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CIRM-FUNDED CLINICAL TRIALS

July 2019

BLOOD DISEASES

Alpha Thalassemia Major

UC San Francisco

Team Lead: Tippi MacKenzie

Dr. MacKenzie is using hematopoietic stem cells (HSCs) to treat babies in the womb who have alpha thalassemia major, a blood disorder that is almost always fatal. The HSCs are taken from the mother's bone marrow and transplanted into the baby before birth. The mother's cells are able to survive and correct the defect in the baby's blood cells, increasing the chances of a healthy birth and improving the chances of having effective treatments after birth.

Beta Thalassemia

Sangamo Therapeutics

Team Lead: Ed Conner

UC San Francisco researchers aim to repair the damaged immune system of children born with severe combined immunodeficiency (SCID), a genetic blood disorder in which even a mild infection can be fatal. This trial will focus on SCID patients who have mutations in a gene called Artemis, the most difficult form of SCID to treat when using a standard bone marrow transplant from a healthy donor. The team will genetically modify the patient's own blood stem cells with a functional copy of Artemis, with the goal of creating a new blood system and restoring the health of the immune system.

Blood Cancer

Angiocrine Bioscience Inc.

Team Lead: Paul Finnegan

Angiocrine is developing a cell therapy aimed to improve the availability and engraftment of blood stem cell transplants for cancer patients who have had their cancerous bone marrow removed by chemotherapy. The cell therapy is made of blood stem cells and endothelial cells, which line blood vessels and are thought to improve the engraftment of the stem cell transplant into the bone marrow. The hope is that this treatment will provide a safer, more tolerable and effective stem cell transplantation that will rebuild the patient's immune system cancer-free

Blood Cancer

Angiocrine Bioscience Inc.

Team Lead: Edward Kavalierchik

Angiocrine will use genetically engineered cells, derived from cord blood, to see if they can help alleviate or accelerate recovery from the toxic side effects of chemotherapy for people undergoing treatment for lymphoma and other aggressive cancers of the blood or lymph system.

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Bone Marrow Transplant And Viral Infection

Children's Hospital of Los Angeles

Team Lead: Michael Pulsipher

Viral infection can lead to fatal complications in patients with weakened immune systems resulting from chemotherapy, bone marrow or cord blood transplant, and other forms of inherited or acquired disorders. A team at Children's Hospital of Los Angeles is testing the feasibility of providing these immune suppressed patients with engineered T-cells to fight these viruses. Donated virus-specific T-cells will be matched to the patient's immune system to help boost their ability to fight off these viruses and to provide longer-term anti-viral protection.

Chronic Granulomatous Disease

UCLA

Team Lead: Donald Kohn

Chronic granulomatous disease is a rare immune disorder that results in severe, recurrent infections that can impact quality and length of life. A team at UCLA uses the patient's own genetically modified blood stem cells to create a new blood supply and a healthy immune system, with the aim of curing patients with this disease.

Leukocyte Adhesion Deficiency-I (LAD-I)

Rocket Pharmaceuticals, Inc.

Team Lead: Dr. Kinnari Patel

Rocket Pharmaceuticals is using blood stem cells to treat infants with Leukocyte Adhesion Deficiency-I (LAD-I), a rare pediatric disease caused by a mutation in a specific gene that affects the body's ability to combat infections. The team will test a treatment that modifies an infant's own blood stem cells and inserts a functional version of the gene. These modified stem cells are then reintroduced back into the infant, with the hope of creating a new blood supply and repairing the immune system, thereby enabling the body to combat infections.

Severe Combined Immunodeficiency (SCID)

UCLA

Team Lead: Donald Kohn

A team at UCLA is using a patient's own blood stem cells to try and repair their damaged immune system. They will use what's called a lentiviral vector to deliver genetic material into the blood stem cells, correcting the genetic flaw that causes SCID. It's hoped this will create a new blood system and a healthy immune system.

Stanford University

Team Lead: Judith Shizuru

A team at Stanford proposes to replace SCID patients' dysfunctional immune cells with healthy ones using a safer form of bone marrow transplant (BMT). Current BMT procedures must use toxic chemotherapy to make space in the bone marrow

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for the healthy transplanted stem cells to engraft. The Stanford team will instead test a safe, non-toxic protein that targets and removes the defective blood forming stem cells. If successful, the procedure could open up similar BMT therapies to patients with other auto-immune diseases such as multiple sclerosis, lupus or diabetes that are generally not candidates for BMT currently.

St. Jude Children's Research Hospital

Team Lead: Brian Sorrentino

St. Jude Children's Research Hospital is teaming up with UC San Francisco to repair the damaged immune system of children born with SCID. They will genetically modify the patient's own blood stem cells, with the goal of creating a new blood system and restoring the health of the immune system.

UC San Francisco

Team Lead: Morton Cowan

UC San Francisco researchers aim to repair the damaged immune system of children born with severe combined immunodeficiency (SCID), a genetic blood disorder in which even a mild infection can be fatal. This trial will focus on SCID patients who have mutations in a gene called Artemis, the most difficult form of SCID to treat when using a standard bone marrow transplant from a healthy donor. The team will genetically modify the patient's own blood stem cells with a functional copy of Artemis, with the goal of creating a new blood system and restoring the health of the immune system.

Sickle Cell Disease

City of Hope (Severe Sickle Cell Disease)

Team Lead: Joseph Rosenthal

Scientists at the City of Hope are conducting a Phase 1 clinical trial testing a stem cell-based therapy for adult patients with severe sickle cell disease (SCD) - a chronic, debilitating blood disease. The therapy involves transplanting blood-forming stem cells from a donor into a patient who has received a milder, less toxic chemotherapy treatment that removes some but not all of the patient's diseased bone marrow stem cells. This allows the donor stem cells to engraft and create a healthy supply of non-diseased blood cells without causing an immune reaction in the patient. The hope is that this treatment will cure patients with more severe forms of SCD who aren't able to benefit from currently available blood stem cell transplants that require the administration of more toxic chemotherapy drugs.

UCLA

Team Lead: Donald Kohn

A team at UCLA is genetically modifying a patient's own blood stem cells to produce a correct version of hemoglobin, the protein that is mutated in these patients, which causes abnormal sickle-like shaped red blood cells. These misshapen cells lead to dangerous blood clots, debilitating pain and even death. The genetically

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modified stem cells will be given back to the patient to create a new sickle cell-free blood supply.

BONE DISEASES

Osteoarthritis

California Institute for Biomedical Research

Team Lead: Leonardo Sahelijo

Researchers at the California Institute for Biomedical Research (CALIBR) have been awarded \$8.447 million to test KA34, a drug that, in preclinical tests, recruits stem cells to create new cartilage in areas damaged by osteoarthritis. CIRM funded the research that developed this technology and now this Phase 1 trial will test this stem cell directed treatment in people with osteoarthritis of the knee, hopefully slowing down or even halting the progression of the disease.

Osteonecrosis

UC Davis

Team Lead: Nancy Lane

A team at UC Davis is testing a drug that directs bone stem cells to the surface of the bone where they then develop new bone tissue and stimulate new blood vessel formation, two defects underlying osteonecrosis. Should this drug prove safe and show signs of effectiveness, it may be tested for the treatment of other bone diseases like osteoporosis.

CANCER, BLOOD

Acute Myeloid Leukemia (AML)

Forty Seven, Inc.

Team Lead: Mark Chao

Forty Seven Inc. is conducting a Phase 1b clinical trial for acute myeloid leukemia patients. Leukemia stem cells have a protein on their surface that enable them to evade being identified and destroyed by the patient's own immune system. This protein also helps these leukemia stem cells survive traditional therapies such as chemotherapy, enabling the cancer to lie dormant for a period before returning and causing the patient to relapse. The team is using a combination of a monoclonal antibody and the drug Azacitidine to make the leukemia stem cells vulnerable to being attacked and destroyed by the immune system.

Nohla Therapeutics Inc.

Team Lead: Colleen Delaney

Nohla Therapeutics is testing a hematopoietic stem cell and progenitor cell therapy called NLA101 to help patients suffering from neutropenia, a condition that leaves people susceptible to deadly infections, after receiving chemotherapy for acute

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myeloid leukemia (AML). The company is currently launching a Phase 2 trial to test this treatment in adult AML patients that have received high-dose chemotherapy.

B Cell Cancers

Stanford University

Team Lead: Crystal Mackall

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 (a type of immune cell) and then genetically engineering them to recognize a protein on the surface of cancer cells, triggering their destruction. In some patients with B cell leukemias, however, cancer cells escape detection by the modified T cells and cause the cancer's reoccurrence. Researchers at the Stanford University School of Medicine have developed an engineered T cell designed to recognize not one, but two, cell surface proteins on cancer cells with the aim of enhancing a patient's response to the therapy and reducing the potential for relapse. In addition, some of the T cells will form memory stem cells that will survive for years and continue to survey the body, killing any new or surviving cancer cells.

Blood Stem Cell Transplants For Cancer Patients

Angiocrine Biosciences

Team Lead: Paul Finnegan

The Angiocrine team is developing a cell therapy aimed to improve the availability and engraftment of blood stem cell transplants for cancer patients who have had their cancerous bone marrow removed by chemotherapy. The cell therapy is made of blood stem cells and endothelial cells, which line blood vessels and are thought to improve the engraftment of the stem cell transplant. The hope is that this treatment will provide a safer, more tolerable and effective stem cell transplantation that will rebuild the patient's immune system cancer-free.

Chronic Lymphocytic Leukemia (CLL)

UC San Diego (2 clinical trials)

Team Lead: Thomas Kipps

A team at UCSD is testing an antibody therapy called cirmtuzumab in a clinical trial study to treat a blood cancer, Chronic Lymphocytic Leukemia (CLL). The antibody recognizes and attaches to a protein on the surface of cancer stem cells. This attachment disables the protein which slows the growth of the leukemia and makes it more vulnerable to anti-cancer drugs. The team is also testing cirmtuzumab in combination with an approved cancer fighting drug called ibrutinib, to target cancer stem cells in a separate clinical trial. The aim is that combining cirmtuzumab with ibrutinib will improve cancer remission and long-term cancer control in patients.

Multiple Myeloma

Poseida Therapeutics

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Team Lead: Matthew Spear

Poseida Therapeutics is testing the safety of a gene modified cell therapy to treat multiple myeloma, the abnormal growth of malignant plasma cells of the immune system. The company's technology is seeking to destroy these cancerous myeloma cells with an immunotherapy approach that uses the patient's own engineered immune system T cells to seek and destroy the myeloma cells.

CANCER, SOLID TUMORS

Brain cancer (malignant glioma)

City of Hope

Team Lead: Christine Brown

A team at the City of Hope led by Dr. Christine Brown is pursuing a Phase 1 trial targeting an aggressive brain cancer called malignant glioma. City of Hope will re-engineer a patient's immune system central memory T cells (TCM cells) to express chimeric antigen receptors (CAR). These CAR-T cells will recognize a molecular marker on the surface of glioma cancer stem cells and kill the tumors. Dr. Brown's award to pursue CAR-T therapy for solid cancers comes at an exciting and opportune time with the recent U.S. Food and Drug Administration (FDA) approval of the first CAR-T therapy, called Kymriah, for patients with acute lymphoblastic leukemia, a deadly form of blood cancer.

Brain metastases (HER2-expressing)

City of Hope

Team Lead: Saul Priceman

A team at the City of Hope led by Dr. Saul Priceman is conducting a clinical trial for the treatment of breast cancer related brain metastases, which are tumors in the brain that have spread from the original site of the breast cancer, expressing high levels of a tumor protein called HER2. The therapy consists of a genetically-modified version of a patient's own T cells, which are an immune system cell that can destroy foreign or abnormal cells. The T cells are modified with a protein called a chimeric antigen receptor (CAR) that recognizes HER2. These modified T cells (CAR-T cells) are then infused into the patient's brain where they are expected to detect and destroy the HER2-expressing tumors in the brain.

Colon cancer

Forty Seven, Inc.

Team Lead: Mark Chao

Forty Seven, Inc. has developed an antibody therapy to block a protein called CD47 that is found on the surface of cancer cells. CD47 acts as a 'don't eat me' signal that tells immune cells not to eliminate the cancer cells. When this 'don't eat me' signal is blocked by the antibody, the cancer cells are 'eaten' and eliminated by the patient's immune cells. Forty Seven, Inc. will combine the anti-CD47

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antibody with cetuximab – a drug used in the treatment of solid tumors – to treat patients with advanced colorectal cancer, hitting it with a 1-2 punch to kill the tumors and prevent any recurrence.

Lung cancer

UCLA

Team Lead: Steven Dubinett

The five-year survival rate for people diagnosed with the most advanced stage of non-small cell lung cancer (NSCLC) is between one and 10 percent. To address this devastating condition, UCLA researchers are using the patient's own immune system where their dendritic cells – key cells in our immune system – are genetically modified to boost their ability to stimulate their native T cells - a type of white blood cell - to destroy cancer cells.

The investigators will combine this cell therapy with the FDA-approved therapy pembrolizumab (better known as Keytruda) a therapeutic that renders cancer cells more susceptible to clearance by the immune system.

Sarcomas and Advanced Solid Tumors

UCLA

Team Lead: Dr. Theodore Nowicki

A team at UCLA is using peripheral blood stem cells (PBSCs) and peripheral blood mononuclear cells (PBMCs) to treat patients with sarcomas and other advanced solid tumors, with an emphasis on patients with late stage or recurring tumor growth that have few options. The team will test a treatment that genetically modifies PBSCs and PBMCs to target these solid tumors. The gene modified stem cells, which have the ability to self-renew, provide the potential for a durable effect.

Skin cancer and other hard-to-treat cancers

UCLA

Team Lead: Antoni Ribas

There are few options for patients whose cancers have metastasized, or spread, due to resistance to current therapies. This team is using gene editing technology to modify a patient's own immune system cells and blood-forming stem cells with the aim of creating a continuous supply of immune cells that can recognize and attack hard-to-treat cancers.

Solid tumors

UCLA

Team Lead: Dennis Slamon

A team at UCLA is testing a drug for the treatment of cancer that works by blocking PLK4, a protein that is important in regulating cell growth, division and death.

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This protein is important for the survival of the cancer stem cell as well as the rest of the cells in a tumor. It is hypothesized that blocking this protein from working in the tumor may stop or even shrink tumor growth.

DIABETES

Type 1 Diabetes

Caladrius Biosciences

Team Lead: Douglas Losordo

Researchers at Caladrius Biosciences will take cells, called regulatory T cells (Tregs), from the patient's own immune system, expand the number of those cells in the lab and return them to the patient to reduce the autoimmune attack on the insulin-producing cells in people with type 1 diabetes.

UCSF

Team Lead: Peter Stock

Dr. Stock's clinical trial at UCSF aims to address current limitations in transplanting beta cells into patients. The trial will be using parathyroid glands to aid in the success and viability of the transplant procedure. Co-transplantation of islets and parathyroid glands, from the same donor, substantially increases beta cell survival, potentially enabling adequate long-term insulin production and removing the need for multiple donors. Additionally, the co-transplantation will occur in the patient's forearm, which allows for easier monitoring and improves the effectiveness and accessibility of islet transplants for patients.

ViaCyte, Inc. (2 clinical trials)

Team Lead: Howard Foyt

ViaCyte is developing cell therapies to replace lost beta cells for people with type 1 diabetes (T1D). The therapies are derived from human embryonic stem cells, which are partially matured into becoming pancreatic tissues (the type destroyed in T1D). The cells are inserted into a small pouch that is transplanted under the patient's skin. The transplanted cells will develop into fully matured beta cells that secrete the hormone insulin, which is needed to keep blood sugar levels at a healthy level. CIRM is funding ViaCyte's two Phase 1/2 trials testing different product candidates. The first product, VC-01, encapsulates the cells and protects them from the patient's immune system. The second product, VC-02, allows the patient's blood vessels to make direct contact with the implanted cells. VC-02 is being developed for patients with high-risk T1D.

EYE DISEASE

Macular Degeneration

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University of Southern California

Team Lead: Mark Humayun

Regenerative Patch Technologies and scientists at the University of Southern California and UC Santa Barbara, are growing specialized cells of the retina from embryonic stem cells, placing them on a single layer scaffold and implanting the combination device in the back of the eye to try to reverse blindness.

Retinitis Pigmentosa

UC Irvine and jCyte, Inc. (2 clinical trials)

Team Lead: Henry Klassen

A team at UC Irvine, is using cells called retinal progenitor cells to repair the damage caused by this vision destroying disease. The cells are injected into the back of the eye and it's hoped they will help preserve the photoreceptors attacked by RP as well as generate new photoreceptors to replace those destroyed by the disease. We funded the Phase 1 clinical trial and are now funding Phase 2.

HEART DISEASE

Duchenne Muscular Dystrophy-Associated Heart Disease

Capricor Therapeutics

Team Lead: Deborah Ascheim

Capricor is using donor cells derived from the heart to treat patients suffering from Duchenne Muscular Dystrophy (DMD), a genetic disorder that leads to progressive muscle degeneration, including heart muscle. One of the leading causes of death for children with DMD is heart failure and the aim of this treatment is to help improve heart muscle outcomes for these patients.

Pulmonary Hypertension

Cedars-Sinai Medical Center

Team Lead: Michael Lewis

A team at Cedars-Sinai Medical Center is using donor cells derived from the heart to reduce two hallmark symptoms of pulmonary hypertension: inflammation and high blood pressure in the blood vessels within the lungs. These conditions make the heart struggle to pump blood to the lungs and over time can ultimately lead to heart failure. The aim of this treatment is to delay the progression of the disease.

HIV-AIDS

HIV/AIDS

Calimmune, Inc.

Team Lead: Geoff Symonds

Calimmune is genetically modifying patients' own blood-forming stem cells (also known as bone marrow stem cells) so they can produce immune cells—the ones

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normally destroyed by the virus—that cannot be infected by the virus. The goal of this treatment is to enable the patients to clear their systems of the virus, effectively curing the disease.

City of Hope and Sangamo Therapeutics

Team Lead: John Zaia

A team at City of Hope and Sangamo Therapeutics is testing a similar method to functionally cure people with HIV. But while Calimmune is using a technique called RNA interference to block the virus, City of Hope/Sangamo are using a technology called zinc finger nuclease – a kind of molecular scissors – to snip out the target gene.

AIDS-related lymphoma

UC Davis

Team Lead: Mehrdad Abedi

A team at UC Davis is taking a patient's blood forming stem cells and inserting three anti-HIV genes into them and then returning them to the individual to help rebuild their immune system. The anti-HIV genes are then passed on to all new immune system cells and make them resistant to HIV. Because AIDS-related lymphoma is linked to the constant immune cell stimulation caused by HIV infection, getting rid of the virus should prevent return of the cancer.

KIDNEY DISEASE

Cystinosis

UC San Diego

Team Lead: Stephanie Cherqui

A team at UC San Diego will be using a gene therapy approach to modify a patient's own blood stem cells with a functional version of a defective CTNS gene that causes the rare disease Cystinosis. The defective CTNS gene causes abnormal accumulation of an amino acid called cystine in all cells of the body, which can lead to multi-organ failure, with some of the earliest and most pronounced effects on the kidneys, eyes, thyroid, muscle, and pancreas. Based on pre-clinical data, the approach is to reintroduce the corrected stem cells into the patient that give rise to blood cells that will reduce cystine buildup in affected tissues.

Dialysis

Humacyte, Inc. (2 clinical trials)

Team Lead: Jeffrey Lawson

Humacyte is using donor cells to create a bioengineered vein needed by people undergoing hemodialysis, the most common form of dialysis. In dialysis a person is connected to a machine that removes waste from their blood. The bioengineered vein is implanted in the arm and used to carry the patient's blood to and from their body during dialysis. Over time the patient's own stem cells start to populate this

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vein, in effect making it part of the patient's own body. In two separate clinical trials, the Humacyte product is being compared head-to-head with the current standard of care as well as a synthetic product that is used by some patients who are not candidates for the standard treatment.

Immune Tolerance in Kidney Transplant Patients

Medeor Therapeutics

Team Lead: Steven Deitcher

Patients who receive kidney transplants must take life-long immunosuppressive drugs to prevent their immune system from rejecting the transplant. Over time, these drugs are toxic and can increase a patient's risk of infection, heart disease, cancer and diabetes. Medeor Therapeutics has developed a stem cell-based treatment they hope will eliminate the need for immunosuppressive drugs in kidney transplant patients. Blood-forming stem cells and immune cells from the organ donor are infused into the patient receiving the donor's kidney. By introducing the donor's immune cells into the patient, the patient's immune system is able to tolerate the donor's kidney, potentially eliminating the need for immunosuppressive drugs that are normally necessary to prevent transplant rejection. Medeor is currently testing this treatment in a Phase 3 clinical trial.

Kidney failure

Stanford University

Team Lead: Samuel Strober

A team at Stanford University will work with kidney transplant patients to see if injecting blood stem cells and T cells (which play an important role in the immune system) from the kidney donor into the kidney recipient can enable the recipient to bypass the need for a life-long dependence on immunosuppressant drugs.

NEUROLOGIC DISORDERS

Amyotrophic Lateral Sclerosis (ALS, also called Lou Gehrig's Disease)

Cedars-Sinai Medical Center

Team Lead: Clive Svendsen

A team at Cedars-Sinai is transplanting millions of genetically engineered stem cells into patients with a degenerative nerve disease called ALS. When transplanted, these cells become astrocytes, the support cells that keep nerve cells functioning. Due to the genetic modifications, the cells also deliver high doses of a growth factor which has been shown to protect nerve cells. The goal of this early stage trial is to test the safety of this astrocyte replacement strategy in ALS patients.

BrainStorm Cell Therapeutics

Team Lead: Ralph Kern

BrainStorm Therapeutics is using mesenchymal stem cells that are taken from the patient's own bone marrow. These stem cells are then modified to boost their produc-

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tion of neurotrophic factors, which are known to help support and protect neurons, the cells destroyed by ALS. The CIRM funding will enable the company to test this therapy, called NurOwn®, in a Phase 3 trial involving about 200 patients.

Spinal Cord Injury

Asterias Biotherapeutics

Team Lead: Jane Lebkowski

Asterias Biotherapeutics uses cells derived from embryonic stem cells to heal the spinal cord at the site of injury. They mature the stem cells into cells called oligodendrocyte precursor cells that are injected at the site of injury where it is hoped they can repair the insulating layer, called myelin, that normally protects the nerves in the spinal cord.

Stroke

SanBio

Team Lead: Damien Bates

SanBio is carrying out a Phase 2 clinical trial using mesenchymal stem cells (MSCs) to help people suffering from chronic disability following a stroke. The MSCs are isolated from the bone marrow of healthy adult donors, and then modified to enhance their ability to promote recovery from injury by triggering the brain's natural regenerative ability. Patients with stroke can suffer from loss of mobility in certain parts of their body and the hope is that this therapy will rescue some of these problems.

CLOSED CLINICAL TRIALS

CANCER

Blood Cancers, Solid Tumors

Stanford University

Team Lead: Irving Weissman

A team at Stanford University is using a molecule known as an antibody to target cancer stem cells. This antibody can recognize CD47, a protein the cancer stem cells carry on their cell surface. The cancer cells use that protein to evade the component of our immune system that routinely destroys tumors. By disabling this protein, the team hopes to empower the body's own immune system to attack and destroy the cancer stem cells. The clinical trial testing this therapy has concluded and has led to another CIRM-funded trial by Forty-Seven, Inc.

Brain Cancer (Glioblastoma)

ImmunoCellular Therapeutics

Team Lead: Anthony Gringeri

ImmunoCellular Therapeutics is targeting six proteins that are found on the surface of cancer stem cells in glioblastoma, a brain cancer. Immune cells from the patient's

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