LifeTrac News Links

- Trends in Transplantation - Volume 3
- Trends in Transplantation - Volume 2
- BiologicTx – the latest addition to LifeTrac
- Trends in Transplantation - Volume 1

Industry Articles

Be The Match BioTherapies – Industry Updates ................................................................. 2

Survival of transplant candidates found to be better than predicted by BODE score (April 2018)........................................................................................................................................ 3

Lung transplant drug enters clinical trial after decades of research (April 2018).......... 4

9 things to know about CAR T-cell therapy (March 2018)............................................ 5

Extended Half-Life (EHL) Products (March 2018) .......................................................... 6

CAR T-Cell Immunotherapy: The 2018 Advance of the Year (March 2018) .............. 7

How Extended Half-Life Treatments Help With Hemophilia Management (March 2018) 8

Reimbursement remains ‘difficult issue’ in CAR T-cell therapy rollout (March 2018).... 9

Masonic Center at U of M Testing New Tech for Enhancing Anti-cancer ‘Killer Cells’ (February 2018) ......................................................................................................................................... 10

NMDP: Jason Carter Clinical Trials Program (February 2018)..................................... 11
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:


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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:


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

News-Medical.net - An AZoNetwork Site

return to Table of Contents
New

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:


News-Medical.net - An AZoNetwork Site

return to Table of Contents
9 things to know about CAR T-cell therapy (March 2018)

February 26, 2018

BY CYNTHIA DEMARCO

CAR T-cell therapy is a new type of cancer treatment offered at MD Anderson through clinical trials and FDA-approved standard of care cell therapy products. But what exactly is CAR T-cell therapy? And who should consider it?

We spoke with Sattva Neelapu, M.D., to learn more. Here’s what he had to say.

Let’s start with the basics. What is CAR T-cell therapy, and how does it work?

CAR T-cell therapy is a type of immunotherapy called adoptive cell therapy. Doctors extract T cells (a type of white blood cell) from the patient’s blood and then add an artificial receptor (called a “chimeric antigen receptor”) to their surface. The receptor functions as a type of “heat-seeking missile,” enabling the modified cells to produce chemicals that kill cancer. And once we infuse them back into a patient’s body through an IV, they begin multiplying and attacking tumor cells.

CAR T-cell therapy can cause some unusual side effects. Tell me about them.

The most common side effect of CAR T-cell therapy is called cytokine release syndrome, or CRS. It’s also known as a “cytokine storm.” About 70-90% of patients experience it, but it’s very short-term and only lasts about five to seven days. Most patients describe it as having a severe case of the flu, with high fever, fatigue and body aches. It usually starts around the second or third day after the infusion. It happens because the T cells have been multiplying and attacking the cancer, causing an immune response in the body.

There’s a very effective remedy for CRS now called tocilizumab, which reverses this side effect fairly quickly. The medicine was originally used to treat rheumatoid arthritis, but has since been approved by the U.S. Food and Drug Administration (FDA) to treat CRS.

The other side effect is known as “CRES,” which stands for “CAR T-cell-related encephalopathy syndrome.” It typically starts around day five after the infusion. Patients can become confused and disoriented, and sometimes may not be able to speak at all for a few days. CRES can be upsetting for patients and their families, but it typically lasts between two and four days, and it’s completely reversible. Patients eventually recover all of their neurological functions.

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


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return to Table of Contents
Extended Half-Life (EHL) Products (March 2018)

March 6, 2018

By Erum Naqvi

The recommended treatment regimen for patients with severe hemophilia to prevent bleeding episodes is treatment to replace the deficient clotting factor. However, currently available replacement clotting factors are limited by their relatively short half-lives and require intravenous injections up to three times a week to maintain protective levels. This can have a negative impact on patient compliance.

Recent research has led to the development of new factor products that are equally efficient with less frequent need for injections — once every week or two. The fewer injections are due to the medication’s increased half-life, which is the amount of time the body takes to reduce the clotting factor to half in the bloodstream. In other words, the structure of the protein factor molecules is altered so the body takes longer to clear them from the circulation, meaning that they work longer after being injected. These drugs are collectively called extended half-life, or EHL treatments.

The clotting factors with extended half-life have several advantages, including reduced injection frequency, increased treatment adherence, and improved clinical outcomes. Long-acting clotting factors also provide an opportunity for improved individualized treatment for hemophilia.

There are three ways of producing EHL products: Fc fusion, PEGylation, and albumin fusion.

Fc fusion
Biogen, in partnership with Swedish Orphan Biovitrum (Sobi), has been granted approval for two new EHL products in which a fragment of Fc, a protein found in immunoglobulin (antibodies), is fused to a recombinant clotting factor. This makes the treatment last for days or weeks instead of few hours. The two approved EHL products synthesized in this way are Alprolix for hemophilia B and Eloctate for hemophilia A.

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

https://hemophilianewstoday.com/hemophilia-treatments/extended-half-life-ehl-products/

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CAR T-Cell Immunotherapy: The 2018 Advance of the Year (March 2018)

January 30, 2018 – Greg Guthrie, ASCO staff

At the start of every year, the American Society of Clinical Oncology (ASCO) releases its *Clinical Cancer Advances* report. This in-depth report is published in ASCO’s *Journal of Clinical Oncology* and covers the major advances made in cancer care over the past year, including prevention, treatment, patient care, and a look toward the future. Each year, leaders in the oncology field also select a single area of research that achieved the greatest progress to name the Advance of the Year.

For the past 2 years, the Advance of the Year was immunotherapy. This innovative shift in the way in which cancer is treated has transformed care for many patients. In fact, ASCO estimates that immune checkpoint inhibitors could save 250,000 years of life for U.S. patients with advanced lung cancer for whom a checkpoint inhibitor could be prescribed. Immune checkpoint inhibitors are medicines that release the brakes on the body’s immune system, unleashing it to fight cancer.

The 2018 Advance of the Year is a new kind of immunotherapy known as chimeric antigen receptor (CAR) T-cell therapy.

What is CAR T-cell therapy?

When the immune system is functioning normally, immune cells move around the body looking for things that don’t belong, like bacteria and viruses. These immune cells search for invaders using “receptors,” which can be thought of as antennae or feelers. When receptors find invaders in the body, special immune cells come in to destroy them. These special cells are called cytotoxic T cells.

Unfortunately, cancer cells are often able to hide from immune cells, which is why the cancer cells can grow out of control. Immunotherapy is a cancer treatment intended to make the body’s immune system able to detect and destroy cancer cells. Immune checkpoint inhibitors have been a successful immunotherapy approach because it pushes the immune system into high gear to fight cancer.

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


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[return to Table of Contents]
How Extended Half-Life Treatments Help With Hemophilia Management (March 2018)

March 6, 2018

By Wendy Henderson

One of the major hurdles people with hemophilia face is complying with their medication schedule — in particular, injecting themselves several times a week with clotting factor.

Clotting factor has a relatively short half-life (the time it takes for a person’s clotting factor levels to drop to 50 percent, which can range from eight to 36 hours). The shorter the half-life, the more often patients will need to administer the factor.

Extended half-life (EHL) treatments allow hemophilia patients to go longer between clotting factor infusions. More extended half-life products are now available for people living with the condition. These products allow patients to have better treatment adherence, need fewer weekly injections, and improved clinical outcomes. Find out more about extended half-life products here.

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return to Table of Contents
Reimbursement remains ‘difficult issue’ in CAR T-cell therapy rollout (March 2018)

HemOnc Today, March 10, 2018

Before the FDA approval of two chimeric antigen receptor T-cell therapies, children and young adults with B-cell acute lymphoblastic leukemia and adults with relapsed or refractory diffuse large B-cell lymphoma were considered incurable.

Chimeric antigen receptor (CAR) T-cell therapy has restored hope for those patients, but at a significant cost to the health care system.

The ELIANA trial showed 76% of children with B-cell ALL who received tisagenlecleucel suspension (Kymriah, Novartis) achieved 1-year OS. In ZUMA-1, 52% of adults with relapsed or refractory DLBCL who received axicabtagene ciloleucel (Yescarta; Kite, Gilead) achieved 18-month OS.

Those durable responses earned CAR T-cell therapy recognition as ASCO’s “Advance of the Year.”

“As a true believer, I absolutely think we’ve just gotten started,” Stephan A. Grupp, MD, PhD, chief of cellular therapy and transplant section and director of cancer immunotherapy at Children’s Hospital of Philadelphia, told HemOnc Today. “If we can see it work in acute myeloid leukemia or even solid tumors, then my optimism about this being paradigm shifting is warranted.”

However, at $475,000 for a single infusion of tisagenlecleucel and $373,000 for axicabtagene ciloleucel, billing and reimbursement impediments could limit recipients to select populations from small pockets of the United States.

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return to Table of Contents
**Masonic Center at U of M Testing New Tech for Enhancing Anti-cancer ‘Killer Cells’ (February 2018)**

A Phase I study has opened for non-Hodgkin lymphoma and multiple myeloma patients.

January 24, 2018

Don Jacobson

The Masonic Cancer Center at the University of Minnesota is leading a clinical charge to unleash “natural killer” cells against blood cancers with the aid of a new kind of Israeli technology which has been shown to enhance their numbers and effectiveness.

Jerusalem-based Gamida Cell Ltd., a startup backed by the Swiss pharma giant Novartis, announced last week it is partnering with the U of M center on a Phase I study of how patients with relapsed or refractory CD20+ non-Hodgkin lymphoma or multiple myeloma fare under a regimen of treatment with natural killer (NK) cells whose numbers have been expanded through the use of the new “NAM” technology.

NAM is short for Nicotinamide, a small molecule which Gamida harnesses in its process to expand the supplies and effectiveness of donated NK cells. These cells, when infused into non-Hodgkin lymphoma and multiple myeloma patients, have shown the ability to attack tumors and bolster the body’s natural immune defenses against cancer.

Using NK cells as anti-cancer agents is an idea which been studied for some years, but there have been two major drawbacks so far: an insufficient supply of NK donor cells, and their short lifespan once infused into the patient. This has served to limit their applicability in clinical settings.

Gamida Cell, however, says it is addressing this problem by using NAM to expand the numbers of NK cells 100-fold while also improving their functionality.

The U of M study will be led Dr. Veronika Bachanova, a hematologist/oncologist who specializes in stem cell transplantation in fighting blood cancers such as leukemia and lymphoma. She said in an issued statement that Gamida Cell’s capabilities could open new doors against blood cancers.

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


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NMDP: Jason Carter Clinical Trials Program (February 2018)
Clinical Trials

1-17-2018

This new program helps patients and families find and join clinical trials.

Patients and families seeking a clinical trial may face many barriers, including complex information, lack of awareness, and potential travel costs. And yet we know that overall survival for some diagnoses is significantly higher for patients participating in clinical trials compared to those who do not. Clinical trials help physicians understand the science behind the medicine, which helps us all get closer to a cure. As patient advocates and educators, we are excited to introduce you to the Jason Carter Clinical Trials Program (JCCTP). This program makes it easier to find and join clinical trials.

One-On-One Clinical Trial Navigation

Scott Kerwin, MN, RN, CCRC, CCRN, is your primary contact when you need help finding a clinical trial for your patients. As a Clinical Trial Patient Education Specialist, Scott provides free one-on-one support by phone and email to help patients and families find and join clinical trials.

According to Scott, the most valuable help he provides is navigating the clinical trial system. “Because of my many years of working in hospitals as a bedside and clinical research nurse, I know how the system works, and I know how to contact the right people in the right roles,” says Scott.

Scott’s support and expertise is not limited to blood and marrow transplant (BMT) trials. He can help you and your patients find any trial for:

- Blood cancers, such as leukemia or myelofibrosis
- Blood disorders, such as sickle cell disease
- Inherited immune system or metabolic disorders, such as severe combined immunodeficiency (SCID) or adrenoleukodystrophy (ALD)
- BMT complications, such as graft-versus-host disease (GVHD)

To contact Scott, call 1(888) 814-8610 or email clinicaltrials@jcctp.org.

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