



Our Mission . . .

To promote awareness of Sanfilippo Syndrome and raise funds to support research aimed at finding a cure.

Sanfilippo Syndrome is a rare and catastrophic genetic disorder . . .

Children afflicted are missing an essential enzyme that breaks down strings of a complex body sugar called heparan sulfate. This sugar or mucopolysaccharide, accumulates in the brain and the body's cells and tissue causing progressive damage.

A Sanfilippo child appears normal at birth and seems to develop normally for the first year or two, but as more and more cells become damaged symptoms begin to appear. Eventually, the build-up of mucopolysaccharides will cause hyperactivity, sleep disorders, loss of speech, mental retardation, dementia and finally death.

Life expectancy for a child with Sanfilippo Syndrome is between 10 to 15 years.

It is estimated that Sanfilippo occurs 1 in 24,000 births. Based on this estimate approximately 200 children will be born with this disorder in the United States this year. Given the average lifespan, there are thousands of families dealing with Sanfilippo in the USA alone.

There is currently no treatment or cure, only determination . . .

BUZZ FOR BEN

Chris Seebeck, a participant in our Birdies for Ben golf fundraiser, was looking for a creative way to raise money. His co-worker, Kris Belken, had been avoiding a barber for sometime and the "mop" he had developed became Chris's inspiration. They decided to offer up their heads—heads of hair that is. But they needed a "big draw", as Chris put it, "to help take their efforts to another level." He convinced Tom Bullen, their Chief Operating Officer to join them. The three challenged the employees and agents of Sun Life Financial Distributors, the company where they work, to meet a dollar pledge goal before a certain date. If each goal was met, they agreed to get a military buzz cut. The clipping would not take place at a barber shop but at the very public sales desk in their Boston, MA office. They set individual goals of \$2,500 for Tom Bullen, \$1,000 for Chris Seebeck and \$500 for Kris Belken.

Chris sent regular company-wide e-mail updates to keep his cohorts from chickening out and to help generate more pledges. Kris did his part by staying far away from sharp objects—extending his mop to historic lengths. In fact, one of the first donations they received was from Kris's mother, who just wanted him to get a haircut. "Shaving our heads was just a gimmick to start the



Kris Belken's famous "mop" pre-buzz

conversation," according to Chris. "Once people heard about Ben and saw your website, it was an easy sell because they felt a personal connection."

On August 16, Tom flew in from his office in New Jersey and the entire sales floor turned out to witness the clippings done by stylist Janine Rossetti, who donated her time. Roars of laughter and shouts of encouragement could be heard as each of the men took turns in the chair. They played to the crowd by having Janine give each a Mohawk before shaving them down to a full military cut. Camera's flashed and the floor was covered in hair. The three had met and exceeded their goals, raising \$5,250 combined. Sun Life added a \$250 match for each of the three bringing the total to an even \$6,000!

Stuart thanked them for putting their heads on the line and offered a single Ben's Dream baseball cap to the one who needed it the most. The crowd voted and it was fitting that Chris, the person who started it all, was the overwhelming choice.

We can only imagine what he will do this year.



Chris Seebeck gets buzzed by Janine

RESEARCH BREAKTHROUGH

Dr. Haiyan Fu,
Columbus Children's
Research Institute,
Ohio State University

By conducting gene therapy on MPS IIIB mice, we have made an adeno-associated viral (AAV) vector as a vehicle to carry genes into cells. This AAV vector contains the normal human NaGlu gene, which is defective in MPS IIIB patients. The vector can produce NaGlu (the enzyme missing in MPS IIIB patients) and leads to the breakdown of the accumulated GAG in cultured MPS IIIB cells and the MPS IIIB mouse brain.

The most critical challenge in developing treatment for central nervous system (CNS) diseases is how to deliver therapeutic reagents (AAV vector) to the whole CNS or broad areas of the brain, and not to just a localized area. The major obstacle to this is the blood-brain barrier (BBB), which prevents large molecules, such as AAV vector and NaGlu enzyme, from entering the CNS tissues. The BBB is closed at birth in humans, but not closed until 21 days of age in mice. Therefore, we tested adult mice making it applicable to even the youngest of human patients, whose BBB is already well formed. Focusing on two major aspects of gene therapy, we have been developing methods to achieve widespread distribution of the AAV gene delivery vectors in the CNS tissues of adult mice and assessing the therapeutic impacts of AAV vector carrying the NaGlu gene on CNS disorders in the MPS IIIB mouse model.

In previous studies, we developed two non-surgical approaches to deliver AAV vectors into the CNS of adult mice; an intravenous (IV) injection procedure that achieved a global distribution of AAV vector throughout mouse CNS, by pretreatment using an IV infusion of Mannitol to temporarily disrupt the blood-brain barrier (BBB) and a broad spread of AAV vector in the CNS by an intracisternal (IC) injection, delivering AAV vector into the cerebral spinal fluid space. These results offered us more effective means to deliver AAV vectors into the CNS for MPS IIIB therapy.

We used the above procedures in AAV gene therapy studies in adult MPS IIIB mice. The mice were treated with AAV2-hNaGlu vector by an IV injection, an IC injection, or a combination of IV and IC injection. The AAV vector resulted in the production of NaGlu enzyme in the brains of all the IC and IV+IC-injected MPS IIIB mice. The NaGlu enzyme subsequently decreased pathological lysosomal storage in these treated MPS IIIB mouse brains. The IV vector injection led to the clearance of lysosomal storage in liver, and partial correction of storage in other somatic tissues. Most importantly, these treatments significantly prolonged the lifespan of MPS IIIB mice to 9.2-15.9 months (IV injected), 10.3-21.5 months (IC injected), and 11.1-19.5 months (IV+IC injected), while non-treated MPS IIIB mice only lived 7.9-11.0 months. Normal mice live approximately 2.5 years. IC and IV+IC injection of AAV vector also significantly improved the behavioral performance, especially learning ability, of MPS IIIB mice. These results suggest that the AAV gene therapy procedures in this

study greatly slowed down the progress of the CNS disease in MPS IIIB mice, though they did not effect a complete cure. The data gained in these studies showed the great potential for using AAV gene therapy to treat MPS IIIB.

In designing our experiments, we considered factors for future human application. Both IV and IC injection are non-surgical and routine medical procedures used in humans. The IV infusion of Mannitol is also a routine medical practice. We calculated the amount of Mannitol and the volumes of injections based on dosages commonly used in patients. The therapeutic effect of these treatments is long-term, we may not need to repeatedly conduct the treatment for MPS IIIB patients. Currently, the IC injection and the combined IV + IC injection of AAV vector are more efficient than IV only for treating MPS IIIB. We are continuing our efforts to improve the efficacy of AAV gene therapy for the CNS disorders of MPS IIIB, by optimizing the vector injection approaches as well as testing them in larger animals, and enhancing the enzymatic functions of AAV-expressed NaGlu to maximize its therapeutic effect.

We feel that we have a therapy in hand that provides meaningful benefits in our MPS IIIB animal model and that the time has come to consider translating our AAV gene therapy procedure from mouse studies into human clinical applications. We are planning to apply for an IND approval from the FDA for a clinical trial in MPS IIIB patients, using the AAV gene therapy vectors and procedures.

FIRST STEPS TO CLINICAL TRIAL

Dr. Fu's words "We are planning to apply for an IND approval from the FDA for a clinical trial" represents the brass-ring for which all researchers strive.

The Investigation New Drug (IND) application process is necessary to gain FDA approval. The IND application must contain pre-clinical data on animal pharmacology and toxicology studies (Dr. Fu's research to-date); clinical protocols which detail the proposed clinical studies that will determine safety and effectiveness testing; and manufacturing information about the composition, stability and controls necessary to produce the drug product. The data gathered during the animal studies and human clinical trials of an IND become part of a New Drug Application (NDA). The NDA application is the vehicle through which sponsors propose that the FDA approve a new drug or treatment for use in the US.

The methods Dr. Fu is using are directly applicable to patients and

appears both safe and effective. The AAV virus being used to deliver the corrected MPS IIIB gene causes no human disease and has been used in over 20 clinical trials without any adverse effects.

It is likely that the lengthy clinical trials ahead will not produce a cure in time for Benjamin. However, that Dr. Fu's research has progressed to this point is a testament to Ben's Dream premise that one determined person can make a difference.

"Dr. Fu's studies have uniquely positioned us to move forward on this for patients with this disease. We have considerable experience with human gene therapy trials and a very good working relationship with the FDA and all of the regulatory agencies that govern gene therapy trials. I am confident that we can bring these studies to the clinic for patients with MPS IIIB in the future."

*Dr. Jerry Mendell, MD, Director, Center for Gene Therapy
Columbus Children's Research Institute*

THE FOUNDATION GIVES THANKS

To the students of Wellesley Middle School for collecting donations at their Famous Romans Wax Museum presentation. They raised over \$400.

To Michael Walsh and Diane Lambert of Walsh & Associates, PC of Concord, MA, for donating their time and energy to help with Foundation tax filings.

To Lewis Hibbs of Baltimore, MD, for collecting donations in lieu of gifts for his 10th birthday.

To Kevin DuBois of Paragon Marketing Group, LLC of Westborough, MA, for putting Ben's Dream on the world-wide-web by donating hosting services for www.bensdream.org.

To Tom Collins, Ben's teacher, who has enriched Ben's life at Perkins each day with unparalleled energy, excellence and love. We wish him luck with his new job. Ben will check up on you!!



Tom Collins and Ben go head-to-head

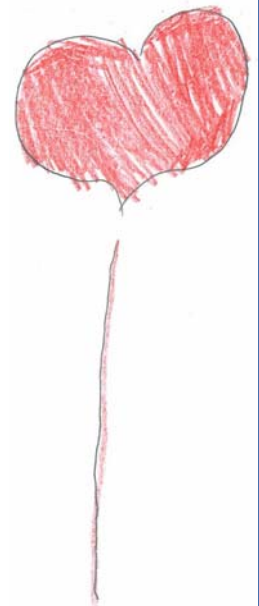
ISABELLE SPILLS HER HEART

My name is Isabelle. Life in my world can be hard. My brother Ben has a sickness called Sanfilippo. He has been crying for at least a year. It is hard to do things when Ben is in a bad mood. Sleeping can also be a problem. Ben is not always a good sleeper. But he is a good brother. I have gotten used to it. It is turning out to be not as much of a problem.

My brother Noah and I like to play ball with Ben. We have a dog too. She is sometimes annoying, but she does help Ben. Ben walks her and my dog lets him hold the leash. When Ben is happy my feelings are that I am happy too. When he is crying I feel sad and sometimes mad. My Mom and Dad are also sad sometimes, but we all feel sorry for him.

In Ben's future when he dies I'll feel sad, but I will know that he will feel better than he did and that is because he won't be crying any more.

Sometimes I wonder what Ben would be like if he didn't have Sanfilippo. It's hard to know what his voice would be like. I wonder what he would look like. I wonder a whole lot of different things, but I know he would always be a good brother.



A Parents Note:

Isabelle is 7-years old and unlike her brother Noah, she has only known Ben while the effects of Sanfilippo have been apparent. She has always been more matter-of-fact about his disorder. But when she wrote this, what immediately stood out was that every single letter "i" was dotted with a heart—while she is our creative child, we've never seen her write like this before . . .

A LOOK INSIDE . . .

I was re-reading a letter my mother sent out last year. In it she wrote, *“Sometimes I am like Scarlett O’Hara with, ‘I will think about it tomorrow.’ It is once again time for Birdies for Ben and I can’t think about it tomorrow. Our Ben is reaching stages in his disorder that we wish we could think about tomorrow but we can’t. It is upon us. We all dreamed the disorder would not develop as we were told it would, but the reality is that it has.”*

This year I seemed to have caught a bit of Scarlett as well. The challenges of Benjamin’s condition grow and change each day and I am left wishing that I could think about them and the things that need to be done tomorrow. But I can’t.

Once again this winter, Ben’s disorder has left him crying by day and sleeping often only 3 hours at night. Some days Ben offers me words but most he is mute and I know his words are likely lost for good. Some days he is able to move across a room freely but more and more frequently his instability forces us to seek the safety of his stroller. And even though I am tired of walking around the block with him, I know the days when he can no longer do so are coming quick. Some days Ben munches on his food fine but most I am mincing up everything and I know decisions like feeding tubes are looming. Some days I wake up feeling driven to tackle the new medical and behavioral problems that are becoming so frequent and not let this disorder win. Most days though, I wake up wishing I could “think about it tomorrow.” But I can’t.

For everyday, I wake up knowing there are so few tomorrows. On those days it is often easier to think of the yesterdays. I would like to share with you some words Ben shared with me in 2003.

From Jennifer’s Journal:

Tonight while trying to cook dinner inside on the stove and outside on the grill, do homework with Noah and manage one of Isabelle’s art projects, Ben was demanding something. With no time to interpret what he needed, I said to him, “Ben I can’t do this now I just need to...” and he interrupted, “What you need, Mommy?” To which I responded, “Sanity, Ben, Sanity. Do you know where we can get some?” And in his sweet little voice he matter-of-factly answered, “The Beer Store.”



Ben in 2002

I hope that his humor and the record of his words bring you the wealth of happiness that remembering them brings to me.

FUNDRAISING NEWS

Pages of Memories



Lisa Wentworth at the scrapbook event

Creative Memories consultants Lisa Cross and Lisa Wentworth once again hosted an unforgettable event benefiting Ben's Dream. The event, now in its 4th year, was held on Nov 19, 2005, at the Italian American Club in Wellesley, MA. It was attended by over 40 dedicated "scrappers" and has raised more than \$25,000 since its inception.

"It is so inspiring to see the same faces year over year. Their dedication helps to strengthen my resolve to raise research dollars for Ben's Dream," remarked Lisa Wentworth.

The day included classes for beginners, a

raffle for pages completed, lunch donated by Panera Bread and dinner by the Cresap family. "2005 was a hard year for the Siedman's with Ben's regression, but today it all came together. We all remembered how important it is to help, and how scrapbooking is really about preserving your family story" added Lisa Cross.

The event continues to be a huge emotional gift to Jennifer. "So many of these women come to this event every year and help me put together pages of Ben's story. In a way it is like they are on this journey with me."

Mixing it up for Ben

Tresca Brothers Sand & Gravel Inc. of Millis, MA is "spinning" with Ben and his dream. The concrete company owned by brothers Bob, John and Steven Tresca is painting one of their mixer's rotating drum with the Ben's

Dream logo and our web address.

The truck, a kind of rotating billboard, is scheduled to be finished by next month and will make an appearance at Birdies for Ben. "Back in February, Bob contacted me about putting the logo on his truck. I met him at an event Grettacole hosted 5-years ago and he remembered our cause. I am so honored," said Jennifer.

Tresca Bros. provides the service free of charge to charities big and small. The logos include organizations like the American Heart Association and lesser-known organizations like Ben's Dream. The drums stay in circulation for approximately 2-years and are Tresca Bros. way of giving back to the community. In an e-mail to Jennifer, Bob wrote, "We're happy to do even this little bit. Hopefully, many people will see the truck and help raise awareness about Sanfilippo."



Mock-up of truck that will hit the streets in June

HOW YOU CAN HELP

A Match for Ben

Investigate your company's matching gift program and double your support for the Foundation. Find out if your company has an Employee Charitable Fund and request a donation be made on behalf of the Foundation.

Give Ben Security

The Foundation can accept donations of appreciated securities through our brokerage account. This may allow you to realize a charitable deduction for the full market value of your securities.

Contact Robert Wofchuck at Ledgewood Capital Management, 603-773-0080.

Buy a Band

Ben's brother Noah has created unique *Dream for Ben* wristbands to raise to raise funds. Each band is only \$3 and all proceeds benefit Ben's Dream. Please specify small, medium or large.

Lend a Hand

Ben's Dream is always in need of professional and technical services. Donations of services saves us from having to purchase them and leaves more dollars for research.

Birthdays for Ben

Do you have a special day coming up? Looking for a unique gift? Make a donation in lieu of a gift and Ben will send you a special birthday thanks.

Host an Event for Ben

Host a local fundraising event to benefit the Foundation or volunteer to help support one of our own events.

We must remember that one determined person can make a significant difference, and that a small group of determined people can change the course of history. — Sonia Johnson

AN UNEXPECTED ROAD

Stuart and I often remark that we are blessed by the wonderful people that Benjamin has brought into our world. We would like to introduce you to Patty and Jessie, two of those wonderful people and share their story. Patty has been a valuable resource to us and others in managing many of the problems encountered by Sanfilippo children. For that and her patient ear we are grateful.

On May 2, 1995, I gave birth to our second child. It was 12½ years after our first child. Even though he was unexpected, the thoughts of another baby brought new joy to our lives. Two years prior, I battled with breast and lung cancer. Going through that and ending up pregnant was a blessing—a sign of life.

Jesse appeared normal at birth; eating and sleeping at regular intervals, laughing and cooing, but by 6-months he was sleeping less and getting constant ear and throat infections. We didn't let those bother us as some children are more prone than others. By 1½, Jesse and I began sleeping on the couch because he was awake much of the night. He seemed to have endless energy. When he did sleep, he snored. So we had his tonsils and adenoids removed.

By 2, we noticed speech development was slow and hearing selective. Testing showed Jesse had a moderate hearing loss. We got hearing aides and enrolled Jesse in a school for the speech and hearing impaired. He thrived there. One day while getting his hearing tested at Kennedy Krieger, an intern commented that Jesse was peculiar looking, and asked me if he was an MPS child. I was taken back by his comment knowing nothing of MPS. He examined Jesse and asked to do urine and blood tests for Hurler (MPS I) or Hunter (MPS II) Syndromes. I knew in my heart that something was not right with Jesse, so I allowed the tests. I went home and read about the two disorders on the Internet. I sat and cried after reading about MPS. Jesse fit so many of the descriptions. I knew the probability was great that he had MPS. Lord, I did not want my son to have any of these diseases. They all sounded so hopeless. Fortunately, tests results came back negative. I was so happy!

At 5, Jesse was developmentally delayed and not potty trained. He was starting to lose skills, his behavior was hyper and he was still not sleeping. The state labeled him Globally Delayed/Mentally Retarded. But I knew in my heart there was more to it. Just before his 6th birthday, the paper ran an article about a girl with Alphasmanosidosis, a rare disorder. It sounded just like Jesse. I e-mailed the girl's father. After we spoke, I took Jesse to see their geneticist. Unlike the intern, he tested for all the MPS disorders. Jesse was diagnosed with MPS IIIA (Sanfilippo Type A). This diagnosis broke my heart and caused every emotion, mostly down. However, it was a relief to finally know why Jesse was the way he was. The worst part was that there was nothing we could do but accept it and treat the symptoms.

After what I call my mourning period, I decided that there had to be something I could do to prolong Jesse's



Jessie Taormino enjoys a ride on his specially designed bike

life and improve its quality. I read everything I could. Learning that the storage affects both the neurological and physical being, I studied how nutrition affects the brain and body. I determined that the best thing I could do for my little man was to build as strong and balanced a brain and body as possible. I did this mostly through vitamins, antioxidants, and healthy organic foods. My thought was not to put anything in his body that he cannot use or get rid of easily. By 7, he started sleeping 7-9 hours every night. What a blessing that was!

Jesse has grown strong and healthy over the past 4-years. He still laughs a lot and says a few words. He is physically active and enjoys his new bike. In June 2005, I decided to try Genistein, a soy isoflavone. Studies done in Poland show that it inhibits GAG synthesis in Sanfilippo. Jesse is the first child in the USA to use this as a therapy and I consult with Dr. Wegrzyn each week. I believe Jesse has not regressed in the past year. He is not cured but his GAG levels have reduced. I have no idea if he will ever regain lost brain functions or if he will be as he is now for 15 or 40 years. But I do know that at 11-years old, he is happy and healthy.

I pray that with all that is being done to find therapies and cures for our Sanfilippo children, we will at least have more manageable lives with our children through each others experiences.

You can follow Jesse via his website:
<http://www.caringbridge.org/md/myjesse>. Many hugs and blessings to you and your families.

Patty Taormino

BIRDIES FOR BEN CHANGES COURSE

The 7th annual Birdies for Ben golf fundraiser saw many changes in 2005, but it's goal remained the same: raise as much money to fund research as possible. And it did. The event brought our seven year total to over \$500,000!

Held for the first time at the Brookline Golf Club at Putterham in Brookline, MA, the event remains our prime fundraiser. Twenty-eight golfers tee'd off on August 22nd and were challenged by the new course. The change in venue did not throw Bob Davis off, who once again took home the prize for lowest score. Golfers were treated to box lunches donated by Mitch Roberts and Panera Bread. This year saw many of the children get actively involved. We had a foursome of teenage golfers who solicited pledges and several children presented money raised from lemonade stands, cookie sales and even their allowance as donations.

With the Siedman residence under construction, Stuart's sister and brother-in-law, Cynthia and Bob Willen of Newton, MA graciously hosted the after-golf picnic. Once

again Taylor Rental of Wellesley supplied the children with hours of bouncing fun in the moonwalk. "Chef" Bob Evans expertly prepared food donated by Captain Marden's, Roche Bros., Star Market and Whole Foods of Wellesley while his sons Rich and Chris kept everyone's thirst quenched through their company Bevinco. Thanks to the efforts of the Shelby's and the Wagner's, guests were treated to a re-broadcast of the UPN-38 Boston story about Noah's wristband initiative.

"No words can adequately express the depth of gratitude we feel for the support, hard work and concern of our golfers. This event is truly the backbone of our foundation. Each year dedicated individuals and businesses come together to help us conquer Sanfilippo. We could not ask for more," says Stuart.

It has been a very challenging year for our family. Ben's needs have grown significantly and the time they consume has increased. When treating Ben's disorder and making decisions about his care, we have always applied the creed, "first do no harm." We

feel that it is also time to apply the same philosophy to our whole family. The time spent running our foundation and hosting events may be time that we now have to dedicate to our family.

Our decision to host a 8th Birdies for Ben was largely the result of Dr. Fu's pending application for clinical trials. Her research progress is proof that our efforts and more importantly yours have been worth all the work. We hope to make this year's tournament the biggest yet and be able to fund Dr. Fu through the Investigational New Drug (IND) application process.



WE NEED YOUR HELP

8th Annual Birdies for Ben Golf Fundraiser

The 8th Annual Birdies for Ben Golf Fundraiser will be held August 21, 2006, at Sandy Burr Country Club in Wayland.

**We need your help to fund
pending clinical trials!**

Please see the reverse side to support a golfer or contact Stuart Siedman at (617) 899-4158 to learn how to become a corporate sponsor.

Your contribution will further research and bring Ben and his friends one step closer to realizing their dreams.

Thank you for your support.

BIRDIES FOR BEN HOW IT WORKS



"Uncle Rob" Carpenter at BB-2005

On August 21, 2006 at the Sandy Burr Country Club in Wayland, MA, a group of golfers will tee-it-up on behalf of Ben and the Foundation in our 8th Annual golf fundraiser.

Not your typical golf fundraiser—the event is designed to raise the maximum and cost the minimum. Golfers secure tax-deductible donations from family, friends and associates for their round of golf to benefit Ben's Dream. The Foundation provides printed materials and fundraising guidance to each golfer and helps them set a personal fundraising goal. Participants pay for their own greens fee and all other event expenses are donated or paid for by Stuart & Jennifer Siedman. This allows the Foundation to use 100% of the contributions to directly support research.

Corporate hole sponsorships are available for \$500, \$1,000 and \$2,000 plus levels.

There are additional corporate sponsorship opportunities through donation of goods and services in support of the event.

If you would like to find out more about becoming a corporate sponsor or if you would like to participate please contact Stuart Siedman at 617-899-4158.



"Birdies for Ben" Sponsor Card

Name _____ Phone _____

Address _____

City _____ State _____ Zip _____

E-mail Address _____

I would like to sponsor _____ for: \$ _____

Check if your company participates in a "Matching Gift" program#:

#Please include your company's Matching Gift form with all necessary employee information.

Make checks payable to Ben's Dream.

Ben's Dream is a public 501(c)(3) non-profit organization so your contributions are tax deductible. Our Tax ID# is 04-3565765.

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