Company's ELiPSE-1 clinical study may proceed to assess CNTY-101 in patients with relapsed or refractory CD19 positive B-cell malignancies. CNTY-101 is the first allogeneic cell therapy product candidate engineered with four powerful and complementary functionalities, including a CD19 CAR for tumor targeting, IL-15 support for enhanced persistence, Allo-Evasion™ technology to prevent host rejection and enhance persistence and a safety switch to provide the option to eliminate the drug product if ever necessary. CNTY-101 is manufactured from a clonal iPSC master cell bank that yields homogeneous product, in which all infused cells have the intended modifications.
- 08.29.22 - ONK Therapeutics Presents Promising In-Vivo Data of its Optimized Affinity CD38 CAR-NK Candidate, Being Developed for the Treatment of Multiple Myeloma (PR)
  - ONK Therapeutics is developing a pipeline of off-the-shelf, optimally engineered natural killer (NK) cell therapies expressing a chimeric antigen receptor (CAR), further modified to enhance tumor homing, persistence and metabolism, and to overcome exhaustion in the tumor microenvironment. Currently it has four programs in pre-clinical development across hematological malignancies and solid tumors. ONKT102 is the company's most advanced program, and is being advanced towards clinical development as a potential treatment for patients with relapsed or refractory multiple myeloma (MM).
- 08.30.22 - Forge Biologics Reports Positive Clinical Data on Brain Development and Motor Function from the RESKUE Novel Phase 1/2 Gene Therapy Trial in Patients with Krabbe Disease at the SSIEM Annual Symposium (PR)
  - FBX-101 clinical data demonstrating safety and initial efficacy from the RESKUE trial is being presented at the 2022 Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium. FBX-101 is a systemic AAV gene therapy following unrelated cord blood transplantation ("UCBT") and has been well tolerated through Day 180. FBX-101 significantly increased GALC enzyme activity in leukocytes and patient exhibited improved motor function and normal brain development
- 08.30.22 - Sangamo Therapeutics Announces Updated Preliminary Phase 1/2 Data in Fabry Disease Clinical Study Showing Continued Tolerability and Sustained Elevated **α**-gal A Enzyme Activity in Five Longest Treated Patients (PR)
  - Isaralgagene civaparvovec, or ST-920, continued to be generally well tolerated across three dose cohorts in the six treated patients. The five longest treated patients exhibited elevated **α**-Gal A activity, ranging from nearly 3-fold to nearly 17-fold above mean normal, up to 15 months as at the last date of measurement. One patient was withdrawn from enzyme replacement therapy (ERT) and demonstrated significantly elevated levels of **α**-Gal A activity at 12 weeks post withdrawal. Since the cutoff date, an additional five patients have been dosed and an additional four patients in the dose escalation phase have been withdrawn from ERT. The Phase 1/2 STAAR study has progressed into the dose expansion phase.
- 08.31.22 - After disappointing Wall Street last year, Denali touts new biomarker data hoping to forge clear path to FDA (Endpts)
  - The South San Francisco biotech reported longer-term results from a Phase I/II study evaluating DNL310, saying the safety profile remained similar to the current standard of care — enzyme replacement therapy — after 85 weeks. Additionally, a larger group of patients gives the company more confidence that early biomarker results from July 2021 indicate the drug is positively affecting Hunter syndrome patients.
- 08.31.22 - Immusoft Announces FDA Clearance of IND Application for ISP-001 for MPS I, the First Engineered B Cell Therapy to Enter into Clinical Trials (PR)
  - Immusoft makes history with the first engineered B cell investigational therapy cleared for human trials. The company seeks to improve the treatment of MPS I, a rare childhood disease, through the use of its Immune System Programming (ISP™) approach that utilizes B cells as re-dosable biofactories for therapeutic protein delivery; the organization has received FDA Orphan Drug Designation and Rare Pediatric Disease Designation for ISP-001 in MPS I (Mucopolysaccharidosis type I) and will launch its Phase 1 study this year