



children's health

A publication for those who support the University of Minnesota Department of Pediatrics and the University of Minnesota Children's Hospital, Fairview

Lifesaving cells

For 40 years, the University of Minnesota has led the way in treating childhood diseases with blood and marrow transplantation

The 4-month-old baby had no other hope. Nearly a dozen relatives had already died from the inherited disorder that prevented his body from making the blood cells needed to fight infections.

His doctor, Robert Good, M.D., Ph.D., had hope. After years of research, the University of Minnesota immunologist was ready to take the big step. In August 1968, Good made history by curing his tiny patient with the world's first successful transplant of bone marrow-derived stem cells from a matched donor.

Since then, Good's successors at the University have continued to lead the way in blood and marrow transplantation (BMT). In 1975, John Kersey, M.D., headed the team that performed the first BMT to treat lymphoma. In the 1980s, University physicians used a patient's own marrow to treat chronic myelogenous leukemia. In ensuing decades, others pioneered the use of umbilical cord blood as a source of cells for transplant and the use of BMT to treat inherited diseases such as Tay-Sachs and osteopetrosis.

And in just the last few months, professor and director of the Division of Pediatric Hematology-Oncology and Blood and Marrow Transplantation John Wagner, M.D., and colleagues opened the doors to a new era when they used stem cells to repair a deadly skin condition.

"The University has made tremendous advances over the years in the field of blood and marrow transplantation," Wagner says. "Yet this is just the tip of the iceberg in terms of what we think this therapy can achieve."

Finding a hot spot for treatment

A 40-year track record of famous "firsts" is nothing to shrug at, for sure. But when Patti
Continued on page 2

Photo by Richard Anderson



Four-year-old Alana Barner received a blood and marrow transplant from a University team including Jakub Tolar, M.D., Ph.D., that reversed her disease.





Cuddihee and Bart Barner of Ashland, Missouri, think about BMT at the University of Minnesota, one image comes to mind: the bright-eyed 4-year-old who pads into their kitchen every morning.

Most blood and marrow transplants done

here are performed to treat cancer. But in the 1980s, University physicians began using BMT to treat inherited metabolic and storage diseases, in which the body is unable to break down certain chemicals, leading to a disabling or deadly accumulation of toxic compounds.

In January 2005, the University's national reputation as a leader in applying BMT to such diseases brought Cuddihee, Barner, and their daughter, Alana, north through ice and snow to Minnesota.

Just days before her first birthday, Alana had been diagnosed with Hurler syndrome, in which the body doesn't break down a molecule that, as it accumulates, damages the brain and other organs. A feverish search of the Internet and medical literature—Hurler syndrome is progressive, so time is of the essence—turned up the University of Minnesota as a hot spot for treating the disorder with BMT.

In April of that year, Alana received a transfusion of umbilical cord stem cells under the direction of BMT physicians Paul Orchard, M.D., and Jakub Tolar, M.D., Ph.D.

"She's doing stupendously," Cuddihee says. Without treatment, Alana would be in a downward spiral of organ failure and neurological damage. Instead, she's learning her colors and ABCs and helping Patti in her veterinary clinic.

"We're beyond thrilled—we're absolutely ecstatic," Cuddihee says. "We feel like the University of Minnesota played a huge, huge role in that."

'Hinge in history'

Today about 75 children each year receive a BMT at the University in hopes of curing cancer, bone marrow failure syndrome, immune deficiency, or an inherited metabolic storage disease.

Last fall, New Jersey toddler Nate Liao received a highly publicized transplant at the University for a revolutionary reason—to cure a life-threatening skin disease called epidermolysis bullosa. EB is a rare genetic disorder in which skin fails to produce a type of collagen that holds the layers of skin together. This means

John Kersey, M.D., performed the first blood and marrow transplant for treating lymphoma in 1975.

5,000 and counting

Truth be told, 11-year-old Gregory Ramsey probably cares more about SpongeBob, Wii, and soccer than about the fact that he's the 5,000th recipient of a blood and marrow transplant at the University of Minnesota.

And his transplant team wouldn't want it any other way. Giving kids like Gregory a chance to do just that—be kids—is a big part of what their work is all about.

Before his transplant last winter, Gregory, who has Fanconi anemia, could not make the blood cells he needed to stay healthy. Now, armed for life with an anonymous donor's cells, he's on the mend.

"It's been a long haul, but not as long as some," Gregory's mother, Mary Ramsey, says. "Our son is OK. We have our life to get back to, better than before."

Gregory, Darren, Christopher, and Mary Ramsey



Photo by Scott Strebble

Family turns grief into hope with research fund

When Jay and Lonni Mooreland of Folsom, Calif., heard about the experimental epidermolysis bullosa (EB) treatment being developed at the University of Minnesota, they knew they wanted their infant daughter, Sarah, to have it.

They also knew the treatment would be risky. Only two other people had undergone the blood and marrow transplant (BMT) aimed at curing the devastating skin disease.

On top of that, 9-month-old Sarah had had some previous kidney and heart problems. But Jay and Lonni felt that the chance of curing Sarah's EB was worth it. Without the transplant, there was a strong chance that Sarah would live a painful life and die of skin cancer in her teens or early 20s.

So the Moorelands moved their family across the country to Minnesota while doctors prepared Sarah for the BMT. But before she could receive the transplant, Sarah died from complications of the chemotherapy that preceded it. Doctors believe that her heart—while it appeared to be functioning well during tests—may have been predisposed to weakness because of her earlier heart problems, her parents say.

“While we are very saddened at her loss and sob daily for her, we felt the potential reward outweighed the risk,” says Jay Mooreland. “We had to try—for her. If people don't step forward and take the risk, doctors will never be able to improve upon their strategies.”

The Moorelands are taking another step to help University physician-researchers improve upon the EB therapy. They've created the Sarah Mooreland EB Fund to provide innovators John Wagner, M.D., and Jakub Tolar, M.D., Ph.D., with funding to continue their work.

So far, about 30 friends and family members have contributed \$50,000 to Sarah's fund. Jay and Lonni themselves have given \$28,000 in Sarah's honor.

“We know Sarah didn't die in vain, but it helps to see how her death can influence others to donate,” says Lonni. “Those donations help the doctors, which ultimately brings hope to other families where there wasn't any before.”

To make a gift to the Sarah Mooreland EB Fund, contact Elizabeth Patty at 612-273-8638 or e.patty@mmf.umn.edu.



Lonni and Sarah Mooreland

that the skin can slough off at the slightest touch and that a cough can cause tears in the lining of the esophagus and stomach.

Alerted to the boy's condition by his mother, Tolar and professor of pediatrics Bruce Blazar, M.D., began laboratory studies to determine whether stem cells could supply the missing collagen. Armed with the lab team's encouraging results, Wagner designed a transplant he hoped would meet Nate's needs.

And it did. In June Wagner made international news when he announced that the transplanted stem cells were producing the missing collagen, and Nate's skin was starting to mend.

“Our work on EB serves as a perfect example of translational bench-to-bedside research and what a team of dedicated researchers, physicians, and nurses can accomplish,” Wagner says. “Stem cell therapies offer hope to so many patients with life-threatening disease. It is my belief that we are at a hinge in history when the practice of medicine will undergo a revolutionary transformation. And it's a privilege to be part of it.”

Nate Liao continues to improve after his transplant.



A new challenge

THE WASIE FOUNDATION

A Philanthropic Legacy



Photo by Richard Anderson

*Richard Vehe, M.D.,
and Bryce Binstadt,
M.D., Ph.D.*

*You can help. To view
a video on the need
for a pediatric rheuma-
tology fellowship, visit
[www.med.umn.edu/
peds/rheum/home](http://www.med.umn.edu/peds/rheum/home).
To make a gift, contact
Cindy Adams Ellis at
612-273-8597 or
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Simple pleasures like chasing a puppy or riding a bike are painful—if not impossible—for the thousands of children who suffer from arthritis or related conditions.

Key to easing their pain and making their lives as normal as possible is specialized care by a pediatric rheumatologist, someone who understands both the disease and the unique circum-

stances of childhood growth and development.

Yet only half of the children in the United States who would benefit from such care receive it. The reason? There are not enough pediatric rheumatologists to go around.

“People simply cannot get access,” says Richard Vehe, M.D., director of the Division of Rheumatology in the Department of Pediatrics at the University of Minnesota.

Nationwide, Vehe says, fewer than 200 board-certified, practicing pediatric rheumatologists care for some 300,000 patients. A 2007 U.S. Department of Health and Human Services study commissioned by Congress concluded that another 200 are needed to provide adequate care and advance research.

The Wasie Foundation, a Minneapolis-based private foundation, hopes to improve that ratio by helping to establish a pediatric rheumatology fellowship program at the University. Through the legacy of Minneapolis trucking pioneer Stanley L. Wasie and his wife, Marie, the foundation has offered to provide a matching gift of \$500,000 if the University can raise \$500,000 by June 30, 2009, for the \$1 million needed to jump-start the program.

The Wasie Foundation supports four health-related areas: schizophrenia, arthritis, cancer, and children’s health issues. Marie Wasie had a sister with severe rheumatoid arthritis and was interested in the health needs of children, so this new fellowship program seemed particularly appealing.

“Ultimately, our goal is for children who have a need for the expertise of a pediatric rheumatologist to have better access,” says Jan Preble, Wasie Foundation director of programs.

The program, which could begin as soon as summer 2009, would aim to begin training three fellows in its first three years. It would also provide opportunities to educate community physicians about rheumatologic diseases and carry out research aimed at improving diagnosis and treatment.

Vehe says the University of Minnesota is an ideal place to set up a pediatric rheumatology training program and develop new leaders in the specialty.

“We have a great group of people interested in immune function, a very strong adult rheumatology program, strong orthopaedic department and rehab programs—all these other assets of major institutions,” he says. “We have a great U. We have great partners in the community. We can do this.”

Locally based, federally funded, and internationally tapped, the Healthy Youth Development–Prevention Research Center teams with community partners to create, test, and spread the word about strategies for helping young people grow up strong.

Adolescence can be a rough ride. Helping to make it smoother is the University of Minnesota’s Healthy Youth Development–Prevention Research Center (HYD-PRC), a collaboration between the Medical School, School of Nursing, and School of Public Health.

Housed in the Department of Pediatrics Division of General Pediatrics and Adolescent Health, the center is one of 33 PRCs established by the Centers for Disease Control and Prevention to better align public health research with community needs.

“Partnering with communities, as opposed to doing to communities, is central to who we are,” says center director and professor of pediatrics Michael Resnick, Ph.D. “Whatever the project might be, the goal is to undertake activities that are community driven.”

Lead Peace is a violence-prevention research and demonstration effort begun in 2006 in partnership with Minneapolis Public Schools, Village Social Services, and Kwanzaa Community Church. Middle-school students participate in service learning and related activities over three years. Surveys will determine the impact.

“The hypothesis we are testing is that this kind of positive involvement can replace some of the bullying and aggressive behavior that kids might use when they don’t get positive recognition,” says Renee Sieving, Ph.D., M.S.N., of the Center for Adolescent Nursing and principal investigator for the project. “What we hope to see is that positive engagement helps to reduce their bullying and violence and helps to bump up their general commitment and success in school.”

HYD-PRC community partners recently identified another need: to build awareness among policymakers of the importance of effective evidence-based sex education. HYD-PRC responded by polling parents statewide about their

attitudes regarding sex education in schools.

“We found that an overwhelming majority of parents across socioeconomic, political, and religious groups want medically accurate sex education ... as long as it includes abstinence, ways of saying no, and ways [youth can] protect themselves if they do have sex,” Resnick says.

True to PRC form, that was not the end of the project. The research team has disseminated the results to school superintendents, teachers, health professionals, and legislators.

Resnick says though HYD-PRC research questions are generated locally, the results often have national or even international impact.

“What we are talking about is the opportunities and the experiences that young people have that help them to become capable and caring human beings,” he says. “If young people become active, engaged people as adolescents, learn to develop new skills, then use those skills to be of help and service to others, they are much more likely to be engaged adults throughout the rest of their lives.”



Eighth graders at Nellie Stone Johnson Community School, including Timeshia Chandler and Destinee Carr (below), packaged rice for hungry kids in Malawi as part of the Lead Peace service learning program.



To see live images of the hospital being built, visit www.uofmchildrenshospital.org/construction.

Photo by Willette Photo Works



Breaking new ground in cures and care for kids

With child- and adult-size shovels in hand, children and representatives from the University of Minnesota, Fairview Health Services, and state and local government on June 18 broke ground on a new, state-of-the-art facility for the University of Minnesota Children's Hospital, Fairview.

The groundbreaking ceremony marked the start of construction on the new hospital building, which is scheduled to open in 2011.

The new facility will replace the current "hospital-within-a-hospital" configuration and unite child and mother services in one family-friendly location. It also will house some of the country's leading clinical programs, including programs in pediatric organ transplantation, blood and marrow transplantation, cancer treatment, and heart surgery.

The new hospital building will be "green," incorporating an environmentally sensitive design, eco-friendly materials, and green spaces, such as a healing garden. It also will be "lean," meaning that it's designed to maximize value for patients and their families by reducing errors, waste, waiting time, and unnecessary transport or movement.

And it's all to provide the highest-quality experience for children and their families in an environment of hope and healing.

"This new building will be the perfect setting for our academic children's hospital to carry out our threefold mission of research and discovery, teaching and learning, and outreach and public service—making it a center of pediatric health for Minnesota's children and families," says University President Robert Bruininks, Ph.D.

University of Minnesota Children's Hospital, Fairview pediatrician-in-chief Aaron Friedman, M.D., thanks Chloe Brunsberg for season tickets to the Minnesota Timberwolves basketball games. Timberwolves mascot Crunch looked on as Chloe presented the tickets on behalf of her parents, Jeff and Sue Brunsberg. Jeff works for Fargo State Bank and Trust. The bank gave employees \$1,000 to "pay it forward" as a holiday gift, and the Brunsbergs generously chose to direct their gift of season tickets, games, and movies to children at the hospital.



Save the date for WineFest No. 14

Because of your support, the Midwest's premier charity wine event has reached a new level. The critical funds raised at WineFest No. 13, held May 9 and 10, helped WineFest top \$7 million raised since the event's conception for innovative research into treatments and cures for childhood diseases at the University of Minnesota.

And this popular wine event keeps getting more popular. For the first time this year, both evenings' events sold out. Guests enjoyed exquisite wine from our Australian winemasters and mouthwatering menus from top Twin Cities chefs.

Don't miss WineFest No. 14, which will prove to be another weekend of top-of-the-line wines, gourmet cuisine, dancing, and auctions—all to benefit children's health. It will be held May 8 and 9, 2009, at the Depot in downtown Minneapolis and will feature honorary winemasters from Ste. Michelle Wine Estates, ranked among the top 10 premium wine companies in America.

For more information, visit www.thewinefest.com.

May 8 & 9
2009



Event co-chairs
Yvette Mullen and
Joanne Jirik Mullen

Photos by Competitive Image

Golf to brighten an ill child's day

Celebrate the kids, families, and health-care leaders who are working to make children's hospital rooms a little more like home through Adopt A Rooms. The third annual A Whole New World Golf Tournament and Celebrity Culinary Experience will be held Monday, September 22, at the TPC Twin Cities in Blaine.

For more information, visit www.upf.umn.edu/news or www.adoptaroom.org.



Fun for kids and adults at Party in the Park



Join the cast of characters from Nickelodeon on Sunday, November 9, at Nickelodeon Universe at the Mall of America for

an exclusive evening of fun and festivities supporting the Department of Pediatrics at the University of Minnesota. Families will enjoy a magical night with unlimited rides on select attractions, delectable dinner and desserts, one-of-a-kind silent auction items, and much more.

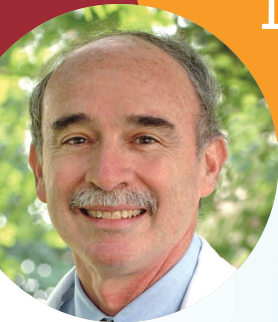
For information about tickets or sponsorships, visit www.upf.umn.edu/news or call 612-273-8643.

What would you like to see in Children's Health?

Send us an e-mail at upf@mmf.umn.edu with "Newsletter idea" in the subject line and let us know!

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On My Mind



If it seems like we've been grabbing a lot of headlines lately, it's because we have.

In the past few months, the innovative physician-scientists here in the Department of Pediatrics have been interviewed by media outlets across the country about a cord blood and bone marrow transplant (BMT) they performed at the University of Minnesota Children's Hospital, Fairview. That transplant has dramatically improved a young boy's genetic skin disease, and we are convinced that we are on a path to curing this devastating illness.

As you'll read in our cover story, the University's BMT program is turning 40 this year. We're celebrating a long history of transplant "firsts"—and celebrating the 5,000-plus people who have received another chance at life through the program.

Perhaps it's not too surprising that these things are happening in a hospital with the only top-ranked pediatric cancer program in the Upper Midwest, according to *U.S. News & World Report*. The

hospital's respiratory disorders care program also ranked among the country's best.

Now we're in the process of building a new children's hospital facility that will strengthen all of our programs by bringing together patient care and clinical research. The University of Minnesota Children's Hospital, Fairview is the only children's hospital in the state that is part of an academic health center. That means members of the hospital's medical team not only deliver the latest innovations in pediatric care, they also create them.

That's the value of academic medicine, and that's why I've dedicated my career to it. We are driven to discover preventions, therapies, and cures to give all children the chance to become healthy adults.

I hope you'll continue to support our work as we keep pushing toward that goal.

Sincerely,
Aaron Friedman, M.D.
Head, Department of Pediatrics
Ruben-Bentson Chair in Pediatric Community Health

children's health Fall 2008

Published twice a year by the Department of Pediatrics and the University Pediatrics Foundation, an affiliate of the Minnesota Medical Foundation

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