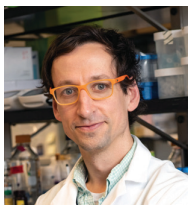
ISSUE 28

St. Baldrick's FOUNDATION | CONQUER KIDS' CANCER

YOU MAKE AN **impact**

SHOT ON GOAL

There's new hope for kids with aggressive brain tumors, thanks to physician-scientist **Carl Koschmann, MD**, and his team. Their research has led to the first FDA-approved treatment for DMG—the deadliest form of pediatric cancer.



Carl Koschmann, MD

There's no cure for diffuse midline glioma (DMG), and kids usually live just nine to 15 months after diagnosis. That's unacceptable to Dr. Koschmann, who has devoted his career to finding a cure. Now, he's closer than ever.

Clinical trials at his University of Michigan lab have led to the FDA approval last August of ONC201—also called Modeyso—a drug that has produced promising results. Dr. Koschmann reports that a portion of kids treated with ONC201, which is taken orally once or twice a week, live longer than expected. He and others have also found that the drug is very well tolerated with few to no side effects.

Dr. Koschmann says this promising treatment is an important step forward. "It's really our first good oral therapy shot on goal, but it's unfortunately not a cure," he explains. "There have been lots of advances for other types of pediatric cancers, like leukemia and neuroblastoma. Now, I feel like we're beginning to climb that hill with DMG. We're putting together the pieces of a puzzle that will eventually lead to kids living longer."

Initially, Dr. Koschmann hadn't planned on a career in pediatric oncology. But then, legendary physician-scientist James Olson, MD, PhD, from Fred Hutchinson CRC/Seattle Children's, urged him to focus on high risk pediatric brain tumors. "Something just struck me about the kids," Dr. Koschmann recalls. "The ability to

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The St. Baldrick's Connection

As a young researcher in 2014, Carl Koschmann, MD, was named a St. Baldrick's Fellow, which supports promising work by young scientists. That grant helped to launch Dr. Koschmann's research career.

Co-director of the Chad Carr Pediatric Brain Tumor Center at the University of Michigan, Dr. Koschmann says St. Baldrick's donors paved the way for his research career: "The St. Baldrick's grant convinced the university that my work was serious and well received. It also gave me confidence in myself as a researcher. I may not be here now without that funding."

Today, he's a big advocate for young scientists, who face increasing challenges due to lack of funding. Dr. Koschmann concludes, "We're seeing a lot of junior scientists leaving the field. It's devastating. We want to keep those young researchers in the pipeline—they're the future of pediatric cancer research. It's more important than ever to invest in organizations like the St. Baldrick's Foundation so we don't lose our momentum."

Love, Dominic

Kira Liples' wrist tattoo is a daily reminder of her late son, Dominic. Scrawled in his childlike printing, it says, "I'll never stop loving you, Mommy." Now, Kira is keeping Dominic's enduring love alive to help other children with cancer.

The Liples family

Before his cancer diagnosis, Dominic was a healthy kid who almost never got sick. Energetic and funny, he loved sports, his friends, and his family—especially his younger brother, Ciarlo, who was born with spina bifida.

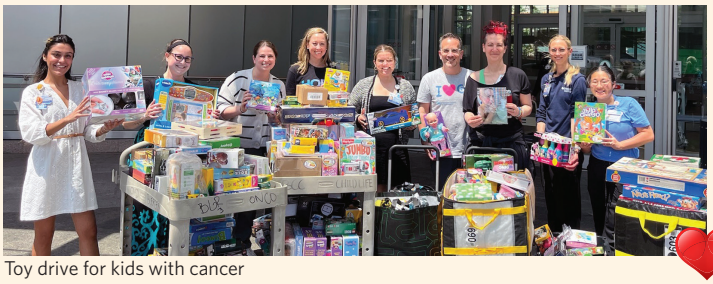
Everything changed on March 7, 2016, when Dominic was diagnosed with diffuse midline glioma (DMG), a rare and extremely aggressive pediatric brain tumor. A couple of weeks before, Kira started noticing minor symptoms—unusual fatigue, red cheeks, unwillingness to tie shoes. But Dominic's health suddenly deteriorated; at a pool party his left arm developed a tremor and he had difficulty swimming. By the next day, Dominic began dragging his left foot.

The St. Baldrick's Foundation Pray for Dominic Hero Fund has raised more than $500,000 for pediatric brain tumor research, with special focus on DMG.

Within 24 hours of the pool party, Kira and her husband, Kenny, received the grim diagnosis: DMG has no cure and most children survive less than a year. Kira recalls, "The neurologist cried when she gave us the diagnosis because she knew there was no hope."

Over the next nine months, Dominic endured several surgeries, radiation, chemotherapy, and an experimental immunotherapy to give him more time. Through it all, Dominic continued to be a source of joy—building LEGOS® with one hand and sharing typical little boy potty humor with his family and medical team. Exactly nine months to the day of his diagnosis, the 8-year-old lost his battle, cradled in Kira's arms.

Despite their grief, those who loved Dominic immediately vowed to honor his legacy. Before the funeral, Kira had his sweet message tattooed on her wrist, fulfilling a promise she had made to Dominic. Several months after Dominic passed, his neuro oncologist announced that she was switching her research focus to high grade glioma in memory of Dominic and several other special children. Kenny has also shaved his head to raise funding for St. Baldrick's since 2017.



Toy drive for kids with cancer

Since Dominic's passing, Kira has organized drives to donate thousands of toys to kids with cancer through Dominic's Toy Chest and serves as membership and outreach coordinator for Momcology, a national nonprofit community-based patient advocacy and support organization founded by mothers of children with cancer. She's also been instrumental in raising funding for pediatric cancer research. The St. Baldrick's Foundation Pray for Dominic Hero Fund has raised more than $500,000 for pediatric brain tumor research, with special focus on DMG. "Right now, children still do not survive a diagnosis of DMG," Kira says. "Brain cancer is the leading cause of death for children with diseases. And it could happen to anyone; Dominic was perfectly healthy until he wasn't. These tumors are still killing our children."

Today, Kira finds hope in lots of things, especially in Ciarlo, now a 15-year-old honor student and talented wheelchair athlete. She spends much of her time advocating on behalf of Ciarlo and other kids with spina bifida, but also devotes a lot of energy to helping families coping with pediatric cancer. Most encouraging was the announcement earlier this year of a promising new treatment for DMG that is giving kids with cancer more time. A doctor instrumental in this discovery, Carl Koschmann, MD, received funding as a St. Baldrick's Fellow in 2016 (see cover story).

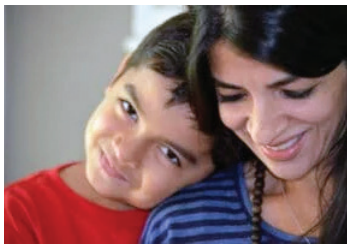
Yet, Kira knows that it's just the tip of the iceberg: "With cuts in pediatric cancer funding on the federal level, the only way we can give any of these families hope is by donating privately through organizations like the St. Baldrick's Foundation. We've raised over half a million dollars, but it's just a drop in the bucket. It's a grain of sand on the beach, compared to what's needed to make a difference."

You can make a difference for kids with pediatric brain cancer. Scan the QR code to make a contribution to the St. Baldrick's Foundation Pray for Dominic Hero Fund today!


Felice and Noah


Ari and Max


Carley and Laura


Asha and Faris


Lorna and Sam

Super Moms

Research to create a new vaccine to treat Ewing sarcoma is underway, thanks to a team of five dedicated mothers who have banded together to advance the study in honor of their late children. The moms—Ariella Ritvo-Slifka, Laura Rutledge, Lorna Day, Asha J. Virani, MD, and Felice Adler-Shohet, MD, have pooled $1.5 million in foundation funding to create the Advancing Cures for Ewing Sarcoma (ACES) Award, the newest grant within the St. Baldrick's Foundation Strategic Research Partnership Program.

Ewing sarcoma is a rare and aggressive type of bone and soft tissue cancer affecting children and young adults. While the five-year survival rate is 70-80 percent for localized cases, that drops to just 20-30 percent when kids relapse. ACES will fund a three-year study proposed by Brian Ladle, MD, PhD, a pediatric oncologist and immunotherapy expert at Johns Hopkins University. Dr. Ladle is developing a new vaccine that would harness the power of the body's own immune system to teach cells to remember, identify, and attack Ewing sarcoma cells—providing a powerful, lasting defense to ward off relapse.

"The St. Baldrick's Foundation is honored to help facilitate this remarkable collaboration," said Kathleen Ruddy, CEO of the St. Baldrick's Foundation. "This is what happens when courage, community, and commitment come together: Bold ideas get funded and hope moves closer to reality."

This amazing initiative is funded through The Alan B. Slifka Foundation in honor of Max Ritvo, a gifted poet with a generous spirit who faced his illness with extraordinary courage and creativity; The Rutledge Cancer Foundation in honor of Carley Rutledge, an avid traveler who loved biology, politics, gardening, and adventuring; The Sam Day Foundation in honor of Sam Day, a bold and adventurous boy with a quick wit, big ideas, and love of life; The Faris Foundation in honor of Faris D. Virani, whose triumphant spirit, creativity, and deep wisdom inspired others far beyond his years; and The Shohet Family Fund for Ewing Sarcoma Research—a St. Baldrick's Hero Fund in honor of Noah Shohet, a guitarist, foodie, and world traveler who taught elementary school in Spain, even while fighting cancer.

Shot on Goal (continued)

treat kids who are extremely sick and the possibility of bringing them back to full health appeals to me. It can feel overwhelming emotionally at times, but it's also very rewarding."

For his study, Dr. Koschmann and his colleagues helped uncover how ONC201 works in DMG cells carrying the H3K27M mutation, which is seen in 80% of DMG tumors, disrupting the energy that fuels cancer cells. Dr. Koschmann says, "ONC201 goes to the mitochondria—the gas tank of H3K27M-DMG cells—and completely disrupts it. We found that this reverses an epigenetic feature in H3K27M-DMG cells, making them behave more like normal brain cells."

"Suddenly, we have long-term survivors. That's never happened before."

Most kids on ONC201 eventually relapse, and now, researchers like Dr. Koschmann are trying to figure out why: "We saw some patients' tumors responding really well, but it's not clear yet why others don't. This is a drug that needs to be studied further so we can figure out which patients it's best suited for. From my experience, the optimal way to build on this would be to use it in combination with other targeted therapies and immunotherapies."

As Dr. Koschmann continues his research, he's encouraged for the future: "We're still learning about this tumor and until now, there's been very little good news for these kids. But suddenly, we have long-term survivors—that's never happened before. We don't know how long it will take to climb this hill, but we're determined to get to the top."

Looking forward, Dr. Koschmann and other scientists are anxiously awaiting the results of a next-stage clinical trial of ONC201 happening overseas. Continuing the work of researchers in the U.S. is also critical. Dr. Koschmann says: "With recent restrictions in federal funding, we're at risk of losing our momentum here in the U.S. This type of research doesn't happen without infrastructure and funding. This is the time for donors to invest and be part of something exciting."

Carl Koschmann, MD, is a board-certified pediatric oncologist at University of Michigan Health C.S. Mott Children's Hospital and co-director of the Chad Carr Pediatric Brain Tumor Center. He is a graduate of the University of Wisconsin-Madison School of Medicine and Public Health. Dr. Koschmann was named a St. Baldrick's Fellow in 2014, a designation that helped to launch his research career.

Ripple Maker

Rick Mondschein and Susan Heard

For Rick Mondschein, supporting pediatric cancer research is a priority. Since 2010, he has participated in eight head-shaving events, raising $27,655 for the St. Baldrick's Foundation. That's impressive—but the Whitehall, Penn., resident wanted to do more. So, he signed up online to make monthly contributions to the foundation. Rick says, "While the head-shaving events raise millions of dollars for pediatric cancer research, much more is needed. As a monthly donor, I am contributing to a dedicated, consistent funding source for research."

At St. Baldrick's, Rick is known as a "ripple maker": An everyday hero who understands that small, consistent gifts can create waves of progress. Monthly donors spread their annual pledge to St. Baldrick's over 12 months to make a big impact without significantly affecting their monthly budget. Monthly donors receive special communications so they can track the impact of their giving.

A retired mathematics teacher, Rick has been moved by the stories of children and parents affected by cancer when he attends head-shaving events to benefit St. Baldrick's. "My shavee experiences have been heartwarming, heart wrenching, incredibly joyous, incredibly sad, exciting, and depressing—all at the same time," he says. "It is always a remarkably moving event that touches everyone present to their core."

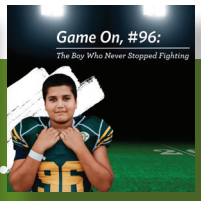
Kelly Forebaugh, St. Baldrick's director of development, notes monthly giving provides a steady and predictable revenue stream that ensures financial stability, while supporting long-range plans. "Monthly giving is a budget friendly option for those who want to make a bigger impact," she says. "You determine what annual amount works best for you and then spread your contribution out over a year."

Rick was first inspired to support the St. Baldrick's Foundation through David Heard, an Honored Kid who passed away from neuroblastoma at age 10 in 2010. David's mother, Susan, is now the senior director of distinguished giving at St. Baldrick's. Today, Rick is more determined than ever to find a cure: "While there are many worthwhile charitable organizations, there are none more important than St. Baldrick's, which is dedicated to saving children's lives. I've never personally experienced childhood cancer. But I believe it is the solemn duty of adults like me to support pediatric cancer research."

Monthly contributions make a powerful impact on the fight against pediatric cancer, Kelly adds: "Monthly gifts do much more than fund research. They create ripples of hope, progress, and possibility. If you're considering making a gift to St. Baldrick's, I urge you to join a community of changemakers whose generosity fuels lifesaving research, funds safer treatments, and gives survivors a brighter future."

Be a ripple maker for kids with cancer! Just scan the QR code to get started.

Let's Connect



Stbaldricks.org/distinguished-giving **Stbaldricks.org/get-involved/business** **Stbaldricks.org/league**

Thank you!

Thank you for supporting lifesaving childhood cancer research. Through your gifts, you are helping the St. Baldrick's Foundation fund the best childhood cancer research, no matter where it's being done. With $368 million in research grants in the U.S. funded since 2005, we are the largest charity funder of pediatric cancer research grants. YOU made progress possible!

Ways to Give

Kids with cancer need cures now, and it starts with you. Donate to fund the most promising childhood cancer research. Give once or monthly. Start today: **stbaldricks.org/ways-to-give**

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Scan here to help find a cure!

The St. Baldrick's Foundation is a volunteer and donor powered charity committed to supporting the most promising research to find cures for childhood cancers and give survivors long and healthy lives.