

September 2022

For all clinical trials open to enrollment at UCI Alpha Stem Cell Clinic (ASCC), please visit: [https://clinic.stemcell.uci.edu/Clinical\\_Trials/current\\_clinical\\_trials.php](https://clinic.stemcell.uci.edu/Clinical_Trials/current_clinical_trials.php).

### **ApicBio Gene Therapy in ALS patients with SOD1 Mutations- Principal Investigator: Namita Goyal, MD – Opening October 2022**

The Phase 1/2 clinical trial which will be initiated in late 2021/ early 2022 is a multi-center , three part study to evaluate the safety, tolerability, and efficacy of intrathecally administered APB-102 in patients with DOD1 ALS mutations: Part I, single ascending dose; part II, randomized, double blind, placebo-controlled; and part III, extended follow-up

### **AskBio Glial Cell Line-Derived Neurotrophic Factor Gene Transfer (AAV2-GDNF) in Multiple System Atrophy (MSA) - Principal Investigator: Nicolas Phielipp, MD**

The purpose of this study is to test the safety of GDNF gene transfer to help researchers find a treatment for MSA-P. We want to find out what effects, good and/or bad, GDNF gene therapy has on people with MSA. The same GDNF gene transfer is being evaluated in separate clinical studies as a treatment for Parkinson's disease (PD). This is the first study of GDNF gene therapy in subjects with MSA.

### **Aspen Neurosciences Developing a Trial-Ready Cohort of Parkinson Disease Patients - Principal Investigator: Nicolas Phielipp, MD**

The study sponsor, Aspen Neuroscience, is developing a technology that can enable a person's skin cells to be turned into nerve cells that make a chemical called dopamine, an important chemical in the brain. The loss of certain nerve cells that produce dopamine causes Parkinson Disease. The main purpose of this study is to obtain skin cells from people with Parkinson Disease and to turn each person's skin cells into dopamine producing nerve cells. Aspen Neuroscience is planning to conduct future clinical trials in which participants in this screening study could potentially have their own dopamine producing nerve cells transplanted into their brains. The purpose of these future trials would be to determine whether these dopamine producing nerve cells could be a treatment for Parkinson Disease. Participation in this study does not guarantee that subjects will be eligible to participate in these future clinical trials or that these clinical trials will occur.

### **Audentes FORTIS Gene Transfer Study in Patients With Late Onset Pompe Disease (FORTIS) - Principal Investigator: Tahseen Mozaffar, MD**

This is a phase 1/2 open-label, ascending dose, multicenter clinical study to evaluate the safety and efficacy of AT845 in adult (aged  $\geq 18$  years) subjects, ambulatory or non-ambulatory, with Late Onset Pompe Disease (LOPD). This study (FORTIS) will evaluate the safety and efficacy of an investigational gene replacement therapy, AT845, in adult subjects with LOPD. Subjects will receive a single dose of AT845 delivered via intravenous (IV) infusion.

### **Direct Biologics, Evaluating the efficacy of EXOFLO in treating moderate to severe ARDS caused by COVID-19- Principal Investigator: Alpesh Amin, MD – Opening October 2022**

Evaluating the safety and efficacy of intravenous administration of bone marrow mesenchymal stem

cell derived extracellular vesicles, EXOFLO versus placebo as treatment for COVID-19 associated moderate to severe Acute Respiratory Distress Syndrome (ARDS).

**Brain Neurotherapy Bio, Inc, a unit of Asklepios BioPharmaceutical, Inc., Open-Label Safety Study of Glial Cell Line-Derived Neurotrophic Factor Gene Transfer (AAV2-GDNF) in Parkinson's Disease – Principal Investigator: Nicolas Phielipp, MD**

The objective of this Phase 1b investigation is to evaluate the safety and potential clinical effect of AAV2-GDNF delivered to the putamen in subjects with either a recent or a long-standing diagnosis of PD.

**Cartesian Descartes-08 Generalized Myasthenia Gravis Clinical Trial - Principal Investigator: Tahseen Mozaffar, MD**

The purpose of this study is to test a possible new cell therapy treatment called Descartes-08 in patients with Generalized Myasthenia Gravis (GMG). This study will test the safety, tolerability, and feasibility of Descartes-08.

**Cartesian Descartes-30 PHASE I/IIa study of Descartes-30 in Acute Respiratory Distress Syndrome Clinical Trial - Principal Investigator: Richard Lee, MD**

Emergency study to test the safety of Descartes-30 cells in patients with moderate-to-severe acute respiratory distress syndrome (ARDS) AND COVID-19.

**Celularity CYNK-001 in Combination with Recombinant Human Interleukin-2 in Adults with Recurrent Glioblastoma GBM - Principal Investigator: Daniela Bota, MD, PhD**

The purpose of this study is to evaluate the safety (any good or bad effects) of CYNK-001 and to assess the effects of CYNK-001 on your GBM. Also, the study plans to figure out the appropriate dose and treatment plan of CYNK-001 and assess whether CYNK-001 can eliminate the disease or keep it stable. CYNK-001 is an investigational product that is a liquid containing natural killer cells (NK cells) from human placental hematopoietic stem cells which have been grown in the laboratory. Natural Killer cells are a type of immune cell that has granules (small particles) with enzymes (a protein that speeds up chemical reactions in the body) that can kill cancer cells. Laboratory tests have shown that CYNK-001 has the ability to specifically kill the tumor cells without killing normal healthy cells.

**Humacyte 005 Human Acellular Vessel for Vascular Replacement or Reconstruction in Patients with Life or Limb-Threatening Vascular Trauma - Principal Investigator: Roy Fujitani, MD**

The main purpose of this study is to evaluate how well a new experimental vascular graft, Human Acellular Vessel (HAV), works when surgically implanted into the subject's arm, leg or torso to repair a damaged vessel and re-establish blood flow. This study will also evaluate how safe it is to use the HAV in this manner. This is a Phase 2, prospective, multicenter, multi-cohort, non-randomized study. There will be a limb cohort and a torso cohort.

**Humacyte 007 Human Acellular Vessel in Subjects with End-Stage Renal Disease - Principal Investigator: Roy Fujitani, MD**

The main purpose of this study is to compare the Human Acellular Vessel (HAV) with arteriovenous fistula (AVF) when used for hemodialysis access. This is a Phase 3, prospective, multicenter, open-

label, randomized, two-arm, comparative study. Subjects who sign informed consent will undergo study-specific screening assessments within 45 days from the day of informed consent.

### **Immunis, Inc, Open Label Use of IMM01-STEM In Patients With Muscle Atrophy Related to Knee Osteoarthritis – Principal Investigator: Dean Wang, MD -Opening October 2022**

An open-label dose escalation study to assess the safety and tolerability of IMM01-STEM in participants with muscle atrophy related to knee osteoarthritis.

### **Regeneron Efficacy and Safety of Pozelimab and Cemdisiran Combination Therapy in patients with symptomatic generalized Myasthenia Gravis (MG) - Principal Investigator: Ali Habib, MD**

This study is focused on subjects with myasthenia gravis (MG). Myasthenia gravis is a disease that causes weakness and fatigue in muscles in the body because the nerves and muscles are not communicating properly. The human body has a substance called Complement C5 (C5). In a healthy person, C5 is involved in fighting infection in the body. In people with MG, the actions of C5 can lead to damage to the body's own muscle and surrounding tissue, where the nerves and muscles meet. The study is researching an experimental combination treatment with pozelimab and cemdisiran to reduce the levels and activity of C5.

### **RegenXBio RGX-314 Gene Therapy in Participants with Neovascular or “Wet” Age-Related Macular Degeneration (nAMD) (ATMOSPHERE) - Principal Investigator: Baruch Kuppermann, MD**

RGX-314 is being studied for its potential to have one single injection that could allow the eye to make its own supply of anti-VEGF continually. Only one eye will receive RGX-314 (the study eye). This will be performed at a hospital or surgical center via local and monitored anesthesia. Once cells receive RGX-314, it is expected that the cells may be able to make their own anti-VEGF. This may decrease the need for future treatment for wet AMD and help stop further vision loss.

### **Sangamo ST-920 a Gene Therapy in Subjects with Fabry Disease (STAAR) - Principal Investigator: Madeleine Pahl, MD**

The purpose of this study is to test the use of a treatment called “gene therapy” to treat Fabry Disease. Fabry disease is caused by mutations in the GLA gene. The GLA gene produces the protein alpha-galactosidase A ( $\alpha$ -Gal A). When  $\alpha$ -Gal A is missing or not working properly, a fat that accumulates in organs called globotriaosylceramide (Gb3). This buildup may cause progressive kidney failure, heart disease, cerebrovascular disease (diseases that impact the blood vessels and blood supply to the brain), skin lesions, and other abnormalities.

### **Spark SPK-3006 Late-Onset Pompe Disease Clinical Trial - Principal Investigator: Tahseen Mozaffar, MD**

Pompe disease is a neuromuscular disease that can often be fatal, with systemic, multi-organ manifestations resulting from loss of function mutations in the gene encoding acid alpha-glucosidase (GAA). The purpose of this study is to test a possible new treatment called SPK-3006 for late-onset

Pompe disease. This study will evaluate the safety, tolerability, and effectiveness of an intravenous infusion of SPK-3006.

**TIGENIX – Takeda Pharmaceuticals Crohn’s Disease: The ADMIRE-CDII Study - Principal Investigator: Nimisha K. Parekh, MD, MPH**

A phase III, randomized, double blind, parallel group, placebo controlled, international, multicentre study to assess efficacy and safety of Cx601, adult allogeneic expanded adipose-derived stem cells (eASC), for the treatment of complex perianal fistula(s) in patients with Crohn’s disease over a period of 24 weeks and a follow-up period up to 52 weeks. ADMIRE-CD II study.

[Click here for more information](#)

**Ultragenyx Pharmaceuticals UX053 for Glycogen Storage Disease Type III (GSD III) Clinical Trial - Principal Investigator: Tahseen Mozaffar, MD**

A Phase 1/2 First-in-human, 2-part Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of Single Ascending Doses (Part 1: Open-label) and Repeat Doses (Part 2: Randomized, Double-blind, Placebo-controlled) of UX053 in Patients with GSD III.

[Click here for more information](#)

# UCI Alpha Stem Cell Clinic

To refer a patient or for more information contact us at (949) 824-3990 or [stemcell@uci.edu](mailto:stemcell@uci.edu).



The UCI Alpha Stem Cell Clinic (ASCC) provides a centralized infrastructure to work with UCI faculty and staff to accelerate stem cell clinical research activities at UCI. The UCI Alpha Stem Cell Clinic:

- Is part of the California Institute for Regenerative Medicine (CIRM) Alpha Stem Cell Clinic network.
- Promotes and facilitates scientific collaborations and interactions at UCI.
- Provides centralized stem cell clinical research infrastructure support at UCI.
- Accelerates the implementation of stem cell clinical trials at UCI.

**UCI** Alpha  
Stem Cell Clinic

[www.clinic.stemcell.uci.edu](http://www.clinic.stemcell.uci.edu) | 845 Health Sciences Road | Irvine, CA 92697