



Rabi Hanna, MD, uses haploidentical transplant to cure even nonmalignant blood disorders — like 22-year-old Jacob Huber's aplastic anemia and PNH.

Innovative Haploidentical BMT Program Expands

The results from Cleveland Clinic's haploidentical bone marrow transplant program, which was launched in 2014, have been so encouraging that the initiative is now being expanded.

"Of the 18 adult and pediatric patients enrolled and treated since the start of the program, we had only two relapses and no deaths," says Rabi Hanna, MD, Director, Pediatric Blood and Marrow Transplant Program. "I think the program has done exceptionally well. No deaths among 18 patients is an excellent outcome. The relapse rate so far is also very low."

Graft-versus-host disease is also remarkably low, Dr. Hanna says. This complication, once common after mismatched haploidentical bone marrow transplants, is now observed in only a few patients thanks to using post-transplant cyclophosphamide. The technique involves administering cyclophosphamide in the immediate days following transplantation to destroy alloreactive immune cells that mediate graft-versus-host and host-versus-graft reactions while sparing hematopoietic stem cells.

[Year One: A New Approach](#)

From its conception, the haploidentical transplant program was novel for many reasons, including its use of a reduced-intensity regimen or myeloablative regimen depending on patients' comorbidities and disease status while still using high-dose cyclophosphamide shortly after transplant.

Using haploidentical donors, i.e., human leukocyte antigen half-matched donors, greatly expands the availability of safe donors, in effect providing almost universal access for patients, Dr. Hanna says. An example of a haploidentical donor is one biological parent donating to a child, or one sibling donating to another.

Cleveland Clinic is one of only a few U.S. centers conducting haploidentical research and transplantation

[Year Two and Beyond: A Broader Mission](#)

Because of the first year's success, the therapeutic horizon is widening. "In the past, we offered haploidentical bone marrow transplant for patients who had leukemia or malignant disorders," says Dr. Hanna. "We then realized that the technique of transplant using post-transplant cyclophosphamide could be applicable to many disorders, malignant and nonmalignant, including sickle cell disease and severe aplastic anemia."

Jacob Huber had battled aplastic anemia and paroxysmal nocturnal hemoglobinuria (PNH) since age 6. At 22, he still had no matching donors for a much-needed transplant. "My older sister was one of my highest matches, with six out of 10 HLA markers," says Mr. Huber, who received her bone marrow in August 2015. "The transplant experience was pretty easy for me. I didn't have complications — just needed to be patient and wait for the results. I was in the hospital one month." Since then, his blood counts have continued to improve, and all signs of PNH are gone.

"Patients with nonmalignant conditions don't stay in the hospital as long as the traditional patient undergoing myeloablative transplantation because the conditioning regimen is less intense," says Dr. Hanna. "So the intensity and the hospitalization are less, and the cost-effectiveness and value of the transplant become higher. This is good for the patient and for the overall healthcare system."

Dr. Hanna will expand using haploidentical transplantation to treat other nonmalignant diseases such as thalassemia and sickle cell disease in addition to inherited metabolic disorders, so every patient who may be potentially cured with hematopoietic stem cell transplant could have access to transplant using this technique.

DEAR COLLEAGUES



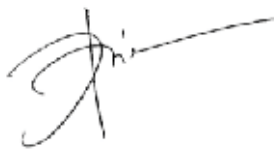
“Our goal is to be available in every way to you and your patients.”

We’re always looking for ways to make your life easier. We’re doing it in big ways, such as making Cleveland Clinic Children’s more accessible in person, online or by telephone. And we’re doing it in small ways, by publishing *Pediatric Update* in a more compact and convenient format. Our goal is to be available in every way to you and your patients.

Small as it is, this issue of *Pediatric Update* is dense with useful information. We’ve got updates on our bone marrow transplant program, facts about pediatric cardiac imaging, guidelines on referring for chronic headache, and much more. I promise you it’s more than worth the short time it will take you to read.

I thank you for your confidence in Cleveland Clinic Children’s, and look forward to continuing to be of help to you in times to come.

Sincerely,



GIOVANNI PIEDIMONTE, MD

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President, Cleveland Clinic Children’s Hospital for Rehabilitation
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DIAGNOSIS

Pediatric Headache

A conversation with pediatric psychologist Ethan Benore, PhD,
Director, Behavioral Headache Treatment Program



Ethan Benore, PhD

Whom should pediatricians refer for chronic headaches?

Any child who experiences two or more headaches per week may benefit from a referral. This includes migraine, tension-type headache, post-traumatic headache or chronic daily headache.

Is behavioral treatment different than medicine?

Behavioral treatments include nondrug approaches to preventing and treating migraine. Psychologists work with a neurologist or primary care doctor to offer this additional level of care.

What can patients expect?


After an initial evaluation, patients undergo six to eight treatments. They can do this individually or participate with a group of children all struggling with headache. Children will learn how their body works and how to

retrain their mind and body to prevent headaches. Kids receive both training and “homework” exercises as they:

- › Master relaxation skills to make their bodies resilient to stress
- › Care for their body with good nutrition, sleep and exercise
- › Conquer stressful life events with coping skills
- › Pay attention to and limit headache triggers

What outcomes are expected?

Research shows behavioral treatment works, reducing the number, duration and/or intensity of headaches. This may help some children reduce their need for medication, and lessen the pain and distress during headaches. Patients leave with a personal plan to stay on top of their headaches with minimal effort.

 [Refer to the Behavioral Headache Treatment Program: 216.448.6253.](#)

CARDIOTHORACIC SURGERY

Introducing the New Chair of Pediatric Cardiothoracic Surgery Hani Najm, MD

IN A NUTSHELL: Dr. Najm brings a distinctly global perspective to Cleveland Clinic Children’s. He trained in the Middle East and Canada and practiced for the past 16 years in Saudi Arabia. He travels incessantly to teach, having delivered 300+ international lectures to date.

PRIMARY CLINICAL INTERESTS: Heart surgery in newborns (particularly procedures for defects such as complex single ventricles and complex transpositions); surgery for adult congenital disease (which involves a growing population needing someone experienced in reoperation).

WHY CLEVELAND CLINIC? I was approached by Cleveland Clinic. It was a difficult decision to let go of my career in Saudi Arabia, but just about any heart surgeon in the world would want to be part of the staff at Cleveland Clinic if asked. As a cardiac surgeon, I’ve always felt I may be the

last resort for my patients, and that’s even more the case at a renowned institution like this.

DOES YOUR GLOBAL EXPERIENCE HOLD LESSONS FOR U.S. SURGEONS? An advantage of practicing abroad is that the limited clinical resources and expertise in many parts of the world give the available surgeons the chance to build considerable volume-based experience. I’ve done more than 250 arterial switch procedures so far, a number few U.S. cardiac surgeons come close to. That type of experience base will help us further enhance Cleveland Clinic Children’s capacity to handle the most highly complex pediatric heart surgery cases.

[Read an entire Q&A with Dr. Najm and view his bio at \[consultqd.clevelandclinic.org/Najm\]\(http://consultqd.clevelandclinic.org/Najm\).](#)

BELOW: *Hani Najm, MD*



HEART HEALTH

Do Plant-Based Diets Also Benefit Kids?

A plant-based diet started young could have lifetime benefits. Michael Macknin, MD, of Cleveland Clinic Children’s, led the first prospective randomized trial of a diet designed to reduce children’s risk of cardiovascular disease.

The plant-based, no-added-fat diet was compared in 30 children (ages 9-18) and one of each child’s parents to the American Heart Association diet, which allows added fat and animal products.

After a four-week period, the study found that children on both diets experienced significant improvements in weight, mid-

arm circumference and myeloperoxidase (though these improvements were higher for plant-based participants). Only children on the plant-based diet also had statistically significant improvements in BMI z-scores, systolic blood pressure, total cholesterol, LDL, high-sensitivity C-reactive protein and insulin. Additional studies are planned with larger sample sizes and including a Mediterranean diet option. Findings were published in *The Journal of Pediatrics*.

[Read more: \[consultqd.clevelandclinic.org/plant-based\]\(http://consultqd.clevelandclinic.org/plant-based\)](#)



Cleveland Clinic Children’s earned national rankings in 10 of 10 specialties in the 2015-16 edition of *U.S. News & World Report’s* “Best Children’s Hospitals” report.



RESEARCH TAKEAWAYS

Here’s what you should know about two new studies by Thomas Frazier, PhD, Director of Cleveland Clinic Children’s Center for Autism:

TAKEAWAY 1.

In families that are affected by autism, pay close attention to the siblings to make sure they’re not affected by autism or something related.

TAKEAWAY 2.

When problems in the family’s quality of life are identified early in the child’s life, broad intervention strategies that include the whole family may lead to better outcomes for the child, parents and siblings.

GASTROENTEROLOGY

Naim Alkhouri, MD, is a realist with a dream. The Cleveland Clinic Children’s pediatric hepatologist is confident that noninvasive testing methods for young patients with nonalcoholic fatty liver disease (NAFLD) are within reach.

One exciting possibility? Breath testing. Dr. Alkhouri published a pilot study in 2015 involving 61 patients, which found that NAFLD produced a distinctive “breath print” that could be identified using selective ion flow tube mass spectrometry.

Now he is investigating whether the technology can determine the severity of NAFLD without the need for liver biopsy. With only 10 percent of his 400+ NAFLD patients having undergone a biopsy, validation will take time. Yet he’s encouraged by the potential of obtaining a quick, immediate diagnosis. “We could develop a smartphone app that would work like a breathalyzer,” he says.

Dr. Alkhouri understands that the overarching issue hampering the diagnosis of NAFLD in children is lack of understanding about the disease’s natural history. “Everything we know is extrapolated from adults,” he notes.

CME & CONFERENCES

You’re invited to attend the following continuing education programs and conferences:

Aug. 29 -Sept. 2

22nd Annual Pediatric Board Review
Cleveland, Ohio

Sept. 16-17

1st Annual Advances in Pediatric and Congenital Heart Care:
From Single Ventricle to Failing Fontan
Cleveland, Ohio

[www.clevelandclinicmeded.com](#) For more CME offerings:

TRANSPLANTATION

Since 1971, Cleveland Clinic Children’s has pioneered advances in organ transplantation. Here is a snapshot of our 2015 volumes.

2015 ORGAN TRANSPLANTS

2015	Adult	Pediatric	All Ages
All Organs	415	22	437
Liver	121	8	129
Intestine	21	2	23
Heart	39	8	47
Lung	92	0	92
Heart/Lung	1	0	1
Kidney	125	4	129
Pancreas	6	0	6
Kidney/Pancreas	10	0	10

BELOW: Naim Alkhouri, MD



“How — or if — the disease will progress in an 8-year-old isn’t known.”

He hopes the development of noninvasive tests will lead to answers. “There’s a huge unmet need,” he says.

[www.consultqd.clevelandclinic.org/NAFLD](#) Read more:

NEW STAFF

We searched for the best of the best — and are proud to announce the arrival of the newest members of our team:



Jessica Cohn, MD

SPECIALTY: General Pediatrics

EDUCATION: Medical degree, Stony Brook University School of Medicine; pediatric residency, Floating Hospital for Children at Tufts Medical Center

EXPERIENCE: Previously in general pediatrics in a community private practice



Stephanie Jennings, MD

SPECIALTY: Pediatric Hospital Medicine

EDUCATION: Medical degree, Rush University Medical Center; pediatric residency, Jacobi Medical Center/Albert Einstein College of Medicine

EXPERIENCE: Working in pediatric hospital medicine for 10+ years, most recently at Advocate Children’s Hospital in Chicago



Chuanchau Jou, DO

SPECIALTY: Pediatric Cardiology

EDUCATION: Medical degree, Kirksville College of Osteopathic Medicine; pediatric residency, University Hospitals Rainbow Babies & Children’s Hospital; pediatric cardiology fellowship, University of Utah

EXPERIENCE: Previously practiced at Primary Children’s Hospital; main focus is general pediatric cardiology with a specific interest in the genetic basis of congenital heart diseases



Roy Kim, MD, MPH

SPECIALTY: Head, Pediatric Endocrinology

EDUCATION: Medical degree, University of North Carolina at Chapel Hill School of Medicine; master’s degree in public health, University of North Carolina (Chapel Hill); internal medicine/pediatrics residency, Cleveland Clinic; pediatric endocrinology fellowship, Children’s Hospital of Philadelphia

EXPERIENCE: Most recently served as Director of the Center for Obesity and its Consequences in Health (COACH Clinic) at Children’s Medical Center, Dallas



Hani Najm, MD

SPECIALTY: Chair, Pediatric and Congenital Heart Surgery

EDUCATION: Medical degree, King Saud University Medical School; rotating residency, King Khalid University; general surgery residency, Ottawa Civic Hospital; cardiothoracic

surgery residency, Toronto Hospital/Hospital for Sick Children; pediatric and adult cardiac surgery fellowships, Hospital for Sick Children/Sunnybrook Health Sciences Centre

EXPERIENCE: Performed > 5,000 open heart operations for adults and children, with a special interest in complex congenital and adult surgery. Strives to lower mortality rates, has achieved an overall mortality rate of less than 1 percent in recent years, including many complex cases. Most recently served at King Abdulaziz Cardiac Center in Saudi Arabia, including appointments as Deputy Chairman, Head of Cardiac Surgery, and Consultant for Adult and Pediatric Cardiac Surgery



Sumana Narasimhan, MD

SPECIALTY: Pediatric Endocrinology

EDUCATION: Medical degree, Lady Hardinge Medical College, Delhi University; pediatric residency, New York Medical College; pediatric endocrinology fellowship, University of Cincinnati Hospital/Cincinnati Children’s Hospital

EXPERIENCE: Main interests include general pediatric endocrinology, puberty, and growth and hormone problems in survivors of childhood cancer; treating puberty issues, growth failure and diabetes; quality improvement



Rajyalakshmi Rambhatla, MD

SPECIALTY: General Pediatrics

EDUCATION: Medical degree, M.P. Shah Medical College & Hospital; pediatric residency, MetroHealth Medical Center

EXPERIENCE: Working in general pediatric practice since 1997 in outpatient and inpatient settings. Special interests in newborn care, ADHD, pediatric asthma, quality improvement, working with residents and medical students.



Mary Wong, MD

SPECIALTY: Developmental & Rehabilitation Pediatrics

EDUCATION: Medical degree, Tufts University School of Medicine; pediatric residency, Massachusetts General Hospital

EXPERIENCE: Working in developmental and rehabilitation pediatrics since 2003, most recently in Boston. Special interests include children who have complex medical problems, ventilators, feeding problems and developmental disabilities.



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