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Children Who Survive Congenital Heart Defects Can Face New Problems as Adults (May 2018)

January 28, 2018 6:00 AM ET

Wendy Wolfson

A few weeks ago, our family gathered for a meeting that we hope will save my sister's life. Our goal was to demonstrate to a hospital social worker that we could take care of her should she get a heart transplant.

My sister Sara is now 50. (NPR isn't using her last name to protect her medical privacy.) For her to get on the transplant list, her anatomy needed to be suitable and her antibody levels low despite prior surgeries. She had to show that she could withstand the grueling transplant process; that she could consistently take her anti-rejection medications; didn't abuse drugs or alcohol; and had a stable home life.

A heart transplant costs about $1.4 million, according to data from the actuarial firm Milliman. And there aren't enough hearts to go around.

Sara had to show that she could pay for three months of living near the hospital for the daily checkups and weekly heart biopsies, as well as the anti-rejection medications she would need daily for the rest of her life.

My sister has the most gallant heart of anybody I know. But her patched up heart has been slowly failing over the last three to four years.

She was born a "blue baby" in 1967 with transposition of the great arteries, a relatively common but serious heart defect, which deprived her body of oxygenated blood. In a procedure called a Blalock-Taussig-Thomas shunt, her doctors made a small hole in her heart to allow oxygenated blood to mix with the other blood in her body. At that point, that was the only option. The doctors told our mother to keep her alive until they improved surgical techniques enough to operate again.

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

https://www.npr.org/sections/health-shots/2018/01/28/579018072/people-born-with-heart-defects-can-have-new-problems-as-adults

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A cancer treatment that one expert called the 'most exciting thing I've seen in my lifetime' just got approved to treat more patients (May 2018)

Lydia Ramsey

5-2-2018

The FDA just approved a cutting-edge cancer therapy to treat another form of blood cancer. It's the second approval for Kymriah, also known as tisagenlecleucel, this time to treat patients with large B-cell lymphoma, and the first for adult patients. An August approval of Kymriah approved the therapy to treat pediatric acute lymphoblastic leukemia in people up to age 25.

The highly personalized treatment is called CAR T-cell therapy (CAR is short for chimeric antigen receptor). It's a type of cancer immunotherapy, which harnesses the body's immune system to take on cancer cells. It removes a person's cells, reengineers them, then puts them back in their body to attack cancer cells.

"I think this is the most exciting thing I've seen in my lifetime," said Dr. Tim Cripe, an oncologist who was part of the FDA advisory committee panel that voted in July to recommend the drug's initial approval.

The approval now puts Kymriah, made by Novartis, into direct competition with Yescarta, another CAR-T cell therapy that was approved in August made by Kite Pharma, a company Gilead Sciences acquired in 2017. There are more than 10,000 adults with large B-cell lymphoma who might benefit from these treatments, Dr. Fred Locke, an oncologist and researcher at Moffitt Cancer Institute in Tampa, Florida told Business Insider in October. That increases the potential use of the medication from the 600 people a year who could initially benefit from Kymriah based on its pediatric leukemia approval.

Yescarta and Kymriah aren't your run-of-the-mill pill — or even a biologic drug, like insulin — that can be mass produced. Since the therapy is made from a person's own immune system, the process can take about three weeks.

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

http://www.businessinsider.com/fda-approves-novartis-kymriah-large-b-cell-lymphoma-cancer-treatment-2018-5

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National Donate Life Month (May 2018)

April 2018

National Donate Life Month (NDLM) was instituted by Donate Life America and its partnering organizations in 2003. Celebrated in April each year, NDLM features an entire month of local, regional and national activities to help encourage Americans to register as organ, eye and tissue donors and to celebrate those that have saved lives through the gift of donation.

National Donate Life Month 2018
For the 2018 National Donate Life Month artwork, Donate Life America was inspired by the image of a rainbow and Maya Angelou's quote, "Be a rainbow in someone else's cloud."

Often following a storm, the presence of a rainbow provides optimism and motivates us to endure through dark times. Similarly, organ, eye and tissue donation is the bridge of comfort and hope between one family's mourning and another's healing — turning tragedy into renewed life. The vibrant Donate Life rainbow in the National Donate Life Month artwork rises from stormy clouds, recognizing that it takes both rain and light to create the gift of a rainbow.

Maya Angelou's message applies to all of us, no matter our background or experiences. We all know rainbows — people that have helped carry us through life and its challenges. In turn, we may also have the opportunity to be rainbows in other people's clouds through the gift of organ, eye and tissue donation. This April, we encourage you to reflect on the lives of those touched by donation and transplantation, and to share its prismatic effect. By registering to be a donor or considering living donation, you can change one ray of light into a spectrum of healing and compassion.

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

https://www.donatelife.net/ndlm/

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Stem Cell Transplant Results Better than Standard Therapy in Severe Scleroderma Patients, Phase 2 Trial Shows (May 2018)

January 18, 2018 by Alice Melao In News.

Results from a Phase 2 clinical trial shows that stem cell transplants can provide better long-term benefits than the standard treatment for patients with severe scleroderma.

Myeloablative autologous hematopoietic stem cell transplant, which uses high doses of chemotherapy and/or radiation before the patient's own stem cells are transplanted, was found to significantly improve survival and quality of life, compared to monthly doses of the immunosuppressive drug cyclophosphamide (sold as Cytoxan, among others).

"This is a major advance in the treatment of severe scleroderma," Karen Ballen, co-author of the study and the director of stem cell transplants at the University of Virginia Cancer Center, said in a UVA news story.

The results of the Phase 2 SCOT trial were reported in an article titled "Myeloablative Autologous Stem-Cell Transplantation for Severe Scleroderma," published in the *New England Journal of Medicine*.

The SCOT study (NCT00114530) enrolled 75 patients with diagnosed severe scleroderma, of whom 97% presented lung involvement, at 26 clinical sites. Patients were randomized to undergo stem cell transplant or cyclophosphamide treatment.

Patients in the transplant group were initially treated with total-body irradiation to destroy the patient's immune cells (myeloablation), followed by chemotherapy, then reconstitution of the immune system with stem cells previously collected from the patient's blood.

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

https://sclerodermanews.com/2018/01/18/stem-cell-transplant-results-better-than-standard-treatment-in-severe-scleroderma-patients-phase-2-trial/

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Novel Agents, CAR T Cells Reinvigorate ALL Landscape (May 2018)

Angelica Welch
Published: Wednesday, Apr 04, 2018

The field of acute lymphoblastic leukemia (ALL) has experienced a wave of new agents, with the approval of 3 novel therapies in the past year alone.

In July 2017, the FDA granted a full approval to blinatumomab (Blincyto) for the treatment of adults and children with relapsed/refractory B-cell precursor ALL, regardless of Philadelphia chromosome (Ph) status. Blinatumomab was then granted an accelerated approval in March 2018 for the treatment of adult and pediatric patients with B-cell precursor ALL who are in remission but still have minimal residual disease (MRD).

Inotuzumab ozogamicin (Besponsa) was approved in August 2017 for the treatment of adults with relapsed/refractory B-cell precursor ALL, and a milestone moment also came in August 2017, when the FDA issued an approval for the first chimeric antigen receptor (CAR) T-cell therapy—tisagenlecleucel (Kymriah) for the treatment of patients up to 25 years of age with B-cell precursor ALL that is refractory or in second or later relapse.

During his presentation at the 2018 *OncLive*® State of the Science Summit™ on Hematologic Malignancies, Jae H. Park, MD, an assistant attending physician in the Leukemia Service at Memorial Sloan Kettering Cancer Center, discussed novel therapies for patients with ALL. In an interview during the meeting, Park shared his insight on the impact of agents such as blinatumomab, inotuzumab ozogamicin, and tisagenlecleucel.

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

https://www.onclive.com/web-exclusives/novel-agents-car-t-cells-reinvigorate-all-landscape

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An off-the-shelf, dual-targeted CAR T-cell products shows promising results in preclinical studies (May 2018)

April 16, 2018, American Association for Cancer Research

FT819, an off-the-shelf, T-cell receptor (TCR)-less CD19 CAR T-cell product that could potentially be made more accessible to cancer patients than conventional CAR T-cell therapies showed positive results in preclinical specificity, functionality, and efficacy studies, according to data presented at the AACR Annual Meeting 2018, April 14-18.

"Chimeric antigen receptor (CAR) T-cell therapy has shown remarkable results in certain cancer patients. However, the therapy is highly personalized, time-consuming to produce, and consists of only enough cells for a single dose treatment with variable quality," said Bob Valamehr, Ph.D., vice president of Cancer Immunotherapy at Fate Therapeutics Inc. "Additionally, today's CAR T-cell therapies only target a single tumor antigen, which can also limit efficacy."

"We aimed to overcome these challenges by demonstrating proof-of-concept for an off-the-shelf, dual-targeted CAR T-cell approach. We started with cells from a healthy donor rather than the patient, created a master cell line, and used the master cell line to produce large quantities of 'universal' CAR19 T cells that are not patient-restricted," Valamehr explained. "These first-of-kind CAR19 T cells, called FT819, can be packaged, stored, and made readily available for treatment of a large number of patients."

The master cell line used to manufacture FT819 is an induced pluripotent stem cell (iPSC) line. The use of a master iPSC line for the production of CAR T cells provides distinct advantages over autologous (using cells from a patient's own body) and allogeneic (using cells from a donor) approaches, Valamehr explained. "A master iPSC line has unlimited capacity to self-renew, and can be banked and renewably used." Valamehr and colleagues used a proprietary platform that they previously developed to create the master iPSC line.

Conventional CAR T-cell therapies use autologous T cells. New approaches under investigation are using donor T cells; however, donor T cells can attack the patient's tissues and organs, resulting in a potentially severe and fatal immune system reaction known as graft-versus-host disease (GvHD). In order to avoid GvHD, it is critical to deactivate or remove the TCR, Valamehr explained.

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

https://medicalxpress.com/news/2018-04-off-the-shelf-dual-targeted-car-t-cell-product.html

Provided by: American Association for Cancer Research

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Emerging Research: Can stem cell treatments help patients with heart failure? (May 2018)

Be The Match BioTherapies · Industry Updates

Emerging research: Can stem cell treatments help patients with heart failure?

March 21, 2018

Heart failure has long been an intractable disease, afflicting 25 million patients worldwide. Most have few options, other than a full heart transplant or surgery to insert a left ventricular assist device.

Now, several companies are advancing clinical trials to see whether stem cell therapy can provide some relief, and early results are promising.

The Australian biotech Mesoblast is conducting a trial in 159 patients who have already had a left ventricular assist device implanted. The therapy consists of millions of stem cells injected or infused via catheter into the heart's left ventricle. Preliminary results were promising, and the U.S Food and Drug Administration (FDA) granted the therapy fast-track review status late last year.

In the UK, Celixir won approval earlier this year to push ahead with a mid-stage clinical trial for its stem cell therapy, dubbed Heartcel, which has shown strong results in earlier studies. The therapy consists of allogeneic immunomodulatory progenitor cells engineered to reduce scarring and regenerate tissue. So far, it's been delivered only during bypass surgery, though Celixir is also working on a formulation that's delivered via catheter. The company will likely seek FDA approval if the new trial succeeds.

Meanwhile, in the U.S., Cleveland-based Athersys is testing stem cell therapies to help patients recover from stroke, and a Belgium biotech, TiGenix, is working on a stem cell treatment to reduce scarring after a heart attack.

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

https://bethematchbiotherapies.com/cell-lines-blog/emerging-research-can-stem-cell-treatments-help-patients-with-heart-failure/?utm_source=subscriber_newpost&utm_medium=email

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In-the-News: March 2018

Be The Match BioTherapies – Industry Updates

April 12, 2018

March was a banner month for gene therapy, as it marked the commercial debut of the first gene therapy to treat an inherited disease – Spark Therapeutics' Luxturna. The drug treats people living with inherited retinal diseases caused by the RPE65 gene.

This medical milestone was chronicled around the country as patients looked forward to gaining more vision, independence and a new perspective on life. For uplifting reads, check out this coverage of patients named Jack, Creed and Toby.

In other gene therapy news, researchers at Duke Health are seeking funding to launch a clinical trial of an experimental treatment for Pompe disease. The therapy uses a modified virus to deliver a gene that produces GAA, a critical enzyme missing in people with Pompe disease. Without that enzyme, patients can't metabolize glycogen so it builds up in the muscles, leading to degradation of the tissue. Untreated, the disease can lead to respiratory problems, heart failure and death. The team at Duke has demonstrated the efficacy of their gene therapy in mice but it has not yet been tested in humans.

Voyager Therapeutics, meanwhile, reported positive data from its small Phase 1b trial for a gene therapy to treat Parkinson's disease, with some participants showing measurable improvement in motor function. Voyager will use the data to set the parameter for dosing in future clinical trials.

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

https://bethematchbiotherapies.com/cell-lines-blog/in-the-news-march-2018/

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Survival of transplant candidates found to be better than predicted by BODE score (April 2018)

March 7, 2018

COPD remains the leading indication for lung transplantation worldwide and accounts for one third of all lung transplants performed. In order to qualify for a lung transplant, patients receive an evaluation and undergo rigorous testing to identify and exclude those with an excessive burden of comorbid conditions. The body mass index, obstruction, dyspnea and exercise capacity (BODE) score is an evaluation used to inform prognostic considerations for potential lung transplantation patients. This scoring system is widely used but has yet to be validated in the context of lung transplant. In a new study published in the journal *CHEST*®, researchers aimed to determine if patients selected as transplant candidates have a better survival rate than the BODE score indicates.

Researchers performed a retrospective analysis of survival according to the BODE score for 4,000 COPD patients in the United Network Organ Sharing database of lung transplant candidates. They compared survival against that observed in the cohort of COPD patients in which the BODE score was originally validated.

They found that in models controlling for BODE score and incorporating lung transplantation as a competing end point, the risk of death was higher in the BODE validation cohort. This shows that patients selected as candidates for lung transplantation survive considerably longer than predicted by the commonly used prognostic estimates extrapolated from the BODE validation cohort. In addition, results indicated that nonrespiratory cause of death was higher in the nontransplant cohort, which supports the idea that comorbid illnesses that are screened out by the transplant selection process contribute a significant amount of morbidity.

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

https://www.news-medical.net/news/20180307/Survival-of-transplant-candidates-found-to-be-better-than-predicted-by-BODE-score.aspx

Source: http://www.chestnet.org/News/Press-Releases/2018/03/BODE

News-Medical.net - An AZoNetwork Site

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Lung transplant drug enters clinical trial after decades of research (April 2018)

March 26, 2018

In the culmination of decades of research at the University of Virginia Health System, doctors have begun human testing of a drug they hope will one day save many lives among lung transplant recipients.

The drug, regadenoson, is already commonly used to image cardiac patients' hearts. But the UVA research suggests it could be put to another, lifesaving purpose: battling ischemia reperfusion injury, in which tissue is damaged by the restoration of blood flow after it has been cut off.

The principal investigator of UVA's trial, Christine L. Lau, MD, called ischemia reperfusion injury the "Achilles' heel of lung transplant."

"Ischemia reperfusion injury directly correlates to the development of chronic rejection, which is the reason why 5-year survival in lung transplant recipients is only about 50 percent," said Lau, a surgeon. "Yet there really are almost no [other] clinical trials in lung transplant. This is our best hope."

Many Years in the Making

UVA surgeon Irving L. Kron, MD, has been working on the problem of reperfusion injury among lung transplant recipients since 1989. That year he performed a lung transplant that he said "couldn't have gone better." Afterward, though, the patient began showing symptoms of reperfusion injury, in which tissue is damaged by the restoration of blood after blood flow has been cut off. The patient survived, but Kron thought there had to be a way to protect patients from the surgical complication – a complication that often leads to organ rejection. He made it his mission to find a solution.

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

https://www.news-medical.net/news/20180326/Lung-transplant-drug-enters-clinical-trial-afterc2a0decades-of-research.aspx

Source: http://newsroom.uvahealth.com/2018/03/26/decades-work-uva-launches-human-tests-lung-transplant-drug/

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