

SCRF NEWS

THE ELISAGRAM

THE SANFILIPPO CHILDREN'S RESEARCH FOUNDATION



Winter 2016

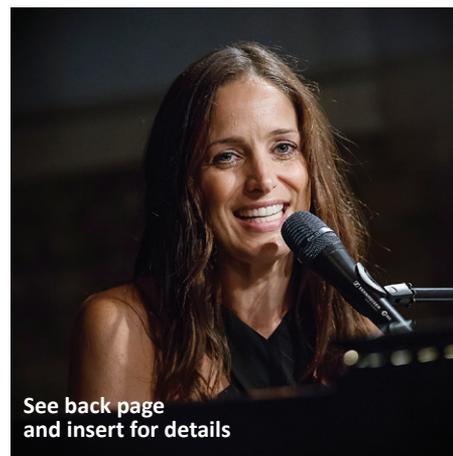
Volume 13.2

The latest news on the fight against Sanfilippo syndrome.

The goal of our Foundation is to fund medical research to find a cure for Sanfilippo syndrome and its related neuro-genetic disorders.

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and insert for details

Holiday Greetings to all our supporters!

As we start a new year, we reflect not only on the blessings and challenges we have embraced, but also think back on the past eighteen years since our daughter Elisa was diagnosed with Sanfilippo syndrome and we founded the SCRF. We have walked an incredible journey through which God has turned our greatest sorrows into our greatest joys. While Elisa's health and abilities have regressed significantly to the point where she is now completely dependent on us for support in every aspect of her life, she has inspired the work of the SCRF, which has supported dozens of research projects worldwide and has been a significant contributor to pending clinical trials on a gene therapy treatment at Nationwide Children's Hospital in Columbus, Ohio.

Our heartfelt thanks to the many volunteers who have contributed their time, talents and gifts and to every donor for enabling us to devote 96 cents out of every one of the over \$6 million dollars we have raised to date directly to research. None of this would be possible without your love and generosity – you have stood by us, believing that the day would come when no parent would ever have to hear the words spoken to us 18 years ago: "There is no money for research into your child's very rare disease, therefore, there is no hope for her."

This past year, too many children with Sanfilippo syndrome have passed away prematurely. This Elisagram is dedicated in memory of our dear friend **Warren Dendekker** from Dundas, Ontario, who passed away suddenly last October. He and his younger sister **Katherine** have inspired the amazing fundraising efforts their family and community have contributed to the SCRF the past three years. Although Warren is no longer with us, his presence will touch us forever.

Elisa graduated from school this past year at the age of 21, a milestone event in her life. She is now home full-time, requiring one-on-one care 24/7. While it is a joy and a blessing to care for Elisa, it absorbs most of Elisabeth's time, which is affecting her ability to accomplish as many SCRF fundraising initiatives as quickly as she would like. Funds to support research are needed more now than ever as scientists are gaining great understanding on how to treat this condition. Our efforts will continue; however, our initiatives and events will most likely change in the future as our focus and priorities shift to Elisa's needs and her care. We are taking things one year at a time.

This coming **Wednesday, April 6th, 2016**, we'll be hosting a special gala evening of celebration and appreciation for everyone who has helped the SCRF accomplish so much at our "**Charmed By Chocolate & Chantal**" gala. This year's event will feature recording artist **Chantal Kreviazuk**, who has graciously gifted us with her talents for the evening. Due to the severity of Elisa's illness, we must sadly say that this year may be our last gala as we have known them for the past 17 years, however we hope to continue fundraising through different events in the years to come. We hope you can join us or support this amazing event in some way. Please save the date and see inside for more details!

We are eternally grateful for your ongoing faithfulness and support and we trust you will continue to remember the SCRF with your charitable giving this year. Please also support our efforts through the gala April 6th, our garage sale May 7th, our marathon run in May, our golf fun-raiser in September, and more.

We wish you and your families many blessings in 2016.

Warm regards, *Randall + Elisabeth Dinton*

Sanfilippo syndrome?

Children with **Sanfilippo syndrome** are missing an essential enzyme that breaks down a complex body sugar called heparan sulfate. This sugar slowly builds in the bones, the brain and other organs, stopping normal development and causing hyperactivity, sleep disorders, loss of speech, mental retardation, dementia, and finally death by the mid teens. There is no cure or treatment yet.

The Sanfilippo Children's Research Foundation

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Life with Elisa

Since our last update about Elisa, her life and ours have changed significantly. We are grateful that she is doing better than expected for a Sanfilippo child of 21 years of age, but we all experience enormous daily challenges.

Elisa appears to be in the last phase of this illness in which her physical and cognitive abilities are very compromised. She requires support for every daily routine, struggles with swallowing and eating, and often needs suctioning as she aspirates on her saliva. However, sleeping (or rather the lack of it) has become her biggest obstacle. Elisa hasn't slept more than a few hours a night for the past six months. After frequent consultations with doctors to find suitable medications to help calm her enough at night to get to sleep, we appear to have found one that is helping, but it requires frequent injections throughout the night, a routine that poses its own challenges.



On the bright side of things, this past June, Elisa graduated from **Park Lane School**, a miraculous moment for any Sanfilippo child and a celebration we only dreamed we would experience. What a joy it was to

see her reach this milestone! After eleven years of receiving amazing care, love and stimulation at Park Lane School, Elisa is now home full-time

with the family. We are thankful for the inspiring personal support workers (PSWs) provided to us from **VHA** on a weekly basis to give us some respite. They graciously help with bathing, physiotherapy treatments and feeding Elisa and they stimulate her the best they can through hugs, songs, stories, laughter, and more. We are also grateful for Emily's House and its amazing staff who care for Elisa during overnight stays when we require respite or travel to conferences. Elisa was at Emily's House for almost a week this past fall when Randall and Elisabeth attended the Global Genes Summit in California.



Seeing changes in Elisa's abilities over the years encourages us to take in every moment and experience with her that we can. Walks in the neighbourhood, pontoon boat rides at the cottage, and snuggles on the



floor are among our favourite times with her. We have learned to enjoy the little things because one day we'll look back and realize they were big things. One doesn't realize the value of a moment until it becomes a memory. We have many memories we look back on fondly: Elisa's smile, eye contact, hugs, snuggles, and the simple sound of her speaking our names. We cherish these moments from the past and look forward to the special ones we have with her in the present.



Memorial Donations

Many donations have been made to the SCRF over the past six months in memory of a loved one or friend. We appreciate these donations greatly and thank those who have made most recent contributions in memory of the following people:

Jane Beverley
Alberto Contardi
Warren Dendekker
Doug Fulton
Dr. Milan Hamza
Lois Humphrey

Margaret Linton
Robert Linton
Maria MacCann
Robert McWhinnie
Sean Pyl
Joan Walsh

The Foundation office would like to know if you:

- Plan to move, and want to give us your change of address so that you can continue to receive newsletters
- Have an idea, comment, auction item or contribution you would like to share
- Require more information about Sanfilippo or the SCRF
- Wish to receive a copy of our Annual Report
- Wish to be taken off our mailing list
- Want to know how you can get involved

Call us at 416-223-1911 or e-mail elisabeth@alifeforelisa.org



Research Progress

Clinical trials of gene therapy in MPS III A and MPS III B at Nationwide Children’s Hospital, Columbus, Ohio

- The team at Nationwide Children’s Hospital has continued its progress toward initiating clinical trials of gene therapy in MPS III A and MPS III B. The team has completed 12-month visits for the 25 subjects enrolled in the MPS III Natural History Study (NHS) for which **Sophie Hamza** from Stratford, Ontario was a participant, visiting the hospital three times this past year for assessments. This natural history study, led by **Dr. Kevin Flanigan**, Center for Gene Therapy, and co-investigator **Dr. Kim McBride**, Center for Human and Molecular Genetics, was a crucial step towards bringing the gene therapy research of **Drs. Haiyan Fu** and **Douglas McCarty**, Center for Gene Therapy, to this point. This study, which was supported in part by The Sanfilippo Children’s Research Foundation (SCRF), was designed to evaluate potential outcome measures for therapeutic trials, and to assess how these measures change in individual patients over time. Preliminary results have been presented at meetings of the American College of Medical Genetics in Salt Lake City in March 2015 and the American Society of Human Genetics in October

2015. The NCH team is currently preparing a manuscript for publication that will present the results to the MPS worldwide research community, and we will present results of the study at the WORLD symposium in San Diego in March 2016.

- Critical data from this study was also included in an Investigational New Drug (IND) application for this novel, non-invasive gene therapy approach that involves delivery of the corrected gene with a simple intravenous injection. This data was submitted to the FDA recently, requesting approval to perform a clinical trial of gene transfer for MPS III B. While the IND application is still under review and the team is still awaiting the formal response from the FDA, they are proceeding with the preparation of an IND submission for the MPS III A clinical trial as well. Both gene transfer trials await completion of production and final release of the necessary viral vector, which is also being funded in part by the SCRF. We anticipate both clinical trials starting this year.

Other SCRF Research Initiatives

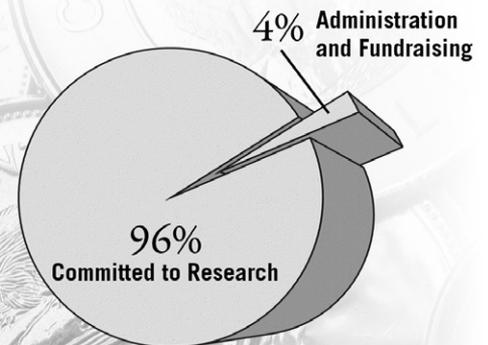
The SCRF continues to support research initiatives at Toronto’s Hospital for Sick Children as well as the University of Victoria in Victoria, BC with **Dr. Francis Choy**. We will share updates on their progress in our next newsletter.

LIFE WITH ELISA PHOTOS Clockwise from left: Smiles from Elisa at home; Elisa at Park Lane School graduation with both sets of grandparents; Elisa relaxing with PSW Dion; Elisa going glamorous in the pontoon boat at the cottage; Elisa with volunteers at Emily’s House Halloween party

Many ways to give to the SCRF

If you’d like to help the SCRF but don’t have much money or time to spare, consider these ideas. They’re easy on your schedule and pocketbook!

- As the year draws to a close, consider reducing your 2015 taxes by donating stocks to the SCRF. By donating securities instead of cash, you can support our cause while reducing your capital gains taxes. Please contact us for more details.
- To mark birthdays, anniversaries or Christmas, give the gift of a donation to the SCRF in lieu of a gift – we’ll send a card to the recipient announcing your thoughtfulness.
- If your company has a matching gift program, they might be willing to double your support of the SCRF.
- Donate to the SCRF through the United Way program at your workplace. Just designate your contribution to the SCRF through the section “Others.” Thanks to those who have already contributed this way—your support has been significant. Our Foundation number for reference is #87391 3628.



Thank you for your generous and ongoing support that enables these projects to happen. TODAY’S research will lead to TOMORROW’S treatments!

We are proud that we are still able to report that 96% of every dollar donated to the SCRF goes directly to research!



Our Foundation's Fundraisers

Sanfilippo Gala 2015 Raises a Revolutionary \$260,000!! Groovy!

So, it was like a hip happening, man. Almost 700 people joined our revolution and helped us raise \$260,000 to find a treatment for Sanfilippo. Check it out – that is the most we ever raised in one night! An anonymous angel donor cat announced he would match our table donations up to \$20,000 so, like, we raised \$10,000 from guys and chicks who just gave from the heart. That's a lot of bread!

Soul sister **Emcee Anne Marie Mediwake** and lots of other righteous cats wore threads in a 60s vibe. **Herbie Kuhn** couldn't make the scene because he was announcing for the Raptors so **Jay Mandarino** filled in at the last minute. He was hip to the crowd and helped us score \$35,000! Thanks, man!



Funkadelic neon flower power was everywhere and peepers were popping! Rockin' tunes. Magic buses. Love, peace and community consciousness. Dudes and dudettes won an iPad and other prizes in the



Yeah Baby! Raffle, and everyone thought the Woodstock Wine Raffle was a gas. The Mad Men Bar poured primo cocktails and **Randall and Elisabeth Linton** channelled Don Draper and January Jones.

The auction blew everyone's mind. There were hundreds of groovy items donated by our sponsors and supporters. Right on, guys! Big thanks to our **new Platinum Sponsor, Aerloc; Gold Sponsors R.S.Kane, Mattel and TD Canada Trust; and our Silver Sponsors Interior Care, Perlane Construction, Verstraete Travel and Cruises, Salivan Landscaping, Higgins Investment Group, Perimeter Fence and Deck, The Bill Carroll Show on AM680 Talk Radio, and Lembit and Karen Janes.** They were far out!



And our 50+ volunteers were the most. Truly outtasight. Love on you all for getting down with us and staying chill when the heat was on. **Sarah Double Miandro** rocks for keeping everything copacetic. A big, boss thank-you to all the beautiful people who helped out to make this shindig shine.

So here is the skinny on this year's jam. Check out the back page and insert for something a little different. Save the date: Wednesday, April 6th. And save your dollars to support us again cuz the kids are counting on us all. It's gonna be totally fab and we'll see you then!



Love, peace and thanks!

Counter-Clockwise from top left: Elisa and Liz Barrington; the love bus on stage; Dr. Tim Miller and Dr. Kevin Flanigan with Elisabeth and Randall; Cindy and Paul Mosey getting their groove on

Honourary Donations

Many thanks for the generous donations made to honour:

- Madeline and Brian Hayes' 55th anniversary
- Sandra Hayes' 60th birthday
- Doreen Kelly
- Elisa Linton's 21th birthday and graduation from school
- Norma and Len Linton's 60th Anniversary
- Randall and Elisabeth Linton's 30th anniversary
- Hilda Lurie's 85th birthday
- Norman and Muriel Macleod's 55th anniversary
- Paul and Jean Macleod's 25th anniversary
- Maureen McCullough's birthday

- Sarah Double Miandro's 50th birthday
- Lynelle Phillips and Jim Clarke's wedding
- Paul and Ruth Pitt's Anniversary
- Quen and Si Quach
- Jack and Shane Reger's birthdays
- Lorna Roberts
- John Saunders
- Anne and Doug Stephen's 45th anniversary
- Ava Stewart and Riley Terro's lemonade stand



Our Caring Community

We are so grateful to all those who want to do something to help us find a cure or treatment for Sanfilippo children. We have an amazing, compassionate community. Every dollar raised is important. Much of the money donated to the Foundation comes from individual donations and from fundraising efforts organized by others.

Our heartfelt thanks to these individuals and organizations for their fundraising efforts:

SCRF Annual Garage Sale

Thank you to the dozens of families who contributed to the SCRF's annual garage sale at the home of the Lintons last May. Once again, crowds from across the city arrived by the hundreds, taking with them used treasures, while leaving over **\$4,000** for the SCRF. Thank you to the many wonderful volunteers who arrived at 7:00 am and didn't leave until clean-up late in the afternoon.

Reduce, reuse and recycle. Please save your unwanted items and we'll gladly receive them in this spring for our annual garage sale on **Saturday, May 7th, 2016.**

The Buzz on Strides for Sophie

On a beautiful sunny June morning last June, approximately 50 walkers and runners gathered in Stratford, Ontario again for a fundraising event that raised nearly **\$20,000** for Sanfilippo research. Most participants were dressed in bumble-bee splendour that raised both awareness and funds. The bumble-bee theme was inspired by the the flight of a bumble bee, that while scientifically impossible to achieve, the bee still flies. And just like the humble bee, not even a cure for Sanfilippo is impossible.



This was the second event held in Stratford where the **Hamza family** live with their daughters **Grace** and **Sophie**, their seven-year-old with Sanfilippo and the inspiration behind this fun event that has brought family, neighbours, friends and supporters together in the race for a cure.

The Hamza family began fundraising for the SCRF only months after receiving the

difficult news of Sophie's diagnosis in 2010. Four short years later, the Hamzas and their community continue to contribute significantly to research by raising funds at various events. Thank you to their amazing community of supporters, with a special thanks to **Dana Robinson** for her unique initiative of organizing her "Excel-ing for Sophie" event at Excel Fitness this year, raising **\$535** through donations at her Wednesday Fitness classes. And to everyone who made donations in memory of **Dr. Milan Hamza**, Sophie's grandfather who passed away last year, a heartfelt thanks to the hope given to Sanfilippo families in his memory.

Getting Tough on Sanfilippo....and Dirty!

Last August, an amazing group of 14 Sanfilippo Warriors participated in a unique and challenging fundraiser – the 2015 Tough Mudder Challenge! It was hard, hot and dirty, and it pushed everyone to their limits. The



Left to right, back row: Cameron Mason, Jessica Linton, Randall Linton, Dr. Tim Miller, Connor Linton, John Neretlis, Elisabeth Linton. **Front row:** Annika Ferwerda, Sarah Pechmann, Brittany Linton, Heather MacGregor, Matthew Linton. **Not Present:** Leslie Carter, John Skain.

team made it together through a physically and mentally challenging 18 km Survivor-style obstacle course, overcoming the challenges of 22 obstacles in the mud. It was all about teamwork, camaraderie and pure people power, and in the end over **\$15,000** was raised for Sanfilippo research. What an amazing accomplishment we achieved together! Thank you to all our participants and sponsors.

Abeona Hits Times Square in Support of Sanfilippo!

Abeona Therapeutics was created in 2013 through a close collaboration with a dozen Sanfilippo foundations to progress the promising medical work of **Drs. Douglas McCarty** and **Haiyan Fu** from Nationwide Children's Hospital in Columbus, Ohio. The collective efforts raised nearly \$5 million dollars by the end of 2014 to start manufacturing a drug and taking steps towards a clinical trial. In May of last year, Abeona Therapeutics was acquired by PlasmaTech Biopharmaceuticals, a publicly-traded organization. This newly-formed entity chose to go forward using the name Abeona Therapeutics and its CEO, **Dr. Tim Miller**, had the opportunity to ring the closing bell at the NASDAQ last June to conclude the trading day, bringing the beautiful faces of Sanfilippo children and their families to the screen at Times Square, New York City.



Clockwise from left: The Hamza family; The SCRF Tough Mudder team; Abeona display on the NASDAQ Times Square video screen

The Sixteenth Annual Fore Elisa Golf Fun-raiser Scores Big...Again!

For the 16th straight year, the SCRF hosted yet another successful Fore Elisa Golf Tournament at a Toronto landmark: The Don Valley Golf Course. This year's tourney was record-breaking, raising over **\$19,000**



for Sanfilippo research. And, once again, after a great day of golfing, the 32 participants were treated to a wonderful dinner at **Mezza Notte Trattoria** on Yonge Street. **Robert and Gus Savibarota** always provide the most enjoyable cuisine in an inviting atmosphere, served with class and attention to detail.

In addition to Mezza Notte, a special note of appreciation is due to some very supportive

sponsors who significantly helped to make the day a success: **Brian Shaw at BDS Consultants, Casio Canada, Macgregors Meat & Seafood, Pacific Links and Salivan Landscaping.**

We look forward to hosting this year's Fore Elisa Golf Fun-raiser on **Monday, September 12th, 2016** and we would love to have you join us. Thanks to all who participated, sponsored the event and supported our golfers!

Exquisite Music in Special Memory of a Beloved Young Boy – Warren Dendekker

Last October, the **Dendekker family** and their community built on the success of two previous musical fundraisers. A memorable evening of classical music, spearheaded by renowned classical pianist **Valerie Tyron** and organist **Chris Teeuwsen**, took place in Hamilton. A stellar line-up of musicians donated their time to showcase their musical talents for over 600 guests, raising funds in support of the SCRF through donations.



Warren Dendekker

In addition to Ms. Tyron and Mr. Teeuwsen, performers included **Janet Obermeyer, soprano; Jenny Enns-Modolo, contralto; Bijan Sepanji, violin; Olga Tcherniak, piano; Emma Rush, guitar; Catherine Robertson, piano; Stephen Piette, clarinet; Daniel Warren, trumpet;** and the **Tactus Vocal Ensemble.**



The unforgettable annual evening was inspired by **Warren and Katherine Dendekker**, siblings who were diagnosed almost three years ago with Sanfilippo syndrome. However,

this year's concert was in performed in memory of Warren, who tragically passed away unexpectedly two weeks before the event. The organizers bravely forged on to do an amazing job despite their unimaginable grief. We thank all who participated, attended, and donated over **\$70,000**, paying tribute to Warren's precious life.

Cultura

Once again, the SCRF was grateful to receive funds from North York's Cultura event last summer. We thank Councillor **John Filion** and local residents who together organized this exciting weekly event at Mel Lastman Square. This

entertaining celebration of food and the arts featured street food, musical performances, street buskers, artists and a free outdoor movie and raised almost **\$800** for the SCRF. Plus, **Eleanor Walters** set



up a table at Cultura to sell many of her hand-crocheted roses, adding to the thousands of dollars she's raised for the Foundation. We thank Eleanor and our city's residents for a fun evening and generous donations!

Willowdale Middle School Comes Through for Us Again!

Willowdale Middle School is an amazing, long-standing community supporter of the SCRF. Jessica and Connor Linton attended grades six through eight there and the school has supported Elisa ever since! Under the direction of the Student Council, WMS worked diligently on unique events this past year, raising another **\$885** to support the SCRF. This school is filled with creativity, big hearts and love! We can't thank you enough for your continued help and dedication to Elisa and Sanfilippo children.

Counter-Clockwise from top left: Golfers dining at Mezza Notte Trattoria; Warren Dendekker; Elisa with Eleanor Walters and friends; Musicians at the Dendekker music event



Upcoming Fundraisers – Save the Date

See our website for more details or contact Elisabeth at www.alifeforelisa.org or 416-223-1911.



Corporate Contributors

Many thanks to these organizations large and small for making financial contributions to help our fight against Sanfilippo:

101 Temptations
 Alliance Marketing
 Ampere Electric
 Applied Systems Technologies Inc.
 Baker Animal Hospital
 Bee Clean
 Begley Door Service
 Beverly Central School Parent Council
 Bondfield Construction Company Ltd.
 Brouwers Painting Ltd.
 Canadian Foundation for Economic Education
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 Cleansales Inc.
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 Constant Controls Ltd.
 DeSousa Painters
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 Nome Izakaya
 Ontario Home Health
 Ontario Securities Commission
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Interior Care Ltd.
 Lazy Poet Creative
 Pettit Integrated
 Wizbot Inc.

Charmed by Chocolate & Chantal



Chantal Kreviazuk

17th Annual Gala Dinner and Auction Wednesday, April 6th, 2016

Please join us for an evening devoted to two of the best things in life: children and chocolate! Be part of this very special milestone event for the SCRF featuring a private concert by Chantal Kreviazuk, a gracious gift from one of Canada's most beloved vocalists. Sample chocolate from exotic regions around the world. Shop for delectable chocolate treats. Enjoy a gourmet dinner with friends and hunt for bargains in our elegant auction!

6pm Reception, 7pm Dinner

The Paramount Convention Centre
222 Rowntree Dairy Rd., Woodbridge
(Hwy 7 & 400 area)
Tel: 905-326-3000
Website: www.peterandpauls.com

Tickets \$195 each
\$210 after March 24, 2016
((\$90 tax receipt))

See insert page for ticket information
and sponsorship opportunities.

Chantal, chocolate, and cheer!