SUMMER 2020

CF news

CYSTIC FIBROSIS NZ

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Kalydeco confirmed for Kiwis, preparing for school, welcome to our fourth Fieldworker, and more!

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For more on our support services, information, advocacy, and research, or to learn about cystic fibrosis, visit cfnz.org.nz.

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Opinions expressed in articles do not necessarily express the official policy of Cystic Fibrosis NZ. The information in this magazine is not intended to take the place of medical advice from your GP or respiratory specialist. Information correct at time of publication.

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Welcome to the CF News Magazine

We've loved putting this magazine together, and we hope you love it too!

In this issue we go in-depth on the fight for access to medicines and what happens next, now that Vertex and Pharmac have reached a provisional agreement to fund Kalydeco. Success stories of Trikafta continue to fill our social feeds, and we were lucky enough to catch up with New Zealander Kirsty who accessed the drug via a clinical trial in the UK. Speaking of clinical trials – CFNZ is working hard to bring clinical trials to New Zealand as part of our research strategy. Turn to page 22 for more on this.

In the Lifestyle section we've covered important topics such as cystic fibrosis related diabetes (CFRD), transitioning to adult care, starting school, and setting goals but also learning to be kind to yourself. We'd love feedback on these articles and welcome any budding journalists to contribute to the next CF News magazine and/or the CFNZ website.

The printed CF News magazine continues to be a valued way of engaging with CFNZ and staying up to date with the latest developments in the world of CF. But not many people know that you can choose to receive the magazine digitally to your inbox, which helps save costs for us and of course a few trees too.

If you'd like to adjust your subscription preferences, enquire about being a part of the editorial team, or have any feedback, let us know by emailing comms@cfnz.org.nz.

Happy reading!

The CF News Team

THANK YOU

We are incredibly grateful to John Illott for its contribution towards the production costs of this issue of the CF News magazine. Thank you for helping to keep our community connected.

John Illott Charitable Trust

We'd also like to thank Grant Davis for capturing the beautiful front cover and page 22 images of the Porter family.
(grantdavis.co)

NEWS IN BRIEF

Green light for Kalydeco in NZ

On 24 January 2020 we received the incredible news that Pharmac had reached a provisional agreement with Vertex to fund Kalydeco (Ivacaftor) for New Zealanders with CF. At the time of printing Pharmac was seeking final consultation from medical professionals with the view to patient access from 1 March 2020. Read the full update on page 22.

Eddie (right) with MP Jenny Marcroft

All DHBs providing PARI BOY following CFNZ-led project

Since the early 1990s, CFNZ has been fundraising for and purchasing the compressors that power nebulisers used for inhaling medication. These were supplied as required to a large portion of people with CF in the country.

CFNZ made the decision in 2018 to exit the funding of equipment as we were aware of the health and safety risks of a patient organisation, with no medical employees, supplying medical devices.

Fundraising for, buying and managing the distribution and maintenance of medical equipment is also a large financial burden for a small charity.

We were also aware of the inequality of care around NZ where some district health boards supplied equipment and others did not.

Over the past eighteen months we've been working with all DHBs to transfer the funding and provision of the PARI BOY SX nebuliser and consumables to the DHBs.

Thanks to everyone from the DHB's, PHARMAC, EBOS and CFNZ, we're pleased to report that all DHB's are now funding this equipment.

The shift means CFNZ can focus on fundraising for other equipment that improves quality of life for people with CF.

Welcoming our fourth CF Fieldworker

In late January we added a fourth fieldworker to the ranks! Chani Venter joins Sue, Jude and Gretchen as the Upper Central Fieldworker, covering the Waikato and Bay of Plenty regions. We are incredibly grateful to the Lighthouse Foundation for funding this role. Meet Chani on page 13.

New faces at the board table

At the October 2019 AGM we welcomed three new members to the CFNZ Board: Rachel Harris as Board Member, Alex McKay as CF Adult Rep, Jaggar Bootten as the Board Intern, and Warwick Murray was appointed as Board Chair. Each brings a wealth of experience, knowledge and passion to the organisation, and we look forward to working with them. Board members Ed Campion and Melissa Skene have stepped down, leaving two vacant positions on the Board. Please enquire with Jane Bollard (ceo@cfnz.org.nz) if you are interested in joining the Board.

Olympian appointed as CFNZ ambassador

Peter Miskimmin has joined CFNZ as sports ambassador. Peter, CEO of Sport NZ, has a wealth of experience as an athlete, coach and sports patron. As a two-time Olympian, Peter has been captain of the NZ Men's Hockey team and played 150 test matches for New Zealand. In his current role Peter has driven an increased focus on community sport and we look forward to working with him to find ways of supporting the CF population through sport.

Post-transplant care

CFNZ recently conducted a research project to better understand the experiences of CF care following a transplant. The feedback received will be used to evaluate post-transplant care and identify any improvements.

24-hour fitness for CF

CLM The Bays recently ran a 24-hour fitness event in support of CFNZ and CFNZ - Auckland Branch. From COMBAT to spin, meditation and reiki, and pool sessions, this fundraiser was an absolute hit. Turn to page 16 to read more.

NEWS IN BRIEF

Meet new CF Nurse, Megan

We're pleased to introduce Megan Brooks, who has moved into a job-share role with Jan Tate at Starship Hospital. Megan has joined us after working at Middlemore Hospital, where she was a nurse educator in the emergency department. However, Megan's not a brand-new face to Starship, she worked there for many years before heading over to Middlemore. Megan was born and raised in West Auckland, and still lives there with her husband and their two little girls.

Welcome aboard, Megan. We're thrilled to have you on the team!

If you have any questions for Jan or Megan, you can reach them Monday to Friday, 8am – 4pm.

A festive success

Passengers travelling through Wellington Airport over the Christmas period were treated to the sight of over 30 unique Christmas trees twinkling across the concourse. Wellington Airport Chief Executive Officer, Steve Sanderson, opened the Gala. "Cystic Fibrosis NZ is a charity I hold dear to my heart, and this event just keeps getting bigger and better," he said.

This year's event raised over $34,000 and CFNZ – Wellington Branch is welcoming tree sponsors for the 2020/21 festival. Contact Wellington@cfnz.org.nz for information.

Dates for your diary

10–16 August 2020

Cystic Fibrosis Awareness Week

CF Week is our biggest fundraising appeal of the year. Get involved!

18–20 September 2020

CFNZ Internal Conference & AGM held in Wellington.

1 November 2020

Applications open for the 2021 Mark Ashford Scholarship and CF Achievers' Awards. Closing date 28 February 2021.

Huffing and coffin all the way to parliament

We are incredibly proud of our CF community members Camilla and Julian (www.rip.kiwi) for their epic tandem ride from Dunedin to Parliament, demanding a Pharmac funding boost.

During their ride up the country, Julian and Camilla spread the word about the lack of medicine funding in NZ and managed to add around 600 signatures to the petition for a reform of Pharmac and to double their budget.

CFNZ would like to express our sincere thanks and admiration to you both for putting yourself out there and for making such a statement to politicians and the NZ public on this important issue. You both spoke so passionately and openly, one could not help but sit up and take notice.

As part of our commitment to advocacy, CFNZ contracted Lorelei Mason to work with us together with Patient Voice Aotearoa. We were delighted to see 30+ media engagements for Julian and Camilla's ride.

SWEAT FOR CYSTIC FIBROSIS

Dust off the lycra, pull on your sweatband, grab a friend... It's time to sweat for CF!

Keep an eye on our social media next month for details of our greatest fundraiser yet!

MARK'S LEGACY

The Mark Ashford Scholarship is awarded each year to a person with CF who's shown excellence in tertiary study and/or shown tenacity to overcome the trials of CF while studying.

Most of the CF community are aware of the award – but what about the man behind it? We spoke with Kathryn, Mark's wife, and his daughter Meghan, to find out more.

An illness defying doctors

"Mark was born 18 October 1960 to immigrant parents," said Kathryn. "Not much was known about CF then, especially in New Zealand.

Mark never let CF slow him down.

"His parents struggled with two sickly children whose illnesses defied doctors. Then, in July 1963 his older sister Catherine passed away at the age of four-and-a-half."

"Dad struggled to put on weight and was incorrectly diagnosed with Coeliac disease as a child," added daughter Meghan. "At age 13 he was finally diagnosed with cystic fibrosis when Professor Elliott came to NZ, and was given pig pancreatic enzymes.

"He seemed to be doing well. He was always on the skinny side, but his lung function was obviously not too bad – he was a smoker until he met my Mum!"

A life well lived

"Mark lived a full life as a child and teen, and never spent any time in hospital other than the three-monthly check-ups at St Mary's Children's Hospital" Kathryn continued. "He really hated going there as a teen/young adult. He was in his mid-20's before the adult clinic at Greenlane hospital was established."

Mark never let CF slow him down.

"All stories about my dad when he was younger, are about getting up to mischief, travelling overseas or playing sports such as squash, tennis, snow and water skiing," added Meghan.

Fletcher Challenge set up the Mark Ashford Memorial Scholarship in his memory.

Mark went on to complete a BSc in Chemistry and then BE in Chemical Engineering at Auckland University. He was then employed by NZ Forest Products Ltd and latterly Fletcher Challenge when they took over the company in Penrose.

While working for the Winstone Wallboards division of Fletcher Challenge Mark wrote 'The Healthy Homes' book which highlighted even back then the need to adequately insulate homes.

"Mark travelled extensively for work to Australia, Japan, the US and Europe. Whilst he loved the trips, they did take a toll on his lungs," said Kathryn.

"By 1995 Mark's health was deteriorating and he never fully recovered after his second pneumothorax."

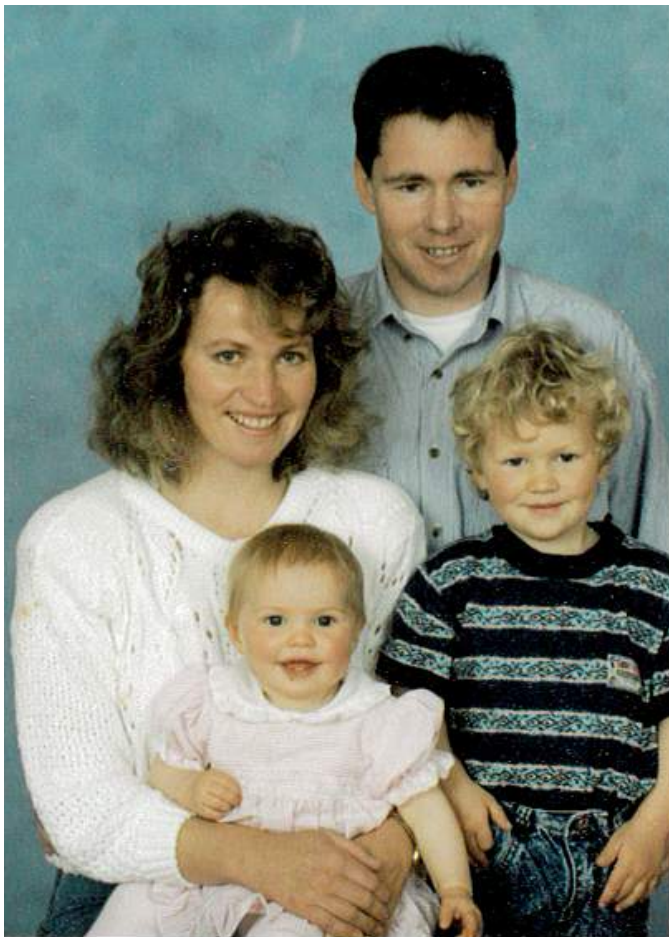
Fletcher Challenge set up the Mark Ashford Memorial Scholarship in his memory and this has been a huge highlight and comfort to his family.

"Mark achieved so much in his short life and his perseverance to overcome his illness and live life to the max is an inspiration to us all," said Kathryn.

A celebration of life

"Through university I made a few friends who have CF and I am humbled by their achievements and zest for life," adds Meghan. "The amazing things that they are achieving are to the credit of the wider CF community who campaign for better treatments, take care of CF families and support one another. With today's advancements in medication and transplants, my dad might still be alive and well.

For my family and me, this award is a celebration of life. We are grateful for the support of the sponsors TelferYoung to allow his legacy to continue. Thank you to CFNZ for including my family in the presentation of this award each year".



"It is incredibly humbling to witness the impact of Mark's legacy over the past 24 years. As people with CF live longer, healthier lives, it continues to be a privilege to administer the scholarship and support New Zealanders with CF to chase their goals, make the most of opportunities, and live life to the fullest".

– Jane Bollard, CFNZ Chief Executive

APPLICATIONS FOR 2020 AWARD

Since 1996 there have been 23 recipients of this wonderful award, helping Kiwis with CF to fulfil their goals and ambitions. We are accepting applications for this years' award which closes 28 February 2020.
To apply visit www.cfnz.org.nz/mark-ashford

WE'RE ON THE LOOKOUT!

The support we receive from our partners is essential and enables us to continue working towards our vision of lives unlimited by CF. We're seeking a new funder (corporate or private!) to sponsor the 2021 Mark Ashford Scholarship (approx. $3,500 p.a.). Contact laura@cfnz.org.nz for more info.

LIFE ON TRIKAFTA

Kirsty Parsons, a Kiwi living abroad, shares her Trikafta journey…

My life before Trikafta

Growing up, I was fortunate Mum and Dad instilled the importance of sticking to strict physio routines and taking all my medication. I've always been an outdoor person, enjoyed being active, and very busy. So, I'd like to say my health wasn't too bad. My lung function was probably about 60%–70%. I've always had a cough but never been too productive. I have one F508del mutation and the other mutation is a very rare one.

A normal day for me after waking up: I'd nebulise hypertonic saline, then PEP, followed by nebulising colistin (with inhalers). All up it would take 30-40 minutes. Apart from Creon at meals, I would get through the day without any treatment. Then in the evenings, I had saline, PEP, colistin again. I take Azithromycin, vitamins, sometimes nasal rinses. From the age of 12 I had a gastrostomy and did overnight feeding. During this time, I also developed CF-related diabetes. Finishing school, I took a gap year in Scotland and got fat on Ben and Jerrys, one way to get rid of a peg! When I was 17 my oxygen sats were quite low, so I had overnight oxygen.

I did find myself really susceptible to colds. Whenever someone at work or a friend had one, it was guaranteed I'd catch it. Most of the time it evolved into an infection which required a two-week dose of oral medications or IVs. Over the years I've had a lot of hospital admissions (mainly two week courses of IV antibiotics). Since leaving school they decreased to about one per year.

Over the last few years, my health has deteriorated a bit. The main reason was being in the UK, doing my masters here, getting a job, travelling and trying to do too much. I was burning the candle at both ends big time! Having CF makes you quite stubborn I think, and I didn't want to accept or admit I needed to take better care of myself nor miss out on any fun. About five months before going on Trikafta I had a cold-turn-infection which I couldn't shake off. IV antibiotics helped a little bit. Eventually, I hit rock bottom (for me). Looking back now it was the sickest I've been. My lung function dropped to 40% at one stage. I was exhausted all the time, walking for more than 30 mins was tough and doing social things with friends was hard work. My cough was really bad, and it made PEP difficult (often impossible) to do. My previous active, busy lifestyle was basically non-existent. I remember dreading hospital appointments, just for the sheer disappointment I'd feel when I couldn't do lung functions tests well.

I hit rock bottom.

I took part in a drug trial!

I took part in a worldwide trial. It was really interesting! I'd recommend anyone to participate in a trial no matter how big or small. I had no idea until I came to the UK how reputable the Manchester CF unit is. The team is huge, and the staff are amazing. For instance, there is an entire CF ward for inpatients, with a dedicated kitchen and chef! In saying that, the care I received at the Auckland CF clinic is very similar.

Straight away I put my hand up for any trials they were running. Most trials are small and don't involve new medicines. While being in Manchester I have been well looked after by one CF specialist doctor in particular, who is very involved in worldwide CF care and drug discovery. After being here for about two years they said there was one trial they'd like me to try for. The process of drug trials is interesting. It's very strict–you must fit a specific criteria, do loads of tests, long regular appointments, and be very diligent in taking the medicine and recording any changes in your health/lifestyle/medicines etc. The initial trial I was admitted on was three months long. It was called a triple combination therapy (Trikafta hadn't been named yet) and patients were either on a placebo or a specific dose of the drug. The goal of the trial at this stage was to understand the best dose to be on.

Didn't quite go to plan…

Unfortunately, I had to be taken off the trial because my health wasn't stable enough, which was gutting. Six months later my doctor said they were opening up spaces for the next stage of the trial. Worldwide there were only 500 places available, and seven research centers. I'm still pinching myself that I managed to get on (there are only two of us in Manchester on it I think

and a handful in the UK)! So, after a phone call I raced up to the hospital and did the initial tests to be completed and registered before my spot was taken. This trial was a 24-week, randomised, double-blind, placebo-controlled trial. I think it's safe to say I was on the placebo! Which was frustrating to be so sick and still have to follow the strict trial process and take these new tablets which were doing nothing.

Having CF makes you quite stubborn I, and I didn't want to accept or admit I needed to take better care of myself nor miss out on any fun.

Then the game changed

The shining light at the end of the tunnel was that every participant on this double-blind was rolled over onto the open study–a two–year trial where every patient is on Trikafta. For me, that started in November 2018. Because it was still in development stages I had to be careful not to say too much to anyone.

The first few months involved three weekly, then six weekly appointments set on strict dates. There I had loads of blood tests to test EVERYTHING (my doctor rang me once asking if I has any bruising or sore muscles which had been picked up in a blood test), a sweat test (to measure the salt levels to detect the activity of the sodium channel protein the CF mutation affects), lung function, ECG, pregnancy test, a survey on lifestyle and mental/emotional health, and a consultation with the doctor. There's loads of paperwork for them to all fill out. Now it has decreased to three monthly visits and phone call appointments in-between visits. Fortunately, Vertex agreed that post-trial, all participants would continue to be given Trikafta until it is fully funded. The doctors worked really hard to get that written into the contract.

What life is like now

It's been 14 months since I first started Trikafta. The initial improvement was by far the best–in fact it shocked me how three small tablets could flip my world around. I had no clue what to expect as there wasn't much publicity about Trikafta by that point. My doctor said that the first few days I could be quite productive. That was an understatement! For the first time ever huge plugs of mucus from every corner and depth of my lungs would just shift. I remember sitting at work (a desk job, so not even using my lungs) and suddenly having a big cough and it would just empty my lungs out like that. After the first week I felt like a new person. Given how sick I was right before going on Trikafta (and not even IVs being able to have a big impact) it was a huge change. After three weeks I had my first appointment and my lung function had improved 13%. It is now 82% (the highest it has been in about eight years).

After the first week I felt like a new person.

The emotional effect it has had on me has been huge too. Like a weight lifted off my shoulders, it's given me a completely fresh start. I am so much happier, can laugh again without going into a coughing fit, and make plans knowing my health will be good. To say it is life-changing is an understatement.

What's next for me?

Trikafta has had a huge impact for me. I'm aware it was all about being in the right place at the right time and luck played a lot in that. It has been approved by the FDA in the US, the UK has fast-tracked it so will be funded here soon too. At the end of this year I'm going to climb Kilimanjaro, the world's highest free-standing mountain where less than 50% of able bodied climbers reach the top. I'll be fundraising for CFNZ and raising awareness about Trikafta in the hope that it will speed up approval for everyone in NZ. I'm also happy for anyone to get in touch with me about my experience with Trikafta or any questions they have.

Email: comms@CFNZ.org.nz to be connected.

AMELIA'S WISH STORY

12-year-old Amelia, who has cystic fibrosis, had one true wish–to meet vet Dr Chris Brown in Australia. When asked "why?" this is what Amelia told our volunteers:

"I have been watching Chris in Bondi Vet since I was about 5-years-old and love seeing him take care of all the animals that come to the clinic. I love animals and we have a dog, a cat, lots of birds and goldfish at home, and I like that Chris looks after lost animals and finds them a home.

It is my dream that one day I could work in a vet clinic like Chris does and look after the animals. I think it's cool that he travels to help animals that people can't afford to look after properly. He is a real inspiration to me."

Amelia's wish to meet Dr Chris Brown was more than just a meeting. Chris talked a lot to Amelia about all the different animals and made Amelia and her family feel very comfortable. At one stage Chris asked Amelia if she would like to assess a new patient that was brought in that morning. During the assessment they talked about what the patient's symptoms were and what medicines he had given them.

Chris took Amelia on a tour of the vet, explaining everything on the way before taking her to the kennels to see a dog that had been brought in. Apparently, it wasn't doing well, but when Amelia was checking it out the dog was lively and playful–everyone said that was because she knew Amelia was coming.

Amelia even got to be Chris' vet assistant during an appointment he had! Throughout the appointment he kept asking Amelia her opinion on what she thought was wrong with the dogs. To top it all off, Chris sent through a Joey video that he secretly organised as Amelia had told Chris kangaroos were her favourite.

Amelia's Mum told Make-A-Wish: "Amelia's wish gave her something positive to look forward to, as all her holidays are to do with hospitals. It was a new and exciting experience that had nothing to do with the day-to-day drag of medical routine."

Make-A-Wish NEW ZEALAND

We help make the wishes of children and teenagers (3–17 years old) with critical medical conditions come true.

Once the completed application form is approved, we will take your child on a unique wish journey towards making their cherished wish come true.

Make-A-Wish NZ has amazing volunteers who will work with your child to **CAPTURE** and uncover what their most cherished wish is. We will then **DESIGN** each journey to create an extraordinary experience that stays true to your child's cherished wish.

Wish **ANTICIPATION** can be as powerful as the wish itself. It builds the excitement and empowers your child to be actively involved in the creation of their wish.

Wish **REALISATION** and delivering a wish beyond your child's wildest imagination is why we do what we do. The positive **IMPACT** of a wish lasts long after the wish is granted and spreads to all of you who are involved in the wish journey.

Every child's imagination is unique, so each journey is designed to create an extraordinary experience that is true to the child.

1. Complete application form online or return via post
2. Make A Wish will contact the child's medical specialist to confirm eligibility
3. Two Wish Volunteers are assigned to the child
4. Wish volunteers meet with the child and their parents/ caregivers to determine one true wish
5. Make A Wish set out to create a unique experience
6. Time varies from wish to wish, depending on the wish and the child's health
7. **Wish granted!**

MEET CHANI VENTER

Cystic Fibrosis NZ is delighted to welcome Chani Venter to the wonderful fieldworker team. Chani is based in Hamilton, covering the Waikato and Bay of Plenty regions.

Let's get to know Chani

What drew you to CFNZ?

I've always had a passion to work with people and to make a difference. For me, work must have meaning. I've been waiting for the right role to come up, where I can make a positive difference… and I think I've found that.

My family and I immigrated from South Africa eight months ago. I applied for another social worker position back then, but the role didn't end up being filled. I saved the recruiter's number in my phone as 'NZ dream job'. The next call I received from 'NZ dream job' was about the CF Fieldworker position. It was meant to be!

What's your pet peeve?

Sitting in a place, sometimes church, and someone behind you sniffs over and over.

Happiest when…

Eating ice-cream with my family.

What is something most people don't know about you?

That I am very adventurous. When adventuring, I am occasionally overly confident to begin with, and it's ended in some very embarrassing moments!

Do you make your bed every day?

Of course. Every bed in the house.

Chani joins the passionate team of fieldworkers at CFNZ; Gretchen, who now focuses on Auckland and upper North Island, Jude, who covers Central Districts, Taranaki, Hawke's Bay and Wellington, and Sue, who covers the South Island.

WE ARE INCREDIBLY GRATEFUL TO THE LIGHTHOUSE FOUNDATION FOR FUNDING THIS ROLE.

24 CLASSES 24 HOURS

CLM The Bays was introduced to CFNZ and the Auckland Branch thanks to Jamie Archibald, who has worked for CLM for over a decade.

They had a pretty exciting plan to hold a 24-hour event running gym classes and raising money and awareness for CF. And that they did!

On 1 November 2019 at 3pm, the first of 24 gym classes began. Each hour was kindly sponsored, and lucky gym-goers were treated to spot prizes through-out the 24 hours. The classes ranged from body balance to cross fit, there were even a couple of swim sessions! At 9pm, the pool turned into a wave-pool when it saw over 40 swimmers taking part in the Aqua class.

The event ran seamlessly, with all staff and attendees given a run-down of why CF was such a great cause, and close to their hearts, thanks to Jamie.

It was such a privilege to work with the amazing team at CLM The Bays. They were all so kind and welcoming but most of all, excited for and believed wholeheartedly in the cause. A special mention to Sammy who kept the 24 hours running smoothly, made sure photos were taken in each class, and even dressed up in a unicorn onesie for the 1am session.

We are pleased to announce that a grand total of $4,700 was raised.

A FEW WORDS FROM CLM

'It was such a great experience raising over $4,700 for Cystic Fibrosis with the 24 hour 24 classes fitness event!

The team at CFNZ were incredibly supportive to help raise awareness (& money) for cystic fibrosis. We have all learnt more about CF and how people are affected.

The members & instructors of CLM The Bays came together with fun, themed classes including a 9pm pool party, 1am BODYBALANCE in pyjamas & 1pm movie theme BODYCOMBAT.

It's impressive to see the community come together–the instructors who worked hard to deliver classes around the clock, the energy from members and the support from local companies.

Overwhelmed with the generous donations from everyone!

We are looking to the next event!' – Sammy Majewicz

CHALLENGE WANAKA HALF A WELCOME CHALLENGE FOR JAKE

Jake and his twin sister Emma.

With a colossal 1.9km lake swim, 90km bike ride, and 21.1km run ahead of him, 21-year-old Jake Gawn is taking the challenge in his stride.

On Saturday 15 February Jake will be competing in the Challenge Wanaka middle distance triathlon as both a test of endurance and to raise funds and awareness for Cystic Fibrosis NZ. In between training, we spoke with Jake to find out a little more.

Anita: Can you tell me a little about yourself?

Jake: I come from Oamaru and my twin sister Emma and I both have cystic fibrosis. I have always been really involved in my sport, playing rugby, squash, taekwondo and everything in between! I'm also studying Sports Technology at Otago University.

I guess my own CF journey is a bit unique because my twin sister also has CF, so I've always been around someone else who is going through the same thing. I am fortunate because I am still able to do things such as sport and going to university.

A: Why did you decide to enter the Challenge Wanaka Half?

J: I've always wanted to do a half Ironman as an individual as a personal challenge to push myself. As well as this, I decided it would be a good opportunity to raise awareness and money for cystic fibrosis.

A: Is this the first time you have taken part?

J: This is my first time as an individual. I have competed in a team four times; twice as the cyclist and twice as the runner.

A: What's the training schedule been like?

J: I train six days a week, anywhere from eight to twelve hours per week. I'm fortunate to have Olympian Tony Dodds of Evolve Triathlon coaching me. It's tough but knowing that I'm doing it to raise money helps to keep me motivated as I don't want to let people down! Also, my partner Kate is participating in the challenge to support me.

A: What's it been like training with cystic fibrosis?

J: It's a rush having to train before and after work, and to fit in all my medication and treatments before work. I need to balance the insulin I take depending on how much training I'm doing. Sometimes sore joints prevent me from training, but I just have to take a rest day.

I've had to ensure I'm eating properly throughout the day and during exercise so that I don't lose too much weight. I have to have enough salt in my diet to make up for the large amounts lost through sweat with all the training. I also drink electrolytes during training.

A: Do you have goals you are working towards?

J: As it's my first one the main goal is just to finish! I would like to do it under six hours, but finishing is the most important target.

A: What advice would you give others who are living with CF?

J: The main thing I would say is not to let CF define you. I know it really sucks at times but in a weird way it has made me who I am. It has made me resilient and I use it to drive me, to do all the things that I have done and will continue to do in the future!

A: And finally, how can people support you?

J: My fundraising page is: givealittle.co.nz/fundraiser/using-my-breath-for-cf. The support so far has been awesome! A huge thank you to Ruby Fresh wetsuits (rubyfreshwetsuits.com) and Aspiring Consultancy who sorted me out with a wetsuit for the challenge, their generosity is amazing. Another big thanks to New Zealand Petfoods (nzpetfoods.co.nz) for paying my entry fee.

Thanks for your time Jake – and best of luck for the triathlon!
Interview by Anita Divers

Jake and his partner Kate

PREPARING FOR SCHOOL

Do you have a little one with CF starting school this year?

Starting school is a big milestone in every child's life, and it often marks the start of your child taking more responsibility for managing their CF.

Last year we met Sophia, a delightful 6-year-old from Hamilton who invited us to spend a couple of days with her and her family. She shares how she manages her CF at school, but mostly importantly she shows us how CF is only a small part of her school day – the rest is taken up with playing with friends, learning and having fun. Watch Sophia's video, but be warned, it's very cute and inspiring!

Watch it here www.cfnz.org.nz/life-with-cf/school

After talking with lots of New Zealand parents of children with CF, we know many parents are worried about infection risks at school. Taken from our guide **Starting School A guide for parents and caregivers of children with cystic fibrosis** here's a few topics we recommend discussing with your child's teacher and school before the big first day:

- Talk with the school and your child's teacher about areas of most concern, such as stagnant water (e.g. fish tanks, vases, pool changing rooms), vegetable gardens and compost.
- Ask if there is another child with CF at the school.
- Ask to be notified of activities where there may be an infection risk, such as impromptu gardening or nature walks. Your child doesn't necessarily need to sit these out, but teachers may need specific guidance from you so they don't over or under-estimate the risks.
- Your child should use their own stationery if pens and pencils are shared – this way you'll know it's only your child biting on the end of their pencil and not everyone else.
- Encourage regular cleaning of chairs, tables, sinks and shared equipment such as iPads.
- Ask about the school's policy regarding sick children attending school and if it is strongly enforced.
- Ask to be notified if there is an outbreak of diarrhoea, gastroenteritis, chickenpox or other infectious diseases. In most cases you don't need to know who it is, just which classroom is affected, so you can make an informed decision about the risks to your child.
- If you and your child feel comfortable to tell classmates about CF, consider sending a letter or email to parents explaining the impact CF has on your child.

- Sick children visit the sick bay – which is not the best place for your child! Discuss with your school on identifying another place your child goes to when they're unwell.
- Encourage the use of alcohol gel in the classroom and to use liquid soap and paper towels for washing and drying hands – no shared towels or bar soap.
- Make sure your child's classroom is well ventilated, dry and free from mould or damp.

Resources to help

You can download a copy of our guide **Starting School A guide for parents and caregivers of children with cystic fibrosis** on our website www.cfnz.org.nz/get-support/resources/guides/. It covers topics such as managing pancreatic enzymes, sport and exercise and school trips.

Also available for download is our guide **Starting School A guide to cystic fibrosis for primary schools and teachers**, available on our website www.cfnz.org.nz/get-support/resources/guides/. This is a great guide to give to your child's school and teachers.

If you'd like a hardcopy of either of these resources please email admin@cfnz.org.nz and we'll post them out to you.

"My son sees heaps of other kids around his school with diabetes and allergies and doesn't think his CF is really any different to this. Every year since he started school, the teachers have explained CF, keeping coughs and illnesses away as much as possible, his tablets and his drinks (Fortini) at the beginning of the school year to the class. No kid has ever really mentioned it to him. If anything, they remind him to take his pancreatic enzymes! He is aged eight now."

SUCCESSES AND CHALLENGES OF LIFE

Claire Scoffield set herself a goal to tackle this summer

Read our Q&A with Claire, where we touch on the successes and challenges of life, goals and climbing mountains.

Tell us about '20 peaks in summer 2020'

After many years of setting goals and not achieving them, I figured why not set a goal that was to do with something I loved, thus making it more likely that I would get there. My lung function has recently declined into the 30's quite quickly, and I needed something to keep me focused on staying active when exercising got more and more difficult, particularly as I now require continual oxygen during exercise.

How many have you walked so far? What's the highest peak you've conquered?

I am currently on 8 peaks. The one with the most elevation gain was Grandview Mountain in Hawea. This was almost 1000m, and 20km in distance. My oxygen concentrator ran out about 2 hours in, so it was definitely the toughest peak so far. The 8 peaks total: 4199m elevation gain (halfway to Everest from sea level) and 20 hours of walking over a distance of 72km.

What's your favourite part about challenging yourself?

I am always up for a challenge, particularly in the outdoors. I love the feeling of achieving something that you felt was not achievable at the start. Particularly with lungs that appear to be in decline, still managing to slowly get there when you were a healthier person is sometimes quite emotional.

What are you finding most difficult?

I think probably I am struggling to manage the emotional side of the challenge. The constant battle with "but I used to be able to do this easily/quickly" is hard. Also, it appears remembering to charge my portable oxygen concentrator to full before leaving the house!

Anything else you want to share?

As everyday life gets a little more difficult, managing to complete a peak each weekend, while working full time and studying part time towards a master's degree can sometimes get a little overwhelming. A challenge on top of this challenge is also knowing when to be kind to myself, and to know when my body needs a break. So many beautiful people with CF I know are constantly pushing themselves to achieve such amazing things every day, and maybe we all need a reminder sometimes that we are allowed to slow down too!

FROM PAEDIATRICS TO ADULTS: A PARENT'S PERSPECTIVE

PIP - MUM

Time flies and before you know it, your child is grown up and is meant to take the reins of their life. When you have a child with CF, this can be a daunting experience. Hayden moving on from the comfort of Starship; under an amazing team that is familiar, supportive and feels life family, to the adult system, can be scary. We started preparing for this next step in his CF journey early. I encouraged him to be more independent, communicate in an open manner, and learn what his medications, treatment and results meant in more detail.

This was difficult as Hayden has struggled with accepting his CF and dealing with treatments. This made the transition to adults a difficult process as I feel he was scared and didn't want to deal with this new independence. We had to let him take control, even if it meant watching him fail to do his treatment properly, or take responsibility communicating issues.

Surprisingly, after all the build-up and fears, his first adult clinic went better than I expected; he was open and honest. He seemed to be more aware of his symptoms, and what decisions should be made when he's unwell. But I won't stop nagging him, as I want him to be able to live the best quality of life as possible.

Now that Hayden's grown up, he's had to attend appointments alone. Getting information out of him afterwards can be a challenge. We've always stayed overnight with him during admissions at Starship, so to prepare for transition he started staying in by himself. This was a big move, but it was important for Hayden to learn how to ask the nurses for support, independently. I am hoping that he'll be more comfortable with stepping up and taking care of himself as he attends more adult clinics and builds a relationship with the team.

Transition is a long and scary process, but we've all been supported very well by both teams. Leaving a place that is like your second home (Starship) and Jan Tate, who has been a godsend to our family and many others CF families, is not something to be taken lightly.

JASON - DAD

There's an old saying, "If you want something done right, do it yourself", but some things are just simply out of your control, despite the influence you think you may have. My son Hayden turned 18 last year, and subsequently has transitioned from the amazing team at Starship Hospital to adult services at Auckland Hospital.

This transition for me, however, started long before his 18th birthday. It started when he began to resent his treatments... when he decided he just wanted to be like every other kid his age and not have to do PEP physio twice a day or take a multitude of meds daily just to maintain a reasonable standard of living. Emphasising the importance, and policing the effort put into treatment is not often appreciated and mostly results in attitude or resistance, but that is (or at least has been) in my eyes, my job... I'm Dad, and I care about his health even if at times, he doesn't.

For the past year, we have been preparing for the responsibility of Hayden's health and well-being to be handed solely to him. He is now an adult. It's his turn to answer all the questions the doctor asks, to list all the meds he's taking, and give a true account of how his health is. My son was always nonchalant about his condition, almost unconcerned. When doctors asked questions to him as a child he would look straight at us to answer, shrug his shoulders, or just say "I don't know". I was concerned that he would only tell them what he wanted them to hear to accommodate what he wanted or to avoid admission. But despite my concern, I am stepping back. The advice I have been given by the CF team at Starship has always been amazing and I'm going with their years of experience and wisdom. I've got to trust him to take control.

I'm still going to gripe if I think he's mucking around during physio, ask him if he's taken his enzymes, remind him of his responsibility toward his health… because I'm dad, and I love him to the core.

TOP TIPS FROM FIELDWORKER SUE

- Know about their health and treatment – make sure they understand the what, where, when, why of their medication.
- During hospital admissions, stay overnight by themselves in paediatrics.
- Talk to health professionals by themselves for a portion of clinic.
- Have a meet and greet with the adult team so you can ask any questions about the adult service before moving there. Have a tour of the different clinic space and CF ward.

TALKING CF-RELATED DIABETES

Cystic fibrosis related diabetes (CFRD) affects around 22% of Kiwis with CF.

Type 1 diabetes occurs when the body's immune system destroys the cells in the pancreas that make insulin, which helps the body use the energy from food. It is commonly diagnosed in childhood and requires you to take insulin.

Type 2 diabetes, which occurs more often in adults who are overweight, is caused by the lack of a normal response to insulin and also the pancreas not making enough insulin.

CFRD shares some features with both Type 1 and Type 2 diabetes. In people with CF, the thick, sticky mucus causes scarring of the pancreas. This scarring prevents the pancreas from producing normal amounts of insulin; so, like people with Type 1 diabetes, they become insulin deficient. In addition to insulin deficiency, people with CF can also develop insulin resistance, especially during periods of acute illness, in patients with liver disease, during pregnancy and when prescribed glucocorticoids.

People with CF who are pancreatic sufficient and possibly overweight may also experience a decline in insulin production by the pancreas as a result of aging.

The prevalence of CF related diabetes increases with age, and affects approximately 50% of people with CF by 40 years of age.

CFRD adds another element to life for people with CF and can feel daunting at times. We spoke with Lizzie to learn about her personal experience of living with CF and CFRD.

What were your early experiences with CFRD?

At my 12-year-old annual review at Starship, I did my first glucose test. It came back a little bit high, so a couple of weeks later I repeated the test, it was confirmed that I had CFRD. In the early days, my diabetes was managed by one injection of protaphane per day, and blood sugar level monitoring with food. It was a secondary issue, and not so front of mind.

As my health declined in my late teens, my diabetes became harder to manage. During admissions, I would require Novorapid (short acting insulin) with meals. After I had my lung transplant I went, as I like to say, 'full-blown-diabetic'. Completely insulin dependent, all the time. This is largely due to the post-transplant medications I am on. It was at this point where my diabetes became something that did have to be front of mind. I was encouraged to test more frequently, and I was eventually taught about carb counting and carb ratios.

How does/did your diabetes affect your day-to-day life in addition to managing your other symptoms?

To be honest, in the early days it didn't affect my day-to-day life too much. It just meant an injection each day and some finger pricks. This just slipped in as another element to the pretty full treatment regime I was already on each day! It came secondary to everything else that comes with CF. But now, since the CF side of things is stable, it's more of an obvious issue that needs to be well managed.

How does the diabetes diet, and the CF diet differ? How do you balance the two?

When I was growing up, it was all about the high fat, high sugar, ultimately high calorie diet. After my transplant, when it became more apparent that I had quite unstable blood sugar levels, and began taking short-acting insulin with food, I had to learn how much insulin to take with what food, and how to bring my sugar levels down by counting back. For people with diabetes, this would be the time to change up your diet, have fewer sweets and carbs… however, the CF and diabetic consultants suggested I should not eliminate these things from my diet, but manage it with insulin, as to not lose the calories and weight. So instead of not having those lollies after dinner, I'd factor them into how much insulin I gave myself. In saying that, I could probably reduce my Mountain Dew intake!

What is the biggest challenge of living with CFRD?

For me, the biggest challenge is having to think about it all the time. Every time I eat, my brain is busy calculating carbs and how much insulin I should give. Although this has been a big part of my life for about 10 years, I'm still learning all the time. I hope one day it'll all be as natural as taking Creon…

What are some of the ways that you've managed your CFRD?

The best thing I have done to manage my diabetes is investing in the Freestyle Libre sensors. This is a little disk on your arm that tests your blood sugar every time you scan it (with your phone! So techno). It also holds eight hours of blood sugar levels and creates a graph so you can see what's happening between tests. I feel like I'm much more equipped with the knowledge of what is happening in my body. Unfortunately, the sensors come at quite a hefty price, and are currently not funded. But it's been a game-changer for me, so the price tag is worth it.

A word from the experts

Dr Amy Liu, PhD, NZRD
Diabetes Dietitian, Auckland Diabetes Centre, ADHB

Lisa Guest, BSc (Hons), NZRD
Adult CF Dietitian, Auckland & regional CF service, ADHB

Importance of diet

A diagnosis of CFRD doesn't alter the usual CF dietary recommendations, and your diet should always favour CF to maintain a good nutritional status and optimise blood glucose control.

The majority of people with CFRD still have higher energy requirements than the non CF population and should aim for 45-65% of their total energy from carbohydrate. If you are under-weight you may need to include refined sugars such as sweet foods, but as these foods cause a rapid increase in blood glucose levels when eaten alone, they should be as part of a meal or substantial snack.

However people with CF who are pancreatic sufficient and possibly overweight should consider avoiding refined sugar from sweet foods and beverages and may want to talk to their CF Dietitian about weight management.

Good nutrition supports good lung health and managing your blood sugar levels reduces your risk of developing diabetes-related complications.

A good diet is important to make sure your body has enough energy to cope with the extra demands of CF. Common CF issues such as inadequately controlled malabsorption, chronic-broncho-pulmonary colonisation from bacteria and fungi, exacerbation of acute lung infection and impaired lung function can also increase your energy needs.

Where possible you should choose carbohydrate foods with a low glycemic index and distribute these evenly and consistently throughout the day to help achieve to help optimal glycaemic control

Carbohydrate foods

Carbohydrate foods are sugars and starch and are the main source of fuel for your body. Carbohydrates break down into sugar in the blood, called glucose.

Not all carbohydrates are the same – some are fast acting and others are slow-release – but both impact your overall blood glucose level. An adequate carbohydrate intake helps to make sure your body is supplied with enough energy and avoids the unwanted surge of glucose release

Some people find carbohydrate counting is helpful to support appropriate carbohydrate intake alongside their individualised insulin regimen to optimise glycaemic control.

Counting carbs

Carbohydrate counting is a meal planning approach used by people who have diabetes, and focuses on carbohydrate as the primary nutrient affecting your post-meal glucose level. There are different types of carbohydrate counting methods and resources used and your CF-dietitian can help work out which one is right for you. The EasyDietDiary app is one resource I've found helpful. This app was produced by the Xyris group who also produce the food analysis programme for Australasia called 'Foodworks'.

A balancing act

The goal for people with CFRD is to have a good nutritional status, good lung health and well controlled glucose levels. It is important to ensure you have established a good nutritional status and stable lung health when working on glycaemic control because poor lung health makes it very difficult to try to manage optimal glycaemic control. it is important any changes in your diet do not cause an unwanted reduction in overall energy intake as this can lead to unintentional weight loss and impact on lung function.

For further advice, talk to your clinical team.

DID YOU KNOW?

From 1 August 2019, the number of funded insulin syringes with needles that can be prescribed per prescription increased from 100 to 200.

Eddie, Beckett, Emma and Otis

ACCESS FOR AOTEAROA
CAMPAIGN FOR PRECISION MEDICINES

On 24 January 2020 the New Zealand CF community received the exciting, and long-awaited, news that Vertex and Pharmac had reached a provisional deal to supply Kalydeco.

"This is a monumental day for the CF community and marks the culmination of many years of lobbying for access to innovative medicines," says Jane Bollard, Cystic Fibrosis NZ Chief Executive.

Pictured on the front cover is Eddie, Emma, Otis and Beckett. The family were instrumental in the Kalydeco for Kiwis campaign.

"We're so excited by the news that Pharmac have reached an agreement with Vertex to fund Kalydeco in New Zealand," said Dad Eddie. "There has been a huge amount of work put into the campaign over the last few years, so this is fantastic reward for everyone's efforts.

"While this has always been the goal of the campaign, it was still a surprise to have everything happen so quickly this year, and knowing that people with CF may benefit from this treatment as soon as March is a hugely exciting prospect," he continued.

"CAN'T BELIEVE IT. TEARS OF JOY, BETTER THAN LOTTO"

"Our son Otis is one of those that will benefit from Kalydeco, and we're excited and hopeful for what may be a very different future for him. We've seen what worked with the Kalydeco campaign, so it is important that the same learnings are applied to the fight for other CF medicines. It's such an exciting time, and we are hugely proud and humbled to have been a part of the campaign!".

So now that the dust has settled on the news – what happens next?

First of all, what exactly is Kalydeco?

Ivacaftor, known by its brand name Kalydeco, is the first medication that fixes the underlying cystic fibrosis defect, essentially turning off cystic fibrosis. Kalydeco represents a breakthrough in cystic fibrosis research; current medications only treat the symptoms.

"UNBELIEVABLE! AFTER SO MANY YEARS I NEVER THOUGHT PHARMAC WOULD DO THIS"

Kalydeco is effective in anyone with at least one of the following nine Class 3 gating mutations: G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P or G1349D, or those with the R117H mutation.

When will Kalydeco become available?

The funding is still subject to a final consultation. If the feedback from the consultation is supportive and the proposal is approved, then people could be prescribed Kalydeco from 1 March this year. It is proposed that the drug will be made available via DHBs and not community pharmacies.

Why did it take so long to get Kalydeco in comparison with other countries?

Drug funding is a complicated issue and each body responsible for assessing and recommending (such as PHARMAC in NZ, NICE in the UK, and PBAC in Australia) each have different rules, criteria and budget thresholds to work with.

"MY FAMILY AND I ARE OVERWHELMED WITH JOY, COULDN'T BE HAPPIER!"

With a list price of over $300,000 per patient, per year, Kalydeco is one of the most expensive drugs available. Pharmac declined

the initial application for Kalydeco back in 2014 citing high cost and limited clinical data.

In contrast, many other OECD countries approved funding for Kalydeco for ages six and over based on the same evidence and list price between 2013 and 2015. Expanded use for two-to-five-year olds was secured in subsequent years.

Some countries, such as the UK, use a system whereby if you meet a certain threshold of benefit it will fund the drug. A commonly used assessment is QALYs or Quality-Adjusted Life Years, which measures the length and quality of life. In the UK, a medicine is funded if it works out at about £30,000 per QALY gained, making the process of funding formulaic and transparent.

Pharmac has no specific level of health benefit which triggers funding. The Pharmac process looks at four areas, the 'factors of consideration' - the need, the health benefits, the costs and savings and the suitability of the medicine, which also includes the use of QALYs. However, Pharmac itself says the factors "are not weighted or applied rigidly" and that "not every factor is relevant for every funding decision" it makes.

Currently New Zealand spends less on medication per capita, waits longer to buy new drugs, and registers fewer new medicines than almost any other nation in the OECD. The IQVIA International Comparisons of Modern Medicines report, commissioned by Medicines New Zealand, ranked New Zealand last for both access to funded medicines and pharmaceutical investment overall.

All these factors combined has made it extremely difficult and time-consuming to secure funding for Kalydeco.

On a positive note, we are pleased that Pharmac has shown the foresight to fund Kalydeco in New Zealand with no age restriction. In Australia for example, Kalydeco is only funded for 12 months and above. Up until August 2019 it was only funded for ages two and above.

It's clear there's a bigger issue with Pharmac – what are you doing to change it?

CFNZ supports the Patient Voice Aotearoa petition to double the Pharmac budget and call for reform of its investment model. We encourage as many people to sign it as possible before it closes on 22 July 2020.

"AMAZING, HUGE THANKS TO THE PEOPLE THAT HAVE PUT SO MUCH EFFORT INTO THIS RESULT, WELL DONE. GREAT RESULT AND THE OTHER TREATMENTS SHOULD FOLLOW, WOW!"

Why didn't you lobby for Orkambi, Symdeko and Trikafta at the same time, or just skip to Trikafta?

There are a few different reasons we campaigned for Kalydeco exclusively in the first instance.

Kalydeco has over a decade of strong, irrefutable data on its benefits for people with CF and presented a solid argument for funding. Given that Kalydeco was declined on lack of evidence in 2014, this indicated that Pharmac was unlikely to consider Orkambi, Symdeko, or Trikafta seriously with its limited years of clinical data.

In addition to this, Vertex will not look at any of these other medications until it has reached an agreement on Kalydeco. This means that getting Kalydeco funded is a critical first step to accessing these other medications. Once we have had the final green light for Kalydeco, we will encourage Vertex to submit an application to Pharmac for Orkambi and begin a concerted effort to secure this drug.

Many countries have also negotiated pipeline or 'portfolio' deals for access to multiple Vertex drugs over a period of years. These deals provide a suite of existing and future drugs that are likely to be developed over the lifetime of the deal. In the Republic of Ireland this pipeline deal has secured them access to Trikafta once the drug receives approval from the European Medicines Agency (estimated to be in 4-5 months' time).

"THAT'S THE BEST NEWS ....EVER, EVER, EVER. THANK YOU SO MUCH TO ALL THOSE WONDERFUL WARRIORS OUT THERE WHO HELPED MAKE THIS HAPPEN."

A pipeline deal for multiple drugs would certainly provide an ideal solution for us in New Zealand. However, our understanding is the current Pharmac funding model and year-by-year budget does not allow for these types of deals.

Can I lodge my own application to get Orkambi / Symdeko / Trikafta funded?

Anyone can lodge an application with Pharmac for drug funding. However, for the application to be considered it needs to be fully supported by the manufacturer with the full documentation on clinical and cost evidence.

Once we have the final greenlight for Kalydeco, we will encourage Vertex to submit an application to Pharmac for

Orkambi and begin a concerted effort alongside the CF community to secure this drug, as we did with Kalydeco.

How can I get involved with the efforts to secure funding for Orkambi / Symdeko / Trikafta?

Our current focus is to support the final consultation process and ensure that Kalydeco is made available on March 1 with no further delay. It is incredibly important that we do not jeopardise or complicate this step.

Once Kalydeco is in the hands of those eligible and that stage is firmly complete, we will develop the next stage of our advocacy campaign for Orkambi, Symdeko and Trikafta and campaign details will be made available at that point.

We've also set up a CF advocacy Facebook group – Access for Aotearoa – which we encourage you to join so that we have an army prepared when the fight for the other CF drugs kicks-off.

Lastly, the campaign for Kalydeco was incredibly compelling because it featured Eddie and Emma fighting for their son Otis. We're on the lookout for families who would benefit from any of these unfunded precision medications who would be comfortable sharing their story. This may be in the form of TV, radio and newspaper interviews, providing written evidence on day-to-day life with CF or supplying photos and/or quotes for us to use. Please email ceo@cfnz.org.nz if you might be interested.

HAVE YOU GOT ANY **OTHER QUESTIONS?**

We're happy to answer them!
Contact Chief Executive Jane Bollard by emailing ceo@cfnz.org.nz or by calling the office on 09 308 9161.

ADVOCACY NEWS

TIME FOR TOBI

In late 2017 CFNZ submitted an application for the funding of Tobi Podhaler, a device similar to an asthma inhaler that delivers tobramycin in around 1 minute compared to about 20 minutes on a conventional nebuliser. The application will be considered at the next PTAC meeting, scheduled for 20 and 21 February 2020.

FAIR FOR RARE NZ TO LAUNCH AT PARLIAMENT

A nationwide campaign to raise awareness for the 300,000 New Zealanders living with a rare disorder is to be launched at the Grand Hall in Parliament on Friday 28 February.

Fair for Rare NZ, the Rare Disorders NZ-led campaign is calling for the establishment of a New Zealand National Rare Disorder Framework, explains chief executive Lisa Foster.

"Rare disorders are often neglected and invisible yet they affect about 6% of the population – half of whom are children. So collectively, rare disorders are not rare," says Ms Foster.

"Those living with a rare disease in New Zealand face inequitable access to diagnosis, treatment and care – particularly when compared to countries such as Australia."

Lisa Foster - Rare Disorders NZ Chief Executive

Rare Disorders NZ and its 140 support groups, including Cystic Fibrosis NZ, are calling on New Zealanders to acknowledge the common challenges faced by people living with a rare disease.

"Furthermore, to honour New Zealand's recent commitments to respective United Nations and Asia-Pacific Economic Co-operation agreements, we are calling for collaborative development of a New Zealand National Rare Disorder Framework to enable responsive healthcare for all people living with a rare disorder. Effective policy can reduce the fragmentation and high level of uncertainty, and offer a clear pathway for cohesive healthcare."

Ms Foster says such initiatives would not only benefit those with a rare disease, but have much further-reaching advantages for wellbeing and contribution to society.

"A National Rare Disorder Framework would identify positive solutions to reduce the costs of chronic health conditions by addressing need earlier, enabling more people to participate in work and the community.

"Such measures would also lessen both the time required away from work and the mental stress for patient carers. All of which would directly benefit the New Zealand economy."

Learn more at www.raredisorders.org.nz

YOUR VOICE. WE'RE LISTENING.

Late in 2019 we gave Deloitte New Zealand the green light to begin collecting information for the Social and Economic Cost of CF report. This report will look at health sector and treatment costs, as well as productivity losses and the cost of caring for people with CF, both formally and informally. It will also consider more qualitative data such as loss of wellbeing and the impact of premature mortality on families.

Thank you to everyone in the CF community who completed the recent cost of CF survey, supported by Rare Disorders NZ and Medicines New Zealand, which provides some of the raw data for the Social and Economic Cost report.

There were 111 people included in the results, approximately 1 in 5 in our community. Of these, 41% were from people living with CF and 55% responses were from parents of children living with CF.

Some of the key findings:

- Almost half reported that their CF impacts a lot on their health and everyday life. For 3 in 4 people they required more than an hour per day for CF related tasks.
- Over two thirds had at least one inpatient day in the last year. For 5 people they spent over 3 months in hospital.
- High health service utilisation with almost all people reported seeing their specialist and requiring diagnostic tests over the last 6 months.
- There was an extensive list of medicines being taken, on average over 6 medicines per person. The most common medicines taken were Creon then salbutamol, pulmozyme, ciprofloxacin, azithromycin and then ibuprofen in that order.
- Generally, care was well co-ordinated and health care professionals had good knowledge about CF.
- A significant impact on wellbeing due to CF, with almost one third often feeling unhappy or depressed, or that they could not overcome their problems.
- While some healthcare costs were being paid out of pocket the amount and range of items varied considerably.

The results of this survey will be used to support our ongoing advocacy work for the CF community in NZ with a focus on highlighting the impact of living with CF and improving access to treatments and health services. We anticipate the report to be available by mid-April 2020.

SIGNATURES FOR LIFE CAMPAIGN

We want NZ to be a place where people who need it can access lifesaving medications. Currently funding for medications in New Zealand is too low, meaning many people miss out.

Patient Voice Aotearoa (PVA) is a collective of patients, caregivers, whānau, advocates and charitable organisations, campaigning together for the rights of New Zealand patients. Patient Voice Aotearoa aims to work collaboratively provide a unified patient voice to inform better health policy and improve access to healthcare.

Signatures for Life is a campaign led by PVA calling on Government to conduct an external reform of Pharmac and to double the budget Pharmac currently receives immediately, with a plan to triple the budget of Pharmac within two years.

This is a unique opportunity to increase access to life saving treatments. It's important we get as many signatures as possible so we can send a powerful message to the Government ahead of the election year. This is our opportunity to be heard and we need to make it count!

Supporting this petition is critically important to our CF community gaining access to the best equipment and medicines, such as nebulisers, portable oxygen concentrators, Tobi Podhalers, liver medicines, diabetes equipment and medicines, as well as game changing modern medicines such as Trikafta, Symdeko and Orkambi. CFNZ is one of over 30 charities putting their full support behind PVA and the Signatures for Life Campaign.

The petition has been extended to 22 July 2020, where it will be presented to Parliament at the Civic Square at 10am. To sign go to: www.sign4life.nz

SPOTLIGHT ON RESEARCH

CFNZ is making progress on a national research strategy designed to deliver real benefits for people with CF.

The strategy is the product of a workshop organised by Cure Kids and CFNZ in 2018. One of the key findings was the need for an interconnected research, clinical and community strategy, and someone to drive it – hence the joint appointment of Dana Felbab working part-time as research development manager. Here's an overview of some of the key aims of the strategy.

CF community portal

In late 2019 we asked the CF community what CF challenges they would like researchers to focus on, and what the research priorities should be. These results are currently being analysed and will be used to set research priorities.

Research community

Some excellent CF research is happening in New Zealand, but funding applications tend to be driven by individual teams rather than as part of a cohesive plan. CF research would benefit from greater coordination within New Zealand as well as stronger links to clinicians and to international efforts. We are currently auditing New Zealand's CF and related research with views to exploit our strengths and unique aspects of CF in New Zealand.

CF clinical and research network

People with CF would benefit greatly if national or international research was translated into improved clinical practice. We have a well-connected paediatric CF network with potential for the adult CF network to develop. CF clinicians currently have very little time available for research. With an adequately supported CF clinical network, however, New Zealand could undertake smaller, practical initiatives that deliver better results for patients, as well as benefiting the international community.

International connections

New Zealand is a great location for clinical trials because many expensive new CF drugs are not used here, creating what is known as a drug-naïve population. Part of the research strategy involves building stronger connections with funders and overseas pharmaceutical companies to explore how New Zealand patients can be more involved in clinical trials.

As a result of the strategy, several Pharmaceuticals have expressed interest in using New Zealand as a trial site. Clinical trial opportunities are being explored, with hopes to appoint a Research Nurse in 2020 to assist with these.

The New Zealand CF data registry, Port CF, is in excellent shape and has proven to be a valuable tool. This anonymous data will allow us to give information on possible trial sites.

MEETING THE NEEDS OF SIBLINGS

CFNZ recently participated in a Massey University study aiming to identify the unmet needs of siblings of children with cancer, cystic fibrosis, or Type 1 diabetes. This research will contribute to the development of targeted services to meet the unmet psychosocial needs of siblings.

SOCIAL ISOLATION STUDY

CFNZ is also participating in an Auckland University of Technology led research project looking to investigate the impact a complex medical condition has on social relationships. It aims to identify any strategies that can be adopted to facilitate social interactions, with results due to be released in early 2020.

RESIDUE AND RESISTANCE

The University of Otago has just completed phase 1 of its research project to learn more about the types of nebulisers used by people with CF for inhaling antibiotics, and how those nebulisers are cleaned after use. The results of the research will help to inform best practice guidelines for the CF community about safe and appropriate disposal of nebulised antibiotic residue, to minimise the chance of creating antibiotic resistant bacteria in the environment.

YOUR QUESTIONS ANSWERED

In late 2019 we asked the CF community to tell us some of their burning CF questions that could be considered as part of our research priority list. There were a few great questions asked, that we can already answer.

Q: Can babies be gene tested during gestation?

A: It is possible to have genetic testing of an established pregnancy at about 11–12 weeks gestation via CVS (chorionic villus sampling) or 15–16 weeks gestation via amniocentesis. If the baby is shown to be a carrier, this can be helpful for them to know so as not to raise anxiety at the time of newborn screening (which can show a positive result for CF carriers) and also to have this information for their child's future reproductive implications. Contact your GP or Genetic Health Service NZ for further information.

Q: What is the cheap, effective way to detect carriers of CF?

A: There is no free population-based screening programme for prospective parents in New Zealand. However, the test can be sought through private clinics. These private clinics charge around $500 for an initial consultation and carrier testing for the most common CF mutations, as well as testing for two other common genetic disorders (Spinal Muscular Atrophy and Fragile X). Clinics can be contacted directly by prospective parents to arrange an appointment. If you or your partner have a family history of CF; for example, someone who has CF or is a CF carrier, you can access free CF carrier testing from your GP.

Q: How do the different gene types affect the body, and is there a relationship between CFTR mutation type and symptom severity?

A: CF is caused by mutations, or changes, in the CFTR gene. This gene provides the code that tells the body how to make the CFTR protein, which is responsible for the salt and water balance in the lungs and other tissues. Whilst there are more than 1,700 mutations of the CFTR gene, many of these share commonalities in the problems they cause in the production of the CFTR protein. Scientists group these mutations into different classes:

- Protein production mutations (Class 1)
- Protein processing mutations (Class 2)
- Gating mutations (Class 3)
- Conduction mutations (Class 4)
- Insufficient protein mutations (Class 5)

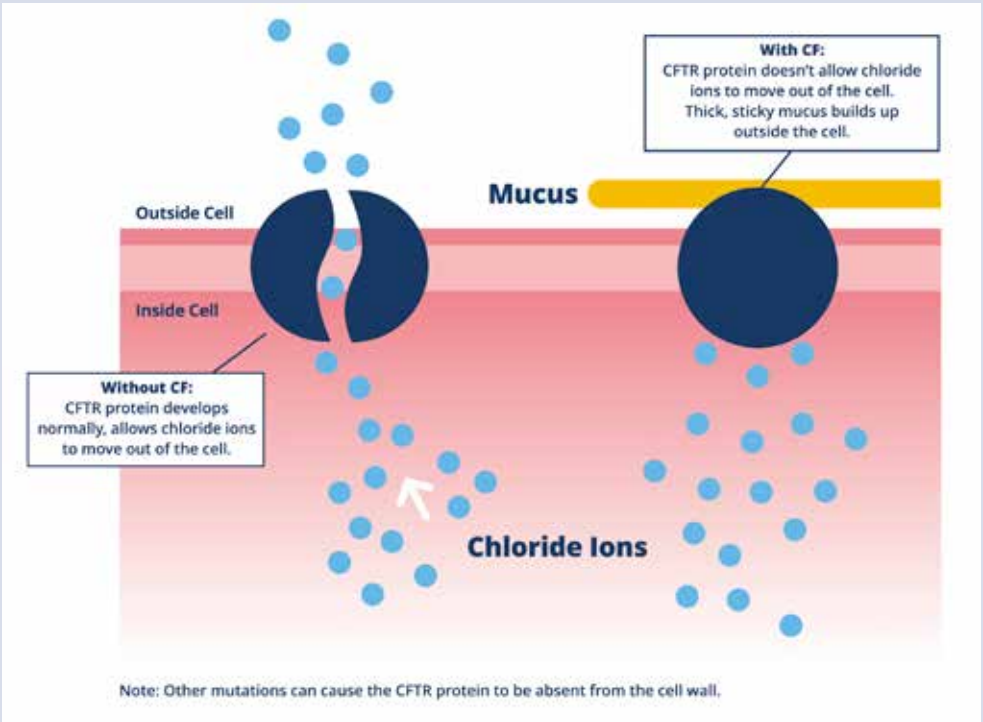
The common gene type F508del falls into the Class 2 mutation, where the CFTR protein is created, but misfolds, keeping it from moving to the cell surface. Correctors such as lumacaftor (in Orkambi) or tezacaftor (in Symdeko) help defective CFTR to fold correctly.

Another example is the gating mutation G551D (Class 3). This is where the CFTR protein is created and moves to the cell surface, but the channel gate does not open properly. Potentiators such as Ivacaftor (in Kalydeco) help open the CFTR channel and help increase the function of normal CFTR.

Whilst a mutation type can tell us how it impacts on the production of the CFTR protein, a mutation type cannot always tell us how severe a person's CF symptoms will be. Some CFTR mutation types can be associated with certain complications, for example issues with the pancreas, but it is not a perfect system.

A great resource on the different variations is the CFTR2 database - www.cftr2.org. This is a global, public registry that provides information about sweat chloride, lung function, pancreatic status, and Pseudomonas infection rate in patients in the CFTR2 database with a CF gene variant or variant combination. It is free to use, and New Zealand has recently been invited to contribute to the database.

For more information on the different mutations, check out www.cff.org/What-is-CF/Genetics/Types-of-CFTR-Mutations



A simple example of the CFTR protein developing normally (left) and abnormally (right) due to a mutation affecting how chloride moves out of the cell.

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