News

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

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:

https://www.mdanderson.org/publications/cancerwise/2018/02/car-t-cell-therapy--9-things-to-know.html

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/

return to Table of Contents

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:

https://www.cancer.net/blog/2018-01/car-t-cell-immunotherapy-2018-advance-year

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.

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

https://hemophilianewstoday.com/2018/03/06/how-extended-half-life-treatments-help-with-hemophilia-management/

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.

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

https://www.healio.com/hematology-oncology/leukemia/news/print/hemonc-today/%7Bbce73f3b-0172-4e05-a109-ba531502b2c6%7D/reimbursement-remains-difficult-issue-in-car-t-cell-therapy-rollout

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:

http://tcbmag.com/news/articles/2018/january/masonic-center-at-u-of-m-testing-new-tech-for-enha?utm_source=SilverpopMailing&utm_medium=email&utm_campaign=012418_TPULSE

return to Table of Contents

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.

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

https://bethematchclinical.org/research-and-news/transplant-enews/resource-connection-enews/jason-carter-clinical-trials-program/?utm_source=resource_connection&utm_medium=email&utm_campaign=jan_enews

return to Table of Contents

In a turf battle for organs, a policy review rattles the national transplant system (January 2018)

By Alan Zarembo

January 3, 2018, 3:00 AM

Tethered to a breathing machine at a Manhattan hospital, 21-year-old Miriam Holman would die without a lung transplant. But her odds of finding a suitable organ were especially low in New York, where waiting times are among the longest in the country.

Just across the Hudson River in New Jersey, patients in far better condition routinely receive lungs much more quickly. Pockets of the South and Midwest also have dramatically shorter waiting times.

The disparities stem from a principle that has always guided the national transplant system: local first. Most organs stay in the areas where they are donated, even if sicker patients are waiting elsewhere.

But a federal judge's recent emergency order in a lawsuit by Holman is threatening to upend decades of organ transplant policy and force places with a relative abundance of organs to start sharing more of them.

With too few donors to meet the demand — last year there were 33,610 transplants while 12,412 patients died on waiting lists or were removed from consideration because they were deemed too sick to survive surgery — transplant centers have long fought over how to allocate organs. California and New York, which have the most severe shortages, have been on the losing side of that battle.

Holman's lawsuit against the federal government has opened a door to change that. The order issued in October by Robert Katzmann, chief judge of the U.S. Court of Appeals for the Second Circuit in New York, spurred the government to immediately broaden access to lungs for many patients across the country. Now, the same legal arguments used in that case are being waged on behalf of liver patients.

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

http://www.latimes.com/nation/la-na-organ-transplant-20180103-htmlstory.html

return to Table of Contents

Organ Transplants Hit an All-Time High in 2017. But It's a Bittersweet Win (January 2018)

By Alexandra Sifferlin

January 10, 2018

Last year, organs were recovered from 10,281 deceased donors—more than a 3% increase from 2016 and a 27% increase over the last 10 years.

Those organs contributed to the 34,768 transplants performed in 2017 using organs from both deceased and living donors—a new record for organ transplants in the United States. The reasons why are both hopeful and concerning.

The new data comes from the United Network for Organ Sharing (UNOS), a non-profit organization that manages the United States's organ transplant system through a contract with the federal government. The number of transplant performed in 2017 marks the fifth consecutive record-setting year for transplants.

"The number of transplants is directly related to the number of donors," says Dr. David Klassen, the chief medical officer of UNOS. "I think who can be a donor has really evolved over the years. The transplant community as a whole has done a really good job looking beyond the usual places of who can be a donor."

Researchers are now finding ways to recover and use organs that would normally be discarded. At Penn Medicine in Philadelphia, there are ongoing clinical trials where people are given organs from donors who are infected with hepatitis C. After the transplant, recipients take a drug that will clear them of the disease. So far, the trial is having positive results.

UNOS also reported that there were a record number of organs recovered for the four most common transplants: kidney, liver, heart and lung transplants.

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

http://time.com/5097377/record-breaking-organ-transplants-2017/

return to Table of Contents

Screening for Critical Congenital Heart Disease at Birth Saves Lives (January 2018)

New study confirms a dramatic decrease in infant deaths

Tuesday, December 5, 2017

Infant deaths from critical congenital heart disease (CCHD) decreased more than 33 percent in eight states that mandated screening for CCHD using a test called pulse oximetry. In addition, deaths from other or unspecified cardiac causes decreased by 21 percent.

Pulse oximetry is a simple bedside test to determine the amount of oxygen in a baby's blood and the baby's pulse rate. Low levels of oxygen in the blood can be a sign of a CCHD.

CCHD screening nationwide could save at least 120 babies each year, according to a new study published in the *Journal of the American Medical Association*. This study is the first look at the impact of state policies to either require or recommend screening of infants for CCHD at birth.

The study, *Association of U.S. State Implementation of Newborn Screening Policies for Critical Congenital Heart Disease With Infant Cardiac Deaths,* shows that states that required their hospitals to screen newborns with pulse oximetry saw the most significant decrease in infant deaths compared with states without screening policies. Voluntary policies or mandated policies not yet implemented were not associated with reductions in infant death rates. The encouraging news is that 47 states and D.C. now have mandatory screening policies in place and one additional state, California, requires screening be offered. These results serve as a reminder to hospitals across the country to remain vigilant in their screening for CCHD.

"More families are able to celebrate special milestones in a child's life thanks to the early identification and treatment of heart defects," said CDC Director Brenda Fitzgerald, M.D. "Screening newborns for critical congenital heart disease in every state, tribe, and territory will save lives and help babies thrive."

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

https://www.cdc.gov/media/releases/2017/p1205-screening-congenital-heart-disease.html

For more information on congenital heart defects, visit https://www.cdc.gov/ncbddd/heartdefects/index.html and https://www.cdc.gov/features/congenitalheartdefects/.

CDC.gov site

return to Table of Contents