






LifeTrac News Links

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Newborns with congenital heart disease have enlarged kidneys (October 2018)

Research counters the expectation that organs of babies with congenital heart disease are smaller than average

PUBLIC RELEASE: 11-SEP-2018

Springer

The hearts and brains of babies born with congenital heart disease are not the only organs affected by this common medical condition. Surprisingly, their kidneys tend to be enlarged at birth, says Gemma Scholes of the University of Melbourne in Australia, who is lead author of a study in the Springer Nature-branded journal *Pediatric Research*.

The research is the first of its kind to investigate the renal development of newborn babies with congenital heart disease.

Congenital heart disease encompasses a range of heart defects and is the most common medical condition occurring in newborns, affecting around nine in every 1000 babies born. Congenital heart disease not only causes defects in the heart, but subsequently may also impair the growth of a baby's brain and body in general. This is due to the "brain-sparing phenomenon", in which the body protects the brain at all costs. In fetuses with congenital heart disease, this means that blood flow is prioritized towards the growing brain, having adverse effects on other organs and the general development of the baby.

Scholes and her colleagues started their study with the hypothesis that fetuses with congenital heart disease will have smaller kidneys. To test this they measured the kidney length of 452 newborn babies by looking at ultrasounds taken before children with congenital heart disease were first operated on.

Surprisingly, the results showed that the kidneys of babies with congenital heart disease are significantly enlarged and were on average 4.5 centimeters long. The kidneys of children who have left heart obstruction were consistently larger than normal. Those with cyanotic heart disease (a range of defects that alter the way in which blood flows through the heart and lungs) tended to have either normal or enlarged kidneys.

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

https://www.eurekalert.org/pub_releases/2018-09/s-nwc091118.php

Reference: Scholes, G.B. et al (2018). Altered in utero kidney development in newborns with congenital heart disease, *Pediatric Research* DOI: 10.1038/s41390-018-0163-0

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Advancing transplantation: Hepatitis C-infected organs safe for transplantation when followed by antiviral treatment (October 2018)

Date: August 6, 2018

Source: University of Pennsylvania School of Medicine

In 2016, Penn Medicine launched an innovative clinical trial to test the effect of transplanting kidneys from donors with HCV into patients currently on the kidney transplant waitlist who do not have the virus, and who would opt in to receive these otherwise unused organs. Recipients were then treated with an antiviral therapy in an effort to cure the virus after transplantation.

A research team co-led by Peter Reese, MD, MSCE, an associate professor of Medicine and Epidemiology, and David S. Goldberg, MD, MSCE, an assistant professor of Medicine and Epidemiology, report full data from the trial which includes 12-month HCV treatment outcomes in 10 patients and six month outcomes in another 10 patients -- all of whom have received a lifesaving kidney transplant, who have been cured of their contracted HCV, and who have reported good quality of life following their transplants. More, the finding that these 20 kidney transplant recipients have kidney function that is similar to recipients of kidney transplants from donors without HCV suggests that the HCV infection did not harm the quality of the transplant.

"This study, and the results, are good news for those in need of a transplant, particularly those patients who were facing tremendous wait times -- often five, seven, even 10 years -- and who were spending so much of their daily lives on dialysis," said Reese. "While larger, longer term studies are important to confirm these results, we can confidently say that hospitals nation-wide could perform hundreds or thousands more transplants if we increased our acceptance of organs from donors with hepatitis C."

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

<https://www.sciencedaily.com/releases/2018/08/180806180024.htm>

University of Pennsylvania School of Medicine. "Advancing transplantation: Hepatitis C-infected organs safe for transplantation when followed by antiviral treatment." ScienceDaily. ScienceDaily, 6 August 2018. <www.sciencedaily.com/releases/2018/08/180806180024.htm>.

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Hepatitis C-Positive Organ Transplants Are on the Rise (October 2018)

In the first five months of 2018, 803 HCV-positive organs were transplanted across the United States.

September 7, 2018 · By Casey Halter

Now that new hepatitis C virus (HCV) treatments promise cure rates upward of 90 percent for those infected, doctors are increasingly considering organs infected with the liver virus. According to a recent report from the Chicago Tribune, HCV-positive organ transplants are on the rise across the country.

According to recent statistics from the United Network for Organ Sharing (UNOS), the nonprofit that runs the nation's transplant system, 803 organs used in transplants in the first five months of 2018 had tested positive for hepatitis C, compared with just 482 hepatitis C-positive organs used in transplants in all of 2013. Last year, 1,491 of the 37,795 organs used in transplants were HCV positive. This year, doctors are well on track to surpass that record figure.

Transplant specialists say the availability of organs from donors with hepatitis C appears to be easing the chronic shortage of organs that has long affected prospective transplant patients across the country. More than 114,000 patients are on waiting lists for organs—and the influx and viability of transplanting HCV-positive organs means far fewer organs will be discarded because of disease.

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

<https://www.hepmag.com/article/hepatitis-cpositive-organ-transplants-rise>

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Rare manufacturing glitch raises concern over CAR-T therapies: study (October 2018)

October 1, 2018 / 10:15 AM / 2 days ago

Julie Steenhuysen

CHICAGO (Reuters) - A single leukemia cell inadvertently got mixed in with a batch of a patient's immune cells that were being manufactured into a CAR-T cell therapy and it acquired resistance to the treatment with deadly results, University of Pennsylvania researchers reported on Monday.

The mishap occurred in a 20-year-old leukemia patient treated with cells manufactured at the university, eventually causing a fatal relapse of the blood cancer, researchers reported in the journal *Nature Medicine*.

The patient was entered in an early-stage trial of CTL019, a treatment eventually licensed to Novartis and sold under the brand name Kymriah, which became the first such gene therapy to win U.S. approval in 2017.

Dr. Marco Ruella of the Center for Cellular Immunotherapies at the University of Pennsylvania, who led the study, said the case was "exceptionally rare," noting that it is the only one out of hundreds of patients treated at Penn.

He said the findings were disclosed to the U.S. Food and Drug Administration and discussed in detail prior to the treatment's approval.

CAR-T therapies, shorthand for chimeric antigen receptor T-cell, are part of a hot new approach to fighting cancer in which doctors remove infection-fighting immune cells known as T-cells, genetically engineer them to recognize and attack cancer, and infuse them back into the patient.

The personalized one-time treatment is complex and expensive, but offers hope for people with certain blood cancers who have exhausted all other treatment options.

Kymriah is approved in Europe and the United States to treat gravely ill children with acute lymphoblastic leukemia (ALL), as well as adults with diffuse large B-cell lymphoma (DLBCL).

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

<https://www.reuters.com/article/us-novartis-kymriah/rare-manufacturing-glitch-raises-concern-over-car-t-therapies-study-idUSKCN1MB334>

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Novel program offers new options for adult congenital heart patients (September 2018)

Reviewed by Alina Shrourou, BSc Aug 29, 2018

Experts at the Vanderbilt Heart and Vascular Institute are forging new ground in the development of a first-of-its-kind program aimed at adults with congenital heart disease (ACHD).

The novel program in Advanced Congenital Cardiac Therapies (ACCT) allows for patients to be evaluated for heart transplantations and ventricular assist devices (VAD) -; a foreign concept to Michael Sharpe, 39, who received a new lease on life in May.

Diagnosed at 9 months old with tetralogy of Fallot, Sharpe had resigned himself to the lifelong condition caused by the combination of four heart defects present at birth. He had already undergone the routine surgeries and procedures to improve the oxygen-poor blood flow out of his heart to the rest of his body.

"I did not know that transplant was an option for congenital heart patients," said Sharpe. "My parents were with me when the doctors mentioned it and they were shocked too.

"This has allowed me the incredible option to watch my son grow up. Evan is my motivator. When they told me that I was in heart failure, the thought of not seeing him grow up hit me the most.

"I know people don't live very long in heart failure and I kept thinking, 'I'm getting maybe two to five more years.' But now? I have 20, 30-plus years. That's the absolute best part."

Sharpe is one of three patients with ACHD transplanted at Vanderbilt within the last year. An additional three patients are currently listed for heart transplant and another three have undergone VAD implants.

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

<https://www.news-medical.net/news/20180829/Novel-program-offers-new-options-for-adult-congenital-heart-patients.aspx>

Source: <https://ww2.mc.vanderbilt.edu/>

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Spark Reports Progress on Hemophilia A Gene Therapy (September 2018)

AUGUST 8, 2018

Spark Therapeutics recently reported preliminary phase 1/2 data for SPK-8011, the company's investigational gene therapy candidate for hemophilia A. The therapy is administered via a one-time intravenous infusion, which is designed to elicit the production of therapeutic levels of factor VIII (FVIII), a protein that is normally deficient in individuals with hemophilia A. Spark Therapeutics' proprietary bioengineered adeno-associated viruses (AAVs) act as delivery vehicles, or vectors, to carry the genetic codes that prompt the FVIII production. The approach being tested in this trial uses a modified novel AAV vector genome (vg) to deliver the corrected FVIII gene into liver cells where the protein is normally generated.

According to a new press release, as of the July 13, 2018 data cutoff, 12 participants in the phase 1/2 trial have received a single administration of investigational SPK-8011. The dose sizes administered to trial participants have varied amongst the study participants with two patients receiving a low dose of 5×10^{11} vg per kilogram (kg) of body weight, three at a mid-range dose of 1×10^{12} vg per kg of body weight and seven at a high dose 2×10^{12} vg per kg of body weight. Across all participants, there has been a 97% reduction in annualized bleeding rate and a 97% reduction in annualized infusion rate. In addition, the first two trial participants, who have been followed for more than one year, have shown stable FVIII activity levels since reaching "plateau" for up to 66 weeks. Monitoring of trial participants is ongoing.

The company also addressed possible immune responses and incidences of elevated levels of alanine aminotransferase (ALT) enzymes in some of the patients – elevated ALT levels can lead to subsequent adverse effects on liver function if not properly addressed. Oral steroids were subsequently administered to positive effect.

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

<https://www.hemophilia.org/Newsroom/Industry-News/Spark-Reports-Progress-on-Hemophilia-A-Gene-Therapy>

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Source: Spark press release dated August 7, 2018

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Societies Release Updated Guideline for Treating Adult Congenital Heart Disease Patients (September 2018)

Document offers guidance for treatment in relatively new, growing population

August 16, 2018 Categories: Scientific Statements/Guidelines

Embargoed until 1 p.m. CT / 2 p.m. ET Thursday, August 16, 2018

WASHINGTON, August 16, 2018 — The American College of Cardiology and the American Heart Association today released an updated guideline for the management of adult congenital heart disease (ACHD) patients.

CHD encompasses a range of structural cardiac abnormalities present before birth and attributable to abnormal fetal cardiac development. Congenital heart defects are the most common type of birth defect. The prevalence of ACHD is growing due to the success of treating these patients during their childhood. Survival to age 18 years is now expected in 90 percent of children diagnosed with severe CHD.

“Patients with ACHD are a heterogeneous population,” said Karen K. Stout, MD, professor of medicine and pediatrics in cardiology at the University of Washington and chair of the writing committee for the guideline. “Although the prevalence of ACHD is increasing, the population of patients with a given congenital abnormality or specific repair may be relatively small, which can make accruing evidence to guide treatment challenging.”

This full revision of the original guideline, published in 2008, incorporates new data and growing ACHD expertise. Despite the difficulty in studying ACHD populations, there is a growing body of high-quality data in these patients to guide the care of this relatively new population. These data were used to develop the recommendations.

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

<https://newsroom.heart.org/news/societies-release-updated-guideline-for-treating-adult-congenital-heart-disease-patients>

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FDA Accepts First Allogeneic CAR T-Cell Therapy Trial (September 2018)

Samantha DiGrande

Newsroom – Published on: August 04, 2018

Celyad, a biopharmaceutical company that focuses on the development of chimeric antigen receptor (CAR) T-cell therapies, recently announced that the FDA accepted its Investigational New Drug (IND) application for CYAD-101, the first non-gene-edited allogeneic clinical program.

Traditionally, CAR T-cell therapies are created by genetically modifying a patient's own immune cells to target specific cancer cells before injecting them back into the patient. However, this can be difficult because researchers aren't always able to collect enough cells from a patient to create the treatment. Conversely, in an allogeneic CAR T-cell therapy, immune cells are instead collected from healthy donors, rather than the patient.

The trial, titled Allo-SHRINK, looks to evaluate the safety and clinical activity of CYAD-101 in patients with unresectable colorectal cancer in combination with standard chemotherapy.

"We are pleased to have achieved this important milestone. Celyad is the first company clinically evaluating a non-gene edited CAR T candidate, which, we believe, offers significant advantages over gene-edited approaches," Christian Homsy, MD, CEO of Celyad, said in a statement.

CYAD-101 is based on features of the company's investigational autologous CYAD-01 CAR T with a novel peptide, TCR Inhibiting Molecule (TIM). This prevents the patients' immune system from recognizing the cells as foreign. The cells in CYAD-01 produce a chimeric receptor called natural killer group 2D (NKG2D) that recognizes multiple tumor proteins.

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

<https://www.ajmc.com/newsroom/fda-accepts-first-allogeneic-car-tcell-therapy-trial>

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Road to Recovery: Woman is Youngest Patient in United States to Receive Face Transplant (August 2018)

AUGUST 14, 2018 / FEATURES & UPDATES

Katie Stubblefield's wit shows through when she describes her face transplant surgery.

"Longest nap of my entire life," she says.

Those unfamiliar with Katie's still-developing speech pattern may not easily make out those words, but her parents are usually there to interpret. Robb and Alesia Stubblefield have been by her side – helping her "take four steps forward, two steps back," as Robb describes it – since Katie, then 18, endured severe facial trauma and significant complications from a self-inflicted gunshot wound on March 25, 2014.

It would take a team of 11 Cleveland Clinic surgeons and multiple specialists to perform the hospital's third face transplant – and its first total face transplant – on Katie. At 21, Katie was the youngest person in the United States to receive a face transplant.

And, indeed, it was extensive: The surgery included transplantation of the scalp, the forehead, upper and lower eyelids, eye sockets, nose, upper cheeks, upper jaw and half of lower jaw, upper teeth, lower teeth, partial facial nerves, facial muscles, and skin – with 100 percent of her facial tissue effectively replaced.

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

<https://newsroom.clevelandclinic.org/2018/08/14/road-to-recovery-woman-is-youngest-patient-in-united-states-to-receive-face-transplant/>

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Face Transplants: The Changing Face of Medicine (August 2018)

AUGUST 14, 2018 / FEATURES AND UPDATES

To many, the human face defines personhood. When it is devastated beyond the reach of conventional plastic or restorative surgery, a patient may lament both the loss of their appearance and simple functional abilities such as smiling, talking, eating, or breathing on their own.

In these rare instances, a face transplant may be the only solution that can sufficiently restore the patient's quality of life and function.

Cleveland Clinic face transplant evolution

A face transplant is an intricately complicated, personalized medical procedure that replaces as much as 100 percent of the recipient's facial tissue with that of a deceased donor. The surgery can integrate many different functional components, such as nose and lower eyelids as well as different tissue types including, skin, muscles, bony structures, arteries veins and nerves.

As of August 2018, around 40 face transplants have been performed worldwide. In 2008, Cleveland Clinic became the first hospital in the United States to perform a near-total face transplant, and remains one of just six U.S. institutions to have conducted the surgical procedure.

Cleveland Clinic doctors performed the initial 22-hour procedure on a 40-year-old woman who suffered severe facial injuries from a gunshot to the face. A Cleveland Clinic surgical team integrated functional facial components and numerous tissue types, including skin, muscles, bony structures, arteries, veins and nerves – encompassing about 77 square inches of transplanted tissue.

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

<https://newsroom.clevelandclinic.org/2018/08/14/face-transplants-the-changing-face-of-medicine/>

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