

Sanfilippo
Children's Foundation



SANFILIPPO CHILDREN'S FOUNDATION - FUNDING RESEARCH, FINDING GEMS

2015 YEAR IN REVIEW



Our vision: Fund medical research so a cure can be found in time for children battling Sanfilippo today and those born with it tomorrow

Welcome

As we conclude our second year of operation we are extremely encouraged in this time of change, expansion, philanthropic gestures and pioneering scientific progress. It's been an extraordinary year for the Sanfilippo Children's Foundation and we couldn't have done it without the help of our exceptional community of supporters. We thought we would show you some of what we have been up to.

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This Year in Review document has been produced by professionals who have discounted and/or donated their time and services. We would like to thank designer Alex Moller and Lindfield Print and Copy Centre.

HIGHLIGHTS WE'VE ACHIEVED TOGETHER

- Partnership sealed with biotech, Abeona Therapeutics Inc (NASDAQ: ABEO) to deliver clinical gene therapy trials
- Invested the first USD\$200,000 with a total of USD\$1m committed (est. AUD\$1.3m) towards first clinical research program
- Australian clinical team engaged to establish trial site in Australia
- Over 5,000 Facebook followers
- Facebook total reach peaks at 40,000 in December 2014
- Over 6,000 supporters on our database
- Foundation ambassador and Aussie singers band together to launch dedicated song
- Extensive media coverage including 5 TV segments, 42 print articles and 12 radio interviews
- 5 major corporations donate over \$100,000 in gift matching alone
- Dedicated office space secured for the Foundation
- 40 community events raising over \$100,000
- 100 peer-to-peer fundraising campaigns raising in excess of \$90,000
- New fully integrated, interactive and supporter friendly website launched
- Family-led “Hope for” campaigns combined raised more than \$140,000
- Welcomed 2 new ambassadors
- Opened an online store and launched a line of SCF merchandise.

THANK YOU TO OUR GEM FUNDRAISERS

MAJOR CONTRIBUTORS



Sponsors and Events that helped raise funds in 2015

SANFILIPPO 1000 – BIG FEET FOR LITTLE FEET

Proudly sponsored by Westpac, a highly motivated team of 7 runners relayed from Melbourne to Sydney and brought home a staggering \$166,000 for the Sanfilippo Children's Foundation, raising immeasurable awareness across both regional and metropolitan Victoria and New South Wales whilst exceeding all fundraising targets.



KCA WALKATHON

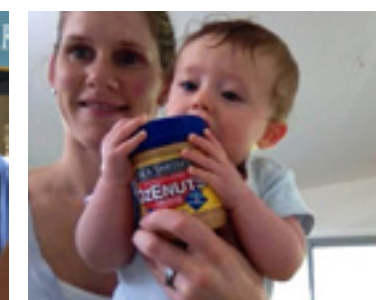
In 2014 Kimberly-Clark Australia (KCA) staff participated in a company and nation-wide KCA Walkathon. So successful was the event and relationship with the SCF, that the KCA team hit the pavement again in 2015 to raise additional funds.

For two years running, KCA staff have participated in a series of fundraising events and activities. Thank you to KCA employee Abby Phillips, who upon sharing the mission of the Foundation, has continued to rally the troops. With KCA matching, this year the event raised \$82,500 towards our research programs, bringing the combined total to \$162,500 over the last 2 years.



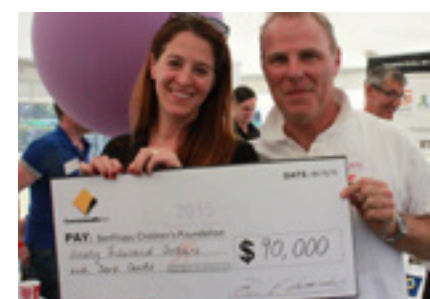
DICK SMITH FOODS

The Sanfilippo Children's Foundation won \$50,000 from the Dick Smith Foods Foundation in its \$1 Million Charity Breakthrough campaign. It is a heart-warming story of people power with SCF supporters taking more 'selfies' and casting more votes than over 450 other eligible Australian charities over a six-month period. The SCF took out top gong in an initiative philanthropist and business man Dick Smith has called the "ultimate crowdfunding".



A W EDWARDS

The Sanfilippo Children's Foundation was nominated by David Oliver as the recipient of the A W Edwards Charity Shield Day, a barefoot bowls event held in North Sydney in October. Thanks to the extraordinary support of A W Edwards, event sponsors and suppliers, the SCF was presented with a cheque for \$90,000.



HIDDEN GEMS GOLF DAY

Due to the success of the inaugural event in 2014, this year golfers supported the SCF at the Hidden Gems Golf Day at Cromer in Sydney. Golfers embraced the day with an Ambrose style event whilst partaking in wine tasting and competitions on course before retiring to the club for lunch and entertainment by ex-cricketer Kerry O'Keefe. With thanks to our volunteer organiser, Stephanie Donohoe, the event raised \$30,000.





“HOPE FOR” CAMPAIGNS

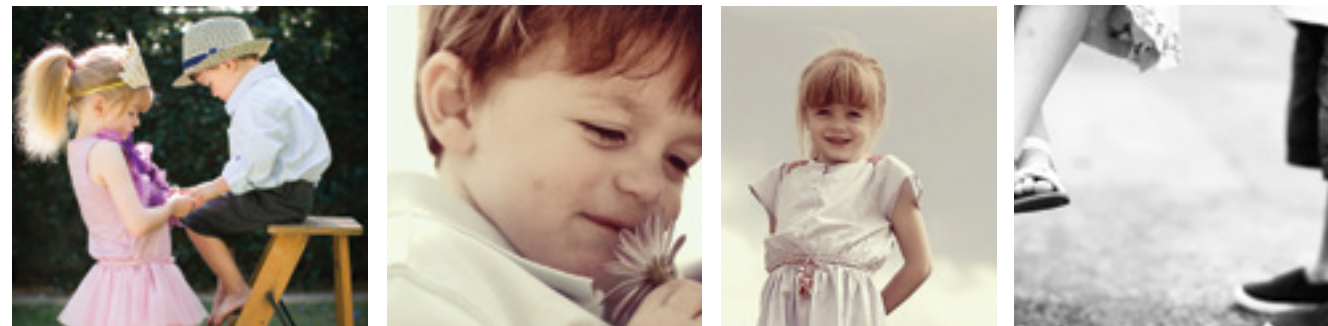
Successful **family-led fundraising campaigns** humanise the face of **Sanfilippo Syndrome**

HOPE FOR ISLA & JUDE

In December 2014, the Sanfilippo Children's Foundation made national and international headlines with the launch of our Hope for Isla and Jude campaign and the release of the charity single **This Woman's Work**. The cover of the Kate Bush song was produced by Foundation ambassador, Sébastien Izambard of Il Divo pop opera fame, together with Sony Music Australia.

Sébastien visited Sydney to produce the single and united a stellar list of Australian artists including Darren Hayes, Natalie Bassingthwaight, Ella Hooper, Diesel, David Campbell, Alex Lloyd, Christine Anu, Luke Steele (Empire of the Sun), Katy Steele (Little Birdy), Casey Donovan, Damien Leith, Nathaniel, Marisa, and the Australian Children's Choir.

The charity anthem was accompanied by a film clip produced by film-maker, Jason van Genderen from Treehouse Creative. The campaign generated over \$50,000 in donations and associated fundraising activity and the film clip has been viewed over 58,000 times. The campaign generated significant media coverage including 4 TV segments (**Sky News**, Channel 9 **Mornings**, Channel 9 **Today Show** and Channel 7 **Morning Show**), 10 print articles and 6 radio interviews.



HOPE FOR SKYE

In February 2015, we introduced Skye Robson with the launch of the Hope for Skye campaign with Skye's family joining the fight to raise funds. Their first #BlueSkyforSkye social media campaign on Rare Disease Day (28 Feb) was a great success with good traction, and generated a series of grassroots events in Ballarat, Victoria, and also in Thailand, where Skye also has family. The Hope for Skye campaign has been profiled in four articles in the **Ballarat Courier** and twice in **The Miner Newspaper**. This year, the Hope for Skye campaign has raised almost \$40,000.



THANK YOU TO OUR GEM FUNDRAISERS

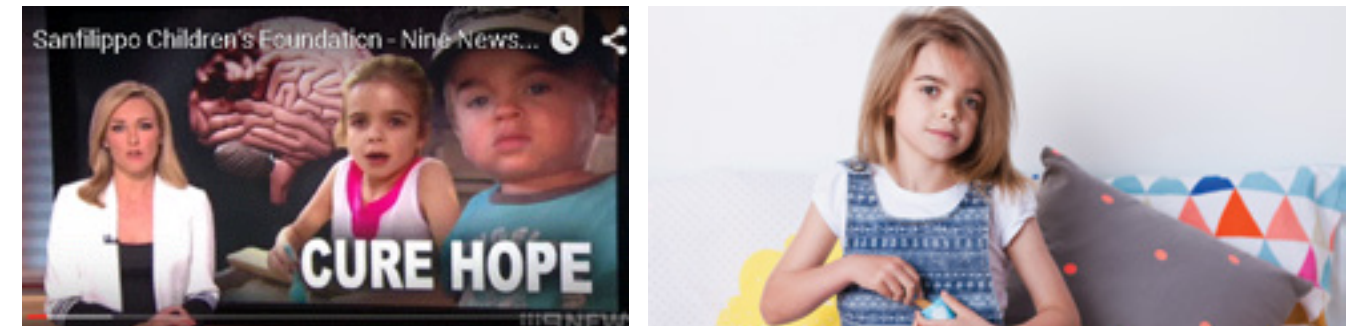
HOPE FOR KYUSS

March saw the launch of the Hope for Kyuss campaign, as the Harris and Johnson families rallied significant community support in regional Victoria. Along with two other children with Sanfilippo (Meckenzie Oelofsen and Skye Robson), Kyuss was featured in a Channel 9 news story about the plight of three families battling this devastating disease. The launch of the Hope for Kyuss campaign was also featured in the family's local newspaper the **Yarra Valley Leader** and successful fundraising events have since included a 'Sex Outta the City' fundraiser, the 'Ky Cup' and the 'Ky-T' high tea fundraisers. Hope for Kyuss has raised over \$93,000 this year.



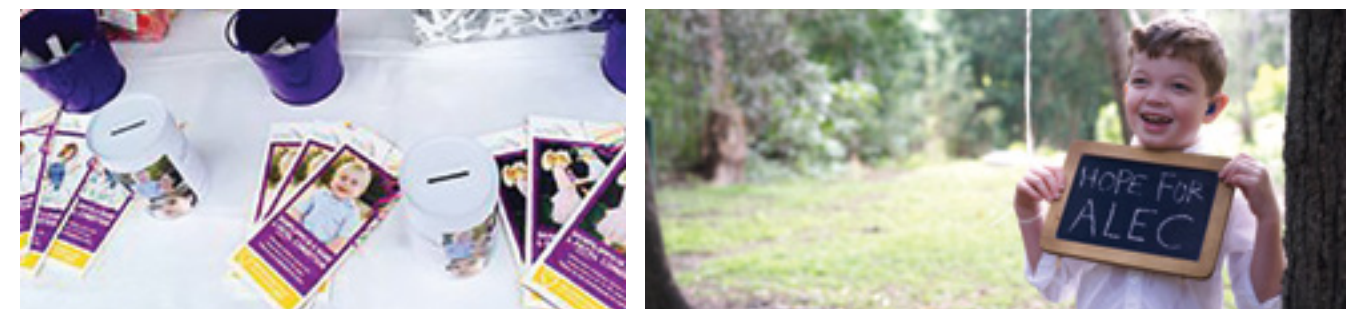
HOPE FOR MECKENZIE

Meckenzie recently turned 8 and to launch her Hope for campaign, Meckenzie's family announced she has 8 birthday wishes - 1. Life, 2. Hope, 3. Treatment, 4. Help, 5. Cure, 6. Awareness, 7. Donate and to 8. Grow Old! Meckenzie has had three articles appear in her local paper, **The Moorabbin Leader**, and also appeared in the Channel 9 news story filed by journalist and Foundation ambassador Sonia Marinelli.



HOPE FOR ALEC

Alec Morrice has Sanfilippo Type C, a rarer form of Sanfilippo affecting only 1 in every 1.5 million live births. Alec was diagnosed at the beginning of 2015. On the front foot in the fight to raise awareness about this devastating disease, Alec's family has been quick to get on-board and all money raised by the Hope for Alec campaign will be put towards advancing research into Sanfilippo Type C. So far this year, over \$11,000 has been raised by Alec's supporters.



OUR GEM FUNDRAISERS

In the spirit of “people-powered medicine”, grassroots community support for the Sanfilippo cause continues to grow and is paramount in realising our goals.

In the last year, the SCF supported approximately 40 community events, raising over \$100,000. Grassroot events have been held across the country by everyday people who we thank for their extraordinary support.

We've had runners, walkers, bakers, riders, dieters, singers, head and beard shavers and even bag-pipers. There have been many events including: trivia, discos, Bunnings BBQs, Scottish dancing, endorsed golf days, band and karaoke nights, car boot sales, book fairs, car park collections, online auctions, gala dinners, street buskers, Christmas parties, cake stalls and group fun runs and walks.

Peer-to-peer fundraising continues to grow with approximately 50 individuals fundraising for the SCF with a personalised fundraising page on the Foundation website. Fundraisers secured sponsorship via physical challenges including fun runs, marathons, obstacle courses and bike rides as well as challenges and dares such as giving up chocolate, wearing a wig for a month, and dying hair purple.

BUNNING'S CAKE STALLS



KY-T (HIGH TEA) FUNDRAISER



MUAY THAI BOXING EVENT



PUB 2 PUB FUN RUN
two years running



DANCE FOR HOPE
two years running



VIOLET DIAMOND BALL,
MELBOURNE



SUN RUN
two years running



SYDNEY TRIVIA
two years running



OPEN GARDEN FUNDRAISER



SEX OUTTA THE CITY
FUNDRAISER

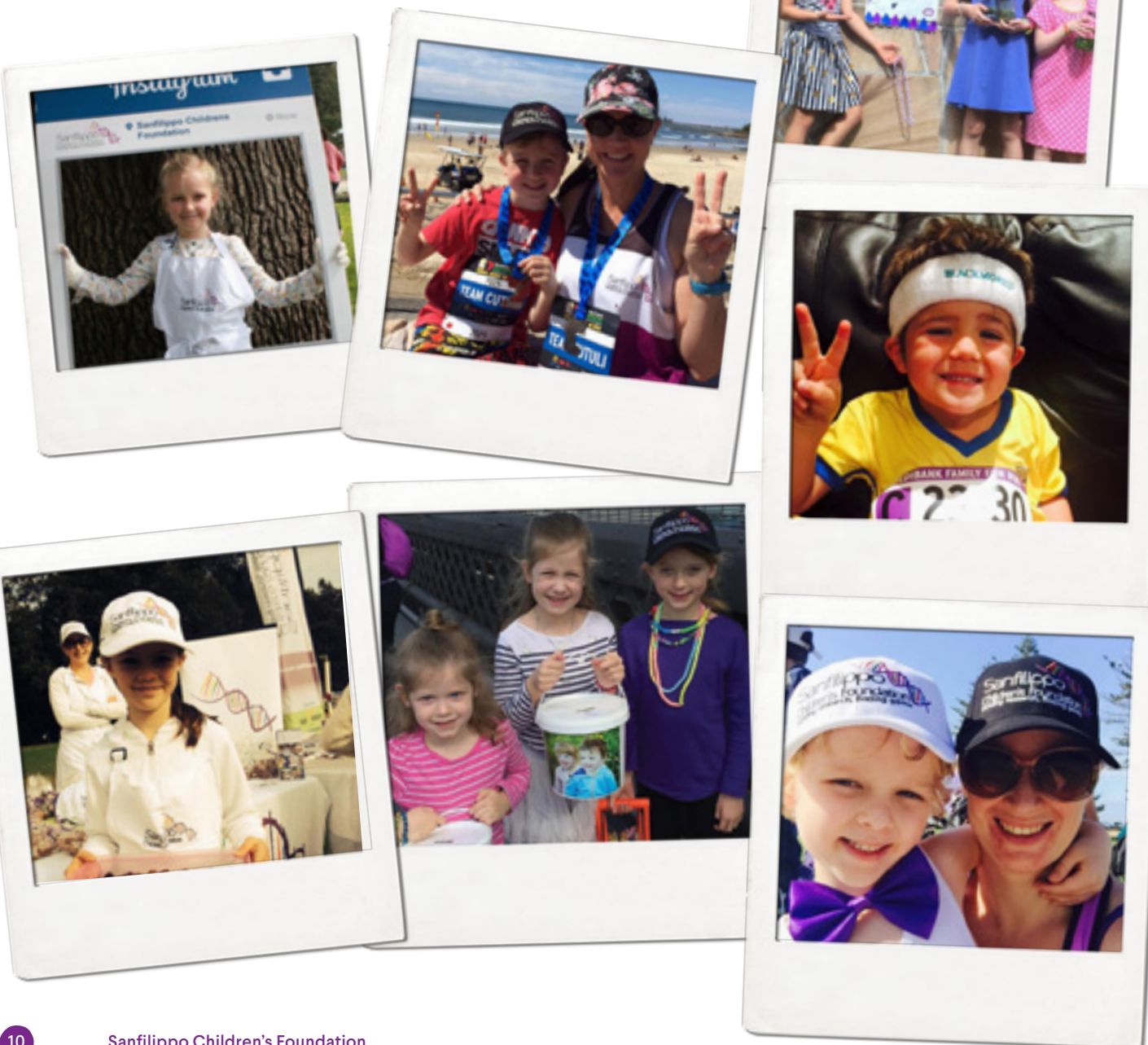


THANK YOU TO OUR GEM FUNDRAISERS

PINT-SIZED PHILANTHROPISTS



Children are often the **Sanfilippo Children's Foundation's** most avid supporters, exercising philanthropy and a **sense of community well ahead of their years**. Fundraisers led by children have included kerbside cake stalls, birthday celebrations in lieu of gifts, busking, making Sydney Harbour Bridge-length loom bands and competing in fun runs.



GEM QUOTES

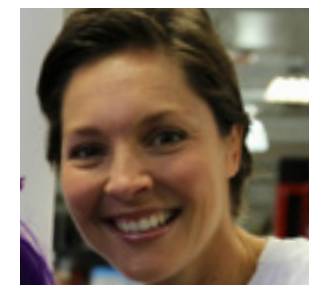
A **few words** from some of our **Gem fundraisers** about their involvement in the Sanfilippo Children's Foundation.



John Burgman
Westpac employee
and instigator of the
Sanfilippo 1000

"I'm linked to SCF via Allan Donnell from playing soccer for Manly Vale. It was absolutely heart-breaking to see a mate and his family deal with the Sanfilippo diagnosis and impossible to comprehend there was so little hope initially offered.

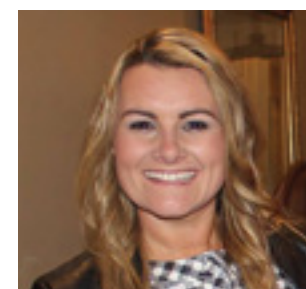
Despite the adversity, it's been inspirational to see the courage, determination and passion from Meg and Al (and the broader SCF community) on not only their quest for a cure but their selfless support of other Sanfilippo families."



Belinda Yates
Bunnings cake stall
organiser & regular
volunteer

"Our babies (Isla and Xavier) were a month old when Megan and I met at mother's group. When the Foundation was created I could see the drive Megan had. Listening to her talk about this, well... it was hard not to be inspired.

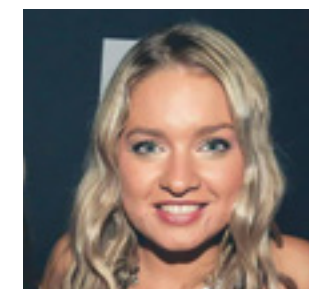
My experience with breast cancer had made me realise how precious life is. I feel very grateful there is a treatment for breast cancer. It really made me want the same for Isla and Jude: a treatment, medicine... and an opportunity for life!"



Peta Goodlet
Event organiser
and Hope for Kyuss
committee member

"I was inspired to help after attending the Violet Diamond Ball. I already knew Kyuss and Nikki's story but left the Ball motivated having learnt more about the disease and the urgent need for a treatment.

With no government funding or the resources of a large charity, this was a cause that needed a little extra help. I couldn't sit on my hands. A group of girls formed the 'Hope for Kyuss' committee. We all bring something to the table and have a mix of skills, contacts and access to various services. There are lots of deserving charities and causes out there but this one is about people-power and that's inspiring."



Rachel Hansen
Paediatric
Occupational
Therapist at Kids OT

"Over the past 12 months, Kids OT has participated in fun runs, painted faces, sold lucky dips and Christmas decorations, got involved in the 'Loom for Sanfilippo' initiative and taken as many Dick Smith selfies as possible.

Not only do our individual team members gain joy from participating, but as a workplace, this has had a hugely positive influence on team morale and relationships. It is something we feel very proud of as a practice."



OUR MEDIA COVERAGE

TV, newspaper, magazine
& radio coverage in 2015

Media coverage of rare disease remains a challenge but the Sanfilippo Children's Foundation has gained significant exposure and continues to make headlines, allowing us to effectively educate the Australian community about our cause. Coverage has largely focused on our innovative fundraising events and both corporate and community activities.

The SCF was profiled across all mainstream media channels this year including 35 print newspaper articles, 7 magazine and e-zine articles, 5 TV segments, and 12 radio interviews. The majority of coverage was generated by the release of the single **This Woman's Work** and the Sanfilippo 1000 event which captured media interest in regional NSW and Victoria.

Other highlights include a radio interview with Alan Jones, articles in two broadsheet dailies **The Sydney Morning Herald** and **The Australian**, as well segments on Channel 9's **Mornings** and **Today Show**, Chanel 9 News Melbourne and Channel 7's **Morning Show**.

Thanks to Ooh! Media, SCF has enjoyed pro-bono digital advertising at select shopping centres, cafés and restaurants, for two years in a row. This generated a 'SPOTTO' campaign in which our supporters photographed our advertisement in public spaces and shared across social media.

TELEVISION MEDIA COVERAGE



PRESS COVERAGE



RADIO COVERAGE



SOCIAL MEDIA

Our online **blogging community** and **social media gems**

Social media is an increasing means of promoting awareness and raising funds for the SCF. Our presence on Facebook in particular has enjoyed marked growth for an organisation our size. Engagement levels sit well above the industry average at 11% and this year we exceeded 5,200 likes. Total reach is on average 10K people but peaked at 40K people during our song launch and the associated Hope for Isla and Jude campaign.

The Dick Smith Breakthrough for Charity initiative was an opportunity to really engage with our online community, as our loyal supporters took action and embraced the collaborative spirit of the social media campaign, taking and submitting more than 2,500 selfies.

The online blogging community has also got behind the Sanfilippo cause, allowing the SCF to utilise this powerful means of communication. An online auction of designer children's clothing hosted by IG (Instagram) fashion blogger Carla Rodan from @Stylingthetribes was a hit, helping us to reach a larger and wider international audience.



MEDIA COVERAGE

Thank you to the following bloggers, online journalists and fashion e-retailers for their support.

@Retro Mummy	Sheree (@captainandthegypsykid)
@ShowandTell	Carla (@stylingthetribes)
@Wheels of Laughter	Andrea Rembeck (@tutudumonde)
@BellaMumma	@sarinassurianojewels
@Waystoamaze	@stuffmumslike
@Kendrascatchycorner	@petitparisians
@kidsfashionblogger	@bloggingmika
Keria & Karianne (@freeandwildchild)	@abeachcottage
Pete & Harlow (@thedaddyfashionstylist)	@champagnecartel
Jodie (@bondi_mumma)	Kristy Withers (@incyinteriors)
Claire (@clairecollected)	@luluandmilly
Miss Maya Photography (@pics_missmaya)	@dimplesandwhispers
Renata (@curatedby)	@rourkeandhenry
Marcia Leone (@notsomumsy)	@havealaughonme
Sandy Zeccardi (@littlezkid)	@uneekkidz
Kerri Sackville	@jaime_king
Rebecca Sparrow	@littlegatherer
Sara Knowles (@theinstashopper)	@saplingchild
	@theinteriorsaddict
	@christieblizzard



SPECIAL THANKS

The Sanfilippo Children's Foundation would like to especially acknowledge some exceptional gems

GRANT GIVERS

The Foundation has been a proud recipient of a grant from these generous foundations and organisations:

Count Foundation Hacket Foundation
Honda Foundation Ripple Foundation



PRO-BONO SUPPLIERS

These suppliers have provided much-valued ongoing time, material and resources to our cause:

Accountants	CXC	Lawyers	Clifford Chance (Jane Ann Gray)
Advertising	Ooh! Media	Photographers	Little Love Photography Miss Maya Pics Hayley Grace Photography Sam Elsom (Captain and the Gypsy Kid) Heartfelt Danielle O'Brien Art Carlma Photography
Auditors	Bentleys	Printers	AAP Print KwikKopy St Leonards Lindfield Print & Copy Centre Print Junction
Graphic Design	Katrina Commyns Jacqui Greig Noleen Lance May Leong Alex Moller Harry Morgan Kevin O'Sullivan Jacinta Weir	Public Relations	Connect PR
Event Production	Jo Bilous		
Grant Writing	Amy McCudden		
Media	Treehouse Creative		

A message from our Founder



We talk a lot about the notion of people-powered medicine and we are seeing it in practise at the Sanfilippo Children's Foundation.

It's been a year packed with activity and one in which great progress has been made. The foundations of our first project are now firmly in place. We have partnered with a US-based biotech (Abeona Therapeutics) which is running a gene therapy program and engaged an Australian clinical team to establish a trial site on Australian soil that can run this program in 2016.

For the first time kids with Sanfilippo and their families have the smallest glimmer of hope that they might face a different future than the one that they thought they were. It means Australian children will receive treatment, a shot at life. And overall science will be that one step further towards an eventual cure.

The work of our Foundation is carried out by a dedicated board, a small but passionate team and a growing group of committed volunteers, from which we have enjoyed the most extraordinary support and generosity. This year in particular we have learned that individual everyday people really can make a difference and make things happen.

Whilst we aren't there yet, thanks to the incredible support of our strategic partners, corporate donors and our extraordinary community, we will get there ...we will get a treatment for kids battling Sanfilippo today and those born with it tomorrow.

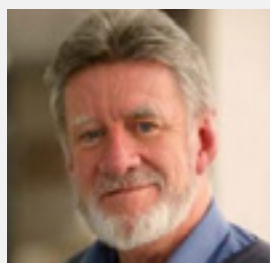
We are full of hope for the year ahead. 2016 looks as if it will be as busy and fruitful as the past year has been. We are excited by what we can achieve together!

With gratitude,

Megan Donnell
Founder of the Sanfilippo Children's Foundation

MEET THE TEAM

The Sanfilippo Children's Team
are a dedicated group of individuals



Professor John Hopwood AM FAA
SCIENTIFIC ADVISOR

Professor John Hopwood recently retired as Director of the Lysosomal Diseases Research Unit (LDRU). He was previously at the Women's and Children's Hospital, then moved to

the South Australian Health and Medical Research Institute (SAHMRI), Adelaide, Australia. The Unit is world-renowned for its research into the diagnosis, treatment and biology of lysosomal diseases including the Mucopolysaccharidoses (MPS) group of metabolic disorders.

Professor Hopwood has dedicated four decades of research into genetically inherited disorders that affect children including Sanfilippo Syndrome. A man of the people, Professor Hopwood is particularly passionate about meeting families and patients living with MPS and learning about their day-to-day challenges with the disease.



Megan Donnell
FOUNDER & DIRECTOR

Megan is responsible for providing strategic vision, leadership and development of the Foundation within the direction set by the Board of Directors. Megan is the Foundation's key liaison between the Foundation, partner organisations, funding groups, associated research and regulatory bodies and patient groups globally.



Ingrid Maack
COMMUNICATIONS & FUNDRAISING
MANAGER (PART-TIME)

Ingrid is our primary communications liaison. She is responsible for social media and external communications in line with the Foundation's mission. In addition, Ingrid manages our community fundraising events and activities.



Nicole Fasseau
OPERATIONS MANAGER (PART-TIME)

Nicole is responsible for general operations, administration, relationship management, corporate programs and broadening the Foundation's fundraising opportunities.



Erene Keriakos
BOOKKEEPER PART-TIME

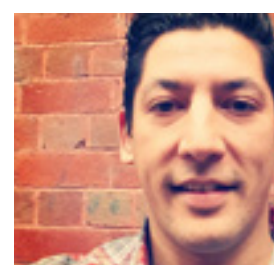
Erene works one day per week providing bookkeeping services for the Foundation.

SCF BOARD MEMBERS



Angeline Veeneman
PATIENT INFORMATION & PROGRAMS

Angeline's focus is on building our knowledge about the disease and associated medical research, and aligning our research strategy accordingly.



Daniel Madhavan
INVESTMENTS & NETWORKS

Daniel's key activities include engaging with support networks to promote foundation awareness and increasing the scale of Foundation investment.



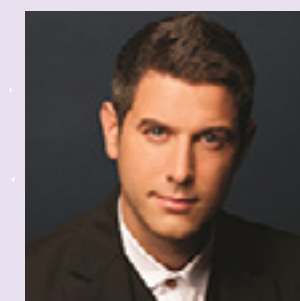
Mark Arnold
STRATEGY & GOVERNANCE

Mark is responsible for strategy and governance of the Foundation. His key activities include defining organisational strategy to achieve the mission and objectives of the foundation and establishing processes to facilitate compliance with governance and regulatory requirements.

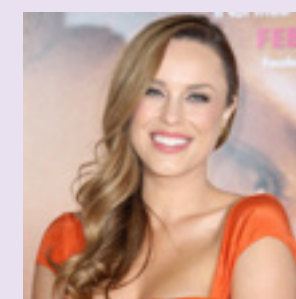
ABOUT THE TEAM



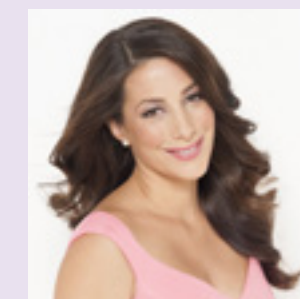
SANFILIPPO CHILDREN'S FOUNDATION AMBASSADORS



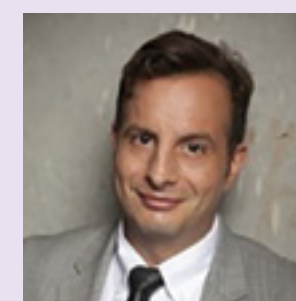
Sébastien Izambard
Singer from pop opera
group Il Divo



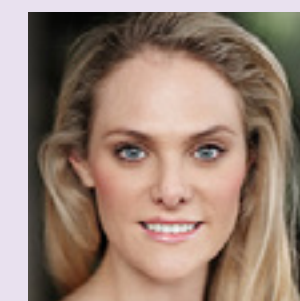
Jessica McNamee
Australian film &
TV actress



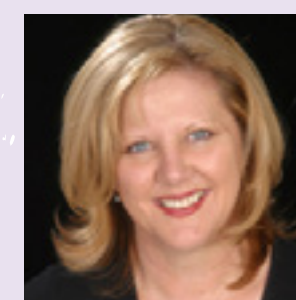
Sonia Marinelli
TV news reporter,
Channel 9



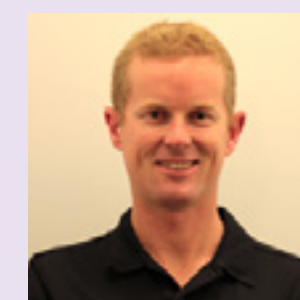
Dan Illic
Comedian & radio host



Catherine Bouchier
Soprano opera singer



Jeannette Rowe
Children's book author



Silas Moss
Sports journalist and
blogger

OUR FAMILY STORIES

Every Family has a story. These are just some of the Australian children battling Sanfilippo Syndrome.



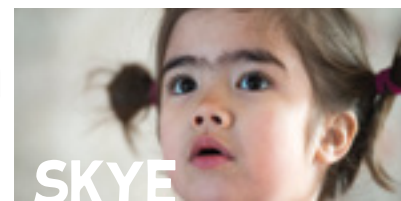
JAYDA

Jayda no longer talks or sings like she once did. Her little sister Lucy sometimes calls Jayda her "baby". Her parents like to watch old videos of Jayda to remember the way she was.



JACOB

Jacob is a gorgeous little man with a heart of gold. He is an affectionate boy, who loves his little sister Alexis. Without treatment, one day Jacob will break hearts but not in the way his grandmother expected.



SKYE

Skye likes dress-ups, AFL and playing with big brother Luke. She has a bubbly and vibrant personality and is always up to mischief. Her family fear one day she will lose her sense of adventure.



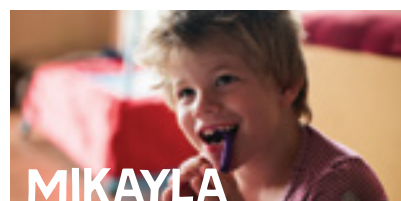
PETER

Peter is a happy little boy who likes to play with his little brother Eliya and sister Natalie. Peter touches many hearts. While he still walks, Peter no longer speaks.



KYUSS

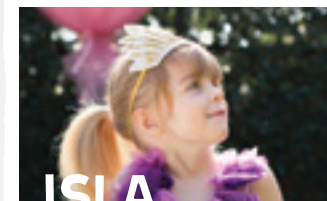
Kyuss can kick a ball, ride a bike and zoom on his scooter. He likes to ride his quad bike on his grandparents' farm. Kyuss has hearing loss but Sanfilippo will steal much more than his hearing.



MIKAYLA

Mikayla loves spending time with her big brother Jake, who sits and reads to her. She is in a wheelchair and eats through a tube. Her parents miss the sound of her voice.

WITHOUT TREATMENT
**THESE CHILDREN
WILL NOT LIVE
TO ADULTHOOD**



ISLA



JUDE

Isla and Jude are brother and sister. They adore each other. Isla loves dressing up and singing. Jude is full of boundless energy and makes friends wherever he goes. It is unimaginable one may lose not only their sibling but also their best friend.



MECKENZIE

Meckenzie is a social butterfly and is full of personality and giggles. Meckenzie's big brother Kyle treasures each and every day he has left with his little sister.



READ MORE

About the experiences
of Australian families
who are impacted by
Sanfilippo Syndrome.

Visit our website:
sanfilippo.org.au



DYLAN

We sadly say goodbye

In 2015 we lost a much-loved Australian child to Sanfilippo Syndrome. Dylan Jessop lost his battle in July. Dylan was just 12 years old but touched the hearts of everyone he encountered in his short but full life.

To honour Dylan and his spirit, the Jessop family together with the Sanfilippo Children's Foundation set up an 'In Memoriam' page, and asked for friends and family to make a donation in Dylan's memory. This initiative generated \$3,350 and donors also left messages of support for the Jessop family.

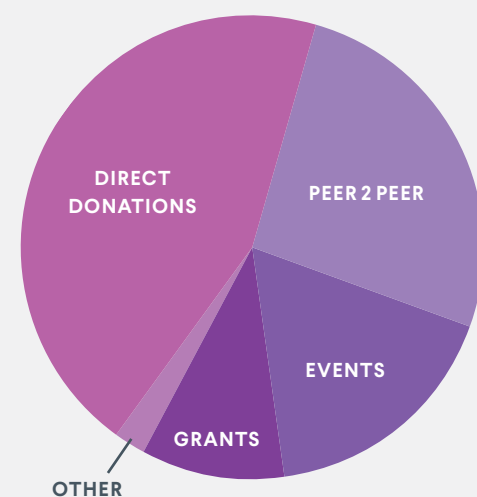
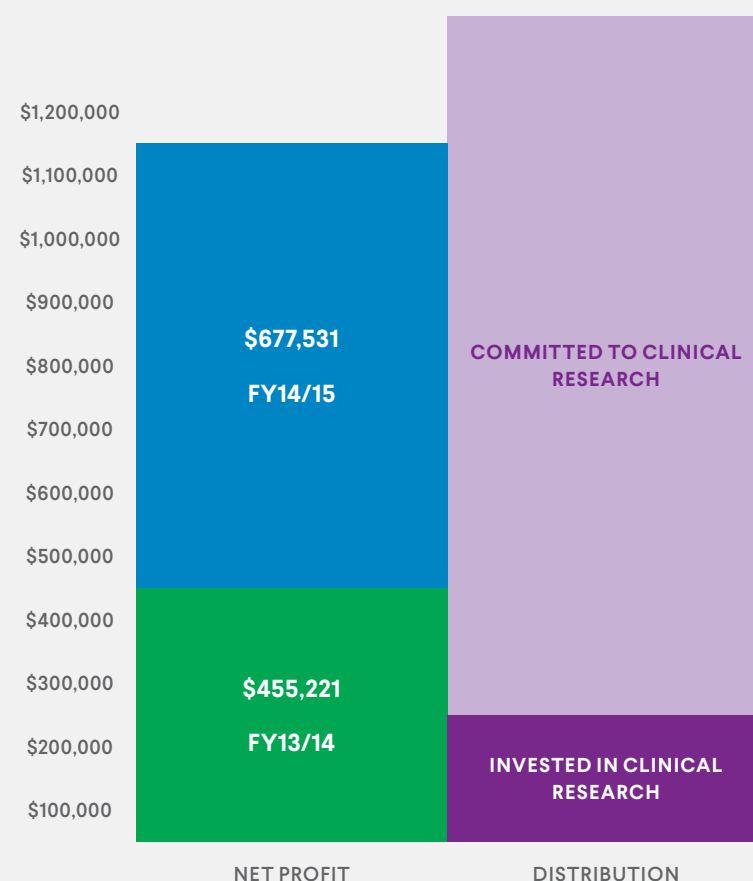
Dylan is sadly missed by his loving parents Matt and Louise and younger brothers Ethan and Ayden.

RIP Dylan Jessop... gone too soon.

THE FINANCIALS

For a full copy of our audited report, please contact us via email - info@sanfilippo.org.au or by phoning **1800 664 878**

	FY14/15	FY13/14
INCOME		
DONATIONS	\$387,996	\$371,825
FUNDRAISING ACTIVITIES	\$384,278	\$121,736
NON-GOVERNMENT GRANTS	\$87,100	\$15,000
INTEREST INCOME	\$13,970	\$2,248
MERCHANDISE	\$6,795	\$912
TOTAL INCOME	\$880,139	\$511,721
FUNDRAISING COSTS	\$106,940	\$42,312
OPERATING EXPENSES		
ADMINISTRATION	\$56,191	\$10,223
MARKETING	\$17,694	\$564
PROGRAMS	\$21,781	\$3,402
TOTAL OPERATING EXPENSES	\$95,668	\$14,189
NET PROFIT (FOR DISTRIBUTION)	\$677,531	\$455,220



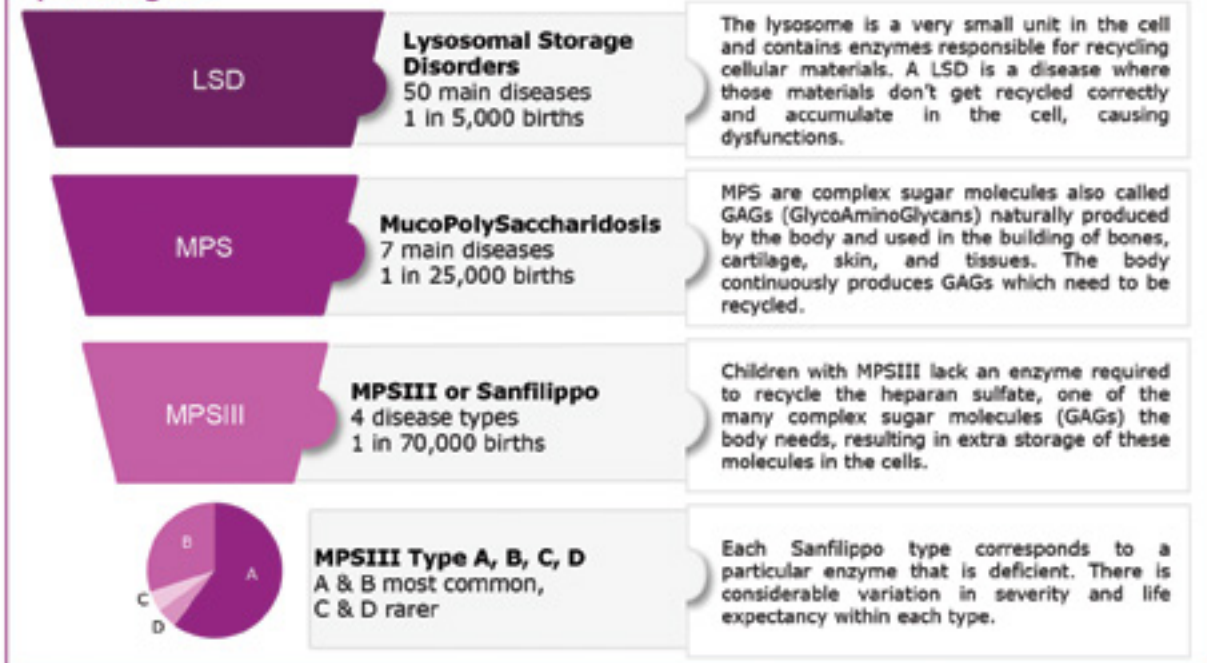
Fundraising sources (FY14/15)

THE DISEASE

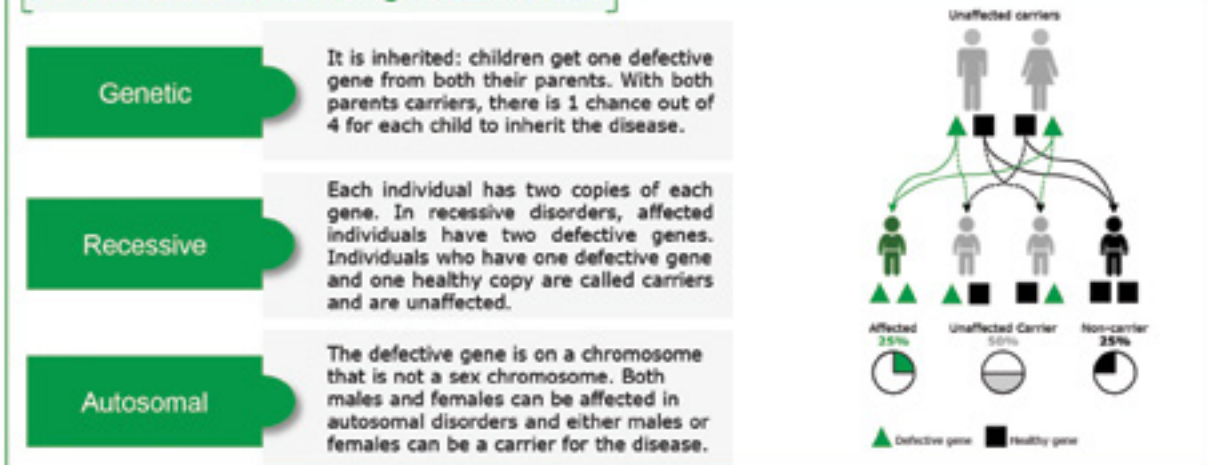
FACT SHEET

What is Sanfilippo (MPSIII)?

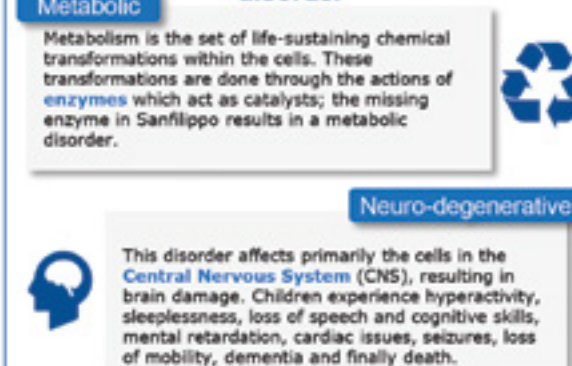
A rare, life-limiting disease that is part of a wider group of pathologies



An autosomal recessive genetic condition



A metabolic & neuro-degenerative disorder

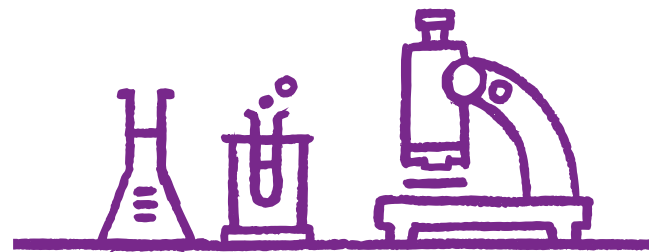


A progressive and fatal disease



The disease does not yet have a cure and only limited palliative treatment currently exist; average life expectancy is 15-20 years. However medical research has recently achieved promising break-throughs with real hopes for the future.

THE RESEARCH



The last year has been very active from a research perspective and brings renewed hope for the whole Sanfilippo community across the world. With multiple programs in early clinical trial stages, there are increasing opportunities for children with Sanfilippo to access experimental treatments that have a real possibility to change their lives and that of their families.

As a monogenic disease that affects the Central Nervous System, Sanfilippo Syndrome is a condition that it is considered as one from which scientists, researchers and biopharmaceutical groups can learn. Finding an effective treatment for Sanfilippo creates application opportunities for other more complex genetic and neurological disorders.

Gene Therapy and Enzyme Replacement Therapy remain the principal therapeutic avenues pursued by research groups. Due to their higher level of incidence, MPSIII Types A and B are the types where research is most advanced. In addition to programs entering the

clinic, there is also a lot of preclinical research being carried out to explore alternative ways and treatments for Sanfilippo Syndrome.

Research activities, especially at clinical trial stage, have historically been largely driven by North American and European research institutions and biopharmaceutical companies. We intend to change this! These groups recognise the value and need to expand their research and trials to other countries such as Australia, with several major programs now evaluating Australian institutions as potential sites for international clinical trial execution.

RESEARCH GROUP	TYPE	STAGE	TRIALS TIMEFRAME	PLANNED TRIAL SITES
Gene Therapy				
Institut Pasteur & Uniqure	B	Phase I/II	Completed in 2015 (results pending)	Europe (France)
Abeona Therapeutics & Nationwide Children's Hospital	A & B	Phase I/II	Commencing 2015 / 2016	USA, Europe (Spain), Australia
Esteve & Autonomous University of Barcelona	A	Phase I/II	Commencing 2016	Europe (Spain)
Lysogene	A	Phase III	Commencing 2016	Europe, USA
University of Manchester & H.A.N.D.S Consortium	C	Phase I/II	Commencing 2017	Europe
Enzyme Replacement Therapy				
Shire	A	Phase IIb	In Progress	Europe, Americas (multiple countries)
Alexion (formerly Synageva)	B	Phase I/II	In Progress	USA, UK
Biomarin	B	Phase I/II	In Progress	Australia, Brazil, Columbia, Europe (Germany, Turkey, Spain, UK), Taiwan
Substrate Reduction Therapy				
University of Manchester (Genistein)	All	Phase III	In Progress	UK
Stem Cell Therapy				
University of Manchester	C	Phase I/II	Start 2016	UK

... AND MUCH MORE YET TO COME. Whilst science is progressing well, providing access to treatments for Australian children with Sanfilippo remains a major challenge, particularly given the inherent uncertainty linked to clinical trials and their progress path. Whilst the prospect of clinical trials coming imminently to Australia represents an incredible opportunity for the Australian patients community, the Sanfilippo Children's Foundation needs to continue its efforts to invest in and support a range of research programs and activities.

OUR RESEARCH PARTNERSHIPS

About our Research Partnership with **Abeona Therapeutics**

In January 2015, the Sanfilippo Children's Foundation finalised a legal agreement and executed its first scheduled contribution of USD\$200,000 (AUD\$236,000) to help fund Abeona's international gene therapy clinical trial for children affected by Sanfilippo Syndrome. In total, the Foundation will contribute a total of USD\$1 million (est. AUD\$1.3 million) towards the funding of Abeona's programs. Contributions are aligned to agreed milestones being achieved and include the establishment of a clinical trial site in Australia in 2016.

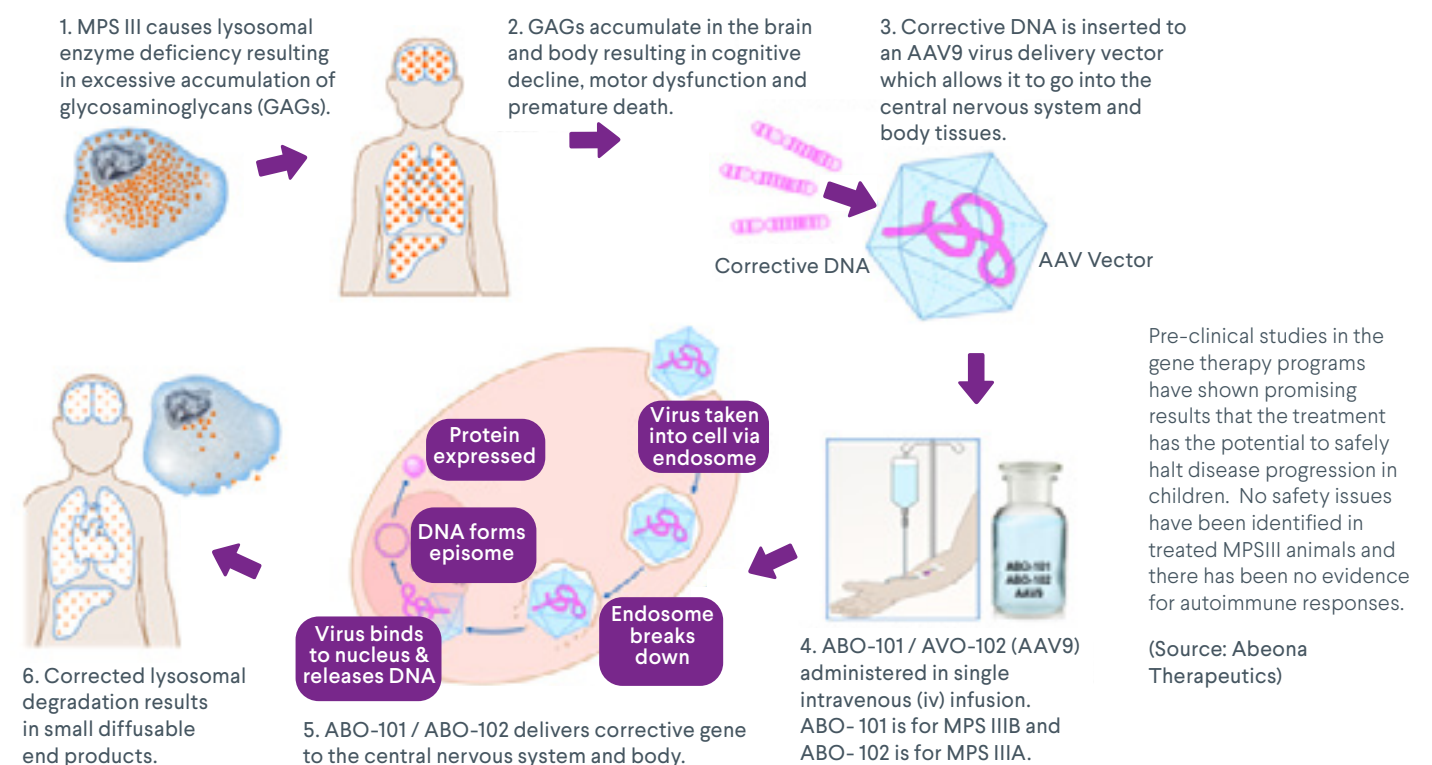
In November 2015, Abeona executives including Tim Miller, CEO and Michelle Berg, Vice President Patient Advocacy travelled to Australia. The purpose of the visit was to engage a clinical team here in Australia, perform due diligence on the preferred clinical trial site and to provide a full briefing to Australian families.

As stated on the Abeona website, Abeona Therapeutics is focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases.

Developing therapies for rare disease requires new approaches and strong collaboration between researchers, industry, regulators and patient groups. Abeona was forged from the company's close collaborations with key stakeholders all dedicated to transforming new biotechnology insights into breakthrough treatments for rare diseases.

How does Abeona's gene therapy treatment work?

Gene therapy is the use of DNA as a potential therapy to treat a disease. In many disorders, particularly genetic diseases caused by a single genetic defect, gene therapy aims to treat a disease by delivering the correct copy of a faulty gene into a patient's cells. The healthy, functional copy of the therapeutic gene then helps the cell function correctly. In gene therapy, DNA that encodes a therapeutic protein is packaged within a "vector", often a "naked" virus, which is used to transfer the DNA to the inside of cells within the body. Gene therapy can be delivered by a direct injection, either intravenously (IV) or directly into a specific tissue in the body, where it is taken up by individual cells. Once inside cells, the correct DNA becomes expressed by the cell machinery, resulting in the production of therapeutic protein, which in turn treats the patient's disease and can provide long-term benefit.





Sanfilippo
Children's Foundation

ABOUT OUR LOGO

Rainbow double helix symbol: The rainbow is a universally recognised symbol for hope and the double helix represents genetics, which underlie this disease, and science upon which our hope for effective treatment is based.

FUNDING RESEARCH, FINDING GEMS

Our logo supports our mission to fund and drive research, leading to effective treatments for our Sanfilippo children. Along the way we look for the gems - those treasured moments our Sanfilippo families share with their children whilst discovering true gems, our supporters assisting in our journey as we all continue the search for the ultimate gem - a cure.



Thank you to our gems - our valued supporters, donors, sponsors, suppliers, volunteers and fundraisers. We couldn't do what we do at the SCF without you!

Contact

Sanfilippo Children's Foundation

PO Box 475

Freshwater, NSW 2096, Australia

E info@sanfilippo.org.au **T** 1800 664 878

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