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T cell engineering breakthrough sidesteps need for viruses in gene-editing (July 2018)

July 11, 2018, University of California, San Francisco

In an achievement that has significant implications for research, medicine, and industry, UC San Francisco scientists have genetically reprogrammed the human immune cells known as T cells without using viruses to insert DNA. The researchers said they expect their technique—a rapid, versatile, and economical approach employing CRISPR gene-editing technology—to be widely adopted in the burgeoning field of cell therapy, accelerating the development of new and safer treatments for cancer, autoimmunity, and other diseases, including rare inherited disorders.

The new method, described in the July 11, 2018 issue of *Nature*, offers a robust molecular "cut and paste" system to rewrite genome sequences in human T cells. It relies on electroporation, a process in which an electrical field is applied to cells to make their membranes temporarily more permeable. After experimenting with thousands of variables over the course of a year, the UCSF researchers found that when certain quantities of T cells, DNA, and the CRISPR "scissors" are mixed together and then exposed to an appropriate electrical field, the T cells will take in these elements and integrate specified genetic sequences precisely at the site of a CRISPR-programmed cut in the genome.

"This is a rapid, flexible method that can be used to alter, enhance, and reprogram T cells so we can give them the specificity we want to destroy cancer, recognize infections, or tamp down the excessive immune response seen in autoimmune disease," said UCSF's Alex Marson, MD, Ph.D., associate professor of microbiology and immunology, member of the UCSF Helen Diller Family Comprehensive Cancer Center, and senior author of the new study. "Now we're off to the races on all these fronts."

But just as important as the new technique's speed and ease of use, said Marson, also scientific director of biomedicine at the Innovative Genomics Institute, is that the approach makes it possible to insert substantial stretches of DNA into T cells, which can endow the cells with powerful new properties. Members of Marson's lab have had some success using electroporation and CRISPR to insert bits of genetic material into T cells, but until now, numerous attempts by many researchers to place long sequences of DNA into T cells had caused the cells to die, leading most to believe that large DNA sequences are excessively toxic to T cells.

To view the full article, please click on this link:

https://phys.org/news/2018-07-cell-breakthrough-sidesteps-viruses-gene-editing.html#jCp

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How to make CAR-T cell therapies for cancer safer and more effective (July 2018)

Researchers are installing safety switches and adding other features to the immune therapy

By Laurel Hamers

9:00am, June 27, 2018

This wasn't 15-year-old Connor McMahon's first time in the hospital. But the 107° fever he'd been running for three days had his dad frightened. The teen was hallucinating, talking gibberish and spouting curses.

"I thought he was going to die," says Connor's father, Don McMahon, who stayed close as his son received and recovered from an experimental treatment for leukemia. "It was really hard to watch." But the fever finally broke, and Connor returned home. Just a month later, in November 2016, he was cancer-free and back on the ice in his hockey skates and pads.

That episode was Connor's third bout with acute lymphoblastic leukemia. The experimental treatment was a last hope for the boy, who was first diagnosed at age 3. He has spent a total of six years of his life receiving chemotherapy. When the cancer came back in 2016, the doctors said the prognosis wasn't good.

At that point, "it was about quality of life, not quantity," McMahon says. But when he and his wife, Michelle, learned about the experimental treatment, called CAR-T cell therapy, the family decided it wasn't time to give up yet.

McMahon enrolled Connor in a clinical trial for a CAR-T cell therapy at Duke University Children's Hospital, five hours from the family's Atlanta home. Days later, doctors extracted immune cells called T cells from Connor's blood and shipped them off to a lab in New Jersey. There, the cells were genetically modified to target and kill the cancer cells coursing through Connor's bloodstream.

A month later, doctors injected those modified T cells into the teen's body, where they multiplied. Over the next few weeks, that five-minute T cell infusion racked Connor's body but also knocked the levels of cancer cells in his bloodstream down to zero.

To view the full article, please click on this link:

https://www.sciencenews.org/article/how-make-car-t-cell-therapies-cancer-safer-and-more-effective

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How is Biotech Innovating to Treat Hemophilia? Let's Review (July 2018)

Clara Rodriguez Hernandez on 13/02/2018

Experts say hemophilia treatment is at a 'precipice of a therapeutic revolution.' From antibodies to gene therapy, here are some of the most innovative technologies in the field that might significantly improve the lives of patients suffering from this bleeding disorder.

Hemophilia is a genetic disorder that impairs blood clotting. When one of the genes that encode for blood clotting factors is absent or deficient, the smallest injury can trigger serious bleeding episodes. Since these genes are located on the X chromosome, the disease is much more common in males than females.

Worldwide, there are over 187,000 people with hemophilia. About 80% of them have hemophilia A, which affects the clotting factor VIII. The second most common form, hemophilia B, is due to a deficiency of the clotting factor IX.

There is currently no cure for hemophilia, once known as the mysterious 'Royal Disease' that monarchs across Europe inherited from Queen Victoria. People living with this disease may require up to 3 infusions per week of clotting factors as a prophylactic therapy, as well as emergency treatment in case of an injury. Still, these patients keep experiencing recurrent bleeding episodes. And 30% of patients with severe hemophilia A develop inhibitory antibodies that leave them unresponsive to the standard treatment — recombinant clotting factors.

But the biotech industry is striving to change this situation, with the incentive of tapping a global market expected to hit €20Bn by 2024. A number of technologies, including therapeutic antibodies, RNAi and cell therapy are fighting to become the go-to treatment; Gene therapy might be the first.

To view the full article, please click on this link:

https://labiotech.eu/biotech-hemophilia-treatment/

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Kymriah Ellicits Prolonged Complete Responses in Advanced DLBCL Patients, Trial Data Show (July 2018)

June 25, 2018

By Joana Carvalho

Kymriah (tisagenlecleucel), a chimeric antigen receptor (CAR) T-cell therapy developed by Novartis, is able to ellicit prolonged complete responses with minor side effects in adults with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), according to new data from a Phase 2 trial.

The results from JULIET (NCT02445248) were in an oral presentation, "An updated analysis of JULIET, a global pivotal Phase 2 trial of tisagenlecleucel in adult patients with relapsed or refractory (r/r) diffuse large b-cell lymphoma (DLBCL)," given at the 23rdAnnual Congress of the European Hematology Association (EHA) on June 16 in Stockholm.

Kymriah was the first cell-based gene therapy to be approved by the U.S. Food and Drug Administration (FDA) to treat relapsing or refractory acute lymphoblastic leukemia (ALL) or non-Hodgkin's lymphoma (NHL) in pediatric and young adult patients up to 25 years old.

ALL is the most frequent type of leukemia in children, and is marked by the bone marrow producing too many white blood cells, or lymphocytes, that fail to develop correctly. This excess of abnormal lymphocytes can cause swelling and damage to internal organs, and prevents the body from producing other blood cells, resulting in anemia and a dampened immune response.

In a similar way, DLBCL — the most common form of NHL — is an aggressive cancer of the lymphatic system caused by accumulation of abnormal B-cells.

To view the full article, please click on this link:

https://lymphomanewstoday.com/2018/06/25/kymriah-ellicits-prolonged-complete-responses-in-patients-with-dlbcl-phase-2-trial-data-show/

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Insurers and Government Are Slow to Cover Expensive CAR-T Cancer Therapy (July 2018)

July 17, 2018 8:02 AM ET

Michelle Andrews

Patients whose blood cancers have failed to respond to repeated rounds of chemotherapy may be candidates for a new type of gene therapy that could send their cancers into remission for years. But the two approved therapies, with price tags of hundreds of thousands of dollars, have roiled the insurance approval process, leading to delays and, in some cases, denials of coverage, clinicians and analysts say.

The therapy involves collecting patients' own T cells, a type of white blood cell, genetically modifying them, and then infusing them back into patients, where they hunt down and kill cancer cells. Known as CAR-T cell therapy, it's been characterized as a "living drug" by some researchers.

Two different CAR-T drugs — Kymriah and Yescarta — were approved by the FDA last year to treat patients whose blood cancers haven't responded to at least two other rounds of treatment.

Kymriah is approved for people up to age 25 with a form of acute lymphoblastic leukemia, the most common cancer in children. Kymriah and Yescarta are both approved for adults with advanced lymphomas.

Researchers report that some critically ill patients who received the therapy have remained cancer-free for as long as five years.

"This is what patients need," says Dr. Yi Lin, a hematologist who oversees the CAR-T cell practice and research for the Mayo Clinic. "With the likelihood of getting patients into durable survival, we don't want to deny them the therapy." She says she receives no personal financial support from the drugs' makers.

To view the full article, please click on this link:

https://www.npr.org/sections/health-shots/2018/07/17/629543151/insurers-and-government-are-slow-to-cover-expensive-car-t-cancer-therapy?utm_source=npr_newsletter&utm_medium=email&utm_content=20180718&utm_campaign=npr_email_a_friend&utm_term=storyshare

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Seattle Children's Hospital launches immunotherapy collaborative to expand clinical trial access at children's hospitals (June 2018)

By Tina Reed | June 12, 2018 8:00am

Four academic children's hospitals have joined forces to create a research collaborative that will make it easier for their patients to access immunotherapy clinical trials and for their researchers to find good candidates for therapies.

Led by Seattle Children's, the new initiative called CureWorks will allow member hospitals to participate in clinical trials and share data and collective expertise with the goal of accelerating progress in developing novel cell therapies.

Children's National Health System in Washington, D.C., Children's Hospital Los Angeles and BC Children's Hospital in Vancouver, British Columbia, are among the founding members.

CureWorks will both facilitate the production of immunotherapy treatments and streamline the clinical trial enrollment and coordination process for member hospitals, officials said.

"We believe a unified effort among leading children's hospitals is the best way to drive the discovery of new therapies for pediatric cancer," said Mike Jensen, M.D., executive director of CureWorks and director of the Ben Towne Center for Childhood Cancer Research at Seattle Children's Research Institute in a statement. "Our hope is that through this collaboration, we'll be able to more quickly develop treatments with fewer side effects, better readmission rates and, ultimately, enable more kids with cancer to grow up and realize their full potential."

To view the full article, please click on this link:

https://www.fiercehealthcare.com/hospitals-health-systems/top-children-s-hospitals-create-collaborative-to-open-better-access-to?mkt_tok=eyJpIjoiTkdVeU56a3paREkyWkRCaCIsInQiOiJPXC8ra25hcHVTNmVlYU42ak4xK1cxTVRZdGhyQ0pxdHZpdEpadDRCVFZJejRWaTY3YUIrWUpOUFRIdGdhSkF1R2g1TVpVMG1KSVUzZHpQMURjNjJ4elAyaWx3WnZ5R29YTjdjZ01OMmMySUhPem5wVlNtMmxmeFBQY0Q1RlMweGoifQ%3D%3D&mrkid=901485

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CLL patient goes into remission thanks to single CAR T cell (June 2018)

Case study provides clues that could help improve response rates

May 30, 2018

The doctors who have spent years studying the case call it "a series of fortunate events." What began as a remarkable response to chimeric antigen receptor (CAR) T cell therapy is now providing evidence about the human genome and immune response that could help turn gene therapy non-responders into responders.

Researchers at the University of Pennsylvania's Abramson Cancer Center say a patient treated for chronic lymphocytic leukemia (CLL) in 2013 went into remission because of a single CAR T cell and the cells it produced as it multiplied, and has stayed cancer free in the five years since, with CAR T cells still present in his immune system. The findings, published today in *Nature*, show the response is tied to where the CAR gene inserted itself into the patient's T cell DNA, a key factor that may help improve response rates to the therapy.

CLL is a type of cancer that starts in cells that become certain white blood cells in the bone marrow, and then move into the blood and lymph nodes. These cancer cells reproduce too quickly and crowd out other cells in the bone marrow. They also don't mature properly and thus don't fight off infection as well as they should. The American Cancer Society estimates there will be about 21,000 new CLL cases in 2018 and around 4,500 deaths from the disease. Many of these patients will undergo a bone marrow transplant, but a potential additional treatment is CAR T cell therapy, which involves collecting patients' own immune T cells, reprogramming them to recognize and kill cancer, and then infusing them back into patients' bodies.

The approach is approved by the U.S. Food and Drug Administration for certain acute lymphoblastic leukemia patients as well as some non-Hodgkin's lymphoma patients, but is not currently approved for treatment of CLL. Patients typically receive three consecutive infusions with increasing doses -- 10 percent, 30 percent, and 60 percent -- to control for cytokine release syndrome (CRS). CRS is a common toxicity associated with CAR T therapy and includes varying degrees of flu-like symptoms, with fevers, nausea, and muscle pain, and can require ICU-level care.

The patient in this report received the first two infusions of 10 and 30 percent, but did not initially respond. "It wasn't until day 50 that the patient experienced CRS, which indicated the CAR T cells were active and may be having an anti-tumor effect," said the study's senior author J. Joseph Melenhorst, PhD, an associate professor of Pathology and Laboratory Medicine in Penn's Perelman School of Medicine and a member of Penn's Center for Cellular Immunotherapies.

To view the full article, please click on this link:

https://www.sciencedaily.com/releases/2018/05/180530133022.htm

University of Pennsylvania School of Medicine. "CLL patient goes into remission thanks to single CAR T cell: Case study provides clues that could help improve response rates." ScienceDaily. ScienceDaily, 30 May 2018. <www.sciencedaily.com/releases/2018/05/180530133022.htm>.

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New study finds hope in understanding and better treating scleroderma (June 2018)

January 10, 2018, Hospital for Special Surgery

Scleroderma is a terribly debilitating disease with no effective treatments and the mortality rates are still upwards of 20%-50%, the highest of any rheumatic disease.

This disabling autoimmune disorder results in inflammation and fibrosis leading to the thickening of the body's connective tissue, including the skin; and for decades its treatment has been symptomatic and, at best, inconsistently effective. But new research by a team from Hospital for Special Surgery (HSS) in New York City may signal hope for patients suffering from the condition.

The mechanism behind systemic sclerosis is not well understood. However, new research published today in *Science Translational Medicine* reveals a potential breakthrough into the cause of this disease, and also provides a possible treatment lead. Led by HSS researcher Dr. Franck Barrat—the Michael Bloomberg Chair and Senior Scientist at HSS - along with clinicians of the Scleroderma center of HSS, the work implicates what are called plasmacytoid dendritic cells (pDCs) in contributing to scleroderma.

Normally pDCs secrete a compound called interferon to help fight off infections. However, as Dr. Barrat's study revealed, in scleroderma patients these cells are chronically activated and infiltrate the skin causing fibrosis and inflammation.

"Plasmacytoid dendritic cells are known to be activated in many other rheumatic conditions, including lupus," explains Dr. Barrat. "But our findings suggest that they participate in both establishing and maintaining fibrosis in the skin as well. This is a very interesting finding as it opens new ways to tackle this condition."

Dr. Barrat found that depleting pDCs in an animal model of scleroderma prevented the disease from forming, while also reversing already existing fibrosis.

The new research also revealed that a receptor on the surface of pDCs called TLR8 is responsible for their increased activity.

To view the full article, please click on this link:

https://medicalxpress.com/news/2018-01-scleroderma.html

More information: M.D. Ah Kioon el al., "Plasmacytoid dendritic cells promote systemic sclerosis with a key role for TLR8," *Science Translational Medicine* (2017). stm.sciencemag.org/lookup/doi/ … scitranslmed.aam8458

Journal reference: Science Translational Medicine

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Study Aims To Show Transplants Between HIV-Positive Patients Are Safe, Save Lives (June 2018)

June 1, 2018 5:00 AM ET

Emily Forman

A large-scale clinical trial launched by the National Institutes of Health in May could pave the way for more HIV-positive patients with kidney disease to receive life-saving transplants.

The trial, called the HOPE in Action Multicenter Kidney Study, will assess the risks of transplanting kidneys from HIV-positive donors into patients living with the virus, says Dr. Christine Durand, assistant professor of medicine at Johns Hopkins University and a principal investigator of the study.

"We have an organ shortage crisis in this country and individuals living with HIV are disproportionately affected," she says. The research will help determine whether the pool of HIV-infected organs is "safe and effective." If so, she says, it would benefit everyone awaiting kidneys.

"Every time someone with HIV gets an organ transplant they move everybody else up on the waitlist," says Durand.

There are 468,000 Americans receiving dialysis for end-stage renal disease. According to Durand's research, an estimated 1.5 percent of those live with HIV. About 1 percent of liver transplant candidates have HIV.

"This means that more than 10,000 HIV positive individuals could benefit from a kidney or liver transplant," says Durand.

The HOPE in Action study will track 160 kidney transplants, half receiving HIV-positive kidneys and half receiving virus-free organs. Recipients will be monitored after surgery for signs of organ rejection, organ failure, and other complications, such as the risk of infecting the patient with more than one strain of HIV.

To view the full article, please click on this link:

https://www.npr.org/sections/health-shots/2018/06/01/615860200/researchers-want-to-show-hiv-positive-organ-donation-is-safe-and-can-save-lives

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Impaired Fetal Environment Linked to Lower Survival after Heart Surgery in Newborns (June 2018)

Published on May 30, 2018 in CHOP News

Children who undergo surgery for congenital heart disease have lower survival rates by 3 years of age if there are specific problems during fetal development, such as hypertension in the mother or the newborn being born preterm or small for gestational age. These problems are considered markers of an impaired maternal-fetal environment (MFE).

"We already knew that an impaired maternal-fetal environment correlates with neonatal mortality and morbidity in children who don't have heart disease," said study leader J. William Gaynor, MD, a pediatric cardiologist at Children's Hospital of Philadelphia (CHOP). "In this study, we investigated the impact of those impairments on child outcomes after infant heart surgery."

Gaynor and colleagues published their study online Feb. 13 in the *European Journal of Cardio-Thoracic Surgery*.

The researchers performed a retrospective analysis of 135 newborns with congenital heart disease (CHD) from a larger study of neurodevelopment outcomes after infant cardiac surgery. Two thirds of the cohort of 135 infants had transposition of the great arteries or hypoplastic left heart syndrome (HLHS). The study team defined an impaired MFE as involving pre-eclampsia in the mother, being small for gestational age, or being born preterm. Pre-eclampsia entails gestational hypertension, plus either protein in the urine or injury to the liver or kidney.

In the study cohort, 28 of the 135 newborns, or 21 percent, experienced an impaired MFE. Those infants had longer hospital stays after surgery than the infants without an impaired MFE. Hospital mortality was similar for both groups, but survival at 36 months of age was significantly lower for children with an impaired MFE (68 percent vs. 96 percent). Within the subgroup of patients with HLHS, survival was also lower among those with an impaired MFE.

To view the full article, please click on this link:

http://www.chop.edu/news/impaired-fetal-environment-linked-lower-survival-after-heart-surgery-newborns

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Prophylaxis Study Results for Boys with Severe Hemophilia A (June 2018)

May 16, 2018

A group of Canadian researchers recently published findings from a clinical study on the use of prophylaxis with a standard half-life recombinant factor VIII (SHL-rFVIII) therapy to treat boys with severe hemophilia A. They investigated an approach to prophylactic therapy in which patients began by receiving weekly infusions of SHL-rFVIII and ultimately transition to "tailored frequency-escalated prophylaxis." The lead investigator of the study was Professor Brian M Feldman, MD of the Division of Rheumatology at The Hospital for Sick Children in Toronto.

A total of 56 boys with severe hemophilia from 12 Canadian treatment centers were included in the study. These 56 participants were recruited as part of on an ongoing enrollment between June 26, 1997 and January 30, 2007. The boys were followed for a median of 10.2 years (to a maximum of 16.1 years) once they began with once weekly prophylaxis using SHL-rFVIII. Participants were eventually treated with escalating frequency, including requisite dose adjustments, in response to breakthrough bleeding events.

Results showed a median annualized joint bleeding rate of 0.95 per year. Joint health measures showed positive outcomes, even for prominent target joints such as the ankle. It was also reported that at some point during the study, 17 (30%) patients had unacceptable breakthrough bleeding as defined by study protocol. In addition, no treatment-related safety events were reported, including central venous catheter (CVC) infections. Feldman and his co-authors concluded that tailored frequency-escalated prophylaxis leads to very little arthropathy and "very good health outcomes" within the World Health Organization's International Classification of Functioning, Disability and Health framework.

To view the full article, please click on this link:

https://www.hemophilia.org/Newsroom/Medical-News/Prophylaxis-Study-Results-for-Boys-with-Severe-Hemophilia-A

Source: RareDisease Report, May 11, 2018

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Five Blood Transfusions, One Bone Marrow Transplant – All Before Birth (June 2018)

By Denise Grady
May 25, 2018

SAN FRANCISCO — In the three months before she was even born, Elianna Constantino received five blood transfusions and a bone-marrow transplant. All were given with a needle passed through her mother's abdomen and uterus, into the vein in her umbilical cord.

Elianna, born Feb. 1 with a robust cry and a cap of gleaming black hair, has a genetic disease that usually kills a fetus before birth. The condition, alpha thalassemia major, leaves red blood cells unable to carry oxygen around the body, causing severe anemia, heart failure and brain damage.

The transfusions in the womb kept her alive, but only treated her illness. The bone-marrow transplant has the potential to cure it. Whether it will succeed is still too soon to tell.

Elianna and her mother, Nichelle Obar, were the first patients in an experiment that pushes the limits of fetal therapy, a field already known for its daring.

If the treatment works, it could open the door to using bone-marrow transplants before birth to cure not just Elianna's blood disease but also sickle cell anemia, hemophilia and other hereditary disorders, some so severe that the prenatal diagnosis may lead parents to end the pregnancy.

Bone marrow is considered a potential cure because it teams with stem cells, which can create replacements for cells that are missing or defective as a result of genetic flaws.

To view the full article, please click on this link:

https://www.nytimes.com/2018/05/25/health/fetal-bone-marrow-transplant.html

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BIVV001 Extends Lifetime of Hemophilia A Replacement Therapy, Early Trial Data Shows (June 2018)

May 30, 2018

By Jose Marques Lopes, PHD

Preliminary results of a Phase 1/2a trial of the hemophilia A experimental treatment BIVV001 show a significant extension of replacement therapy lifetime in the blood, Bioverativ announced.

The ongoing, and still recruiting, open-label, multicenter EXTEN-A study (NCT03205163) is evaluating the safety, tolerability, and pharmacokinetics of a single intravenous injection of low- and high-dose BIVV001 in adult hemophilia A patients ages 18-65. Pharmacokinetics refers to the study of drug absorption, distribution, metabolism, and excretion in the body.

Factor replacement therapy for hemophilia A provides the normal version of a clotting protein called factor VIII (FVIII), which is defective or missing in patients.

BIVV001 (rFVIIIFc-VWF-XTEN) combines a region of a blood-clotting protein called von Willebrand factor and a molecule called XTEN to extend the lifetime of recombinant (lab-made) FVIII in circulation.

Bioverativ says BIVV001 is the only treatment candidate able to overcome the von Willebrand factor ceiling, which is thought to limit the half-life – the time required for a 50% reduction in a compound's blood concentration – of current therapies.

The potential therapy is intended to provide once-weekly or less prophylactic (preventive) dosing for hemophilia A patients.

The findings, presented at the World Federation of Hemophilia (WFH) 2018 World Congress, held recently in Glasgow, Scotland, revealed that the low dose (25 IU/kg) BIVV001 prolonged the FVIII half-life to 37 hours in four men after a washout period.

To view the full article, please click on this link:

https://hemophilianewstoday.com/2018/05/30/bivv001-may-extend-lifetime-hemophilia-replacement-therapy-early-data/

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