

thank you for your support of



THE CREATING HOPE ACT

Thank you for supporting The Creating Hope Act.

Championing The Creating Hope Act was an act of faith and commitment by you. It was a wonderful example of the democratic process, in which people across the political spectrum came together to express their hopes that better drugs could be developed to cure our sickest children. It was an example of Members of Congress responding with innovative legislation to create a chance for our children with serious diseases to live longer, healthier lives.

Thank you. Without you, we could not have achieved passage of the Creating Hope Act, passed as Priority Review to Encourage Treatments for Rare Pediatric Diseases, Section 908 of the Food and Drug Safety and Innovation Act of 2012, 21 U.S.C. Sec. 360ff.

Sincerely,

Nancy Goodman
Executive Director
Kids v Cancer
kidsvcancer.org



During our hard-fought efforts to save our son, we came to realize that Jacob and other children with pediatric cancer are at a serious disadvantage when it comes to research for new drugs.

My son, Jacob, was your typical, 8-year old boy when he was first diagnosed. He loved to play video games with his brother when he was allowed. He loved any sport with a ball, though he wasn't particularly able. He always did his homework and rarely turned it in. He was a passionate rock keyboard player and vocalist.

As a result of his treatments, Jacob suffered severe neurological and cognitive impairments, including an inability to speak or, for a time, move. He was wheelchair bound, he lost gross and fine motor skills, and he experienced significant memory deficits. Jacob had unmanaged pain, anorexia, nausea, baldness, and multiple infections. He was hospitalized for months at a time. And still, unbelievably, he never complained and just focused on his friends, his family, and attending

school as much as he could. Jacob died two years later at age 10.

During our hard-fought efforts to save our son, we came to realize that Jacob and other children with pediatric cancer are at a serious disadvantage when it comes to research for new drugs. In Jacob's memory, I started Kids v Cancer.

I am very grateful for the support you provided in our effort to author and advocate for Section 908 of the FDA Safety and Innovation Act, based on The Creating Hope Act.

I look forward that one day when children like Jacob will be able to live full, healthy, and productive lives.

Nancy Goodman

JACOB

We lost our beautiful and loved grandson, Jacob, who at the age of eight was diagnosed with medulloblastoma.

An adorable red head, Jacob somehow found a way to always be included in the most unlikely of groups and have everyone delighted to be with him, whether it was with older boys playing ball or a father/son duo. He sang, danced and played his heart out, full tilt.

That call on Monday afternoon at 4:00 to say he had brain cancer changed us all. Now, four years since he died, Jacob's presence is still with us all the time, mostly with his pre-illness sunny face and playful way. But when I saw this picture – his beautiful smiling face looking out, it wrenched me back to the reality of what we have lost. I miss him so, so much. Usually the



deep pain comes at the anniversary of the diagnosis or of his death, but a picture can bring me back too.

I remember Jacob as he was when he was ill and I remember his courage. I remember how his young brother, Ben, was the one always allowed under the blanket when Jacob covered his face. I remember how true a friend a little brother can be. I remember the love and constant care his mom and dad gave him. His mom always says, "all parents do this."

Thank you to all of you who have supported The Creating Hope Act. My deepest hope is that it will allow the possibility that children and families will have a chance at being spared what we have gone through.

Judi and Ed Goodman
Grandparents of Jacob Froman

My deepest hope is that The Creating Hope Act will allow the possibility that children and families will have a chance at being spared what we have gone through.



During the last years of Seamus's life, he struggled with hearing loss, neuropathy, and chronic pain.

SEAMUS

On July 9, the same day this act was passed, my 21-year-old brother, Seamus, lost his eleven-year battle with medulloblastoma, a form of brain cancer.

Seamus had undergone extensive chemotherapy and radiation, as well as participated in three clinical trials, over his eleven years of treatment. At the end of his journey, the long-term side effects of the drugs he had taken greatly impeded his quality of life. During the last years of Seamus's life, he struggled with hearing loss, neuropathy, and chronic pain.

I hope that in the future cancer treatments can become both more effective and less toxic, so no other family has to endure the torture of watching their loved one lose a battle to this awful disease.

By passing The Creating Hope Act you have given hope to so many families. Thank you.

Shannon O'Connor

Thank you very much for passing The Creating Hope Act. This law is very important to my family because my 3-year-old daughter, Abby, was diagnosed with optic pathway glioma when she was only 6 months old.

We have been told that she will be on and off chemotherapy for the rest of her life, which could be five more years or fifty: no one can predict.

Most children become more and more disabled and blind over time and eventually succumb to side effects and complications from chemotherapy and radiation, rather than from the disease itself.

Abby has already endured one year of extremely toxic chemotherapy and is currently stable. We hope that future treatments for her will be more effective and less dangerous.

Thank you for giving frightened families of children with cancer some hope that their children may live normal lives.

Thank you for giving frightened families of children with cancer some hope that their children may live normal lives.

Melissa Farber

ABBY





DANIEL

Thank you for helping make Daniel's wish come true, for helping these other kids.

I'm writing on behalf of Daniel French, my grandson, because he is unable to write. He is unable to write because we lost him to cancer on February 25, 2011. It's been hard on all of us, especially his mother and brothers and sisters.

Daniel fought cancer for three years. He had a primitive neuroectodermal brain tumor, his third one, when he died.

Daniel was a very loving, 14-year-old guy when he passed. He wanted to help other kids, so they wouldn't have to go through this.

So I'm writing this for Daniel. Thank you for helping make Daniel's wish come true, for helping these other kids.

Thank you,

Brenda Denton

AJ

For a couple years, my right shoulder really ached. It ached from throwing thousands of footballs to my 14-year-old son, Alex John (AJ), trying to lead him just right on deep post patterns, to keep up with his speed. And it ached from catching the big lug at the end of each of our sessions, as he would run and jump into my arms, yelling, "The Bills win the Super Bowl, the Bills win the Super Bowl!" Just us dreaming.

It ached from trying to pitch as fast as I could so he wouldn't hit me, but he always did. It hurt enough that I slept only on my left side, and if I rolled over on it, it would wake me in the night. But I didn't care. How could I stop doing those things? I loved it.

My new problem is that my shoulder slowly but surely stopped aching. Now what keeps me awake at night is my broken heart. You see, I have no one to throw those passes to, no one to brush back anymore. AJ left us on January 5, 2008, a victim of childhood cancer.

I could tell you about the greatest son ever, about how funny, smart, athletic, musical, animal loving, and, as we came to know during those dark nights, how evolved he was. I could tell you about how he fought cancer, so strong and graceful. And it would break your heart.

Instead, I will tell you that after five years of learning more and more about childhood cancer, after five years of seeing so many other children fight, so many parents and siblings devastated, I am just as heartbroken and even more outraged.

I'm outraged at the lack of funding for research, family support, and long-term survivors. I'm outraged that the National Cancer Institute directs less than \$200 million of its \$5.2-billion-dollar budget to childhood cancers, which in turn kill more kids every year than cystic fibrosis, muscular dystrophy, asthma, and AIDS combined!

I'm outraged when I see an 80-percent cure rate quoted — because we don't hear about relapsed neuroblastoma patients, where the options are so limited, or DIPG, where there is literally no option.

I'm outraged that two-thirds of those five-year survivors face dramatic and devastating side effects (secondary cancers, major organ damage) from the "cure."

Thank you for helping to diminish my sense of outrage.

Robert Piniewski
AJ's dad
People Against Childhood Cancer
PeopleAgainstChildhoodCancer.org



Thank you for helping to diminish my sense of outrage.



For many kids like our son,
Max, the drugs that are
currently used just don't work.

Thank you for your support of The Creating Hope Act.

For many kids like our son, Max, the drugs that are currently used just don't work. That's because they were designed for a completely different type of adult cancer — and handed down to use on kids where there's "hope" that they work.

We need better drugs for our kids, to help them live full, productive lives. The incentives provided by The Creating Hope Act will help to spur childhood cancer-specific drug development, so that kids like Max get access to cutting-edge therapies developed by the smartest minds in science and medicine.

Andy and Melissa Mikulak
Parents of Max
June 30, 2001 – August 31, 2008
Forever 7

MAX

JORDAN

Thank you for your support in passing The Creating Hope Act. We will be forever grateful.

Our daughter Jordan was born on September 17, 1999. She was beautiful and loving and very intelligent, but that was before the word medulloblastoma was added to our vocabulary. When Jordan was 26 months old, in November 2001, we learned that this horrible cancer had taken root in her little brain.

When she was almost four, Jordan's cancer relapsed and we were forced to choose between losing our baby girl and saving her life but crippling her intelligence forever. We She began proton radiation treatments.

The good news is that the radiation and chemotherapy saved her life.

The bad news? Jordan is 13 years old and reads at a late-second-grade level. Socially, she behaves like a 5-year-old. She wears hearing aids and gets a daily injection for growth-hormone deficiency. She will most likely be living with us for the rest of her life and will never be a wife, a mother, or even hold a job. The jury is still out on whether or not Jordan will develop a secondary cancer, suffer organ damage, or have strokes and seizures.

We love our daughter with all of our hearts, but we have had to mourn the child she should have been. We have had to let go of the dreams we had for her when she was born and find new goals, new hopes that are attainable with her limited potential. So much is beyond her ability.

With sincere and heartfelt gratitude,

Angela, Guy, Jordan, Ben, and Emma Atherton



We were forced to choose between
losing our baby girl and saving her life
but crippling her intelligence forever.



THOMAS

What I learned over the past two years of his life is that modern medicine is not as impressive as I'd previously thought.

Our son, Thomas Cowden, died this past April from medulloblastoma and complications from treatments.

His surgery went well, but after radiation, things got progressively worse. Three months after his treatments ended, he was diagnosed with radiation necrosis in his brainstem. Tom gradually lost all muscle function. He was 15.

What I learned over the last two years of his life is that modern medicine is not as impressive as I'd previously thought.

Thank you and God bless you!

Sincerely,

Nelson Cowden

KARISSA

Thank you so much for passing The Creating Hope Act. I am an eight-year childhood cancer survivor. I was diagnosed with nasopharyngeal carcinoma when I was 13 and was treated with chemotherapy and radiation therapy.

While my twin brother started eighth grade, I started chemo. It was devastating. I had it rough in chemo: I threw up at least 15 times over a period of up to two weeks during each round.

When I started radiation, I thought it would be easy compared to the agony of chemo, but I was wrong. It was its own kind of torture.

My biggest fear is my cancer coming back and having to go through that treatment again, but I can only pray that I am one of the lucky ones who continue to be healthy.

I now spend my free time working with other sick kids like me. It breaks my heart to see them have to go through the same misery I did. There have not been any new developments in the treatments, but I am confident that with the passing of this act that will change.

Thank you for passing this act and for doing everything you can to help fight cancer! You are truly making a difference in the lives of so many.

Sincerely yours,

Karissa Carey



I now spend my free time working with other sick kids like me.



BRIANNA

My name is Bri Commerford. I am 14 years old and just started high school. I am also a pediatric cancer survivor. When I was 9, I was diagnosed with stage IV Hodgkin's lymphoma.

Being a kid with cancer was completely horrible. I missed out on two years of school, playing sports, hanging out with friends — basically just being a kid. Instead, I spent most of those two years in hospitals and clinics, where I had tons of infusions, transfusions, and surgeries; I had numerous hospital stays, because the treatment made me so sick.

I am now four years cancer free and living my life to the fullest. No kid should have to endure the pain and suffering that comes with a cancer diagnosis.

I am on a mission to help other kids with cancer. When I was the 2010 Hyundai Hope on Wheels National Youth Ambassador, I met directly with many Members of Congress, asking for their sponsoring of The Creating Hope Act.

I am grateful to Congress for all the tireless efforts in having The Creating Hope Act be signed by the president.

I am proud to be an American teenager and look forward to a world without cancer!

With much appreciation and *hope* for a day without cancer,

Bri Commerford

No kid should have to endure the pain and suffering that comes with a cancer diagnosis.



BRANDEN

Thank you from the bottom of our hearts. It is about time our babies, children, teens, and young adults get the help they so desperately need.

Our middle child, Branden, was diagnosed with acute lymphocytic leukemia a week after his 17th birthday.

After Branden's initial hospital stay, he came home, depressed, with a port in his chest. He didn't move around at all. It wasn't too long before Branden snapped out of it and was happy to rejoin his senior class. The following spring he played five parts in a musical, caught up with all the work in his AP classes, and graduated with honors. He received a Bright Futures Scholarship and is going to a local college now. He has his whole life ahead of him.

Only when Branden is finished with his treatment will we find out whether his leukemia is gone for good. He's definitely weak, but he never complains and never asks for special attention. When people find out that he is living with a life threatening illness, they are shocked.

Thanks for fighting for our children. We never, ever want to see another wasted human life.

Love and respect and appreciation,

Elizabeth and Arthur, Kristin, Branden, Glenn Akens

Thanks for fighting for our children. We never, ever want to see another wasted human life.



DUSTIN

The treatment our son was on employed the same drugs that have been used since the 1950's or '60's.

We lost our precious son Dustin in 2001 at age 11 to rhabdomyosarcoma. The treatment protocol that our son was on employed the same chemotherapy drugs that have been used since the 1950s or '60s. We hope that some obviously much-needed progress will be made as a result of The Creating Hope Act.

Once again, thanks.

Victor and Traci Henderson

MINTY

Thank you for supporting The Creating Hope Act.

I watched my daughter's body slowly be taken over by brain tumors, despite six months of high-dose chemotherapy. The last two months of her life were like a living death. There are truly no words to describe the horror of watching your 1-year-old slowly shut down, while trying to figure out how much morphine to give.

I hope that drugs can be developed so no child has to go through what mine went through, and no parent has to suffer this immense pain of watching his or her child die from cancer.

I hope that drugs can be developed... so no parent has to suffer this immense pain of watching his or her child die from cancer.

Sincerely,

Mei Lai Hippiisley Coxe,
Minty's mummy





JONATHAN

I want to thank you for supporting The Creating Hope Act, Section 908 of the Food and Drug Administration Safety and Innovation Act, S. 3187.

Two years ago, cancer was the last thing on my mind. Having just finished my sophomore year at Harvard, I was working in Beijing, climbing the Great Wall, and looking forward to going back to school in the fall. It was a joy-filled summer with friends, marred only by the occasional knee pain I had been told was tendonitis.

Today, I can barely go a day without thinking about cancer. The knee pain was not tendonitis; it was a malignant tumor. I will never forget the day the oncologist said those three words, "You have cancer." He sent me home on crutches. If I broke my fragile leg, the cancer would spread, and the doctors would have to amputate my leg.

Thirty years ago, they would have amputated my leg as standard procedure. But immense advances in surgery and prosthetics have let me keep my leg. I have a cutting-edge artificial knee and a titanium femur. It is nothing short of a medical miracle. Yet, the chemotherapy regimen I was on was positively ancient. Of my three chemotherapy drugs, the newest was approved by the FDA in 1978. My knee is from 2011, so why were my drugs from 1978?

Now, I'm lucky enough to be adjusting to a life after cancer. Since I finished chemotherapy and relearned how to walk, I had the privilege of interning at the White House. I just started my senior year and am back with my friends and going to class. Normalcy has never been so exciting. I can only hope that one day, every child I saw in the pediatric cancer ward will get the chance to see a life after cancer. The Creating Hope Act makes it more likely they will.

Thank you.

Jonathan Yip

I have a cutting-edge artificial knee and a titanium femur. Yet, the chemotherapy regimen I was on was positively ancient.



JACOB

I had the privilege of taking care of and loving Jacob Froman. He was an energetic, spunky, compassionate boy. He spent many of his days running the streets of Manhattan with his younger brother, Ben, rooting on the Collegiate basketball team, grabbing a slice of pizza at the corner shop, and thriving on music and being in a rock band. He was extremely bright, with a fabulous sense of humor.

Jacob was diagnosed at 8 years old with medulloblastoma, a form of brain cancer. This was absolutely devastating. The tragic news was received on a Sunday; he was in surgery that Monday. The limited options available weren't able to cure him, and Jacob passed away at the age of 10.

It is difficult to express the magnitude of how deeply grateful I am to everyone who supported The Creating Hope Act.

It is comforting and reassuring to know that our children have hope, thanks to you.

Much appreciation,

Jennifer Flynn

It is comforting and reassuring to know that our children have hope, thanks to you.



ADRIENNE

That first morning with no treatments to take and no pills to swallow: it's exhilarating. It is impossible not to think, "I am finally ready to begin my life."

Thank you so much for helping to get The Creating Hope Act passed. You have given so much hope to so many children affected by pediatric rare diseases.

Staring down a long treatment plan—anywhere from one to three years for kids with cancer—can be very difficult, especially with a child's limited life experience. Long days and dark, endless nights are often spent wondering, "Is this really worth it?"

Doctors, parents, neighbors, and friends constantly provide assurance, always insisting that things will be fine. With luck, the days pass quietly, one chemotherapy treatment blending into the next.

Those who are among the "lucky" ones finally reach that last treatment. It's hard not to be a bundle of nerves, unsure of feelings; it's quite common to be both terrified and ecstatic.

That first morning with no treatments to take and no pills to swallow: it's exhilarating. It is impossible not to think, "I am finally ready to begin my life."

Sincerely,

Adrienne Westcott

October 10 began like any other day for my family. Our 16-year-old daughter, Adrienne, was like most teenage girls. She had been looking forward to Homecoming — the dinner, the dance, the date, and her dress — but she came home earlier than expected, saying she was very tired. A few hours later, Adrienne woke my husband and me complaining of pain in her hip. We gave her Advil. The next morning, when Adrienne again complained of pain in her hip, I took her to the emergency room. The ER physician said to give it twenty-four hours and continue the Advil. The next day Adrienne insisted on returning to the ER.

Four hours later, to our horror, Adrienne was diagnosed with acute lymphocytic leukemia. The following day she began grueling and aggressive chemotherapy. Adrienne endured two years and three months of chemotherapy, numerous blood transfusions, and painful side effects and complications.

Adrienne is one of the "lucky" ones; she survived this insidious disease. That said, there are long-term side effects experienced by survivors of pediatric cancers. Many times we hear folks discuss which is worse: the disease or the cure. I watch my 24-year-old suffer from long-term side effects, having physically and emotionally faced a life-threatening disease as a teenager. If only it were me, I have frequently wished, and not my child.

I applaud the passing of The Creating Hope Act. How great it would be if children like Adrienne don't have to suffer adult hand-me-down drugs and their devastating side effects.

Please accept my heartfelt appreciation of your support.

Marcia Westcott

If only it were me, I have frequently wished, and not my child.



ZACHARY

We must harness our love and energy and make a commitment to cure childhood cancer.

I want to thank you for the role you played in passing The Creating Hope Act.

Before our son Zachary was born, we hoped simply, as most parents do, to have a healthy child.

Our dreams were shattered when the doctors found a plum-sized tumor in his tiny 4-year-old brain. Hope of better days ahead got us through Zach's brain surgeries and when Zach emerged from surgery unable to do anything other than sit in a dark room. Even the slightest noises made him cry. Our fears were great while we hoped for the return of our little boy's laugh and smile. Against all odds, Zach regained the ability to swallow and speak, to hold a pencil to smile. Our hopes were destroyed when Zach relapsed. Finally, his battle-weary body told us the fight was over.

I mourn all the things he'll never get to be or do. Zach dreamed of becoming a bird scientist, a baseball player, and a violinist. With his wit and sense of humor, he surely had a future in late-night comedy. With his attention to detail, he could have been an artist, an editor, or an engineer. With his sharp mind and compassionate heart, the options were endless.

Three years after Zach's passing, his sister Kendall is 7 and struggling to understand why we couldn't find a cure for Zach. She misses her best friend and partner in mischief in the kitchen and the garden. Alex, Zach's older brother, lost his best friend and perhaps the only person who will ever see him as flawless. Zach adored his brother in the way only a little brother can. My husband and I will spend each day of our next fifty years missing Zach.

If we had a penny for every loving wish and prayer said for a child with cancer, we could cure this disease. Instead, we must harness our love and energy and make a commitment to cure childhood cancer. Thank you for all you have done.

With much gratitude,

Anne White

SAM

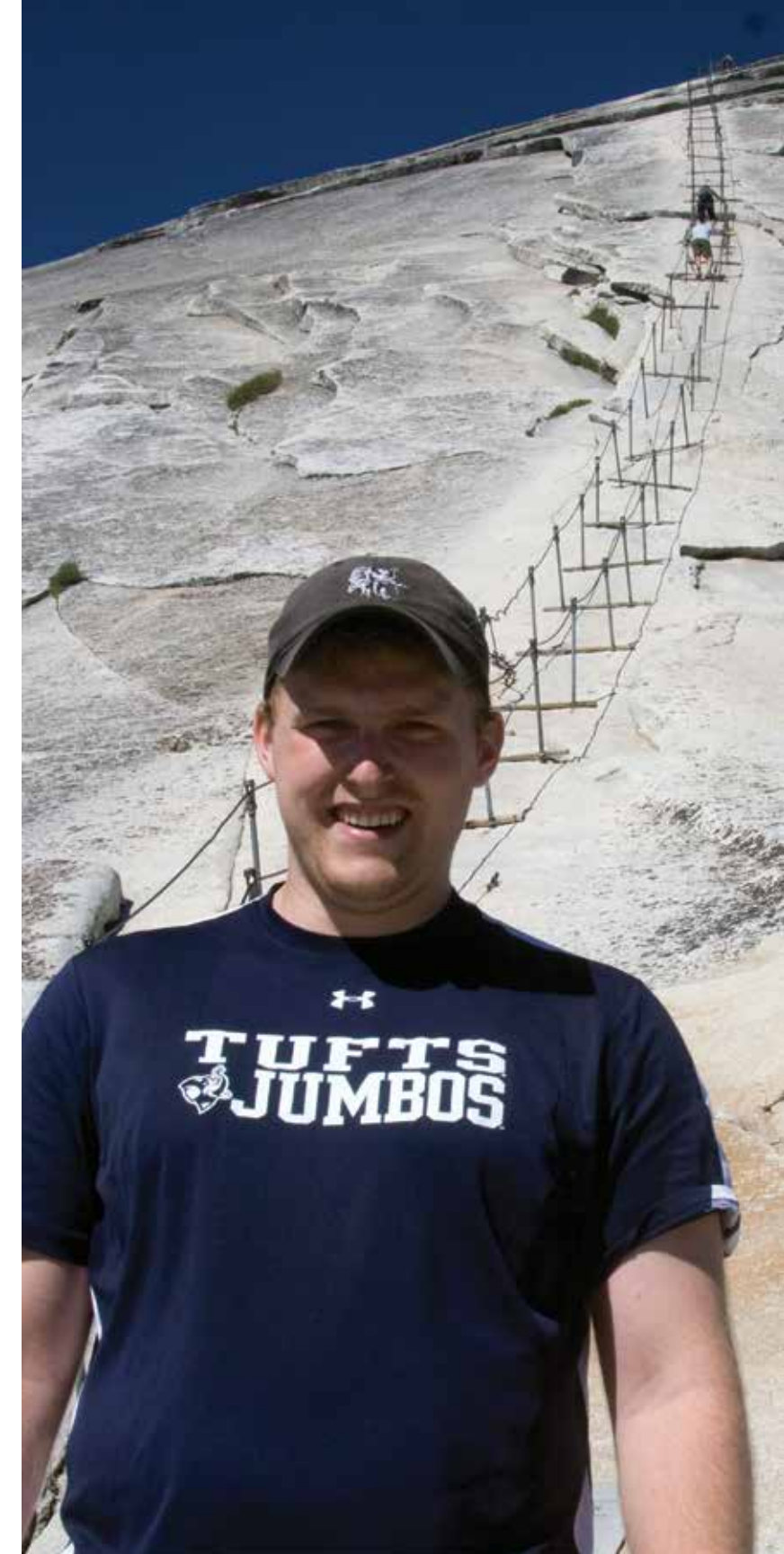
Thank you for passing The Creating Hope Act. At age 15, my son Sam was diagnosed with acute lymphoblastic leukemia.

Sam was able to graduate from high school and go on to major in both biochemistry and environmental studies at Tufts University. Sam is now five-plus years off treatment and has been in remission for over eight years. He is currently employed as a research assistant at Children's Hospital in Boston, where he is investigating new treatments to help repair kidney damage. When you save a young life, it opens up a world of possibilities for what that child may accomplish.

When you save a young life, it opens up a world of possibilities for what that child may accomplish.

Thank you for passing The Creating Hope Act. It will help to improve the cure rates for the many kids who face terrible diseases at a time in their lives when they should be having fun and learning in school.

Douglas Johnson
Father of Sam Johnson





CAMERON

I believe he will be the first to be cured of this type of cancer.

When he was an infant, he always had a smile. “Very healthy,” the doctor said. He had most beautiful red hair you would ever see — like a beautiful summer sunrise. His bright blue eyes are full of curiosity. He was smart, sweet, and wonderful.

Then one day I took him to the doctor for vomiting, which the doctor attributed to a sinus infection. A couple months later, he had a seizure. I took him to the hospital, and our world was flipped upside down.

He had fluid on the brain, so the doctors did emergency surgery to drain the fluid. When they put a shunt in, to divert the fluid to his stomach. Two weeks later, he had a mass removed from his spine and a tethered spinal cord fixed. A long week later, on March 14, 2010, he was diagnosed with anaplastic astrocytoma.

He is my hero — so strong and courageous. He has seen angels, but they have told him this isn’t his time. That gives me hope. I hope we find a cure for this awful beast we call cancer.

I believe miracles happen every day. I believe he will be the first to be cured of this type of cancer. You see, when a child is diagnosed with anaplastic astrocytoma, their chance of survival — well, there isn’t any. I pray that he is the one who overcomes this.

Love always,
his mom,
Tabitha Farmer

TEDDY

I’m including two pictures of my son Teddy. One was taken in August 2009, when we were on our annual family vacation to Block Island. Teddy adored Block Island and always looked forward to going there — eating seafood, hiking, riding the waves, and being with his family.

The other picture was taken in January 2010. Teddy was at Yale New Haven Hospital, undergoing the fourth of seven rounds of high-intensity chemotherapy that his doctors — and we — hoped would help cure him of the cancer that was discovered growing in his body in November 2009.

After feeling “off” for a few weeks, Teddy was diagnosed with an incredibly rare type of cancer called undifferentiated sarcoma.

Teddy underwent high-intensity chemo. Our vibrant, smart, loving little boy became sad and scared. He missed his school, his friends, and his brother and sister.

After two life-threatening operations, all visible tumor had been removed. We followed up with radiation therapy and more chemo, and then waited, praying that somehow any cancerous cells left would be destroyed.

We didn’t have to wait long. Less than a month after his chemo and radiation finished, a scan showed that Teddy had multiple new tumors.

We tried desperately to get access to other, low-toxicity chemo drugs, and met with the red tape and great expenses.

We ran out of time, and on August 16, 2010, Teddy fell asleep and did not wake up.

Losing a child is worse than I ever imagined it could be. Almost two years out I still feel such deep pain and sadness that it takes my breath away.

It would be easy to dig a hole, climb into it, and never come out again. But I won’t. What I will do is try my hardest to ensure that other families are spared the agony of watching a child become sick and die, with no hope.

Thank you for your support of The Creating Hope Act, from one family that knows too well what it feels like to have no hope.

Sincerely,

Jessica Gerber



Thank you for your support of The Creating Hope Act, from one family that knows too well what it feels like to have no hope.



My son David was almost 9 when he was diagnosed with a glioblastoma multiforme, the same kind of tumor Ted Kennedy had.

David had surgery the following day. We were devastated. I learned that knowing what the future holds can be a very heavy burden.

David was able to attend third grade for half-days, with me in attendance as his aide. His classmates were overjoyed to see him. A wheelchair was on hand. David had a leg brace and a walking stick. His hair was nearly gone. His speech was slow and deliberate, though his sense of humor was intact. David had to teach himself how to write with his left hand. He needed help showering, getting dressed, tying his shoelaces, and opening a sandwich bag. He would often sigh and say, "Mom, did you ever think that you'd have to help me with this stuff at my age?"

Half way through fourth grade, an MRI showed that there was a growth. Surgery wasn't an option. David just returned to school.

DAVID

On June 12, 2012, David passed away, leaving a gaping hole in our world.

My son's classmates will soon be having fifth-grade pictures taken. Their progression into adulthood will be charted photographically. But David will forever be frozen in fourth grade, wearing a blue-striped shirt and eyeglasses, in need of orthodontics; he'll never be older than he is in that photograph. He will never shave, drive, vote, date, or graduate from high school.

David will forever be frozen in fourth grade...

The drugs available to David were helpful only in delaying the inevitable. I truly hope that other children will have a better chance, thanks to The Creating Hope Act.

Sincerely,

Kathleen M. English

FAITH

In Faith's last few weeks with us, life for her was becoming a struggle. However, she fought to the very end. We know that God was holding her in the palm of His hands.

We witnessed her spirit rise the moment we removed the oxygen. She closed her eyes and went into a deep, heavy-breathing sleep. All that remained on the hospital bed was her shell. In short, absent a miracle, Faith had no chance. God wanted her.

What are we going to do now? Faith's fight is over. Ours has just begun. We'll be praying and seeking the Lord's guidance as we march forward. We'll be working with organizations to help raise awareness for The Creating Hope Act and help raise funding for research.

Without a doubt, we are a better family. We have blossomed from this journey. Many new friends and relationships have formed. Our purpose in life is now clear, and our need to be closer to God and our faith has deepened. May the good Lord continue to bless you and grant you His grace to accept the things you cannot change. Peace and our prayers are with all of you.

Sonia and Charles Griffin, Elijah Griffin



We know that God was holding her in the palm of His hands.



WILLIE

We appreciate this important tool to bring new cures to patients with these rare diseases.

My son, Willie Tichenor, died in 2006 of osteosarcoma at the age of 19. Willie was a showman and a prankster, a master of both hilarity and sincerity. He related to people of all ages, and he brought them all together. He made the ordinary extraordinary, the mundane amusing, and desperation hopeful. Willie led us through his illness. His hope became our hope. We saw through Willie's eyes and became hopeful ourselves.

Out of pain and loss, Willie's family and friends established the What Would Willie Want (QuadW) Foundation. QuadW redirects the lens of hope that had focused on curing Willie to finding better treatments and cures for sarcoma, as well as supporting his passions for transformative mission and higher education. Willie was unorthodox, and QuadW is taking an unorthodox approach to philanthropy. We are serious, critical, humorous and energetic, or as we have said in our mission statement, we employ "the light-hearted but whole-hearted" approach that Willie would have wanted.

QuadW applauds the passage of The Creating Hope Act. Because Willie, like so many others, ran out of treatment options, we appreciate this important tool to bring new cures more quickly to patients with these rare diseases.

Lisa Tichenor

HUNTER

When my son Hunter was 14, he was diagnosed with a type of brain tumor called a germ cell tumor.

Although Hunter would lose his hair because of treatment, he finally got the mohawk he always wanted. He maintained his sense of humor with the help of friends and family. He made it to his eighth-grade graduation and celebrated with his friends. Then it was on to radiation. Then it was off to high school. Hunter loved playing sports, but the treatment and steroids left him tired, weak, bloated, and no longer feeling like the athlete he had been. Despite all the adversity, Hunter remains strong, determined, and still Hunter.

Teenagers fighting or surviving cancer have different issues. They are no longer little kids, but they are not yet adults. They face unique challenges — physical, emotional, academic and social. How do they find their way back?

I want to thank you for hearing the voices of our children. Your actions will save lives.

Gratefully,

Mindy Brooks



Hunter... finally got the Mohawk he always wanted.



GETHIN

Thank you for your recent efforts and hard work on promoting and passing The Creating Hope Act.

Our son was diagnosed with anaplastic large cell non-Hodgkin's lymphoma a year ago. He was only 18 months old. We had been living in Houston, Texas, for less than five months, and we were blindsided.

Since then, we have been on a rollercoaster journey of discovery. To see your toddler, just learning to explore the world on his two feet, attached to an IV delivering toxic chemicals and an oxygen tank is enough to break anyone's heart.

Our son required many, many months of in-patient chemotherapy. Relatives flew in from England on a rotating basis to care for our other child. We did not leave our son's bedside, unless he was sleeping, and then for only a couple of minutes. He had a severe reaction to every round of his treatment protocol. He needed supplemental oxygen.

We are extremely thankful that our son is now in remission but the road has not been easy.

The bald heads that everyone sees are just a symbol of what is being destroyed on the inside — not just the unhealthy cells, but the healthy ones, too. We have to poison our children's bodies in order to save their lives.

This legislation represents the opening of a door that has been shut for far too long. We hope that this signals the emergence of better treatments for our children.

Thank you from the bottom of our hearts.

Rebecca Sinnatt and Gordon Rutter
Proud parents to a very brave little cancer warrior

To see your toddler, just learning to explore the world on his two feet, attached to an IV delivering toxic chemicals... is enough to break your heart.

My boys are my life, and I can't imagine life without them.

I want to thank you for passing The Creating Hope Act.

My 3-year-old son, Chase, was diagnosed at age 2 with medulloblastoma, a form of brain cancer. Before that day he was a happy and very energetic, normal 2-year-old.

Chase had had a few occasions of unexplained vomiting and dizzy spells. His doctor had ordered an MRI. At the time, I had thought it was overkill.

After the tumor was found, we were sent to the Riley Children's Hospital in Indianapolis, about a three-hour drive from home. It was the longest drive of my life.

Since then, Chase has endured two brain surgeries, one spinal surgery, six rounds of chemotherapy, a stem-cell transplant, and 30 rounds of proton-beam radiation. He now has no clear evidence of disease. I do not know what is in store for our future, but we can sleep easier knowing he will have access to the medicines he needs to fight this disease.

Chase is the younger of my two sons. My boys are my life, and I can't imagine life without them.

Kimberly Eaden

CHASE





LYDIA

No words can say how much this means to me and other families of kids fighting cancer.

Thank you from the bottom of my heart for passing The Creating Hope Act and giving children with cancer a chance to have less abrasive drugs.

My little heroine, Lydia Disbro, has been fighting Philadelphia chromosome–positive acute lymphoblastic leukemia for a year now. She has had a very rough road and has two more years of treatment ahead of her.

To know that there is a good chance that they will develop drugs for children that will have few side effects when they are older is such a gift. No words can say how much this means to me and other families of kids fighting cancer.

Once again, thank you!

Jodi Forcum

RYAN

My son, Ryan Christopher Kennedy, entered eternal life on May 26, 2012, two days after his tenth birthday and after five years and three months of living with ependymoma.

Who would have thought that the word breath could break my heart into pieces? I guess it makes sense, considering that I lay every day with my sweet little boy, listening to his every breath, for his every movement, wondering if this would be the last day I would be with him. Wondering how life would be after he was gone. Wondering, *Did I do the right thing? Make the right choices? How will I ever breathe myself after he is gone?*

I had no idea which breath would be the last. We sat there, my arms wrapped around him, stroking his face. Listening and counting. The gaps between breaths became longer — 30 seconds, 35 seconds, 45 seconds. When I told the nurse I thought he was gone, he gasped so deeply I chuckled. “You can still hear me,” I said to him. “Ryan,” I said, “it is time to trust Mommy and let go. It will be okay.” In that moment, he was gone.

Until that moment, I had taken breathing for granted. But sitting there, helpless, trying to find a way to comfort Ryan as he struggled for breath, my whole perspective was shattered. The very thing that sustains our life, breath, can be gone in the blink of an eye.

Thank you for creating hope that future young cancer patients might have a positive outcome.

Kimberly Kennedy



I had no idea which breath would be the last.



When I heard that The Creating Hope Act was passed and that drug companies would be offered incentives to make drugs especially for pediatric cancer patients, I was touched and overjoyed.

I am a cancer survivor and am happy to be alive to see this bill being passed. When I was 2-and-a-half years old, I was diagnosed with acute lymphoblastic leukemia. My memories of two and half years of treatment are quite limited, though my parents still remember vividly. After that, I started kindergarten, my chemotherapy treatments ended, and I was cancer free. I was experiencing a “normal life.”

When I was in first grade, the leukemia came back stronger than ever. I remember being bald and that horrible feeling in my legs when I would stand up and immediately lay back down because the pain was unbearable. But thankfully, I made it through another three years of chemotherapy medically unscathed. I am one of the lucky ones.

I am one of the lucky ones.

The Creating Hope Act can help ease the troubles of future children who will have to face this incredibly hard challenge. Thank you from the bottom of my heart.

Sincerely,

Claire Tedesco

CLAIRE

LUKE

My name is Luke Weber. I am almost 11 years old and have had rhabdomyosarcoma, a rare cancer, since I was 3. During the last eight years, I have been stuck with a needle in my chest or in my finger about a thousand times, and I am tired of it.

I thought my cancer was cured twice before, but it came back last year. Now I am doing well — I had a good summer and am back in school.

Unfortunately, my treatments have had many bad side effects, like height loss, hair loss, hearing loss, and loss of strength in my legs. All these affect what I like to do most: listen to interesting facts (it can be hard for me to hear) and play sports, especially basketball (my height and lack of strength make me slower than other kids my age). I think it is cool that my brother can do fun things with his hair, but my permanent hair loss prevents me from doing that.

I still get to do lots of fun things. I just wish the medicines that I took didn't have to have so many side effects. And even though the side effects have already happened to me, I hope that the next kids who get cancer don't have to go through the same things I went through.

Thank you. Because of you, kids like me will hopefully get good medicines that both kill all the cancer cells in our bodies and leave all the other cells healthy.

With warm regards and admiration,

Luke Weber



Because of you, kids like me will hopefully get good medicines that both kill all the cancer cells in our bodies and leave all the other cells healthy.



DIANA

We need more research for cancers affecting children.

My daughter, Diana, has been in a three-year battle with thyroid cancer. She was 7 when we found out she had cancer, and now she is 10. She was the first child under 10 to need three radioactive iodine (RAI) treatments.

Now Diana's cancer has grown resistant to RAI treatments. We have no real options, because there are so few kids who have battled with thyroid cancer, which usually affects adults, that little research has been done on the subject.

We need more research for cancers affecting children.

Karen Rodenbeck

JACOB

I want to express my personal thanks to you for your support of The Creating Hope Act.

As a bereaved parent, it is so important to know that other people care, and that they recognize that innovative and compassionate research is one way that future heartbreak can be avoided.

My son Jacob was 14 months old when he was diagnosed with a brain tumor. He was given a 40 to 60 percent chance of survival. Believe it or not, that sounded pretty good to us.

The treatment seemed to do its work, and he came through the transplant with flying colors. He got well quickly, and for six precious months we enjoyed our boy.

Unfortunately, two months after his second birthday, the tumor recurred. The doctors were stunned – which gave us some solace – and there was really nothing they could do, except palliative measures. Jacob was no longer in a cure category.

About three months later, at the age of 2-and-a-half, he was dead.

I have included a picture of the mosaic we had made in Jacob's memory. The center panel of our

sleeping boy is based on a drawing by his grandmother. The next ring depicts many of the things he loved – his juice cup, books, apples, trucks, balls, homemade waffles, and, especially, music.

The sun with the white bird in front of it represents him as the “son” and his sunny personality, while referring to his last word, “bird,” which he said as he pointed to the ceiling of his hospital room. The outer ring holds the hippo, rhino, and elephant characters I created to entertain him during long hospital stays, along with the trees I liked to draw with and a rising and setting sun.

We wanted this work of art to reflect that even though he was very little when he died, Jacob was a whole person. We'll never know everything that person would have done in the world. He died in 1999, and we are still learning to live without him.

Thanks for taking the time to read this, and for your support of this serious issue.

Best,

Jeremy Shatan



We wanted this work of art to reflect that even though he was very little when he died, Jacob was a whole person.



KATIE

Thank you for supporting The Creating Hope Act.

When my beautiful daughter was just 15 months old, she was diagnosed with a brain tumor. We were whisked after midnight in an ambulance from the local hospital to the regional children's hospital, where she underwent emergency brain surgery and another brain surgery three days later.

Then at age 3, she received radiation to her whole brain and spine. The radiation and chemotherapy worked, but she lost some of her intelligence in the process.

Life moved along until she was 14, when she developed two new cancers. Her right ovary and fallopian tube were removed. The neuro-oncologist said not to worry about the second tumor, as the tumor could not be removed. But when she was 22, a routine scan indicated the second tumor has grown.

We are now in fact-finding mode and trying to determine whether we do another craniotomy, stereotactic radiosurgery, or both. She now understands what a craniotomy is, and the fact that stereotactic radiosurgery involves "drilling four holes in your skull and attaching a halo while you are awake, and then screwing your head to a table for a 40-minute radiation procedure."

So that is where we are now — decisions, decisions — operating in that surreal state of, "I can't believe this is happening to us again."

Sincerely,

Connie Norman
Sent from the Vortex of Cancer Hell

So that is where we are now—decisions, decisions—operating in that surreal state of, "I can't believe this is happening to us again."

Thank you very much for passing The Creating Hope Act. This was very important to our family, because at age 15, my beautiful teenage daughter, Maeve, was diagnosed with osteosarcoma, bone cancer.

As with most pediatric cancers, there were no risk factors that explained why she had this very aggressive cancer. And when I found out that she would be treated with decades-old drugs that worked for some kids but not for others, I was terrified.

When I found out that she would be treated with decades-old drugs that worked for some kids but not for others, I was terrified.

Maeve went through 18 rounds of in-hospital chemo, three minor surgeries, and one major surgery. She lost her long, blond hair and most of her sophomore year of high school but gained a large scar and awful experiences no child or adult should ever have to face.

Today, she is college bound and healthy (knock wood), but we live with the great uncertainty of long-term side effects of the old drugs with which she was treated.

Thanks for giving frightened families of children with cancer hope that their children may be treated with more effective and less toxic drugs. This is such a great act!

Amy Williamson

MAEVE





HANNAH

I am writing to thank you for your efforts in passing The Creating Hope Act.

My daughter Hannah was diagnosed in 2007, at 7 years of age, with a malignant brain tumor. Hannah had several surgeries, full head and spinal radiation, chemotherapy, and countless other procedures.

Now 13, Hannah is cancer free and a cancer survivor.

While the treatments used to rid my daughter of the cancer were effective, they also caused irreversible side effects. Hannah struggles physically, academically, and emotionally. Hannah is nauseated every morning and takes medicine for nausea and seizures.

She attends school in a special-education class and has many accommodations to allow her to keep up. She learns new information at a rate of only 30 percent of that of her peers. She is determined to keep the pace and workload of her friends, but it is a daily struggle for her.

Hannah's dream is to own her own bakery. However, she can't make change, she can't tell time, she can't recite her multiplication tables, and she can't run. Hannah can't organize her thoughts, can't write a complete sentence, and can't complete her homework on her own. It's uncertain she'll ever have a job.

And due to her treatments, we also wonder if Hannah can ever have children of her own.

No child should have to experience the pain and debilitating effects of cancer treatments. While pediatric cancer statistics show that survival rate is on the increase, those statistics mean nothing if the life after cancer isn't a quality one.

My sincerest thanks,

Kimberly Prokop
*Mother to cancer survivor
Hannah Prokop*

Survival statistics mean nothing if the life after cancer isn't a quality one.



CARSON

Carson had moxie.
A bold charming confidence
An indomitable spirit
A disarming smile that would warm a room

It was a gift to teach him about life

Then he fought, 1170 days. And he taught us
To live without guile
That courage comes from God
To embrace moments of goodness in the midst of pain

Then he whispered
Don't let them bury me
Make sure they study those tumors
If those tumors can help the next kid not die like I am, I'd like that
It's hard to have cancer.

Then he died. Young and fearless.

Annette Leslie
Carson's mother

He taught us to live without guile, that courage comes from God.



SKY

The day that forever changed my perspective on life was July 23, 2005.

“Please call the ER at Children’s Medical Center” is the message that I will forever recall. My 5-year-old daughter, Sky, had been having headaches and had had an MRI earlier that day. I called the hospital right away.

The voice on the other end said, “We found a large mass on your daughter’s brainstem, and you need to bring her to the ER immediately.”

Sobbing uncontrollably, I crawled to Sky’s room and gently lifted her from her bed. Holding her in my arms, I began to pray. I told Jesus that I knew His love for her was even greater than the love that I had for her. I asked for His mercy for Sky.

Sky was admitted to the hospital for surgery. For a month, she lived in the ICU. She received a shunt. She received proton-beam radiation to her brain.

Two years passed. Our lives began to resume some kind of normalcy.

Then, a new tumor was found on Sky’s spine. The fear of death and despair flooded my mind again. Sky was given an even grimmer prognosis. I cried when she lost her hair and tried to save every strand that I combed away.

Today, Sky is a remarkable 12-year-old with an angelic presence. She has never asked, “Why me?” Sky now speaks publicly and writes about brain tumors for the school newsletter. She has taught our family about faith and perseverance.

Sky continues to inspire others to trust in God and to lean not to their own understanding or will. I find myself so often gazing at her in awe. I feel so blessed to have been chosen to be her mother.

Sonya Strong

I asked for His mercy for Sky.

THE CREATING HOPE ACT: PRIORITY REVIEW TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES.

SECTION 908 OF THE FDA SAFETY AND INNOVATION ACT OF 2012, 21 U.S.C. SEC. 360FF.

BACKGROUND

Very few drugs are being developed for children with cancer and other rare diseases. In the past 20 years, there have been only two initial FDA approvals for drugs to treat any pediatric cancer – Erwinase and Clofarabine.

By enacting the Creating Hope Act – the Rare Pediatric Disease Priority Review Voucher Program, Section 908 of the FDA Safety and Innovation Act – Congress created a market incentive for the development of drugs for rare pediatric diseases through the establishment of a priority review voucher. Under this program, a company or institution that develops a drug or biologic for a rare pediatric disease and receives FDA approval for that drug or biologic also receives a voucher. That voucher comes with rights to FDA priority review for any *other* drug or biologic, including a large market adult drug or biologic that would otherwise receive standard review. This would result in the other drug or biologic getting to market many months earlier, creating significant value. The voucher is fully transferable.

The Creating Hope Act voucher is based upon a tropical disease priority review voucher program that was signed into law as a provision of the Food and Drug Administration Amendments Act of 2007. The Creating Hope Act

improves upon that program by providing an enhanced voucher value through unlimited transferability of the voucher, as opposed to one time transferability; and through a shorter notification period to the FDA before using a voucher, from one year in the tropical disease voucher program to 90 days in the Creating Hope Act. In addition, the Creating Hope Act provides greater certainty to drug developers earlier in the drug development process by providing for a designation process, which the neglected tropical disease voucher program does not offer.

HOW TO RECEIVE A DESIGNATION AND A PRIORITY REVIEW VOUCHER

The Creating Hope Act provides for a designation process to create greater certainty earlier in the drug development process that a drug or biologic would qualify for a voucher, should it be approved.

The Office of Orphan Products Development within the Office of the Commissioner, which manages orphan designations, may be contacted to receive an authorization for a designation of a Rare Pediatric Disease Product Application and a Rare Pediatric Disease. The request for a Creating Hope Act designation could be made as early as the same time a request for a designation of orphan drug status or fast track status is made. The application for a Creating Hope Act designation is likely to require similar sources of data as an orphan designation. The language of the Creating Hope Act provides that once a designation application is filed, the FDA would have 60 days to make a determination as to whether to grant the designation.

Pursuant to the language of the Creating Hope Act, a sponsor of a drug or biologic may request that the disease for which it is developing the drug or biologic be designated as a “Rare Pediatric Disease.” In addition, the sponsor may request that the drug or biologic be designated as a “Rare Pediatric Disease Product Application.” These designations would lead to a voucher if the drug or biologic is approved.

The criteria for a Rare Pediatric Disease are that the disease primarily affects individuals aged from birth to 18 years; and that the disease is rare within the meaning of the Orphan Drug Act. An orphan designation would satisfy the second criteria.

The criteria for a Rare Pediatric Disease Product Application are that a drug or biological product:

- Is for the prevention or treatment of a Rare Pediatric Disease;
- Contains no active ingredient that has been previously approved in any other application;
- Is submitted under section 505(b)(1) of this Act or section 351(a) of the Public Health Service Act;
- Itself qualifies for priority review;
- Relies on clinical data derived from studies examining a pediatric population and dosages of the drug intended for a pediatric population; and
- Does not seek approval for an adult indication before or at the same time as the pediatric indication.

HOW TO TRANSFER A PRIORITY REVIEW VOUCHER

Unlike the tropical disease voucher which is only transferable once, the Creating Hope Act priority review voucher has unlimited transferability and may be exercised for other drugs or biologics that the sponsor may be developing, or for drugs and biologics developed by another sponsor.

HOW TO EXERCISE A PRIORITY REVIEW VOUCHER

A sponsor that wishes to exercise a priority review voucher would likely have a large market adult drug or biologic that would otherwise be reviewed by the FDA pursuant to a standard review. By exercising a priority review voucher, the sponsor would then be able to receive a priority review for that drug or biologic. This would get the drug or biologic to market more quickly, thereby generating significant value.

The priority review voucher program does not alter the criteria for a FDA approval. Drugs subject to priority review must still conform to all the FDA requirements to be approved.

A sponsor of a drug or biologic application that is the subject of a priority review voucher shall pay a user fee. The amount of the user fee shall be based on the difference between the average cost incurred by the FDA of a priority review and the average cost incurred by the FDA of a standard review. This amount shall be adjusted annually to reflect current costs.

The Creating Hope Act provides that the sponsor must notify FDA of its intent to submit a drug application with a priority review voucher at least 90 days prior to submission. The user fee shall be due upon the notification.

Pursuant to the Creating Hope Act, the FDA may revoke a priority review voucher awarded if the Rare Pediatric Disease Product Application for which such voucher was awarded is not marketed in the United States within a year.

FOR MORE INFORMATION

Further questions may be directed to Nancy Goodman at nancygoodman@kidsvcancer.org, 646-361-3590. ■

FOOD AND DRUG ADMINISTRATION SAFETY AND INNOVATION ACT § 908

(CODIFIED AT 21 U.S.C. § 360FF)

§ 360FF. PRIORITY REVIEW TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES

(a) Definitions. In this section:

(1) PRIORITY REVIEW. The term “priority review”, with respect to a human drug application as defined in section 379g(1) of this title, means review and action by the Secretary on such application not later than 6 months after receipt by the Secretary of such application, as described in the Manual of Policies and Procedures of the Food and Drug Administration and goals identified in the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2012.

(2) PRIORITY REVIEW VOUCHER.

The term “priority review voucher” means a voucher issued by the Secretary to the sponsor of a rare pediatric disease product application that entitles the holder of such voucher to priority review of a single human drug application submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] after the date of approval of the rare pediatric disease product application.

(3) RARE PEDIATRIC DISEASE.

The term “rare pediatric disease” means a disease that meets each of the following criteria:

(A) The disease primarily affects individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents.

(B) The disease is a rare disease or condition, within the meaning of section 360bb of this title.

(4) RARE PEDIATRIC DISEASE PRODUCT APPLICATION.

The term “rare pediatric disease product application” means a human drug application, as defined in section 379g(1) of this title, that—

(A) is for a drug or biological product—

(I) that is for the prevention or treatment of a rare pediatric disease; and

(II) that contains no active ingredient (including any ester or salt of the active ingredient) that has been previously approved in any other application under section 355(b)(1), 355(b)(2), or 355(j) of this title or section 351(a) or 351(k) of the Public Health Service Act [42 U.S.C. 262(a), 262(k)];

(B) is submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)];

(C) the Secretary deems eligible for priority review;

(D) that relies on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population;

(E) that does not seek approval for an adult indication in the original rare pediatric disease product application; and

(F) is approved after July 9, 2012.

(b) Priority Review Voucher

(1) IN GENERAL. The Secretary shall award a priority review voucher to the sponsor of a rare pediatric disease product application upon approval by the Secretary of such rare pediatric disease product application.

(2) TRANSFERABILITY

(A) IN GENERAL. The sponsor of a rare pediatric disease product application that receives a priority review voucher under this section may transfer (including by sale) the entitlement to such voucher. There is no limit on the number of times a priority review voucher may be transferred before such voucher is used.

(B) NOTIFICATION OF TRANSFER.

Each person to whom a voucher is transferred shall notify the Secretary of such change in ownership of the voucher not later than 30 days after such transfer.

(3) LIMITATION. A sponsor of a rare pediatric disease product application may not receive a priority review voucher under this section if the rare pediatric disease product application was submitted to the Secretary prior to the date that is 90 days after July 9, 2012.

(4) NOTIFICATION

(A) IN GENERAL. The sponsor of a human drug application shall notify the Secretary not later than 90 days prior to submission of the human drug application that is the subject of a priority review voucher of an intent to submit the human drug application, including the date on which the sponsor intends to submit the application. Such notification shall be a legally binding commitment to pay for the user fee to be assessed in accordance with this section.

(B) TRANSFER AFTER NOTICE. The sponsor of a human drug application that provides notification of the intent of such sponsor to use the voucher for the human drug application under subparagraph (A) may transfer the voucher after such notification is provided, if such sponsor has not yet submitted the human drug application described in the notification.

(5) TERMINATION OF AUTHORITY.

The Secretary may not award any priority review vouchers under paragraph (1) after the last day of the 1-year period that begins on the date that the Secretary awards the third rare pediatric disease priority voucher under this section.

(c) Priority Review User Fee

(1) IN GENERAL. The Secretary shall establish a user fee program under which a sponsor of a human drug application that is the subject of a priority review voucher shall pay to the Secretary

a fee determined under paragraph (2). Such fee shall be in addition to any fee required to be submitted by the sponsor under subchapter VII.

(2) FEE AMOUNT. The amount of the priority review user fee shall be determined each fiscal year by the Secretary, based on the difference between—

(A) the average cost incurred by the Food and Drug Administration in the review of a human drug application subject to priority review in the previous fiscal year; and

(B) the average cost incurred by the Food and Drug Administration in the review of a human drug application that is not subject to priority review in the previous fiscal year.

(3) ANNUAL FEE SETTING. The Secretary shall establish, before the beginning of each fiscal year beginning after September 30, 2012, the amount of the priority review user fee for that fiscal year.

(4) PAYMENT

(A) IN GENERAL. The priority review user fee required by this subsection shall be due upon the notification by a sponsor of the intent of such sponsor to use the voucher, as specified in subsection (b)(4)(A). All other user fees associated with the human drug application shall be due as required by the Secretary or under applicable law.

(B) COMPLETE APPLICATION. An application described under subparagraph (A) for which the sponsor requests the use of a

priority review voucher shall be considered incomplete if the fee required by this subsection and all other applicable user fees are not paid in accordance with the Secretary’s procedures for paying such fees.

(C) NO WAIVERS, EXEMPTIONS, REDUCTIONS, OR REFUNDS. The Secretary may not grant a waiver, exemption, reduction, or refund of any fees due and payable under this section.

(5) OFFSETTING COLLECTIONS.

Fees collected pursuant to this subsection for any fiscal year— (A) shall be deposited and credited as offsetting collections to the account providing appropriations to the Food and Drug Administration; and

(B) shall not be collected for any fiscal year except to the extent provided in advance in appropriations Acts.

(d) Designation Process

(1) IN GENERAL. Upon the request of the manufacturer or the sponsor of a new drug, the Secretary may designate—

(A) the new drug as a drug for a rare pediatric disease; and

(B) the application for the new drug as a rare pediatric disease product application.

(2) REQUEST FOR DESIGNATION.

The request for a designation under paragraph (1) shall be made at the same time a request for designation of orphan disease status under section 360bb of

this title or fast-track designation under section 356 of this title is made. Requesting designation under this subsection is not a prerequisite to receiving a priority review voucher under this section.

(3) DETERMINATION BY SECRETARY.

Not later than 60 days after a request is submitted under paragraph (1), the Secretary shall determine whether—

(A) the disease or condition that is the subject of such request is a rare pediatric disease; and

(B) the application for the new drug is a rare pediatric disease product application.

(e) Marketing of Rare Pediatric Disease Products

(1) REVOCATION. The Secretary may revoke any priority review voucher awarded under subsection (b) if the rare pediatric disease product for which such voucher was awarded is not marketed in the United States within the 365-day period beginning on the date of the approval of such drug under section 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262].

(2) POSTAPPROVAL PRODUCTION REPORT.

The sponsor of an approved rare pediatric disease product shall submit a report to the Secretary not later than 5 years after the approval of the applicable rare pediatric disease product application. Such report shall provide the following information, with respect to each of

the first 4 years after approval of such product:

(A) The estimated population in the United States suffering from the rare pediatric disease.

(B) The estimated demand in the United States for such rare pediatric disease product.

(C) The actual amount of such rare pediatric disease product distributed in the United States.

(f) Notice and Report

(1) NOTICE OF ISSUANCE OF VOUCHER AND APPROVAL OF PRODUCTS UNDER VOUCHER. The Secretary shall publish a notice in the Federal Register and on the Internet Web site of the Food and Drug Administration not later than 30 days after the occurrence of each of the following:

(A) The Secretary issues a priority review voucher under this section.

(B) The Secretary approves a drug pursuant to an application submitted under section 355(b) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] for which the sponsor of the application used a priority review voucher under this section.

(2) NOTIFICATION. If, after the last day of the 1-year period that begins on the date that the Secretary awards the third rare pediatric disease priority voucher under this section, a sponsor of an application submitted under section 355(b) of this

title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] for a drug uses a priority review voucher under this section for such application, the Secretary shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a document—

(A) notifying such Committees of the use of such voucher; and

(B) identifying the drug for which such priority review voucher is used.

(g) Eligibility for Other Programs. Nothing in this section precludes a sponsor who seeks a priority review voucher under this section from participating in any other incentive program, including under this chapter.

(h) Relation to Other Provisions. The provisions of this section shall supplement, not supplant, any other provisions of this chapter or the Public Health Service Act [42 U.S.C. 201 et seq.] that encourage the development of drugs for tropical diseases and rare pediatric diseases.

(i) GAO Study and Report

(1) STUDY

(A) IN GENERAL. Beginning on the date that the Secretary awards the third rare pediatric disease priority voucher under this section, the Comptroller General of the United States shall conduct a study of the effective-

ness of awarding rare pediatric disease priority vouchers under this section in the development of human drug products that treat or prevent such diseases.

(B) CONTENTS OF STUDY. In conducting the study under subparagraph (A), the Comptroller General shall examine the following:

(I) The indications for which each rare disease product for which a priority review voucher was awarded was approved under section 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262].

(II) Whether, and to what extent, an unmet need related to the treatment or prevention of a rare pediatric disease was met through the approval of such a rare disease product.

(III) The value of the priority review voucher if transferred.

(IV) Identification of each drug for which a priority review voucher was used.

(V) The length of the period of time between the date on which a priority review voucher was awarded and the date on which it was used.

(2) REPORT. Not later than 1 year after the date under paragraph (1)(A), the Comptroller General shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate, a report containing the results of the study under paragraph (1). ■

The Creating Hope Act

Priority review to encourage treatments for rare pediatric diseases.

SECTION 908 OF THE FDA SAFETY AND INNOVATION ACT OF 2012, 21 U.S.C. Sec. 360ff.

KIDS  CANCER