# Current Clinical Program Portfolio

Apr-18

| Award Number, PI, Institution | Program | Trial Stage | Indication | Therapeutic | General Disease Area | General Class of Approach | Funding (ICOC Approved) | Therapeutic Cell (for Cell Therapy) | Cell Source | Rationale | Project Goal | Award Start Date | Projected Award End Date | Percent Time Into Award |
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| **NEURO THERAPEUTICS** | | | | | | | | | | | | | | |
| **Neurologic Disorders: Injuries** | | | | | | | | | | | | | | |
| SP3A-07552 Lebkowski, Asterias Biotherapeutics | Strategic Partnership III | Ph 1/2a | Spinal Cord Injury | Allogeneic oligodendrocyte progenitors | Neurologic Injury | Cell Therapy | $14,323,318 | Oligodendrocyte Progenitors | Allogeneic | Up to 12,000 Americans suffer a spinal cord injury each year. Leads to a high level of permanent disability and decreased life expectancy. Currently no approved therapies. Cells derived from embryonic stem cells used to heal the spinal cord at the site of injury. The stem cells mature into oligodendrocyte precursors that are injected at the injury site where it's hoped they repair the myelin that protects the nerves in the spinal cord. | Safety. Dosing. Efficacy - motor improvement. | 10/1/14 | 9/30/18 | |
| CLIN2-10344 Bates, SanBio | Clinical Trial Stage Projects | Ph 2b | Ischemic Stroke | Modified bone marrow-derived mesenchymal stem cells (MSCs) | Neurologic Injury | Cell Therapy | $19,998,580 | MSC | Allogeneic | Stroke is a major cause of long-term disability and there are no proven medical treatments for chronic stroke. Intracerebral delivery of modified MSCs provides a well tolerated treament with the potential to improve motor function in these patients | Safety and efficacy compared to sham surgery - improvement in motor activity on stroke affected side. | 8/1/17 | 6/30/20 | |
| CLIN1-09433 Steinberg, Stanford | Late Stage Preclinical Projects | IND | Ischemic Stroke | H9 ESC-derived neural stem cells | Neurologic Injury | Cell Therapy | $5,300,000 | NSC or NPC (ESC-derived) | Allogeneic | Stroke is the leading cause of adult disability. There is no medical therapy that promotes stroke recovery. Cells derived from H9 ESC act via secretion of paracrine factors to modulate brain repair processes in preclinical stroke models. | Obtain an active IND | 8/1/17 | 7/31/19 | |
| **Neurologic Disorders: Neurodegenerative** | | | | | | | | | | | | | | |
| DR2A-05320, CLIN2-09284 Svendsen, Cedars-Sinai | Disease Team Therapy Development, Clinical Trial Stage Projects | Ph 1/2a | ALS (Amyotrophic lateral sclerosis) | Allogeneic neural progenitor cells genetically modified with GDNF | Neurodegenerative Disorder | Genetically Modified Cell Therapy | $17,842,617, $6,154,067 | NSC or NPC | Allogeneic | ALS is a devastating disease with no cure. This cell therapy intends to support sick motor neurons via astrocyte replacement and pro-survival growth factors. Allogeneic neural stem cells, genetically modified to express GDNF, injected into the spinal cord. | Safety. Dosing. Efficacy - Lower limb strength | 4/1/17 | 3/31/20 | |
| CLIN2-09894 Kern, Brainstorm | Clinical Trial Stage Projects | Ph 3 | ALS (Amyotrophic lateral sclerosis) | Autologous MSCs cultured to enhance secretion of growth factors (NurOwn) | Neurodegenerative Disorder | Cell Therapy | $15,912,390 | MSC | Autologous | ALS is a fatal neurodegenerative disease for which there is currently no adequate treatment. Autologous MSCs are propagated ex vivo and induced to secrete neurotrophic factors. NurOwn cells are returned to the patients in the target area of damage. Previous trials showed safety and encouraging signs of efficacy. | Safety and efficacy of three repeated doses. | 8/1/17 | 7/31/19 | |
| **Eye Disease** | | | | | | | | | | | | | | |
| DR3-07438 Humayun, USC | Duane Roth Disease Team Therapy Development III | Ph 1 | Adult Macular Degeneration | Allogeneic functionally polarized hESC-derived RPE monolayers on synthetic substrate | Eye Disease | Cell Therapy, Combination | $18,922,665 | RPE | Allogeneic | Age-related macular degeneration is a progressive disease resulting in death of the retinal pigment epithelium (RPE) causing distortion to central vision and eventually to legal blindness. Incidence - 1:1359 in the US. Approach is replacement therapy with viable RPE cells delivered on a synthetic membrane mimicking native state with RPE cells on Bruch's membrane. | Safety. Efficacy - slow disease progression, maintain and restore visual acuity | 8/1/14 | 3/31/19 | |
| LSP1-0835 Wang, Cedars-Sinai | Late Stage Preclinical Projects | IND | Retinitis Pigmentosa | Subretinal injection of human neural progenitor cells | Eye Disease | Cell Therapy | $4,954,514 | NPC | Allogeneic | Retinitis pigmentosa (RP) is a progressive retinal degeneration that affects over 1.5 million people worldwide. Unfortunately, treatment is still rather limited. A single sub-retinal injection of human neural progenitor cells offers dramatic preservation of vision. Grafted Cells survive for an extended period, secrete pro-survival factors and extracellular matrix, reduce oxidative stress response and preserve vision and RPE integrity. | Obtain an active IND | 8/1/15 | 9/30/17 | |
| DR2A-05739 Klassen, UC Irvine | Disease Team Therapy Development | IND, Ph 1/2a | Retinitis Pigmentosa | Allogeneic retinal progenitor cells | Eye Disease | Cell Therapy | $17,306,668 | RPC | Allogeneic | Retinitis pigmentosa (RP) is a severe form of blindness that runs in families with an incidence of 1:4000. Good target for stem cell therapy due to the defined loss of specific cells. Proposed mechanism: Rescue the light sensing photoreceptors. | Safety and efficacy - visual acuity. | 1/1/13 | 12/31/17 | |
| CLIN2-09698 Klassen, Jcyte | Clinical Trial Stage Projects | Ph 2b | Retinitis Pigmentosa | Allogeneic retinal progenitor cells | Eye Disease | Cell Therapy | $8,295,750 | RPC | Allogeneic | Follow-on study based on Phase 1/2a clinical trial. Continue to assess safety and establish efficacy. | Safety and efficacy - improvement in visual function at 12 months. | 2/1/17 | 1/31/21 | |
| CLIN1-08686 Deng, UCLA | Clinical Trial Stage Projects | IND | Corneal Blindness | Cultivated autologous human limbal stem cells on human amniotic membrane | Eye Disease | Cell Therapy | $4,244,211 | LSC | Autologous | Limbal stem cell deficiency results in inability to heal following ocular surface injury leading to corneal opacity. Cultivated autologous limbal stem cells transplanted back to the patient allow restoration and maintenance of a normal corneal surface. | Obtain an active IND | 8/1/16 | 11/30/18 | |
| **BLOOD & CANCER THERAPEUTICS** | | | | | | | | | | | | | | |
| **Blood Disorders** | | | | | | | | | | | | | | |
| CLIN2-09183 Mackenzie, UCSF | Clinical Trial Stage Projects | Ph1 | Alpha Thalassemia Major | Maternal bone marrow derived HSC transplant in utero | Blood Disorder | Cell Therapy | $12,131,817 | HSC | Allogeneic | Untreated alpha thalassemia major is almost universally fatal in utero. Current treatment requires in utero blood transfusions and monthly blood transfusions for life or a bone marrow transplant if a suitable donor is identified. The proposed treatment is a maternal bone marrow transplant in utero that takes advantage of maternal-fetal immune tolerance, and may provide a definitive cure. | Safety and feasibility, efficacy. | 8/1/17 | 7/31/22 | |
| CLIN2-08231 Kohn UCLA | Clinical Trial Stage Projects | Ph 1/2 | X-linked Chronic Granulomatous Disease. | Lentiviral vector modified autologous CD34+ hematopoietic stem/progenitor cells via transplantation & engraftment | Blood Disorder | Genetically Modified Cell Therapy | $7,402,549 | HSC | Autologous | CGD prevents white blood cells from killing foreign invaders. Patients have persistent, untreatable tissue infections. Affects 1:200,000 in US. Usually diagnosed before age 5, without treatment children die before age 10. Project plan is transplantation of severe X-CGD patients that lack matched donors using gene-corrected autologous HSCT. | Primary: Safety and Efficacy. Secondary: Restoration of immune function | 9/1/15 | 8/31/20 | |

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| DR3-06945 Kohn, UCLA | Duane Roth Disease Team Therapy Development III | Ph 1 | Sickle Cell Disease | Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated addition of a hemoglobin gene that blocks sickling | Blood Disorder | Genetically Modified Cell Therapy | $13,935,441 | HSC | Autologous | An inherited mutation in the hemoglobin gene causes red blood cells to "sickle" under conditions of low oxygen. Affects 1:500 African-Americans and is common in Hispanic-Americans. Median survival is 42 years for males and 48 years for females. More than 80% of patients lack an HLA-identical sibling donor. Project plan is genetic correction of adult bone marrow hematopoietic cells by adding a novel therapeutic hemoglobin gene that blocks sickling of the red blood cells. | Primary: Safety, feasibility. Secondary: Hematopoietic Recovery; RBC function; Quality of life assessment | 7/1/14 | 6/30/18 | |
| CLIN2-09339 Kohn, UCLA | Clinical Trial Stage Projects | Ph2 - registration | ADA-SCID (severe combined immune deficiency) | Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated addition of human ADA gene | Blood Disorder | Genetically Modified Cell Therapy | $20,000,000 | HSC | Autologous | In ADA-SCID allogeneic HSCTs from non-matched sibling donors are a high risk procedure. Efficacy of chronic enzyme replacement therapy is uncertain in the long-term. Preliminary data indicates that OTL-101 may significantly improve outcomes compared to available therapies. | Primary: Safety. Secondary: Efficacy, gene marking, immune reconstitution. Registrational trial. | 1/1/17 | 12/31/21 | |
| CLIN2-09504 Sorrentino, St. Jude's | Clinical Trial Stage Projects | Ph 1/2 | X-SCID (X-linked severe combined immunodeficiency) | Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated correction | Blood Disorder | Genetically Modified Cell Therapy | $11,924,780 | HSC | Autologous | Catastrophic immunodeficiency disorder caused by mutation in IL2RG; Without a curative transplant-based therapy, X-SCID is lethal typically in first year of life. | feasibility. Secondary: Efficacy; gene marking; immune reconstitution | 4/1/17 | 3/31/22 | |
| DR2A-05365 Shizuru, Stanford | Disease Team Therapy Development | IND, Ph 1 | Conditioning regimen for allogeneic HSC transplantation for SCID (Severe Combined Immunodeficiency) | MAb that depletes endogenous HSC | Blood Disorder | Biologic | $19,068,382 | --- | --- | Monoclonal antibody that targets CD117 and promotes engraftment of hematopoietic stem cells. Could replace toxic conditioning regimens and enable chemotherapy-free transplants. Enabled donor cell HSC engraftment and cure of disease in an animal model of SCID. | Safety. Dosing. Efficacy - HSC engraftment, immune reconstitution. | 8/1/13 | 7/31/18 | |
| CLIN1-08363, Puck, UCSF | Late Stage Preclinical Projects | IND | ART-SCID (Artemis-deficient severe combined immunodeficiency) | Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated correction | Blood Disorder | Genetically Modified Cell Therapy | 4,268,865 | HSC | Autologous | Primary immune deficiency due to Artemis gene. Most difficult to treat by allogeneic hematopoietic stem cell transplantation (HSCT) due to increased sensitivity to alkylating agents and radiation. Autologous gene modified HSCT transplantation to overcome allogeneic stem cell transplant difficulty. | Obtain an active IND | 5/1/16 | 10/31/17 | |
| CLIN1-10084, Porteus, Stanford | Late Stage Preclinical Projects | IND | Sickle Cell Disease | Autologous HSC, genetically corrected ex vivo by CRISPR-mediated correction | Blood Disorder | Genetically Modified Cell Therapy | $5,194,431 | HSC | Autologous | Gene editing using CRISPR-Cas9 technology has the potential to correct the sickle cell mutation. | Obtain an active IND | 11/1/17 | 4/30/19 | |
| CLIN2-10847 Rosenthal, COH | Clinical Trial Stage Projects | Ph 1 | Sickle Cell Disease | Allogeneic haploidentical HSC | Blood Disorder | Cell Therapy | $5,742,180 | HSC | Allogeneic | Transplant of blood-forming stem cells from a donor to a patient that has received a milder, less toxic chemotherapy conditioning regimen that removes some but not all of the patients diseased bone marrow stem cells. The donor cells are depleted of T immune cells to allow engraftment without causing an immune reaction in the recipient. | Safety. Efficacy. Mixed chimerism. | 04/1/18 | 4/30/22 | |
| CLIN2-11031 Conner, Sangamo | Clinical Trial Stage Projects | Ph 1/2 | Beta Thalassemia | Autologous HSC, genetically corrected ex vivo by zinc finger nuclease mediated correction | Blood Disorder | Genetically Modified Cell Therapy | | HSC | Autologous | Beta thalassemia is a severe form of anemia caused by mutations in the hemoglobin gene. Patients require life-long blood transfusions and have a life expectancy of only 30-50 years. The Sangamo therapy takes a patient's own blood stem cells and, using a gene-editing technology called zinc finger nuclease (ZFN), provides a functional copy of the hemoglobin gene. The modified cells are given back to the patient which potentially will eliminate the need for chronic transfusions and the associated complications. | Safety and tolerability. Efficacy, change from baseline HbF levels, frequency and volume of RBC transfusions. | | | |
| CLIN2-10830 Cowan, UCSF | Clinical Trial Stage Projects | Ph 1/2 | Artemis-deficient severe combined immunodeficiency | Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated correction | Blood Disorder | Genetically Modified Cell Therapy | | HSC | Autologous | Artemis-deficient severe combined immunodeficiency is a genetic blood disorder in which even a mild infection can be fatal. It is the most difficult form of the disease to treat. The UCSF team will genetically modify the patient's own blood stem cells with a functional copy of the Artemis gene, with the goal of creating a functional immune system. | Safety and efficacy. Multilineage engraftment persistence and B cell reconstitution. | | | |
| **HIV/AIDS** | | | | | | | | | | | | | | |
| DR1-06893 Symonds, Calimmune | Disease Team I | Ph 1/2a | HIV/AIDS | Autologous HSC transduced ex vivo with a lentiviral vector engineered to express an shRNA against CCR5 & a fusion inhibitor. | HIV/AIDS | Genetically Modified Cell Therapy | $8,278,722 | HSC | Autologous | Cal-1 increases the number of HIV-protected cells in the body. Uses shRNA to CCR5 and C46 to confer cellular resistance to HIV infection. | Safety. Efficacy - slow disease progression, mitigate need for ART. | 2/1/13 | 7/31/16 | |
| CLIN2-08289 Abedi, UC Davis | Clinical Trial Stage Projects | Ph 1 | HIV/AIDS | Gene modified HSCs via a lentiviral vector that encodes a triple combination of HIV-resistance genes and a tCD25 pre-selective marker | HIV/AIDS | Genetically Modified Cell Therapy | $7,402,549 | HSC | Autologous | Lentiviral vector encodes a triple combination of HIV-resistance genes and a pre-selective marker. Vector transduced CD34+ cells will safely engraft, divide and differentiate in vivo into mature myeloid and lymphoid cells. | Safety. Efficacy - immune reconstitution, viral load and HIV status. | 9/1/15 | 8/31/19 | |
| SP3A-07536 Zaia, City of Hope | Strategic Partnership III | Ph 1 | HIV/AIDS | Autologous HSCs genetically modified to disrupt CCR5 | HIV/AIDS | Genetically Modified Cell Therapy | $5,583,438 | HSC | Autologous | Autologous hematopoietic stem cells gene edited ex vivo to eliminate expression of HIV entry co-receptor CCR5. Cells carrying disrupted CCR5 provide a renewable, long-lasting source of HIV-1 resistant immune cells. | Safety. Efficacy - engraftment. | 4/1/15 | 3/31/19 | |
| **Hematologic Cancers** | | | | | | | | | | | | | | |
| DR3-06924 Kipps, UCSD | Duane Roth Disease Team Therapy Development III | Ph 1 | CLL | Monoclonal antibody (anti-ROR1) targeting CLL cancer stem cells | Hematologic Malignancy | Biologic | $4,179,600 | --- | --- | Cancer is a leading cause of death in CA. Many cancers resist current therapies due to therapy-resistant cancer stem cells (CSCs). Discovered a protein, ROR1, present on CSCs but not on normal healthy cells. Developed an antibody, cirmtuzumab, that is specific for ROR1. Project plan is to treat chronic lymphocytic leukemia with cirmtuzumab. | Safety. Dosing. Follow on trials will include other cancers and will test cirmtuzumab alone or in combination with other anti-cancer therapies. | 6/1/14 | 11/30/17 | |
| CLIN2-10192 Kipps, UCSD | Clinical Trial Stage Projects | Ph 1b/2a | B Cell Cancers | Monoclonal antibody (anti-ROR1), combined with tyrosine kinase inhibitor Ibrutinib | Hematologic Malignancy | Biologic | $18,292,674 | --- | --- | Cancer is a leading cause of death in CA. Many cancers resist current therapies due to therapy-resistant cancer stem cells (CSCs). Discovered a protein, ROR1, present on CSCs but not on normal healthy cells. Developed an antibody, cirmtuzumab, that is specific for ROR1. Project plan is to treat chronic lymphocytic leukemia or mantle cell carcinoma with cirmtuzumab in combination with ibrutinib. | Evaluate dosing and complete response rate. | 10/1/17 | 3/31/22 | |

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| CLIN1-08342 Davis, Angiocrine Bioscience | Clinical Trial Stage Projects | IND | Hematologic malignancies including leukemia and lymphoma | Matched cord blood derived hematopoietic stem and progenitor cells expanded by co-culture with genetically modified endothelial cells. | Hematologic Malignancies | Cell Therapy | $3,797,117 | Expanded CD34+ stem and progenitor cells from cord blood and gene-modified endothelial cells | Allogeneic | CD34+ hematopoietic Stem and progenitor cells engraft into the bone marrow of patients, rebuilding a new blood and immune system after appropriate preparation called myeloablation. The endothelial cells used in the co-culture are thought to aid the engraftment of the stem and progenitor cells into the bone marrow via secretion of angiocrine factors. The remainder of the cord blood cells in the cell product also aid in the engraftment as well as provide anti-viral and anti-bacterial effects after transplantation. | Obtain an active IND | 4/1/16 | 12/1/17 | |
| CLIN2-10386 Finnegan, Angiocrine Bioscience | Clinical Trial Stage Projects | Ph 1b | Hematologic malignancies including leukemia and lymphoma | Matched cord blood derived hematopoietic stem and progenitor cells expanded by co-culture with genetically modified endothelial cells cells. | Hematologic Malignancies | Cell Therapy | $5,000,000 | Expanded CD34+ stem and progenitor cells from cord blood and gene-modified endothelial cells | Allogeneic | CD34+ hematopoietic Stem and progenitor cells engraft into the bone marrow of patients, rebuilding a new blood and immune system after appropriate preparation called myeloablation. The endothelial cells used in the co-culture are thought to aid the engraftment of the stem and progenitor cells into the bone marrow via secretion of angiocrine factors. The remainder of the cord blood cells in the cell product also aid in the engraftment as well as provide anti-viral and anti-bacterial effects after transplantation. | Safety. | 10/01/17 | 12/31/21 | |
| CLIN1-09776 Junutula, Cellerant | Late Stage Preclinical Projects | IND | AML | Anti-CLL1 antibody linked to a DNA binding payload. | Hematologic Malignancy | Antibody-drug conjugate (ADC) | $6,863,755 | --- | --- | ADCs are intended to target and kill only the target cancer cells and spare healthy cells. ADCs are composed of an antibody linked to a cytotoxic payload or drug. After the ADC binds to the target cell and is internalized, the cytotoxic drug is released and kills the cancer cell. CLL1 is highly expressed on leukemia stem cells but not on normal cells. Binding of the anti-CLL1 ADC results in targeted killing of leukemia stem cells. | Obtain an active IND | 8/1/17 | 1/31/19 | |
| CLIN2-09574 Delaney, Nohla | Clinical Trial Stage Projects | Ph 2 | Chemotherapy-induced neutropenia in the AML setting | Ex-vivo expanded Umbilical cord blood hematopoietic stem and progenitor cells | Hematologic Malignancy | Cell Therapy | $6,922,109 | Expanded CD34+ stem and progenitor cells from cord blood | Allogeneic | The product is an ex vivo expanded hematopoietic stem and progenitor cell therapy capable of providing bridging (temporary) hematopoietic support to protect patients against infections. It is intended for AML cancer patients undergoing chemotherapy that results in neutropenia. | Evaluate effect on the rate of infections associated with Chemotherapy-Induced Neutropenia in AML patients and determine optimal dose | 11/01/17 | 11/30/19 | |
| CLIN2-10144 Chao, 47Inc | Clinical Trial Stage Projects | Ph 1b | AML | Anti-CD47 monoclonal antibody in combination with azacitidine | Hematologic Malignancy | Biologic | $5,000,000 | --- | --- | CD47 is overexpressed on cancer and cancer stem cells. It stops phagocytic macrophages from eliminating these abnormal cells by delivering a potent "don't eat me" signal. Hu5F9-G4 is a humanized monoclonal antibody (mAb) that binds to CD47 and blocks the "don't eat me" signal, thereby enabling macrophage-mediated phagocytosis of the cancer cells. | Phase 1b trial; determine optimal dose; safety and efficacy in combination with azacitidine | 11/01/17 | 5/31/21 | |
| Pulsipher, CHLA | Clinical Trial Stage Projects | Ph 1/2 | Viral infection | Partially HLA-matched virus-specific T cells | HSC transplant-related infection | Cell Therapy | $4,825,587 | T Cell | Allogeneic | | | 12/01/17 | 11/30/22 | |
| CLIN2-10395 Spear, Poseida | Clinical Trial Stage Projects | Ph 1 | Multiple myeloma | CAR-T | Hematologic Malignancy | Cell Therapy | $19,997,927 | CAR-T | Autologous | MM is a treatable but typically incurable plasma cell malignancy that is usually fatal. Currently available therapeutic options have limitations in efficacy and are generally associated with significant toxicity and complications. Hence, there remains an unmet need for effective and durable MM therapy. CAR-T immunotherapy is emerging as an important potential therapeutic approach for cancer, including MM. Being stem cell memory CAR-T cells, the treatment could potentially produce long term control. | Determination of maximum tolerated dose. | 12/01/17 | 12/31/21 | |
| CLIN2-10846 Mackall, Stanford | Clinical Trial Stage Projects | Ph 1 | B Cell Cancers | CAR-T | Hematologic Malignancy | Cell Therapy | | CAR-T | Autologous | Chimeric Antigen Receptor (CAR) T Cell Therapy is an innovative cancer therapy with very encouraging response rates in patients. The therapy works by isolating a patient's own T cells and then genetically engineering them to recognize a target protein on the surface of cancer cells, triggering their destruction. In some patients with B cell leukemias, however, cancer cells are able to remove the target protein to escape detection by the modified T cells, thereby causing the cancer's reoccurrence. Stanford researchers have developed an engineered T cell designed to recognize not one, but two, cell surface proteins on cancer cells with the aim of overcoming loss of the target protein and enhancing a patient's response to the therapy and reducing the potential for relapse. | | | | |
| **Solid Cancers** | | | | | | | | | | | | | | |
| DR2A-05309 Ribas, UCLA | Disease Team Therapy Development | IND, Ph 1 | Advanced tumors (Synovial Sarcoma, Melanoma, Ovarian) | Autologous HSCs and T cells genetically modified to express an anti-tumor T cell receptor. | Solid Tumor | Genetically Modified Cell Therapy | $19,999,563 | HSC | Autologous | There are few options for patients whose cancers have metastasized due to resistance to current therapies. Engineering of patient's own blood-forming stem cells to produce a continual supply of the immune system cell to attack cancer. | Primary: Safety and feasibility. Secondary: Persistence of gene-marked anti-cancer immune cells | 4/1/14 | 11/30/20 | |
| DR3-07067 Slamon, UCLA | Duane Roth Disease Team Therapy Development III | Ph 1 | Solid Tumor | Small molecule mitotic inhibitor targeting serine/threonine kinase to eliminate both tumor cells and cancer stem cells | Solid Tumor | Small Molecule | $6,924,317 | --- | --- | Solid tumors are the most prevalent form of cancer, and are a major cause of death worldwide. The small molecule being developed inhibits the activity of a protein required in tumor cell lines and cancer stem cells (CSC). It is hypothesized that inhibiting the CSC can prevent tumor regrowth after treatment. | Determination of maximum tolerated dose and recommended Phase 2 dose. Safety. PK. Efficacy in solid cancers. | 5/1/14 | 4/30/18 | |
| CLIN2-09577 Chao, 47Inc | Clinical Trial Stage Projects | Ph1b/2 | Solid Tumor | Anti-CD47 monoclonal antibody + cetuximab | Solid Tumor | Biologic | $10,234,048 | Ab | _ | CD47 is overexpressed on cancer and cancer stem cells and prevents their elimination by phagocytic macrophages by delivering a potent "don't eat me" signal. Hu5F9-G4 is a humanized monoclonal antibody (mAb) that binds to CD47 and blocks its interaction with its receptor, thereby enabling phagocytosis of cancer cells. Anti-CD47 is highly synergistic in combination with other anti-cancer therapies including tumor-targeting mAbs such as cetuximab. | Safety. Dosing. Efficacy - objective response rate (ORR) | 1/1/17 | 12/31/21 | |
| CLIN2-10248 Brown, COH | Clinical Trial Stage Projects | Ph 1 | Malignant Glioma | T cells engineered to target cancer stem cells | Solid Tumor | Genetically Modified Cell Therapy | $12,753,854 | CAR-T | Autologous | Gliobastoma (GBM) is lethal with 5 year survival rate is only 5.5%. CAR-T are "living drug" with potential to actively seek out and destroy malignant cells. This study clinical study will investigate two different routes of local delivery of CAR-T cells to target and eliminate IL13R expressing tumor cells | Safety, Feasibility, persistence, biodistribution and biological activity | 11/1/17 | 10/31/21 | |

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| CLIN1-10893 Abbot, Fate | Late Stage Preclinical Projects | IND | Advanced solid tumors | iPS-derived NK cells | Solid Tumor | Cell Therapy | $4,000,000 | iPS-NK | Allogeneic | | | 04/1/18 | 6/30/19 |
| **ORGAN SYSTEMS THERAPEUTICS** | | | | | | | | | | | | | |
| **Bone Disorders** | | | | | | | | | | | | | |
| DR2A-05302 Lane, UC Davis | Disease Team Therapy Development | Ph 1 a/b | Osteonecrosis | Synthetic molecule, LLP2A-Ale, to enhance homing of endogenous bone marrow MSCs to bone surface | Bone Disorder | Small Molecule | $19,999,867 | --- | --- | Femoral head osteonecrosis (aka avascular necrosis) is a disease caused by loss of blood supply to the bone, leading to bone cell death, end stage hip arthritis and total hip replacement. There is an unmet need for treatment of this disease, that affects individuals at prime of life (peak age 35 years). This small molecule therapeutic recruits bone forming cells to site of damage, where they serve the dual function of laying down new bone, and stimulating revascularization to prevent further bone cell death. | Safety, tolerability. Determine PK. Determine PD effects on bone turnover, biomarkers. Determine immunogenicity. | 1/1/13 | 12/31/17 |
| **Cartilage Disorders** | | | | | | | | | | | | | |
| CLIN1-09472 Wang, Cellular Biomedicine Group | Late Stage Preclinical Projects | IND | Osteoarthritis | Allogeneic adipose-derived MSCs | Cartilage Disorder | Cell Therapy | $2,291,976 | MSC | Allogeneic | | Obtain an active IND | 3/1/17 | 3/31/18 |
| CLIN1-08309 Schultz, Calibr | CLIN1 | IND | Osteoarthritis, cartilage injuries | Small molecule injected intra-articularly that promotes resident cartilage mesenchymal stem cell differentiation into chondrocytes | Cartilage Disorder | Small Molecule | $1,667,832 | --- | --- | | File an IND | 9/1/15 | 3/31/17 |
| CLIN2-10388 Sahelijo, Calibr | CLIN2 | Ph 1 | Osteoarthritis, cartilage injuries | Small molecule injected intra-articularly that promotes resident cartilage mesenchymal stem cell differentiation into chondrocytes | Cartilage Disorder | Small Molecule | $8,447,523 | --- | --- | | | 12/01/17 | 11/30/20 |
| **Cardiovascular & Vascular Disorders** | | | | | | | | | | | | | |
| DR2A-05735 Smith, Capricor Inc. | Disease Team Therapy Development | Ph 2 | Heart dysfunction after myocardial infarction/Chronic heart failure | Allogeneic cardiosphere derived cells | Cardiovascular Disease | Cell Therapy | $19,782,136 | CDC | Allogeneic | Heart failure is a progressive disease with a high risk of mortality. Cardiosphere-derived cells (CDCs) reduce scar size after heart attack in preclinical animal models and in a prior clinical trial. | Primary: Determine whether treatment is safe and causes reduction in cardiac scar size in patients with heart failure after a heart attack. Secondary: Assess for other structural or functional cardiac benefits. | 1/1/13 | 12/31/17 |
| CLIN2-09444 Lewis, Cedars-Sinai | Clinical Trial Stage Projects | Ph1a/b | Pulmonary Arterial Hypertension | Allogeneic cardiosphere derived cells | Vascular Disease | Cell Therapy | $7,354,772 | CDC | Allogeneic | Pulmonary arterial hypertension (PAH) is a progressive condition with no cure, survival is poor. Cardiosphere-derived cells (CDCs) decrease wall thickening of lung small blood vessels in preclinical studies. Improvement in lung blood vessels is expected to reduce cardiac right ventricular dysfunction. | Primary: Safety. Secondary: Exploratory efficacy measures of right ventricular function. | 1/1/17 | 4/30/21 |
| CLIN2-08334 Ascheim, Capricor, Inc. | Clinical Trial Stage Projects | Ph 2 | Duchenne muscular dystrophy cardiomyopathy | Allogeneic cardiosphere derived cells | Skeletal Muscle Disorder | Cell Therapy | $3,376,259 | CDC | Allogeneic | Heart failure is a leading cause of death for Duchenne muscular dystrophy patients. Cardiosphere-derived cells (CDCs) decrease myocardial fibrosis, improve cardiac function and induce regeneration of heart muscle in preclinical models of DMD. | Primary: Safety and tolerability in DMD patients. Secondary: Structural or functional cardiac benefits, quality of life improvements. | 4/1/16 | 10/31/18 |
| DR2A-05394 Wu, Stanford | Disease Team Therapy Development | IND | Ischemic heart failure | Allogeneic hESC-derived cardiomyocytes | Cardiovascular Disease | Cell Therapy | $19,060,330 | CM | Allogeneic | 5.7 million Americans suffer from heart failure, and the end stage 2 year survival rate is 50%. hESC-CM promote new blood vessel formation and improve cardiac function in preclinical models of heart failure. | Obtain an active IND for a first-in-human trial in heart failure patients. | 4/1/13 | 3/31/18 |
| **Diabetes & Complications/Metabolic** | | | | | | | | | | | | | |
| AP1-08039 Foyt, ViaCyte Inc. | Accelerated Pathway I | Comparability Trial | Diabetes: Type 1 | Allogeneic hESC-derived pancreatic cell progenitors in a device implanted subcutaneously | Endocrine Disorder | Cell Therapy, Combination | $16,603,160 | Pancreatic endocrine progenitor | Allogeneic | Diabetes mellitus affects 370 million people worldwide. Disproportionately affects certain minority groups and the elderly. Current therapy is self-administration of insulin. Diabetes costs in CA are tens of billions of dollars each year. Directed differentiation of embryonic stem cells to pancreatic precursor cells. Project plan is transplantation of pancreatic precursor cells that generate functional islet tissue in vivo that can respond to insulin levels in a more physiological manner than direct insulin replacement. | Primary: Safety. Secondary: Efficacy. | 1/1/15 | 12/31/17 |
| CLIN2-09730 Losordo, Caladrius | Clinical Trial Stage Projects | Ph 2 | Diabetes: Type 1 | Autologous ex vivo expanded polyclonal regulatory T cells | Endocrine Disorder | Cell Therapy | $12,211,255 | T-reg | Autologous | Children with T1D face lifelong struggles with glycemic control and, despite careful management, an increased risk of severe complications. No therapy that maintains or restores pancreatic beta islet cell function is currently approved. Evidence indicates that regulatory T-cells (T-regs) maintain immune balance at least in part by control of differentiation of multipotent progenitor/stem cells. | Primary: Safety. Secondary: Efficacy. | 4/1/17 | 7/31/20 |
| CLIN1-08671, D'Amour, Viacyte | Clinical Trial Stage Projects | IND | Diabetes: Type 1 | hESC-derived pancreatic progenitor cells delivered in a device that allows direct vascularization of the cell therapy | Endocrine Disorder | Cell Therapy, Combination | $3,984,164 | Pancreatic endocrine progenitor | Allogeneic | There are over 100,000 people in the US with type 1 diabetes so severe that they are at constant risk of hospitalization and/or death. Within months after administration, this product could provide a source of insulin producing beta cells to restore those patients' blood sugar to normal healthy levels and save their lives. | Obtain an active IND and trial start up | 8/1/16 | 7/31/17 |

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| CLIN2-09672, Foyt, Viacyte | Clinical Trial Stage Projects | Ph 1/2 | Diabetes: Type 1 | hESC-derived pancreatic progenitor cells delivered in a device that allows direct vascularization of the cell therapy | Endocrine Disorder | Cell Therapy, Combination | $20,000,000 | Pancreatic endocrine progenitor | Allogeneic | There are over 100,000 people in the US with type 1 diabetes so severe that they are at constant risk of hospitalization and/or death. Within months after administration, this product could provide a source of insulin producing beta cells to restore those patients' blood sugar to normal healthy levels and save their lives. | Primary: Safety and Tolerability | 10/1/17 | 12/31/20 |
| **Kidney Disorders** | | | | | | | | | | | | | |
| CLIN2-08938, Lawson, Humacyte, Inc. | Clinical Trial Stage Projects | Ph 3 | Renal dialysis | A Human Acellular Vessel in Patients Needing Renal Replacement Therapy: A Comparison with ePTFE Grafts as Conduits for Hemodialysis (HUMANITY) | Endocrine Disorder | Device | $9,999,528 | | Allogeneic | Synthetic vascular access grafts for hemodialysis in kidney patients are associated with thrombosis, infection and abandonment. Human Acellular Vessel (HAV) is made of extracellular matrix from human smooth muscle cells, similar in composition and structure to native tissue. | Primary: Safety and tolerability, rate of patency of the graft and rate of interventions needed to restore patency. | 8/1/16 | 7/31/21 |
| CLIN2-09688, Lawson, Humacyte, Inc. | Clinical Trial Stage Projects | Ph 3 | Renal dialysis | A Human Acellular Vessel in Patients Needing Renal Replacement Therapy. | Endocrine Disorder | Device | $14,082,865 | | Allogeneic | Synthetic vascular access grafts for hemodialysis in kidney patients are associated with thrombosis, infection and abandonment. Human Acellular Vessel (HAV) is made of extracellular matrix from human smooth muscle cells, similar in composition and structure to native tissue. | A Comparison with AV Fistula | 11/01/17 | 3/31/22 |
| CLIN2-09439 Strober, Stanford | Clinical Trial Stage Projects | Ph 1 | Transplant tolerance | Donor CD34+ and CD3+ T cells for immune tolerance to HLA mismatched kidney donors. | Immune tolerance, transplant | Cell Therapy | $5,069,674 | HSC | Allogeneic | Unmet medical need for allogeneic kidney transplants. Need to eliminate chronic rejection/allograft nephropathy that causes gradual loss of kidney (50% of graft loss by 12-15 years in HLA mismatched recipients). Eliminate the lifelong need for anti-rejection drugs that have numerous cumulative side effects. | Primary: Safety. Secondary: Preliminary efficacy. | 2/1/17 | 1/31/21 |
| Clin1-09230 Cherqui, UCSD | Clinical Trial Stage Projects | IND | Cystinosis | Ex vivo transduced autologous human CD34+ hematopoietic stem cells for treatment of cystinosis | Cystinosis | Genetically Modified Cell Therapy | $ 5,273,189 | HSC | Autologous | Cystinosis is caused by a genetic mutation that allows an amino acid, cystine, to build up in and damage the kidneys, eyes, liver, muscles, pancreas and brain of children and adults. Current therapy only delays progression of the disease, has severe side effects and people taking it still require kidney transplants, and develop diabetes, neuromuscular disorders and hypothyroidism. The goal is to take blood stem cells from people with cystinosis, genetically-modify them to remove the mutation, then return them to the patient to create a new, healthy, blood system free of the disease. | Obtain an active IND | 11/1/16 | 10/31/18 |
| CLIN2-10411 Deitcher, Medeor | Clinical Trial Stage Projects | Ph 3 | Transplant tolerance | Donor CD34+ and CD3+ T cells for immune tolerance to HLA mismatched kidney donors. | Immune tolerance, transplant | Cell Therapy | $18,763, 585 | HSC | Allogeneic | Unmet medical need for allogeneic kidney transplants in HLA-matched patients. Eliminate the lifelong need for anti-rejection drugs that have numerous cumulative side effects. | Efficacy and Safety | 3/1/18 | 12/31/22 |