



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 life-span, there are thousands of families dealing with Sanfilippo in the USA alone.

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

ONE BROTHER'S DETERMINATION

The golf clubs are put away, the remnants of the family picnic scattered in the yard, the moonwalk is quiet for the first time in many hours but the glow of the 5th Annual Birdies for Ben is still reverberating throughout the Siedman household. Noah, age 9, is excited and proud of the outcome of the day. So many people working so hard to find a cure for Ben makes a big impression and his wheels start to turn. He wants to be involved and what follows is truly amazing.



Soliciting the help of his life-long friend Olivia Garber of Needham, the two conceived of a charity fun run for kids to benefit Ben's Dream. The two enterprising 4th graders sought out a location for the run and held weekly meetings to plan their strategy. They set a goal—get 50 kids to run, charge \$5.00 each and raise \$250.

Noah wrote and published a flyer. He initiated a letter writing campaign to get businesses to donate goods and prizes. Both he and Olivia wrote letters to the Principals of Fiske Elementary and Newman Elementary asking for their support. Noah presented a short speech to each of the 4th and 5th grade classes at his school. They talked pointedly about the devastating impact of Sanfilippo with reporters from the Community Newspapers in both towns and with the Boston Globe. Noah told them, "When I went to the other classes, everyone asked what will happen to my brother when he's older. It's a

question I don't want to answer but sometimes I have to."

Sunday, October 19th arrived and more than 120 elementary age kids came to run. Noah and Olivia began the day by taking Ben's hand and inviting everyone – runners, parents and spectators to join them in a walk around the track. Over 300 people walked that lap with Ben, a symbolic gesture that will be imprinted in many minds forever. When the day was over and the trophies handed out, the goal of \$250 became a reality of \$8,000! "I never thought I would raise that much. All my friends supported me so much," said Noah.

Noah and Olivia are examples of the difference anyone, child or adult, can make if they have passion and determination for what we are trying to accomplish.

Please join us in our determination.

A LOOK INSIDE . . .



June 21, 2004

Today was Benjamin's last day in public school. He won't be back next year.

As I was standing with Pam, his friend, companion and educator for the past 5-years (I can't bring myself to call her his aide), I watched him walk the schoolyard holding hands with one of his classmates. All the other children were running around – excited by the prospect of summer vacation, but this girl was taking time away from everything else to walk with Ben. Watching I sensed that she knew holding his hand was more important . . . she could always join in later or next year. Pushing back a well of emotion, I smiled. His last day.

Who will walk with him next year when everyone at school has special needs? Turning this over in my mind, I watched a second classmate take Ben's other hand. I tried to exchange pleasantries with other parents, but my gaze kept coming back to Ben and his friends. They had broken away from the hubbub and were heading toward the fields. Their little group had grown to five. Watching, I expected the group dynamic to change. I was sure talk between the others would distract them from Ben, he would become a bystander and they would rejoin the celebration. I was wrong. Two more classmates left the schoolyard to join his group.

Standing next to me, I heard Pam telling me how good the kids are at gently changing Ben's direction. I could see Ben's smile. The entire group was smiling and I couldn't help but smile as well. The moment was broken only when everyone was called to lineup for their formal dismissal. This was Ben's last.

Ben, I don't know if we will find you friends like this again. I don't know if we ever really did. It's more likely that they found you and you found them. All I can do is try my very best and watch to see you work your magic smile in the next adventure. You always do.

Ben's Dad

BIRDIES FOR BEN

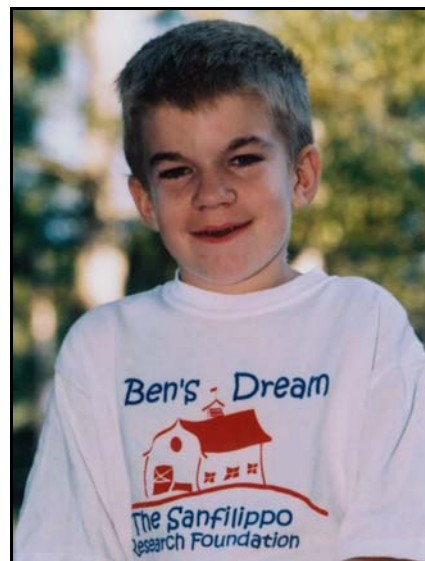
"Birdies for Ben" remains the cornerstone of our fundraising efforts. In just five years, this event alone has raised over \$400,000 for research.

Each year when August rolls around and we try to sort out all the logistics for the event we have moments when we ask, "Why are we doing this?" When we watch Benjamin and see the skills he's lost we wonder, "Is it worth it?" But then we remember all that we've accomplished in the past five years and see the smiles Ben still offers and we know why!

The 5th Annual "Birdies" held at Sandy Burr Country Club in Wayland, was a grand success. Occasional downpours did not dampen the spirits of this devoted group of golfers. The tournament welcomed many new golfers and

challenged them to out-play and out-raise the ever-faithful group who participate each year. But in the end, long time tournament supporters took the prizes for lowest score and most dollars raised.

Participants in the event are asked to solicit pledges for their round of golf. When asked why the Foundation chose this over a larger pay-to-play style event Stuart remarked, "Our efforts on behalf of Ben and the Foundation have always been based on individuals making a difference. The golf event is a great example of how a small, dedicated group can raise more money than a traditional tournament while at the same time allowing for the personal contact with Ben and our family that means so much to us."



The sun came out for the post-golf barbecue at the Siedman's and the golfers families came to celebrate another successful tournament. Everyone enjoyed an

(Continued on next page)

WE NEED YOUR HELP

6th Annual Birdies for Ben Golf Fundraiser

The 6th Annual Birdies for Ben Golf Fundraiser will be held August 23, 2004, at Sandy Burr Country Club in Wayland.

We need your help to reach our six-year goal of \$500,000 raised for research.

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" Continued from previous page)

HOW IT WORKS

abundance of food graciously donated by Roche Bros, Star Market, Whole Foods and Captain Marden's and the golfers wet their whistles with refreshments donated by The Tap of Boston. Sounds of glee could be heard throughout the yard as kids bounced the night away in the moonwalk donated by Taylor Rental. "Each year when I look at the crowd in our yard, many of them good friends and yet so many of them people who only know us through our cause, I am overwhelmed by a sense of how fortunate I am. Benjamin has this devastating disorder and because of that each day my heart aches, but he has brought so many wonderful people into my life to help ease that pain. This event really brings it all right to our backyard," says Jennifer.

On August 23, 2004 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 6th Annual golf fundraiser.

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 and each golfer sets a personal fundraising goal. They pay for their own greens fee and all other event expenses are donated or paid for by Stuart & Jennifer Siedman so 100% of the contributions received goes to supporting 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"

Name _____ Phone _____

Address _____

City _____ State _____ Zip _____

E-mail Address _____

I would like to sponsor the round for: \$ _____

Check if your company participates in a "Matching Gift" program[‡]:

[‡]Please include your company's Matching Gift form with all necessary employee information.

Please make checks payable to Ben's Dream.

FUNDRAISING NEWS



Hope Comes in a Jar

In honor of Ben's 8th birthday his classmates at Sprague Elementary School collected a huge jar of coins. Presentation of the jar brought tears to Jennifer's eyes and hope to her heart. "They have been kind, caring and concerned classmates. They look beyond Ben's disorder and appreciate his talents and gifts. As we prepare to move Ben to his new school, I know he will miss holding hands with all his special friends."

Ben's Dream Goes International

It was a night of dancing, drinks and new friends. Hearing about Ben and the Foundation through Deb Condon, a neighbor of the Siedman's, Kerry Welch, Gabby Poirier and Z Gallagher hosted an evening at The International in Boston benefiting Ben's Dream. Despite frigid cold temperatures, well over 100 people turned out and helped raise over \$2,500. "Kerry had never met Ben, Jennifer or Stuart before that night, but she was excited to be helping. As corny as it sounds, there are a few less strangers in the world now and a whole lot more awareness of Sanfilippo and Ben's Dream," remarked Deb.

Going the extra mile Kevin Kramich, another friend of the Welch's organized a small silent auction for the event. There were a number of sports items auctioned off including Bruin's tickets and autographed hockey sticks.

This event truly illustrates the generous nature of people and how lucky the Foundation is to be the benefactors of such kindness. "I am continuously overwhelmed by the generous spirit of people who hear our story and take the steps to help us," says Jennifer.



Making Memories

The 3rd Annual "All-Day Crop for Ben" was the largest yet with 60 people attending and raising over \$10,000 for research. Held at the Italian-American Club in Wellesley, the event draws "scrapers" from all over Massachusetts. Scrapbook Consultants, Lisa Wentworth and Lisa Cross offer classes to beginners, support to veteran scrapers, and a popular raffle for pages completed. This year's event also included donations from a photographer and Longaberger Basket representative.

"This event has become my favorite. It gives me a whole day to reflect on my family, record their images and my memories, and it raises money for research. I know I will cherish my scrapbooks of Ben more and more in the years to come"

The 4th Annual Crop for Ben is already in the "layout" stage – again scheduled for mid-November. Contact Lisa Wentworth at 781-237-9121 for more information.

FUNDRAISING OPPORTUNITIES

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, 908-252-2890.

Send a Note

Walter See of DaisymaeGallery recently turned his paintings into note cards. View his paintings on our website and then place your order. A set of 8 cards is only \$15 and all the proceeds benefit Ben's Dream.

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

THE FOUNDATION GIVES THANKS . . .

To Pam Davis who has been Ben's aide at school for the past 5-years. Her commitment and love for Ben has made those precious years in public school a true victory over Sanfilippo Syndrome and the greatest gift anyone could give Ben.

To the staff at Sprague Elementary School in Wellesley who not only enriched Benjamin's world and appreciated every day spent with him but embraced whole-heartedly all the foundation's fundraising efforts.

To Ben's big brother Noah for being determined to make a difference for Ben. Your energy will always produce excellence to ordinary things in extraordinary ways.



To Monica (10) & Susan (8) Roberts for hosting a fun-fair in their Wellesley neighborhood in honor of Ben.

To the students in Ms. Firger's fifth grade class at the William Mitchell Elementary School in Needham who read over 28,000 pages in a four-week read-a-thon "Giving Project" that raised over \$735 in donations.

To the Wellesley Firefighters for making Ben's 8th birthday a "4-Alarm" event.

To the students of Saint Joseph's Elementary School in Needham for demonstrating "Faith in Action" and supporting the Kids Fun Run for Ben.

RESEARCH UPDATE

Dr. Haiyan Fu, University of North Carolina—Chapel Hill

The critical issue in studies on therapeutic development for MPS IIIB (Sanfilippo B) and other severe forms of MPS is how to efficiently deliver therapeutic materials in to the central nervous system (CNS). The presence of the blood-brain barrier (BBB) is the greatest obstacle in developing treatment for ubiquitous CNS diseases. Previous studies showed that intravenous injection of AAV vectors into neonatal MPS VII mice resulted in the correction of lysosomal storage in both somatic and CNS. However, in humans, the BBB is well developed at birth. It is therefore important to study efficient CNS delivery techniques, in order to treat the neurological disorders in MPS IIIB patients.

We have been studying the therapeutic effect of AAV viral vectors for the CNS disease as well as more recently the somatic disease of MPS IIIB, using the MPS IIIB knock-out mouse model. Efforts have

been made to investigate the means of CNS AAV gene delivery for achieving a broad/global distribution of the AAV transgene expression. The long-term goal of this study is to develop effective therapies for CNS diseases in patients with MPS IIIB.

AAV vector appears to be an ideal gene delivery system for gene therapy studies on MPS IIIB. The AAV-NaGlu vectors constructed in our laboratory were proven to transduce both somatic and CNS tissues in vitro and in vivo. The critical issue in gene therapy study for the treatment of CNS disease in MPS disorders is how to achieve broad or global distribution of the therapeutic vectors in the CNS. With generous support from Ben's Dream, we have been focused on studying gene delivery methods to improve the distribution of AAV vectors in the CNS.

Mannitol is a known blood-brain barrier interruptive reagent. Intravenous infusion of highly concentrated mannitol is routinely used to reduce the intracranial pressure in patients with traumatic brain diseases. Mannitol has been used in preclinical studies to achieve the entrance of a wide

range of substances into brain, including enzymes, antibodies, and viral vectors. Intra-arterial infusion of mannitol caused no obvious BBB damage. Recently, we studied the effects of IV mannitol infusions on the distribution of AAV-mediated gene expression in mouse brain after intravenous or intracisternal AAV vector delivery, in order to achieve broader spread of the vector.

Based on our studies, we anticipate that AAV gene therapy offers promising future in developing therapy for MPS IIIB. The therapeutic efficiency of AAV gene therapy for MPS IIIB will be greatly improved by combining IV and IC injection, using combination of AAV1 and AAV2 vector, using more powerful and ubiquitous promoter, and studying the mechanisms involved in post-translational modification of NaGlu.

The studies have been done by Dr. Haiyan Fu in the laboratory of Dr. Joseph Muenzer, with the collaboration of Dr. Richard J. Samulski of Gene Therapy Center at University of North Carolina at Chapel Hill.

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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