

Ben's Dream



The Sanfilippo
Research Foundation

Barnraising News

The latest news on Benjamin Siedman and the grassroots, "Barnraising" effort to find a cure for Sanfilippo Syndrome

Summer 2007

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 . . .

IF YOU BUILD IT, THEY WILL COME

No, we didn't get Kevin Costner but we got something better! We got Mike Pollak and the Ben's Dream White Sox. An avid baseball player, Ben's teacher, Michael Pollak approached us about having his Boston Baseball League team adopt Ben and the Foundation as their cause. Pollak also gained the support of Brett Rudy, team Manager and President of the league. "I think it is fantastic that we can help raise awareness for Ben's Dream by playing baseball. Players keep asking me what it's all about. That's proof that it's working," commented Rudy. Pollak went on to single-handedly raise \$5,000 in sponsorship money contributing to the Foundation's mission to raise money for research.

The team started the season off in a tournament on Doubleday Field in Cooperstown, NY, and as of publication is headed to the playoffs – a standing they attribute in part to the "Luck of Ben". They have proudly worn the Ben's Dream logo on their jerseys all season and a Sanfilippo Research Foundation banner hangs at all of their games. There is a link on the league's website (www.bostonbaseball.com) bringing awareness of Sanfilippo Syndrome to the very extensive Boston baseball audience.

At a game early in the season Ben was

presented with an official baseball mitt by player John Byrne. Ben and his brother Noah gave each team member a wristband. "It is an honor to be sponsored by Ben's Dream this season. Ben's family has shown us incredible courage and integrity in dealing with their difficult situation . . . that is something we strive to carry onto the ball field each game," said Matt LoPresti, starting pitcher for the team.

About his experience this season, Pollak remarks, "I have had the pleasure and privilege of knowing Ben and his family since the fall 2004 when I joined the Perkins staff. My time with Ben has been incredibly meaningful, gratifying, fun and at times, challenging. Every step of the way, Ben demonstrates an unconquerable spirit, a great love of life and a wonderful sense of humor. Ben and the Siedman's have become family to me. As I learned more about Sanfilippo and the work of Ben's Dream in raising awareness and funds for research toward a cure, I thought about ways that I could help."

On-Deck Circle

*Columbus Day weekend charity baseball tournament – for details go to:
www.bensdream.org/events.html*



The Boston MBL 2007 Ben's Dream White Sox

SANFILIPPO BENefit CONCERT

Saturday, October 6, 2007 @ Harpers Ferry – Allston, MA



Ticket prices are \$15 in Advance and \$20 at the Door and go on sale August 28th. Order tickets online through Harpers Ferry at:

<http://harpersferry.musictoday.com>
or 800-594-TIXX (8499)

Unable to attend? Make a \$50 donation and we will send you an autographed copy of Ari Hest's album *The Break-In*. For details visit:

www.bensdream.org/hestforben.html

On Saturday, October 6, 2007 at the Harpers Ferry Club in Allston, MA, Columbia recording artist Ari Hest (www.arihest.com) and fellow artists Mieka Pauley (www.mieka.com) and Tim Blane (www.timblane.com) will play a benefit concert on behalf of Ben and the Foundation.

Coming off the recent release of his latest album, *The Break-In*, Ari just returned from a tour in the UK & Ireland. He became aware of Ben and the Foundation through his childhood friend and Ben's teacher Michael Pollak (see *One Determined Teacher*).

Mike introduced Stuart & Jennifer to Ari after a performance where plans for the benefit started to take shape.

Ari contacted his friend Mieka Pauley who also agreed to perform.

Mieka has a new album of her own, *Elijah Drop Your Gun*, due out this fall, but she is still able to take time out of her busy schedule to play at the benefit.

Adam Klein of Callanan & Klein Communications completed the picture by adding Tim Blane to the bill and securing the Harpers Ferry venue. Like Ari, Tim is coming off a new album of his own with his recently released *Clockwork* CD.



ONE DETERMINED TEACHER – ONE SOULFUL SOUND

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

Recording artist Ari Hest, a childhood friend of Ben's teacher, Michael Pollak, has agreed to lend his soulful sound to a benefit concert for Ben and the Foundation. Michael and Ari first met on the little league fields of Riverdale, a town in the northwest section of the Bronx. They later attended the same prep school in NYC where they were teammates on the baseball field.

"I was the lone freshman on the varsity baseball team and was very intimidated. Ari was a junior at the time. He went out of his way to take me under his wing and make me feel like an important part of the team." remembers Pollak. That year the team won the league championship with Hest, the big lefty, on the mound for the deciding game.

This spring in a trip to NYC to see Hest perform, Pollak told Hest about Ben, Sanfilippo Syndrome, and his experience teaching a child like Ben. He invited Hest to come to Boston to play in the Columbus Day tournament as a member of the Ben's Dream White Sox. He

asked Hest to consider performing a benefit concert on behalf of the Foundation. Hearing about Pollak's love for Ben, commitment to help fund research and knowing his passion for baseball Hest remarked, "When Mike asked me to play a show, I knew it was something I wanted to be a part of. It just so happened that baseball was involved as well. Before music came along I had a far reaching aspiration of playing pro ball...I look forward to both the show and lacing up my cleats for a triumphant return to the field and all for a worthy cause."

About Hest's commitment to a pro-bono performance, Pollak remarks, "That's just the kind of person that Ari has always been. He's a team player, always willing to sacrifice of himself for the benefit of others. With all the success he has achieved as a musician, he remains the same caring, humorous, and kind person that I got to know in high school."

Caring is also how the Siedman's would describe Michael, "The opportunities for fundraising that Mike has created for the Foundation are only trumped by the love and dedication he has for our son."

Michael is truly an example of what one determined individual can do.

A LOOK INSIDE . . . BIG BEN



Ironically, Ari Hest has a song titled “Big Ben.” His website says he wrote it for a friend who was going through a rough time and he offered the clock in England as a reminder of all that is stable and lasting. And yet, the lyrics strike a cord and resonate in my heart. They describe perfectly what it is like to have a child with Sanfilippo.

*Someone please explain
How days so plain
Became so fickle and dangerous
Have gathered an unsettling dust
Have beaten up the both of us.*

From the moment your child is diagnosed, life is altered. Days that were once plain are now “fickle and dangerous.” The child sitting on your lap is no longer just your boy. He’s a boy who might never. A boy who, if he does, you cannot stop yourself from wondering . . . for how long? Life with Sanfilippo gathers “an unsettling dust” that covers even the everyday things that you do.

In 2001 when Ben was playing tee ball like every other 5-year-old, I described that feeling, “And then it hit me. Sometimes he is so regular. Simultaneously, I was filled with an unyielding sense of sorrow and overwhelming joy. I find the regular moments in life intensely profound and extremely painful. Even as I ache for them to last, I wonder if it wouldn’t be easier if they never tempted me at all.”



Ben is 11 now and his days of tee ball have passed. So many of the things doctor’s warned us would come are here. Over the years many of you who have shared this journey with us ask how Stuart and I face the daily challenges of our lives. Honestly, there are days when it feels as though Sanfilippo has “beaten up both of us” and nothing can drag me out of the sadness that fills my heart and the frustration that fills my head.

And then I hear Ben’s contagious laugh bellowing out from his special room and I know he got the joke on his Arthur video. Or I hear him say, “seven, eight, nine” as he counts along with Nanny from a 101 Dalmatians. And I feel victorious. At that moment, I know how it is that I have the courage to celebrate each day, good or bad, happy or sad, spent with Ben. Courage is the gift Ben has given me.

And again Ari’s lyrics resonate . . .

*Think Big Ben, Think Big Ben.
Don’t go down, don’t go down that easy!*

Jennifer.



RESEARCH UPDATE

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

In our gene therapy research, we have made an adeno-associated viral (AAV) vector for use as a vehicle to carry NaGlu genes into cells. We have also developed two non-surgical vector-delivery procedures, intravenous (IV) and intracisternal (IC) injection. These procedures enabled us to efficiently deliver the AAV vector into the central nervous system (CNS), to reach broad areas of both the brain and spinal cord in mice. Using an IC injection alone or the combination of an IV and an IC injection of AAV vector, we have been able to significantly extend the survival, and also improve the behaviors of MPS IIIB mice. The AAV vector entered the CNS and produced NaGlu enzyme in brain and spinal cord. We believe that we have a working procedure for treating MPS IIIB, which could significantly improve quality of life, though it did not yield a complete cure of the disease. Considering the potential benefits of these treatments for patients, we are working toward future clinical trials. We are currently seeking funding from NIH (and

other sources if possible) to support this translational step from laboratory procedure through FDA approval.

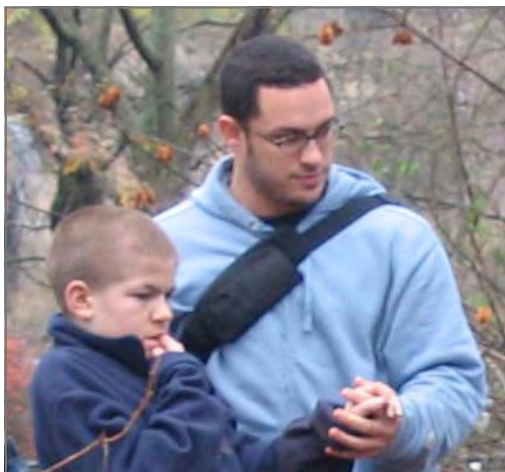
In our last round of experiments, an IV infusion of AAV vector following mannitol pretreatment prolonged the survival of MPS IIIB mice, but did not improve their behavior. We have recently optimized our IV vector delivery procedure, which led to approximately 10-fold increase of the AAV-CNS entry. With the continuous support from Ben's Dream – The Sanfilippo Research Foundation, we have been assessing the therapeutic impacts of an IV AAV vector injection in MPS IIIB mice, using the recently optimized conditions. These experiments are ongoing.

As mentioned above, we have developed working therapeutic procedures for treating MPS IIIB, but did not completely cure the disease in mice. This may be due to the complex mechanisms of cellular damage initiated by the enzyme deficiency in MPS IIIB. Our recent studies showed that the lack of NaGlu enzyme has affected several hundred genes in MPS IIIB mice, and we believe that these changes contribute to the pathology of the disease. This raises the possibility of slowing down the progression of the disease by correcting some of these

factors independently, or in combination with the gene therapy. Therefore, one of our main efforts will be to identify the most critical factors from these affected genes and put them in consideration for therapeutic development. We anticipate that these efforts will enhance the impacts of AAV gene therapy and yield more efficient therapeutic benefits for MPS IIIB.

Earlier this year Dr. Fu submitted her application for funding to the NIH, the first step towards clinical trials. The application was evaluated using the standard NIH review criteria (42 CFR 52h.8) of significance, approach, innovation, investigator and environment.

In May, Dr. Fu received her Summary Statement from the committee. This statement includes a summary of the panel's discussion, a priority score and any human and animal concerns the panel had. The priority score reflects the relative strengths and weakness of the application. Dr. Fu's application did not receive funding (not unusual) but her priority score indicated that with revisions her research should be resubmitted. She hopes to resubmit this October.



I LIKE MIKE

Our thanks to Michael Pollak for challenging Ben to learn new things each day and for having the wisdom to love.

For Ari and for baseball and for you.



THE FOUNDATION GIVES THANKS

To Michael Walsh and Diane Lambert of Walsh & Associates, PC of Concord, MA, for donating Foundation tax filings.

To Matt and Laura Weiler for honoring Ben on their wedding day by asking for donations in lieu of gifts.

To the Youth Group at Saint Thomas the Apostle of West Hartford, CT, for raising \$1,000.

To Isabelle S. and Sage W. for selling cookies for Ben's Dream.

To the Wallace Family, another University of Rochester connection, for their commitment to their son's and generous donations.

To Tom Collins and Kate Kerr for sharing Bands for Ben with their wedding guests.

To Adam Klein of Callanan & Klein Communications of Boston, MA, for donating his rock-n-roll event expertise.

To Kevin DuBois of Paragon Marketing Group, LLC of Westborough, MA, for putting Ben's Dream on the world-wide-web.

FUNDRAISING NEWS

Hamburger Helpers



Chris Seebeck & Jason Lang chow down

Jon Sullivan ate his way to a victory during the first ever, "Burgers for Ben" cheeseburger eating contest. The brainchild of Chris Seebeck, a long time supporter of the Foundation, the event was held on September 12, 2006 at the Vault in Boston, MA. Ten contestants were challenged to eat 15 sliders in the quickest time. Jon "Big Dig" Sullivan dominated the field finishing all 15 in 30 minutes. The Vault generously donated 150 of their famous sliders for the event.

The contestants, all employees of Sun Life Financial Distributors in Boston, MA, challenged their coworkers to purchase pledge tickets. A donation of \$10 earned a person a ticket that they could then use to "bet" on one of the participants. They could buy as many tickets as they wanted and pledge them in any or all contestants buckets. When the contest was over, Seebeck promised to pull one ticket from the winning contestant's bucket. The holder of that ticket was the winner of a 50/50 raffle worth up to \$2,000.

To drum-up interest, crazy bios were posted for each contestant – noting such things as proudest eating accomplishment, preference

for boxers or briefs and celebrity look-a-like. The contest was very popular – even more so than last year's haircutting challenge with hundreds of tickets sold.

With bellies full and half-eaten burgers all around, Stuart Siedman pulled the winning ticket from Jon Sullivan's bucket. Amidst cheers (and a few belches!), Chris Seebeck had the honor of announcing Justin Wilson as the raffle winner. Wilson, deeply moved by Ben and his story, generously donated his winnings to the Foundation. After Sun Life matching gifts, the event raised over \$5,000 for research.



Jon "Big Dig" Sullivan on his last bite!

Memory Makers

Once again consultants, Lisa Wentworth and Lisa Cross hosted a fabulous day-long scrapbooking event benefiting the Foundation. In its 6th year, the event continues to be a favorite of Jennifer's. "I love this day. It provides me with a whole day to reflect upon the year's events with my family. Capturing those memories and putting them together in a book is something I know I will treasure

forever."

Over 40 dedicated attendees arrived at the Italian American Club in Wellesley ready to add a page to this year's Ben scrapbook. "This year so many people volunteered to do a page that Jennifer had to start a second scrapbook. We filled one book with Ben's first 10-years and look forward to completing one for his next 10," said Lisa Cross.

The attendees were treated to lunch donated by Mitch Roberts of Panera Bread, desserts from Quebrada Bakery and dinner donated by the Wentworth Family.

The 2007 Crop for Ben is scheduled for November 17th. "The support and commitment to this event continues to grow – the date has held firm for the past few years – the Saturday before Thanksgiving 10AM–10PM. It is endearing to hear the stories of how people have rearranged their holiday plans to ensure their participation," remarks Lisa Wentworth.

Crop for Ben is a staple in the Foundation's fundraising efforts raising approximately \$5,000 each year for a cumulative total of \$30,000 dollars raised for research. If you would like more information on how to attend the event contact Lisa at:

www.mycmsite.com/lisawentworth



Lisa Cross & Lisa Wentworth

MEET JESSIE

Six degrees, they say that is all it takes to connect people. This year we would like to introduce you to the Aufman family also from Massachusetts. In an almost surreal coincidence we discovered that not only are we both faced with the challenges of Sanfilippo but that Cari and Matt also attended the University of Rochester, as did both Stuart and I.

Just days after my husband and I discovered I was pregnant with our second child, we were devastated to learn that our beautiful 2-year old daughter, Jessie, had MPS III-A. We also learned there was a 25% chance that our second child would have Sanfilippo as well. Jessie's doctor said there was nothing we could do for Jessie.

After two weeks of feeling completely helpless, I decided to search the internet for help and support. I quickly found the website for Ben's Dream and was relieved to know there was a family nearby that knew what we were going through. While speaking with Jennifer for the first time, she mentioned that Duke University was researching whether children with Sanfilippo could benefit from stem-cell transplant. I sent an e-mail that day to the head of the transplant program at Duke, and she responded the same day. Within a month, we were in Durham for extensive evaluations, which determined that Jessie was a suitable candidate for transplant.

A stem-cell transplant is similar to a bone-marrow transplant, except donor umbilical cord blood is used instead of bone marrow. The transplant is preceded by an intense course of chemotherapy to destroy the patient's immune system. Following transplant, patients receive a myriad of strong drugs to prevent rejection of the donor stem cells and stave off infection. The objective is for donor cells (which don't carry the Sanfilippo mutation) to produce a new immune system that will produce the enzyme missing in children with Sanfilippo. *Transplant is considered a treatment for Sanfilippo, not a cure.* It cannot reverse the damage the disease has already done to the child's brain, but the doctors at Duke are hopeful that transplant will allow children with Sanfilippo to live longer, healthier lives and to avoid some of the disease's most severe effects. Jessie received her transplant in June 2005.



Recovery from transplant is grueling and dangerous; sadly, some children succumb to infection or other complications. Although we had some setbacks on the way, Jessie's recovery went reasonably well. Still, the process took its toll on her. For over a year, Jessie remained in isolation because of her compromised immune system. Moreover, her cognitive skills declined sharply in the months following transplant, most likely because the stress that the transplant process placed on her body exacerbated the effects of Sanfilippo on her brain.

After nearly eighteen months, Jessie finally came off all her transplant-related medications. She has fully recovered from transplant and is a happy child. She has a loving relationship with her younger brother, who does not have Sanfilippo. Jessie will never be "normal," and she is unlikely to have a normal lifespan. We are hopeful, however, that we will be blessed for years to come with Jessie's joyful presence in our family.

Although a few Sanfilippo children have benefited from transplant, doctors question whether the potential benefits outweigh the risks. The Foundation does not endorse any medical procedure. If you would like more information please contact Dr. Kurtzberg at Duke University.

BIRDIES FOR BEN

On August 21, 2006, the Sandy Burr Country Club in Wayland, MA, welcomed us back for our 8th Birdies for Ben golf tournament. Golfers were greeted by Stuart with a muffin breakfast for 9:30 am tee off. This year's also featured two "teenage teams" bringing the total golfers to 40 – our largest crowd yet.

With boxed lunches donated by Panera Bread and golfer goody bags that included products donated by Sam Adams, Dunkin Donuts and EMC, the golfers were well equipped to play their best round yet. Nature provided a beautiful day, thus depriving many of much needed excuses on the course. Bob Davis had his first serious challenge in years, but still managed a share of low round with Roy Pottle when both posted a 79. Pottle also captured the longest drive award with the cart GPS showing 297 yards from the tee (we've sent it in for testing). Chris Shelby measured his shot in feet, not yards, in taking closest-to-the-pin honors. Off the course, Rookie of the Year went to 16-year old Chris Wong who out-raised many adults bringing in over \$1,500.

The post-golf party returned to the Siedman's newly landscaped yard, which included food donated by Captain Marden's, Roche Bros, Star Market and Whole Foods. The Evans family was once again instrumental in the success of the picnic providing their expert grilling services and the beverages for the event and Taylor Rental kept the children bouncing all night long.

About the continued success of the event, Tom Hitchcock remarked, "Birdies for Ben is an extraordinary charity golf and family experience. While similar events with 3-4 times the participants raise \$20-\$40K, Birdies routinely raises \$70-\$90K with only 20-28 players, none of whom are wealthy or write huge checks. The difference? Passion. We know how dreadful this disease is for both the kids and their families. We know that even this small amount of money is making a difference in the progress of research."

Hitchcock went on to say that relative to other larger non-profits, the Foundation's commitment to deliver dollars raised to research rather than expenses makes it easier to get sponsors. "We love that every



Winston Wong follows his shot at Birdies for Ben 2006

dime we raise . . . all of it . . . goes to research, not expenses." Since its inception, all expenses for Birdies for Ben have been either donated or paid for personally by the Siedman's.

The summer has come and gone – August 20th, the 3rd Monday of the month and the day we normally host Birdies was a beautiful day, perfect for golf – sunny and cool. It was a day we spent wondering if we had made the right decision when we decided not to host a 9th annual tournament this year—our single largest fundraising event. Over the past year, we have heard from many fellow Sanfilippo families, attended a few conferences, and talked to researchers. All of whom have concurred that we are making a difference. We have also heard from so many of you who urged us to "tee-off" again.

We know that spending this year taking a "vacation" from Birdies for Ben has made a difference for our family and for Ben. We also know that our work as a Foundation is not done until the day we can tell the parents of a newly diagnosed child that there is a cure. This year we have included in our newsletter an annual appeal. We hope that you will consider this appeal a "virtual" Birdies for Ben. The research has come a long way but we have not hit that hole-in-one yet. Thank you for your continued support.

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 Sweatshirt

We have a number of grey Hooded and non-Hooded sweatshirts with the Ben's Dream logo still available. They are \$25 each. Send an e-mail to Jennifer, jennifer@bensdream.org, with size preferences and she will get back to you with availability.

Buy a Band

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

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.



BENefit Concert

Saturday, October 6, 2007

@ HARPERS FERRY

156 Brighton Avenue
Allston, MA



ARI HEST with MIEKA PAULEY & TIM BLANE

Advance Tickets \$15 - At-the-Door \$20

Order Tickets at 800-594-TIXX (8499)

or <http://harpersferry.musictoday.com>



Ben's Dream



P.O. Box 81268

Wellesley, MA 02481-0002