PROJECT EVOLUTION

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Advanced Therapies
Clinical & Commercial Approvals

REACH OUT TO BRYAN KENNEDY AT BK@PROJECTEVOLUTION.ORG WITH ANY QUESTIONS

- 01.31.23 - Artiva Biotherapeutics Receives FDA Fast Track Designation for AB-101 (PR)
  - AB-101 is a cord blood-derived, allogeneic, cryopreserved, ADCC-enhancing NK cell therapy candidate for use in combination with mAb or innate-cell engagers. Granted Fast Track designation by U.S. FDA for the treatment of relapsed/refractory non-Hodgkin lymphoma of B cell origin, in combination with rituximab.
- 01.31.23 - Early signs are positive for CureVac and GSK's mRNA collaboration (thepharmaletter)
  - mRNA specialist CureVac has reported positive data from key Phase I programs in COVID-19 and seasonal flu. The German company is working with GSK on the vaccine programs, under collaboration inked in 2020, where GSK took a 10% stake in the company for $163M, in addition to making a significant upfront payment and committing to substantial milestone payments.
- 01.30.23 - Nanoscope Therapeutics Receives Fast Track Designation by the FDA for MCO-010 for the Treatment of Stargardt Disease (PR)
  - In September 2022, enrollment was completed in the Phase 2 open-label STARLIGHT clinical trial of MCO-010 in six patients with advanced vision loss due to a clinical or genetic diagnosis of Stargardt disease. Six-month data from the trial are expected in Q1 2023.
- 01.30.23 - Moderna's RSV Vaccine Snags FDA Breakthrough Therapy Nod (Biospace)
  - Based on the pivotal Phase III ConquerRSV trial, the mRNA-based vaccine, mRNA-1345, demonstrated 83.7% efficacy against RSV lower respiratory tract disease in older adults. The company plans to submit for regulatory approval in the first half of 2023. mRNA-1345 employs the same lipid nanoparticle envelope as Moderna's COVID-19 vaccine, Spikevax
- 01.27.23 - XyloCor Advances Cardiovascular Gene Therapy Space with Positive Angina Results (Biospace)
  - The company reported positive topline results from Phase II of Phase I/II trial of its gene therapy for refractory angina. The EXACT trial, its lead gene therapy asset, XC001, met safety and efficacy goals. Six-month data from 28 patients with refractory angina showed the therapy hit several key metrics. This included a reduction of ischemic burden as measured by cardiac positron emission tomography imaging.
- 01.26.23 - Sana Biotechnology Gets FDA Clearance of IND Application for SC291, a Hypoimmune-modified, CD19-targeted Allogeneic CAR T Therapy for Patients with B-Cell Malignancies (PR)
  - The goal of the hypoimmune platform is to overcome the immunologic rejection of allogeneic cells, which if true for SC291 may result in longer CAR T cell persistence, and a higher rate of durable complete responses for patients with B-cell lymphomas or leukemias, company expects to report initial clinical data this year as well as submit a second IND from the platform this year for SC262, a hypoimmune-modified CD22-targeted allogeneic CAR T therapy.
- 01.26.23 - XyloCor Therapeutics Reports Positive Topline Safety and Efficacy Results from Phase 2 EXACT Clinical Trial of XC001 Novel Gene Therapy for Refractory Angina (PR)
  - XC001 is a one-time gene therapy designed to reduce ischemic burden by creating new blood vessels in the heart. In the Phase 2 portion of the EXACT trial, evidence of the drug's mechanism of action was demonstrated by the reduction in ischemic burden was accompanied by an improvement in total exercise duration, an important measure of exercise capacity. Prior to treatment, almost all subjects had marked limitations on ordinary physical activity. Six months after treatment, nearly half of all subjects were able to conduct ordinary physical activity without causing angina.
- 01.26.23 - DiscGenics Announces FDA RMAT Designation Granted to IDCT for Degenerative Disc Disease (PR)
  - The RMAT designation is based on positive two-year clinical data from DiscGenics's first-in-human study of IDCT. As previously reported, the study demonstrated IDCT's potential to safely increase disc volume and provide rapid, durable improvements in low back pain, function, quality of life, and pain medication usage out to two years post-injection in patients with lumbar DDD.
- 01.26.23 - Bristol Myers Squibb Announces TRANSCEND CLL 004 Trial of Breyanzi® Met Primary Endpoint of Complete Response Rate in Patients with Relapsed or Refractory Chronic Lymphocytic Leukemia (PR)
  - BMS will complete a full evaluation of the TRANSCEND CLL 004 data and work with investigators to present detailed results at an upcoming medical meeting, as well as discuss these results with health authorities.
- 01.25.23 - Capricor Therapeutics Announces Positive 18-Month Results from Ongoing HOPE-2 Open Label Extension Study of CAP-1002 in Duchenne Muscular Dystrophy Patients (PR)
  - Capricor is the developer of cell and exosome-based therapeutics for the treatment and prevention of muscular and other select diseases. The positive 18-month results from its ongoing HOPE-2 open-label extension study in patients with later-stage DMD. Data from the study continues to show evidence for disease modification with statistically significant differences in the Performance of the Upper Limb.
- 01.24.23 - Cullinan Oncology Announces U.S. FDA Clearance of IND Application for CLN-978, a Novel T-Cell Engager for the Treatment of Relapsed/Refractory B-cell Non-Hodgkin Lymphoma (PR)
  - The study is a Phase 1, open-label, dose-escalation and dose-expansion study designed to evaluate the safety and efficacy of CLN-978 in patients with relapsed/refractory B-NHL. In addition, the company's IND submission remains on track for CLN-617 (IL-2, IL-12 fusion protein) in 1H 2023.
- 01.24.23 - ImmPACT Bio Announces FDA Clearance of IND for Novel Bispecific CAR to Treat Aggressive B-cell Lymphoma (PR)
  - ImmPACT Bio, a clinical-stage company developing transformative logic-gate-based CAR T-cell therapies for treating cancer announced clearance of its first IND application by the U.S. FDA for IMPT-314, a bispecific "OR-Gate" autologous CAR T-cell therapy targeting the B-cell antigens CD19 and CD20. IMPT-314 will be studied in a Phase 1/2 clinical trial in patients with aggressive B-cell lymphoma, including diffuse large B-cell lymphoma.

- 01.23.23 - TScan Therapeutics Announces FDA Clearance of Three IND's Applications for the Treatment of Solid Tumors (PR)
  - Primary IND for solid tumor program, T-Plex, supports simultaneous use of multiple TCRs to create customized, multiplexed TCR-T cell therapies based on target and HLA expression. INDs for TSC-204-A0201 and TSC-204-C0702 introduce the first two TCRs into TScan's ImmunoBank, targeting MAGE-A1 on HLA types A*02:01 and C*07:02, respectively
- 01.23.23 - REGENXBIO Announces Phase I/II AFFINITY DUCHENNE™ trial of RGX-202, a Novel Gene Therapy Candidate for Duchenne Muscular Dystrophy, is Active and Recruiting Patients (PR)
  - The company is also enrolling newly active observational screening study, AFFINITY BEYOND, evaluating AAV8 antibody prevalence in boys with Duchenne. Commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center to be used in the clinical trial. RGX-202 is a potential one-time AAV Therapeutic for the treatment of Duchenne and includes an optimized transgene for a novel microdystrophin and REGENXBIO's proprietary NAV® AAV8 vector
- 01.23.23 - First US leukemia patient dosed with Smart Immune's investigational SMART101 cell therapy (PR)
  - Phase I/II trial will assess safety and efficacy of SMART101 in accelerating immune reconstitution to fight infection and relapse. The trial is a multicenter, open-label, first-in-human study expected to enroll up to 36 adult and pediatric patients with haematological malignancies, and has been designed to assess the safety and the potential to improve clinical outcomes of hematopoietic stem cell transplantation.
- 01.23.23 - Neurogene Announces FDA Clearance of IND for NGN-401 Gene Therapy for Children with Rett Syndrome (PR)
  - NGN-401 is the first investigational AAV gene therapy candidate to be administered to pediatric patients using Neurogene's proprietary Expression Attenuation via Construct Tuning (EXACT) gene regulation technology. EXACT, developed in collaboration with the University of Edinburgh, is a self-contained, transgene regulation technology that can be tuned to deliver a desired level of transgene expression within a narrow range, and is compatible with viral and non-viral delivery platforms.
- 01.18.23 - Exegenesis Bio Announces FDA Clearance of Investigational New Drug (IND) Application for EXG102-031; A Novel Gene Therapy for the Treatment of neovascular Age-Related Macular Degeneration (nAMD) (PR)
  - EXG102-031, a recombinant adeno-associated virus (rAAV) based gene therapy that is being studied for the treatment of neovascular Age Related Macular Degeneration (nAMD), the leading cause of severe vision loss and irreversible blindness worldwide.
- 01.12.23 - Adaptive Phage Therapeutics Announces First Patient Dosed in the PHAGE Clinical Trial Evaluating Phage Therapy in Cystic Fibrosis-related Respiratory Infection (PR)
  - The company announced the first patient dosed in the PHAGE clinical trial, evaluating bacteriophage therapy in adults with cystic fibrosis who carry Pseudomonas aeruginosa in their lungs. The trial is evaluating whether the bacteriophage, or "phage," therapy is safe and able to reduce the number of bacteria. The trial is being conducted by the Antibacterial Resistance Leadership Group which consists of more than 100 leading experts, and is funded by the NIH). The investigational phage therapeutic, was developed by the Walter Reed Army Institute of Research and is licensed to and manufactured by APT. The trial is expected to enroll a total of 72 patients at multiple sites across the country.
- 01.06.23 - Cellenkos Announces First Patient Dosed with CK0804 Cell Therapy in LIMBER-TREG108 Clinical Trial (PR)
  - CK0804 is a novel allogeneic, CXCR4 enriched, T regulatory cell therapy product that utilizes Cellenkos' proprietary CRANE™ technology to generate disease-specific products. The LIMBER-TREG108 trial is part of a development collaboration between Cellenkos and Incyte. The trial evaluates CK0804 as an add-on therapy to ruxolitinib in patients with myelofibrosis who experience a suboptimal response to ruxolitinib.
- 01.06.23 - Celularity's Placental-Derived Allogeneic Cell Therapy Provides Clinically Meaningful Benefit and Durable Biological Effect in Patients with Moderate to Severe Crohn's Disease in Phase 1, Phase 1b/2a and Phase 1b Studies (PR)
  - Legacy studies employing a single treatment course of two infusions seven days apart demonstrated greatest benefit in three early studies. Data support further investigation of novel genetically modified cell therapy in Crohn's disease
- 01.05.23 - Orchard Therapeutics Announces U.S. FDA Clearance of IND Application for OTL-203 in MPS-IH (PR)
  - The study is a multi-center,clinical trial designed to evaluate the efficacy and safety of OTL-203 in patients with MPS-IH compared to standard of care with allogeneic hematopoietic stem cell transplant (HSCT). A total of 40 patients with a confirmed diagnosis of Hurler subtype of mucopolysaccharidosis type I (MPS-IH).The study is powered to demonstrate superiority of OTL-203 over HSCT.

About Us:

PROJECT EVOLUTION

Project Evolution is an innovative, end-to-end, HUMAN CAPITAL platform that enables leading Life Scientific organizations to identify and secure the talent required to drive innovation. Our collaborative platform is powered by the industry-leading capabilities of Berke Executive Search and GTS Scientific. The highly-successful Joint Venture bridge Executive and Contingent placement and enable organizations of all sizes the ability to secure highly specialized talent quickly and efficiently.

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