RARE DISORDERS NZ

IMPACT REPORT

2024 - 25

Amplifying the collective voice

raredisorders.org.nz

OUR MISSION

To enable and empower people with rare disorders to best benefit from services and therapies available in New Zealand, and to champion their collective voice, advocating for provision of world leading evidence based health, disability, education and other services.

OUR VISION

Best possible health and wellbeing for people and whānau living with rare disorders in New Zealand/Aotearoa.

Our Values

Aroha – we will demonstrate love, compassion, empathy and respect for people living with rare disorders and we will support and encourage our stakeholders to do likewise

Whakamana – we will advocate assertively and constructively with strength and resilience, supporting communities of people living with rare disorders to be similarly empowered

Manawa rahi – we will steadfastly and stout-heartedly maintain an unwavering evidence-based commitment to the RDNZ cause

Kotahitanga – we will exhibit unity, cohesion and collaboration both internally as a team of staff and volunteers, and externally in our relationships both with New Zealand's rare disorders community and supporters, and internationally.

Tiakitanga – we will do everything we can to sustain, protect and respect the viability and reputation of our organisation, our fellow team members, those living with rare disorders and the physical, cultural and spiritual environments which sustain us

CHAIR'S REPORT

The past 12 months have been packed with challenges for our team, but it is pleasing to look back on how well we've overcome them; how we've innovated, advocated, and thrived in the face of them. Healthcare, education, and disability support have all been under pressure, and many services have been reduced. Vulnerable populations such as people living with rare disorders tend to suffer first - and most, under these conditions, and so our staunch advocacy at such a time is critical.

What has that advocacy looked like? Our team has made submissions to parliament on multiple bills including most recently the changes to the Pae Ora legislation; they have established a Clinical Advisory Panel; carried out regular engagement with Pharmac, Manatū Hauora | Ministry of Health, and Te Whatu Ora | Health NZ; set up a Cross-Party Parliamentary Group on Rare and Undiagnosed Disorders; and ushered a Rare Disorders Research Network into being.

Throughout these workstreams I see a clarity of purpose and exemplary representation for rare, whether that is in the constant advocacy for the Rare Disorders Strategy to be implemented, in the effective media representation and fundraising throughout Rare Disorders Month, or in the regular hui the team holds with support groups. In this work they have been building trust, awareness, and momentum at every step.

We achieved these because we are not only working to support rare people, we are rare people. This was certainly how I felt as I was introducing the Governor General, Her Excellency The Rt Hon Dame Cindy Kiro at our awards ceremony in March. What a privilege it was to attend that fine event, and to be able to say thank-you, publicly, to all of the advocates who raise their voices for rare.

In recognising the year's achievements, I would of course like to note the leadership of our Chief Executive Chris Higgins. Not only has Chris led the establishment of the research and advisory groups, with the rest of the RDNZ team he's also relentlessly pursued the implementation of the Rare Disorders Strategy. In June he embarked on an overseas tour to establish stronger connections with rare disorder groups and researchers around the globe.

If that wasn't enough, he was then appointed to the Pharmac Consumer and Patient Working Group, which is a panel established by Pharmac as a response to the Kerry Prendergast report, itself derived from workshops held in late 2024 where I was fortunate to represent us.

Chris has also played an instrumental role in ensuring we hold true to our vision of becoming a Te Tiriti organisation. Our board has a deep commitment to embedding Te Tiriti principles in our kaupapa, and in doing so to become more inclusive for all. I want to thank our trustees Sam La Hood and Awhina Hollis for their work in this area.

We were sad to see the departure of Maurice Roberts from the team after three and a half years of service. His ability to find funding for us, and his down-to-earth advice, have been essential to our growing profile and range of initiatives. I'll miss his pragmatism, warmth, and humour and we wish him well for the future.

I must also at this point note my own departure. I am, unfortunately, stepping down as of the AGM so that I can navigate a permanent move to Australia. As is often the way with volunteering I feel I have gained far more from being involved with Rare Disorders NZ than I've ever given, an involvement that gave me purpose at a time when, after my diagnosis, I really needed it. I've met some incredible people and have been privileged to share in their stories. Serving in this role has been special to me, and I'm thankful to everyone that has helped along the way (and there have been many of you).

I am filled with great optimism for the future. The board of trustees will be in excellent hands as Ariane Tuapola takes over the role of Chair, supported by Deputy Chair Awhina and our other amazing trustees. This is a good thing as the coming year will also present plenty of challenges. We will need to draw upon our collective wisdom and strength if we are to see our vision for the health and wellbeing of people and whānau living with rare disorders in Aotearoa become a reality. But that is what makes us special: we are rare, but we are together, and together we will create lasting change.

James McGoram

CHIEF EXECUTIVE'S REPORT

The past year has truly been a year of two halves for Rare Disorders NZ (RDNZ). The first half began with a significant milestone: the publication of New Zealand's first Rare Disorders Strategy (RDS) by Manatū Hauora | Ministry of Health in July 2024. This achievement followed over fifteen years of RDNZ advocacy and a year of intensive consultation with the rare disorders community.

While the release of the Strategy was a cause for celebration, it was tempered by frustration at the lack of commitment to its implementation from Manatū Hauora, Te Whatu Ora | Health New Zealand, and the Minister of Health. In response, RDNZ stepped up to fill the leadership gap, guided by the Strategy's themes and our own priorities.

Personal highlights included commissioning Costello Medical to analyse Māori and non-Māori responses to our 2023 Voice of Rare Disorders survey, through the lens of Te Aka Whai Ora Whānau Voice 2023 report. This work underlines our commitment to ensuring that no one is left behind as the RDS is implemented.

We also engaged with Pharmac's new leadership, including Associate Minister of Health Hon David Seymour and Chair Hon. Paula Bennett, who have shown a commitment to implementing the RDS. While funding and a specific assessment pathway for rare disorder medicines remain challenges, building a valued collaborative relationship with Pharmac based on trust, empathy and respect is an essential initial step.

Another highlight was learning about the potential for whole genome sequencing (WGS) to transform rare disorders diagnoses.

RDNZ is represented on a Liggins Institute reference group trialling WGS for unwell newborns at Starship Hospital, contributed to Te Whatu Ora's draft genomics strategy, and showcased WGS at our successful parliamentary roundtable event in May. We also welcomed the two main New Zealand WGS suppliers as members of our Round Table of Companies.

The second half of the year heralded RDNZ's silver jubilee, celebrating 25 years of advocacy and support. Rare Disorders Month provided an opportunity to celebrate our achievements at Government House, honouring contributors such as founder John Forman, the first recipient of our Lifetime Achievement Award. Special thanks go to our patron, Her Excellency, The Rt Hon Dame Cindy Kiro, for hosting and presenting awards.

Rare Disorders Month also saw the Rare Disorders Research Network agree to establish an RDNZ hosted National Mirror Group, a step toward joining the European Rare Diseases Research Alliance.

Financially, the year was tough, requiring us to draw more on reserves than planned. Despite this, the dedication of our staff—Alex, Alanna, Angela, Gemma, Lewanna, Kim, Maurice, and Susan—ensured we continued to make good progress in implementing our annual plan. Thank you.

I also want to thank our many donors and financial supporters, RDNZ's support group leads who consistently volunteer their time and lived expertise which adds so much value to the work that we do, and members of our Clinical Advisory Panel who, supported by Chair Professor Stephen Robertson, have also been very generous with their knowledge, time and expertise.

In closing I must gratefully acknowledge the support of RDNZ's Board of Trustees. Board Chair, James McGoram has been great to work with, and I'll miss his support as he steps down from the role in September. I've also very much valued working with Awhina Hollis as tangata whenua deputy chair, and particularly her guidance as we've progressed on our journey of becoming a better Te Tiriti partner.

Chris Higgins

Celebrating Achievements, **The year in numbers**

654
enquiries answered

7
new support groups joined

27
meetings with alliances, networks and advocacy groups

24
meetings with government officials and MPs

42+
meetings/events with support groups

14
submissions on policy proposals

50+
news media pieces throughout the year

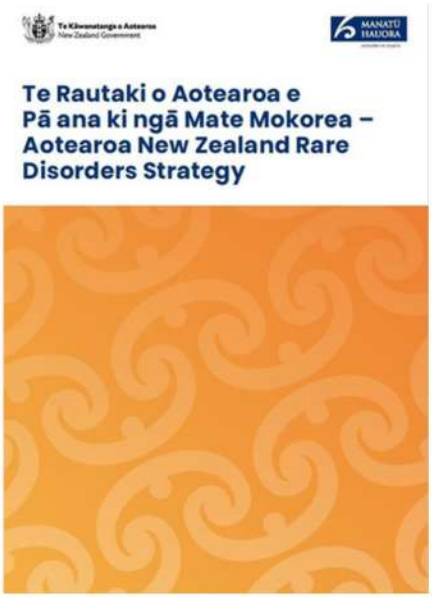
900
new social media followers

Campaigning for full implementation of New Zealand's Rare Disorders Strategy

With the Aotearoa New Zealand Rare Disorders Strategy (RDS) launching in July 2024 Rare Disorders NZ's advocacy work for the year ahead was clear – ensuring that the agencies responsible for implementing the RDS developed an action plan for implementation.

The RDS stipulates the agencies responsible are:

- Manatū Hauora | Ministry of Health
- Te Whatu Ora | Health New Zealand
- Te Pātaka Whaioranga | Pharmac
- Te Tāhū Hauora | the Health Quality & Safety Commission



Early on we endeavoured to gather the government agencies tasked with implementing and monitoring the Strategy to discuss implementation.

This was however, unsuccessful due to Te Whatu Ora | Health New Zealand declining to engage while undergoing a 'reset'. This led to Manatū Hauora | Ministry of Health unwilling to engage until Te Whatu Ora | Health New Zealand was ready.

Despite continued correspondence initiated from Rare Disorders NZ to relevant officials as well as the Minister of Health throughout the year, an action plan has yet to be developed. This has been incredibly disappointing for the rare disorder community.

Rare Disorders NZ therefore took matters into our hands to get traction.

RARE DISORDERS NZ-LED INITIATIVES TO IMPLEMENT THE STRATEGY

Petition

We channelled the rare disorder community's collective frustration at the lack of action into a petition calling for urgent action. Manatū Hauora | Ministry of Health invested two years developing the RDS and without implementation, it is not worth the paper it's written on. The petition will remain open until March 2026 or until an action plan for implementation is developed by the implementing agencies.

Roundtable on diagnosis and access to rare disorder medicines

Rare Disorders NZ hosted a roundtable at parliament on diagnosis and access to rare disorder medicines with executives from Manatū Hauora | Ministry of Health, Te Whatu Ora | Health NZ, Pharmac, specialists, industry, parliamentarians and consumer representatives. Both the Minister of Health and Minister responsible for Pharmac also attended and spoke to the plenary.

Data and Digital

We initiated discussions with Data Standards at Te Whatu Ora | Health NZ about rare disorder coding in the nationwide Shared Digital Health record. Primary care software increasingly supports SNOMED CT, which is capable of capturing rare disorders information. ORPHAcodes have been added to SNOMED CT, which are internationally recognised codes for over 6,500 rare disorders. We would like to see 100% adoption of SNOMED CT across primary, secondary and tertiary care and we will continue to advocate for the capability and requirement to record a patient's specific rare disorder in their health record.

Pharmac

We have made significant progress improving consumer input in Pharmac's assessment processes and successfully advocated for specialists regularly getting in front of the Rare Disorders Advisory Committee to speak from the clinical perspective of the value of rare disorder medications being considered.

We have also been working closely with Pharmac to update their definition of a rare disorder. Up until now, Pharmac only recognised a disorder as being rare if it affects fewer than or equal to 1 in 50,000 persons - a definition usually reserved for ultra-rare conditions. With the release of the Rare Disorders Strategy New Zealand had for the first time an official definition of what constitutes a rare disorder, and Rare Disorders NZ has been working to ensure Pharmac's definition aligns.

RARE DISORDERS NZ-LED INITIATIVES TO IMPLEMENT THE STRATEGY

Te Whatu Ora | Health NZ National Clinical Networks

Rare Disorders NZ initiated discussions with the National Clinical Networks team at Te Whatu Ora | Health NZ about how a Rare Disorder Reference Group at Te Whatu Ora could be progressed as a step towards a leadership and coordination mechanism for rare disorders in the health system.

The National Clinical Networks team have been given the green light to establish such a reference group, though currently with no additional funding or resources. We will continue to engage to support the establishment of the group and advocate for committed resourcing to further advance this initiative moving forward.

Establishment of Cross-Party Parliamentary Group on Rare and Undiagnosed Disorders

To strengthen our advocacy work, we successfully established New Zealand's first Cross-Party Parliamentary Group on Rare and Undiagnosed Disorders (CPGRD).

Members of Parliament from across the political spectrum came together for the first meeting in March, hosted by Hon. David Seymour, Associate Minister of Health. National MP Hamish Campbell was elected Chair of the group, and Labour MP Hon. Dr. Ayesha Verrall MP was elected Deputy Chair.

The purpose of the group is to increase awareness of rare and undiagnosed disorders within the New Zealand Parliament and support policies and initiatives that enhance equity, wellbeing, and social inclusion for people living with rare or undiagnosed disorders, and their families. The group will hold quarterly meetings with Rare Disorders NZ providing secretariat support.

RAISING AWARENESS AND UNDERSTANDING OF RARE DISORDERS & STRENGTHENING RDNZ'S PRESENCE AS THE COLLECTIVE VOICE FOR RARE

With 2025 being Rare Disorders NZ's 25th year we took the opportunity to celebrate with an awards ceremony on Rare Disease Day hosted by our patron Her Excellency The Rt Hon Dame Cindy Kiro at Government House in Wellington.

Support group leads, representatives from partner organistions, officials, past and present board members and staff and other stakeholders came together to celebrate how far we have come over the past 25 years.

The following individuals who have made a significant difference to the health and wellbeing of people living with rare disorders in Aotearoa New Zealand were recognised:

Lifetime Achievement Award – John Forman, founder of Rare Disorders NZ
Outstanding Advocacy Award – Sue Haldane, parent and patient advocate
The Collaborative Leadership Award - Denise Astill and Jacki Morris from Foetal Anti-Convulsant Syndrome New Zealand
Rare Disorders Research Award - Professor Stephanie Hughes of Otago University

GLOW UP AND SHOW UP FOR RARE DISORDERS

RARE DISORDERS MONTH

Our annual Rare Disorders Month campaign once again rallied Aotearoa to *Glow Up and Show Up* during March for the rare disorder community to help us bring rare disorders out of the darkness and into the light.

Reaching every corner of the motu, schools, workplaces and community groups showed up through fundraising events; craft breweries got behind the Rare Beer Challenge; buildings lit up; health professional students reflected on how they could contribute to improving the wellbeing of rare, and rare disorder researchers connected.

FORTUNE FAVOURS RARE BEER CHALLENGE 2025

Rare Disorders NZ was once again blown away by another outstanding Rare Beer Challenge run and hosted by Fortune Favours to raise funds and awareness for Rare Disorders NZ.

Now in it's fifth year, 14 craft breweries entered, all vying for the title of Rare Beer Champion. Over $8,000 was raised, and each participating brewery did an amazing job of raising awareness of rare through their creative social media campaigns.

35

local events held around the motu for rare disorders

44

media pieces on rare disorders

51

buildings/monuments lit up for rare

Spotlight

ESSAY COMPETITION FOR STUDENTS

The greatest challenge? Combating complacency in a stretched health system. Rare disorders are easy to deprioritise—until they're personal.

A poignant phrase written by 6th year medical student Yuhan Chi in her winning entry to Rare Disorders NZ's essay competition during Rare Disorders Month.

For the past two years, Rare Disorders NZ has run an essay competition for health professional students during Rare Disorders Month to encourage students to reflect on, engage with and learn about rare disorders.

This year, 18 entries were received, responding to the following essay prompt:

> *In 500 words or less, consider how you see yourself contributing to one or more of Rare Disorder NZ's seven strategic priorities to improve the health and wellbeing of people living with a rare disorder. Reflect on what you think might be your biggest challenge in this area over the next 25 years of your career and how you will overcome it.*

Yuhan Chi's essay, Weaving a Whāriki of Hope: *My Vision for Rare Disorder Care in 2050, Aotearoa* stood out due to Yuhan's thoughtful reflections of the patient experience when living with a rare disorder, and her drive as a future clinician to improve the patient experience.

Yuhan is truly grateful to have won first place, and says it is an honour to contribute to raising awareness of rare disorders and to reflect on how, as future clinicians, she and her peers can make a difference.

RARE DISORDERS RESEARCH NETWORK

Following the success of our inaugural Rare Disorders Research Network event in early 2024, we formally established the network leadership group led by co-chairs Rare Disorders NZ Chief Executive Chris Higgins and Associate Professor Phillip Wilcox of University of Otago.

Currently sitting at over 50 members and growing, the research network enables researchers working with rare disorders to connect, share learnings and explore opportunities for collaboration.

A second event was held during Rare Disorders Month in 2025 with two themes comprising development of a rare disorders research strategy, and affiliation with the European Rare Diseases Research Alliance (ERDERA) through forming a New Zealand National Mirror Group.

CLINICAL ADVISORY PANEL

Rare Disorders NZ advocates on a wide range of issues on behalf of people living with one or more of over 7,000 disorders. It is imperative we understand the issues we advocate on correctly and that our messaging is based on accurate knowledge and evidence.

To support this intent, we were pleased to establish a Clinical Advisory Panel, consisting of an interdisciplinary team of experts who are willing to donate their time to offer advice to the team to help inform our decision making and messaging.

STRENGTHENING RDNZ'S PRESENCE AS THE COLLECTIVE VOICE FOR RARE DISORDERS IN NZ

ENQUIRIES LINE

Central to Rare Disorders NZ's work is providing an enquiries line for the rare disorder community, offering guidance and navigation through the health and social systems.

> *"Thank you so much for the speedy reply. Your email was full of great advice which I have taken onboard."*

Our team has in-depth understanding of the unique challenges faced by those living with rare disorders and has excellent knowledge of supports available. We are also often able to connect people with others with the same condition, which for many has a big impact on their wellbeing.

> *"Thank you so much this is very helpful & yes I would really like to talk to someone else who has this if possible. Really appreciate this."*

654
enquiries answered

154
support groups in our collective

7
new support groups joined the collective

CONSULTING ON THE LIVED EXPERIENCE

We regularly consult with our support group collective to help inform our advocacy work, and often prior to meetings with officials or when writing submissions on policy proposals to ensure we are always guided by lived experience.

42+
meetings/events with support groups

24
meetings with government officials & MPs

14
submissions on policy proposals

BIENNIAL SUPPORT GROUP LEAD HUI

On 2 August Rare Disorders NZ hosted our biennial Support Group Lead hui in Wellington. It was an opportunity for support group leads from around the country to get together, share learnings and come away with new tools to help support their roles.

Most of the support group leads voluntarily run their support group while managing their own condition or caring for someone with a rare condition. Through their invaluable efforts they create a sense of community for those feeling alone and misunderstood, offering somewhere for people to connect, ask questions and share information.

At the hui the group leads received an update on the most recent developments on the Rare Disorders Strategy from Rare Disorders NZ, and also from Te Whatu Ora regarding the next important stage – implementation of the Strategy.

"Fantastic opportunity to network and meet the other support leads."

The next sessions offered some upskilling to help support attendees in their roles as leads of support groups, including media training for advocacy purposes and building compassion resilience.

The day concluded with a Q & A session with RDNZ board members.

"It's fantastic and so worthwhile. You always get so much from it. The speakers are all very different."

BECOMING AN EFFECTIVE AND RELEVANT TE TIRITI PARTNER

A MĀTAURANGA MĀORI LED APPROACH TO IMPLEMENTATION OF THE RDS

As we work to get the Rare Disorders Strategy implemented, we will be driving the call for a mātauranga Māori led approach to implementation. Taking this deliberate approach will help to ensure Māori living with rare disorders will not be left behind when systemic improvements are implemented. Getting systems right for Māori living with rare disorders means getting it right for everybody.

To support this work, RDNZ has commissioned Costello Medical to undertake a more detailed comparison of the Māori vs non-Māori results from our 2023 survey, and to include the findings of Te Aka Whai Ora Whānau Voice 2023 Summary report.

On our journey to become a better Te Tiriti partner, the Rare Disorders NZ team, including the board, participated in an externally facilitated workshop, exploring our values and promoting mutual understanding and expectations for operating as a better Te Tiriti partner.

MĀORI RESEARCH AND CLINICAL EXPERTISE

We have stipulated in the Terms of Reference of the Rare Disorders NZ's Research Network leadership group that it must be co-chaired by a Māori rare disorder research specialist. Associate Professor Phillip Wilcox of University of Otago holds the position.

Similarly, we are seeking a co-chair for our Clinical Advisory Panel with kaupapa Māori expertise.

INCREASING MĀORI REPRESENTATION ON THE BOARD

We have welcomed new board member Samantha La-Hood who will work with fellow board member Awhina Hollis on the establishment of a Māori Advisory Panel that can support and provide advice to the team.

ENSURING RDNZ'S LONG TERM FINANCIAL AND REPUTATIONAL VIABILITY AND SUSTAINABILITY

FUNDRAISING MANAGER JOINS THE TEAM

With the funding landscape for charities becoming increasingly precarious, Rare Disorders NZ appointed Fundraising Manager, Gemma Jackson, in mid-2024 to generate and maximise revenue from individual giving and other donations.

Rare Disorders NZ has not had a strong fundraising programme in the past, and while it takes time to build such a programme up, it was heartening to have raised over $10,000 in our first two major appeals.

ROUND TABLE OF COMPANIES

The Round Table of Companies provides an important forum to foster transparent working relationships with industry and encourage open communication between industry and the rare disorder patient community. We were thrilled to welcome new members Chiesi, Novartis, Oxford Nanopore Technologies and Illumina this year.

Thank you to members for your continued engagement - Alexion, Amicus, Biogen, BioMarin, Chiesi, Illumina, MSD, Novartis, Takeda, Vertex, Oxford Nanopore Technologies

THANK YOU

We are extremely grateful to the following foundations and organisations who continue to support our important work for the rare disorder community, despite the financially challenging times that continue to be felt across the board.

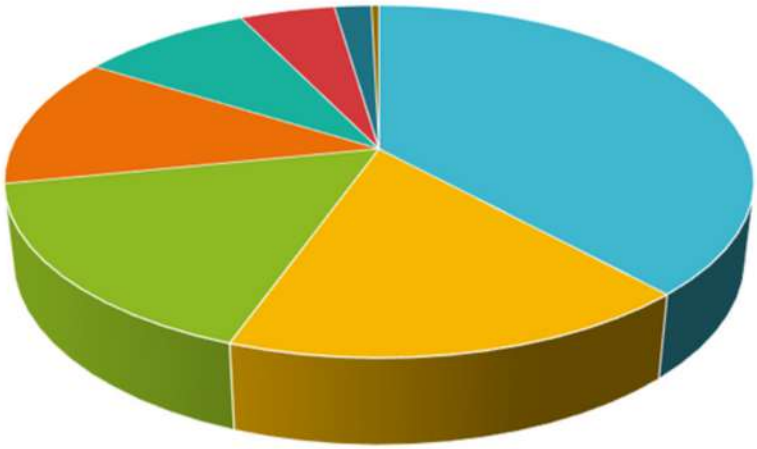
One Percent Collective
Pub Charity Ltd
Hugo Foundation
COGS Committees
Rare Disease Month sponsorship: BioMarin, Takeda, Alexion, Vertex
Parliamentary Roundtable sponsorship: Alexion, Biogen, Vertex
Bluesky Community Foundation
The Lion Foundation
Trust House Foundation
TG Macarthy Trust
One Foundation
Frimley Foundation
Aotearoa Gaming Trust
Four Winds Foundation
Kiwi Gaming Foundation
EM Pharazyn Trust
FH Muter Trust
Foundation North
Grassroots Trust Central South Canterbury Trusts

FINANCIAL RESULTS

Summarised statements of financial performance for year ended 30 June 2025

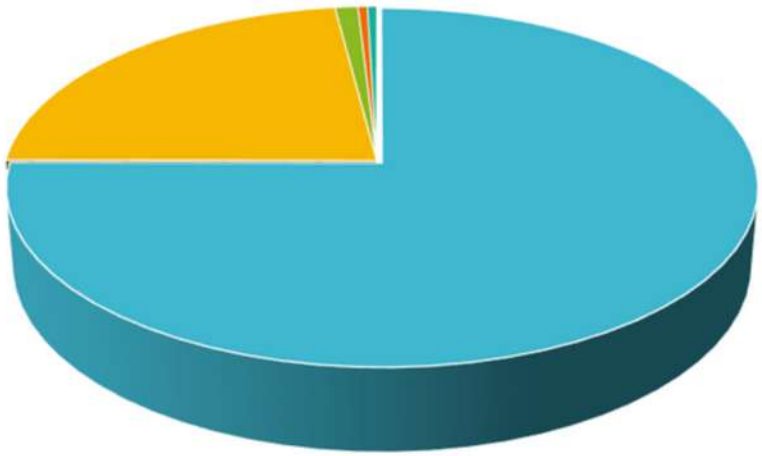
	2025	2024
	$	$
General grants	165,622	242,437
Revenue from commercial activities	74,500	63,728
Government service delivery grants/contracts	71,098	103,541
Donations and fundraising	52,284	63,869
Sponsorship	38,500	0
Interest	21,251	25,668
Non-government service delivery grants/contrac	8,260	9,870
Capital grants	1,716	0
Total Revenue*	**433,231**	**509,113**
Operating Expense		
Employee remuneration and related expenses	432,455	389,696
Service delivery	131,939	117,800
Other expense	6,459	8,987
Commercial activities	2,667	0
Fundraising	2,648	937
Total Expenses*	**576,167**	**517,420**
Surplus/(Deficit) for the Year*	**-142,937.00**	**-8,306**

Income Y/E June 2025



- General grants
- Revenue from commercial activities
- Government service delivery grants/contracts
- Donations and fundraising
- Sponsorship
- Interest
- Non-government service delivery grants/contracts
- Capital grants

Expenses Y/E June 2025



- Employee remuneration and related expenses
- Service delivery
- Other expense
- Commercial activities
- Fundraising

STAFF

Chris Higgins - Chief Executive
Kim McGuiness - Relationship Manager
Angela Nielsen - Communications Manager
Lewanna Pentecost - Navigator
Susan Langston - Finance Manager
Maurice Roberts - Business Development Manager
Gemma Jackson - Fundraising Manager
Alexandra Nicholson - Administration Officer
Alanna Peck - South Island Coordinator

BOARD MEMBERS

James McGoram - Chair
Awhina Hollis - Deputy Chair
Rosemary Marks
Bice Awan
Martin Hanley
Ariane Tuapola
Samantha La-Hood
Nivedita Sharma Vij