

RARE DISORDERS NZ

**ANNUAL
REPORT
2021/2022**



HIGHLIGHTS 2021/2022



Fair for Rare call heard - Government commits to develop a Strategy for Rare Disorders

RDNZ and its collective's persistent Fair for Rare call for a national framework for rare disorders was firmly acknowledged, with the Government recognising the Pharmac Review's recommendation to develop a strategy for people living with rare disorders.



New Zealand adopts UN Resolution on "Addressing the Challenges of Persons Living with a Rare Disease and their Families"

In December 2021, 193 countries, including New Zealand, adopted the first UN resolution that acknowledges the specific challenges of people living with a rare disease and their families.



RDNZ carries out largest-ever survey on rare disorders in New Zealand

In November 2021, RDNZ's bi-annual Voice of Rare Disorders survey, on behalf of Patient Organisations, received 718 responses, making it the largest survey of its kind in New Zealand and filling a major data gap on living with a rare disorder.



New Governor General becomes patron of Rare Disorders NZ

Her Excellency, The Right Honourable Dame Cindy Kiro, GNZM, QSO, Governor-General of New Zealand, accepted RDNZ's invitation to become patron of Rare Disorders NZ.



RDNZ educates health professionals at New Zealand's largest GP conference

For the first time, Rare Disorders NZ was invited to run a workshop at the General Practice Conference & Medical Exhibition to educate health professionals on how best to support patients living with a rare disorder.



Rare Beer Challenge fundraiser biggest yet

A record 18 breweries were involved this year in the now annual Rare Beer Challenge fundraiser for RDNZ, hosted by craft brewery Fortune Favours. The event helped to raise awareness of RDNZ through a Stuff article and TVNZ Breakfast live-cross, and over \$10,000 was raised.



Supporting a growing collective

Over collective has grown to 138 patient support groups, ranging from 2 – 3 members per group to 450 members.



Enquiries line

We responded to 540 enquiries over the year - an increase of 36% from the previous year.

CHIEF EXECUTIVE'S REPORT

Tēnā koutou katoa,

I begin this report with jubilation for the decision made by Minister of Health, Hon Andrew Little, to develop an inaugural 'Strategy for Rare Disorders' in Aotearoa.

This fantastic achievement follows years of advocacy from previous leaders of our charity, our dedicated team members, our board trustees and particularly all the courageous rare disorder leaders who spoke out to share their stories, start petitions and demand equity. This acknowledgment by government and resulting actions can ensure that the transformed health system will include the needs of those with rare disorders, by creating appropriate pathways, mechanisms, and inclusive practices. A strategy enables the collective voice to be heard, and there can be clarity of the combined challenges that impact those with rare disorders and the reasons why the system often fails them. The time for inclusion and equity for the population with rare disorders has begun!

Reflecting on the past year, there has been incredible energy and dedication to lift awareness of the specific needs facing persons with rare disorders, and the word that resonates most is Kotahitanga, Unity, as ultimately each one of us are threads within the larger web of life, and what affects one, affects all.

This unity has been demonstrated with growing public support and with the adoption of the first ever United Nations Resolution to 'addressing the challenges of persons living with a rare disease and their families' in December.

The independent review into Pharmac called for a unified response from all those hoping for access to effective innovative medicine treatments for smaller populations within a system no longer fit for purpose. This led to a robust independent review spelling out discrimination and inequities for those with rare disorders in New Zealand and recommending solutions. Next comes engagement and action in an exciting phase of hope for investment in medicines that reduce the burden economically, socially, and morally.

The Ministry of Education's Highest Needs Review has provided a collaborative engagement model to address inequities founded in the acceptance of the intertwining between social determinants of health and the need for a more flexible, and nuanced approach for children who may not tick boxes, yet have specific needs.

It has been a year of change with a focus on health equity, on opportunities to do things better with inclusion at the core, so no one is left behind. The question on many lips is how government investment will be actualised, how it will partner and collaborate with all key stakeholders to get the outcomes and improvements it seeks.

As I step back from my CE position with Rare Disorders NZ I remain fully committed to supporting a brighter future for those on the fringes.

Lisa Foster
Chief Executive

BOARD CHAIR'S REPORT

Kia ora koutou.

The past 12 months have been tumultuous. We've seen some seismic shifts in the way healthcare is delivered in Aotearoa, we've lived through another year of the pandemic which has isolated so many in our vulnerable community, and we've seen a great deal of media focus on the challenges of those living with chronic illness or disability. It is with pride that I reflect on how well RDNZ has navigated this challenging time.

We've made great use of the opportunities available to amplify the voice of our community - whether through media and public events (such as during the Rare Beer event) or through our strong appearance at the GP Conference earlier this year. Coupled with an excellent article by two of our trustees in NZ Doctor magazine, and multiple TV and radio interviews and news articles throughout the year, we have successfully grown the profile of both RDNZ and rare disorders in the national consciousness.

This work has also made our advocacy carry more weight. Encouraging effective advocacy is a core focus for the board, and we have seen some amazing work being done towards removing systemic barriers to healthcare, treatment, recognition and support for rare.

Perhaps the biggest example was the large amount of space given to rare disorders in the Pharmac Review; a testament to the strong advocacy of many - our staff, our support group leads, and members of our board among them.

The ripples caused by this report put us in an excellent position to advocate for substantial reform – beginning of course with the development of a strategy for rare disorders which the Minister has now instructed the Ministry of Health to develop.

These big picture developments are encouraging, but there has been underlying anxiety about our sustainability as an organisation in light of the continued paucity of government funding. The good news is that we have been able to diversify our revenue (thanks in large part to our skilled grant-writer), set some ambitious targets, and continue our work with the support groups who make up our collective. We have taken on new staff who have all performed admirably and who have engaged with our vision and purpose, and we now have a plan for an expanded public awareness and fundraising campaign for rare disease day next year.

It is therefore with some sadness that we farewell our remarkable Chief Executive Lisa Foster after more than five years of dedicated service. Lisa, you have done an incredible job and have laid the foundations for much success to come. We also farwelled one of our trustees earlier this year, Joanna Lusk. Jo has given incredible service to RDNZ over many years, including being board Chair.

I have great confidence that RDNZ increasingly has the systems, the vision, and most importantly the people to achieve our goals for the coming year. Our past success shows us just how much we can achieve.

James McGoram
Chair.

Mission Statement

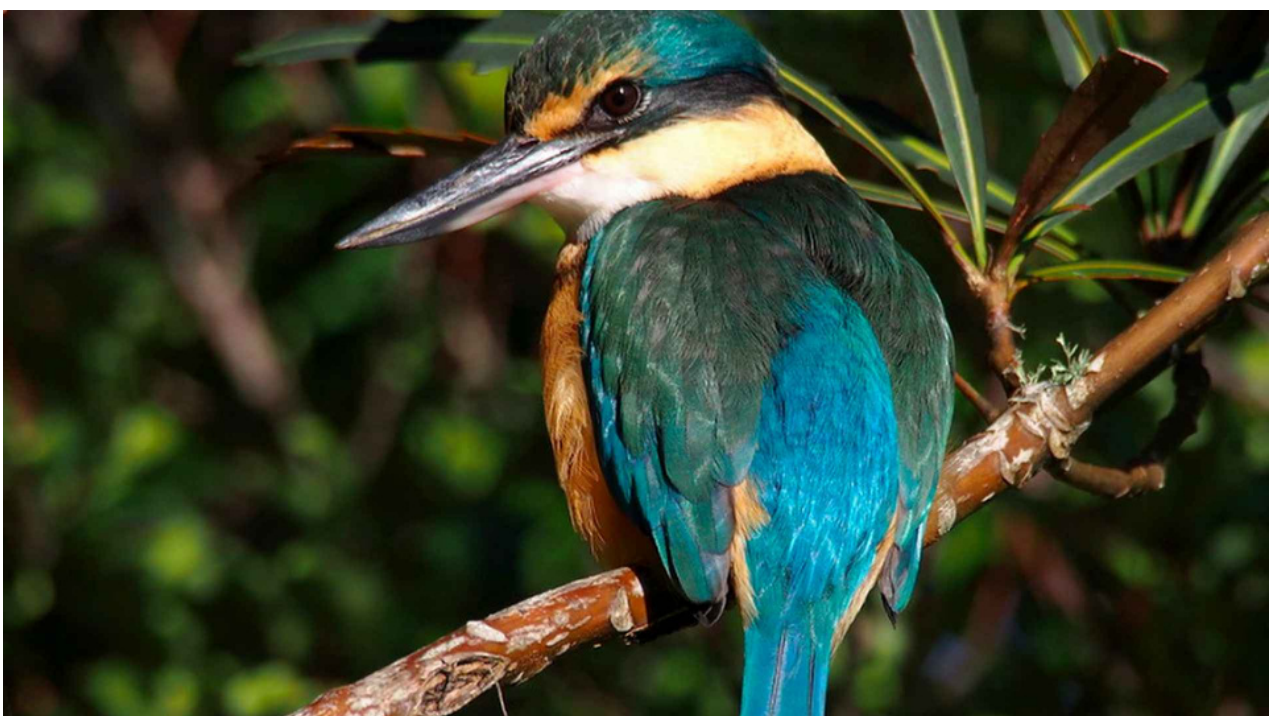
Amplify the collective voice to improve healthcare and wellbeing for people and their whānau living with a rare disorder

Our Organisation

The New Zealand Organisation for Rare Disorders (NZORD) was established in September 2000, and changed its name to Rare Disorders NZ (RDNZ) in 2019. RDNZ is the respected voice of rare disorders in New Zealand.

We are the only national organisation specifically focused on supporting the 300,000 New Zealanders who live with a rare condition and the people who care for them.

RDNZ is the connector hub for families, health professionals, researchers and Government. We provide a strong common voice to advocate for health policy and a healthcare system that works for all people with rare conditions.



RDNZ Strategic Plan

RDNZ's work plan focuses on four strategic aims:

1. Connecting all those in the rare disorder community to ensure improved outcomes, including mental health and wellbeing;
2. Campaigning for a New Zealand National Rare Disorder Framework;
3. Raising awareness and understanding of rare disorders and strengthening our presence as the collective voice for rare disorders in New Zealand;
4. Ensuring RDNZ is financially viable and sustainable long-term.

These aims are underpinned by RDNZ's core value of Mahi Tahi (collaboration, cooperation, joint ventures).



1

Connecting all those in the rare disorder community, to ensure improved outcomes, including mental health and wellbeing

Living with a rare disorder can feel incredibly isolating. We are proud to provide a space for people to turn to for guidance and advice. We empower individuals and support groups to get their voices heard to stimulate meaningful systemic change, and to engage in advocacy initiatives to improve greater awareness and understanding of living with a rare disorder. We also build connections between patients, health professionals, researchers and others with a shared interest in this area.

Enquiries

Our enquiries line is the first direct point of contact for most, but we continue to see growth in the number of followers on our Facebook page, and also in visits to our website. Through our website, social media platforms and our bi-monthly newsletters we provide centralised, up-to-date information on a wide range of rare disorder news and developments; the only national organisation in Aotearoa New Zealand to do so.



The growing number of enquiries we receive through the website and over the phone reflects increasing awareness of RDNZ and the support we offer. We have particularly noted an increase in requests for help with how to get a diagnosis, and of people looking for rare disorder specialists.

Our Collective

We have a collective that is strong, unified, engaged and growing. Currently at 138 groups, these groups range from 2 – 3 members to 450 members. Over the past year we have welcomed 22 new groups to our collective, strengthening our collective voice and broadening our reach.

We also bring together groups with common interests and needs, such as medicines access or rare cancers, to share experiences, concerns and collaborate on ideas for creating lasting change in the system. Having this direct connection to such varied and diverse disorders confirms our crucial position as the central connector hub for all New Zealanders living with a rare disorder.

“

Fragile X New Zealand has appreciated working alongside RDNZ during the year to improve the lives of people living with rare disorders like Fragile X syndrome. It has been helpful to work as a collective, particularly around making submissions on legislation, including significant legislation like the Pae Ora (Healthy Futures) Bill. We appreciate the ongoing support from the team at RDNZ and to be part of a collective voice of rare disorders.

Andrea Lee, Executive Director, Fragile X New Zealand

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Māori Engagement Officer

In late 2021, we employed a Māori Engagement Officer to improve our engagement with the Māori community, and to better understand how we can most effectively support the Māori rare disorder community. This has led to a new private Facebook group for Māori who live with a rare disorder and their whānau, and already we are developing new relationships with Māori whānau.

138

rare disorder
support groups

collectively reaching over

30k

people living with a
rare disorder

representing over

1,600

different rare disorders

International Children's Day

To mark International Children's Day, RDNZ ran a colouring competition for children from the rare disorder community and asked them to include messages on their artwork to the Prime Minister about their experiences and challenges, along with their hopes and dreams.

The pictures were presented to Labour MP Dr. Liz Craig, to deliver directly to Prime Minister Ardern to tell her what it means to live with a rare disorder.



2

Campaigning for a New Zealand National Rare Disorder Framework

“ 'A strategy for rare disorders needs to be developed' – Minister of Health Andrew Little. ”

It was a watershed moment in June for the rare disorder community to hear the Minister of Health announce that a strategy for rare disorders is needed, and that he would be instructing his officials to engage with those with lived experiences to develop one.

This announcement followed the release of the Pharmac Review, which emphasised New Zealand's lack of a coordinated approach to rare disorders as a major challenge for the community, and endorsed RDNZ's call for a National Rare Disorder Framework, including the seven strategic priorities identified.

How the Ministry will go about developing the strategy and what it will include is yet to be seen, but the announcement was a significant step in the right direction and an acknowledgement to all those who have fought so hard for recognition, that their message has been heard. Getting to this point has taken years of advocacy work, petitions, submissions and meetings with decision-makers at the highest level. Some key pieces of work RDNZ has done in this space over the past year are outlined below.

Submissions

RDNZ always ensures the rare voice is heard in any proposed initiatives, changes to legislation or matters before select committees that are relevant to our community. Alongside our own, RDNZ also supports others in the rare community to make submissions.

Submissions and feedback we have delivered in the past year:

- [Health Select Committee Submission on petition to develop a National Rare Disorder Framework](#)
- [Submission on content for the 2023 New Zealand Disability Survey](#)
- [Submission on Pae Ora \(Healthy Futures\) Bill](#)
- [Submission and inclusion within Ministry of Education Highest Needs Review](#)
- [Submission to the Mental Health and Wellbeing Commission](#)
- [Feedback to the Ministry for the Environment Plastic Straw Phase Out Review](#)
- [Feedback to Pharmac on access to COVID-19 anti-viral medications](#)

Engaging with Government

As the only national organisation representing all New Zealanders who live with a rare disorder and the people who care for them, government agencies benefit from the wealth of knowledge and expertise we have on living with a rare disorder in Aotearoa from our strong and engaged collective. We utilise every opportunity to share this knowledge and engage at the highest level to ensure the rare voice is heard.

Over the past year, our meetings with government entities have included:

Health and Disability Review Transition Unit

The announcement of the health system reform raised many questions from the rare community around whether the reform would finally lead to inclusion and more equitable health outcomes for those with rare disorders. RDNZ's CE attended a Future of Health workshop led by the Health and Disability Review Transition Unit, and enquired about connection and inclusion of a representative from the rare disorder community to ensure the health plan considered their unmet needs.

Disability Rights Commissioner

In November, support group representatives and RDNZ attended a meeting arranged by Paula Tesoriero, Disability Rights Commissioner with Ministry of Health Deputy Director General, to explain current system barriers faced by people with rare conditions in relation to access to disability and support services. Further discussion is needed on inclusion within disability for those with rare disorders.

Pharmac meetings

RDNZ has quarterly meetings with members of the Senior Leadership Team at PHARMAC to ensure a continuing dialogue with the sector. We submit queries from our collective and other stakeholders and report back to our networks. During a turbulent period of review for Pharmac, we have consistently and clearly expressed the need for a system which can offer pathways for access to modern medicines, not just for those with rare conditions, but for all New Zealanders.

Ministry of Health

RDNZ's CE facilitated a Ministry of Health-hosted meeting in November with the rare community to give relevant departments in the Ministry the opportunity to learn about the needs of people living with a rare disorder. This meeting was attended by a number of support group leads from the rare community who shared their experiences of living with a rare disorder.

“RDNZ supported us individually and our Pemphigus New Zealand group during Pharmac's consultation process on the proposal to widen access to rituximab for us. They also advocated for us and served as a bridge with Pharmac to help us get answers to many of the queries we had about the decision of approving this medication and understanding the Special Authority for Subsidy. Now this medication has been used by some of our members, improving considerably our quality of life. - Janneth Gil, Pemphigus NZ”

3 Raising awareness and understanding of rare disorders and strengthen our presence as the collective voice for rare disorders in NZ

This year saw RDNZ provide evidence on the unmet needs for those living with rare disorders in Aotearoa, through a white paper based on our 2021 Voice of Rare Disorders Survey. It clarifies the unmet needs and invisibility of the rare disorder population, and offers leaders the opportunity to develop specific cohesive actions within our systems to change this picture.

We have also this year turned our attention to primary health care providers, as they are often the first point of contact for people seeking help with diagnosis, accessing treatment, and requesting information about a condition. The value of having a well-resourced and supported health practitioner who is aware of rare disorders and able to link with key tools to help their patient cannot be under-estimated and is the main priority in the UK National Strategy for Rare Disease. We have been working to help health professionals understand the perspective of the rare patient and ensure they are aware of the multitude of international resources available.

We have also been actively engaged in a number of international events, grown our advocacy assets and further strengthened our relationship with Fortune Favours.

Educating health practitioners

Two of our board members co-wrote a comprehensive article in NZ Doctor, published in October, on how to treat rare disorders, to bring about more awareness among health professionals of rare disorder patients and how to approach these cases.

In June, RDNZ presented a workshop for the first time at the largest annual gathering in New Zealand of GPs at the General Practice Conference and Medical Exhibition. The workshop was the second most sought-after at the conference, highlighting the huge demand for more knowledge in this area. RDNZ also had a stall, and included flyers in delegate bags.

BE RARE AWARE

A RARE PATIENT WILL ENTER YOUR CLINIC SOON

1 IN 17 of your patients has a rare disorder

ABOUT HALF of those affected by rare disorders are children

72% of rare disorders are genetic

You may be the first point of contact for someone at the beginning of their life-changing journey of living with a rare disorder. Your approach can mean all the difference to how their journey is experienced; from getting a diagnosis to accessing the right care.

You do not need to know every disorder, but you can walk alongside your patient, listen, and be open to learn with them. Rare Disorders NZ is here for you and your patient. We are a source of centralised information on rare disorders and can connect you and your patient to relevant resources, networks and support.

VISIT WWW.RAREDISORDERS.ORG.NZ

Voice of Rare Disorder Survey 2021

With no official mechanisms currently collecting data on rare disorders in Aotearoa, there is a major data gap that stands in the way of any meaningful progress for the rare community. As long as the rare community are not being measured, they remain invisible to health plans and budgets.

In an effort to fill this data gap, we carried out our biannual survey of living with a rare disorder in New Zealand. Anyone living with a rare disorder in New Zealand was strongly encouraged to take part, as well as whānau and carers. The survey received 718 responses, surpassing the previous 2019 survey with an overall 149% increase in response, and making it the largest-ever survey of patient-reported outcomes for people with rare disorders in New Zealand.

The results painted a concerning picture of isolation, lack of timely diagnosis, poor treatment access, lack of coordinated care, significant carer impact and for many, being lost in the system.

On Rare Disease Day we released our white paper on the survey results, and we will continue to distribute widely to key stakeholders and use over the coming year in our advocacy work to back our calls for meaningful change.

Key findings from the 2021 survey

- Over half took longer than one year to get a diagnosis, and for one in five, it took over 10 years.
- For 60-75% of people, their disorder makes a number of everyday activities difficult.
- High utilisation of healthcare services, with 81-87% having seen a specialist or GP in the 70 days prior to survey.
- Many have been in hospital over the last 12 months. One in three were in hospital for an average of 13 days, and one in 17 were in ICU for an average of 7 days.
- There were few accessible, effective modern medicines available for the majority of people, with most being used to relieve pain and inflammation.
- 70% had a decrease in income and limited employment options due to their disorder.
- One in three often felt unhappy and depressed and felt they could not overcome their problems.

International Events

Our international connections have continued to grow stronger over the past year, particularly through the number of key global events the RDNZ CE has presented at over the year.

- Rare Disease International's Global Rare Disease Day Online Event at the World Expo in Dubai;
- Asia Pacific APAC Blood Summit;
- The American Rare Disease Day event '24 hours of Rare';
- 2021 RARE Patient Advocacy Summit;
- Pinnacle Workshop and conference in conjunction with Rare Cancer Australia;
- And contributing to a podcast for clinicians in the Asia-Pacific region who treat rare bleeding and neurological disorders (rare epilepsy) where a key topic was Patient Advocacy.

In addition, as a member of the Board of the Asia Pacific Alliance of Rare Disease Organisations (APARDO), our CE has been actively involved in APARDO's work in the Asia-Pacific region, where the focus this year has been on rare disease unity and advocacy across the region.

Speaking at UN event about Spinal Muscular Atrophy

In October, RDNZ was honoured to be invited to speak alongside Patient Voice Aotearoa at an international virtual event to bring attention to Spinal Muscular Atrophy (SMA) – a rare, inherited neuromuscular disease. The event was initiated by the Permanent Mission of Ukraine to the United Nations and attended by senior UN representatives, including the President of the UN General Assembly and Rt Hon. Helen Clark; then President of the Executive Board of UNICEF. Other stakeholders participating included representatives of diplomatic missions in New York, major UN entities such as UNICEF, WHO, UNDP and UNFPA, pharmaceutical company representatives, patient advocacy groups and NGOs.

This event was a significant opportunity for RDNZ to be heard on the world stage; to raise awareness about this devastating disease and the discrepancies in access to treatment.

Collaboration with Fortune Favours

RDNZ is extremely grateful for our growing relationship with local craft brewery Fortune Favours. They now host two annual fundraising events to support RDNZ; the Rare Beer Challenge, and a Tattoo Art Auction with the Wellington Tattoo Convention.

Rare Beer Challenge 2022

The Rare Beer Challenge has built on its momentum to become bigger and better than ever. 18 craft breweries got in behind the event this year, and along with professional judges who offered their time pro bono, and Hills Hats who donated a custom-made trophy hat for the winner, there was a great sense of community support for our work and the people we support. The event was one of Fortune Favours' busiest nights ever, highlighting not only the popularity of the event but also how many people we were able to reach. Over \$10,000 was raised. An article in Stuff, which included an interview with our CE Lisa Foster, and a TVNZ Breakfast live-cross with Chair James McGoram, gave RDNZ further great exposure.

Tattoo Art Auction

Fortune Favours also hosted a Tattoo Art Auction as part of their collaboration with the Wellington Tattoo Convention. Tattoo artists from around New Zealand donated tattoo art, which was auctioned on 16 June at Fortune Favours, with auctioneer Nu Taramai donating his time to the cause. At this event, \$3,500 was raised for RDNZ – another great result and hugely appreciated effort.



4 Ensuring RDNZ is financially viable and sustainable long-term

Government funding

We continue to be in a period of reduced funding from the Ministry of Health with just under \$66,000 a year until 30 June 2023. With the recent announcement of the Government's intention to engage with the rare disorder community to develop a strategy for rare disorders, we expect our funding to be increased to reflect the size of the community we represent and the vital contribution we can make towards the development of a strategy, as the connector hub and expert on rare disorders.

Grant Writer

We employed a Grant Writer in October to source and apply for applicable grants, and to identify new opportunities for grant applications. This has been incredibly valuable for RDNZ, as many of our projects would not have been able to go ahead without grant funding.

We would like to thank the following foundations for supporting our work through grants in the past year:

- The Lion Foundation
- Pelorus Trust
- Trust House Foundation
- One Foundation
- Grassroots Trust Central Ltd
- Aotearoa Gaming Trust
- Kiwi Gaming Foundation Ltd
- Four Winds Foundation
- Lottery Community Grants
- Pub Charity

Round Table of Companies

Our Round Table of Companies (RTC) is a group of pharmaceutical companies with a common interest in rare disorders and orphan drug development. RDNZ set up the RTC to create a forum for a long-term relationship between RDNZ and those companies operating within the health sector that have an interest in rare disorder treatments, drugs, medical devices, food supplements or health services. This relationship is governed by clear policy and principles. The focus is on updates on the current context within New Zealand for equity for rare disorders. The Round Table of Companies holds two meetings annually.

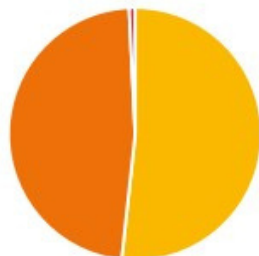
The membership to the RTC provides a valuable source of funding for RDNZ and helps us to carry out activities that are of benefit to rare disorder patients. The RTC members cannot influence in any way RDNZ's policy, positions or decisions, whether explicitly or implicitly.

FINANCIAL RESULTS

Summarised statements of financial performance for year ended 30 June 2022

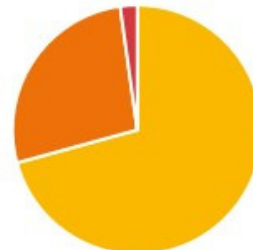
	2022	2021
	\$	\$
Donations and fundraising	178,441	217,224
Revenue from providing goods and services	163,968	102,180
Other revenue	2,619	3,410
Total Revenue*	345,028	322,814
Operating Expense		
Volunteer and employee related costs	208,482	188,153
Costs related to providing goods or services	79,720	46,990
Public fundraising and other expenses	6,470	3,249
Total Expenses*	294,672	238,392
Surplus/(Deficit) for the Year*	50,357	84,422

Income Received Y/E June 2022



- Donations and fundraising
- Revenue from providing goods and services
- Other revenue

Operating Expenses Y/E June 2022



- Volunteer and employee related costs
- Costs related to providing goods or services
- Public fundraising and other expenses

Rare Disorders NZ Team

Staff

Lisa Foster – Chief Executive
Susan Langston – Business Manager
Kim McGuinness – Relationship Manager
Angela Nielsen – Communications Manager
Maurice Roberts - Grant Writer
Julian Laking - Māori Engagement Officer
Lucy Bennett - Administration Officer

Board of Trustees

James McGoram (Chair)
Bice Awan
Carol Gernhoefer
Martin Hanley
Joanna Lusk
Rosemary Marks
Stephen Robertson
Awhina Hollis-English

Contact us

Rare Disorders NZ
PO Box 14-313
Kilbirnie
Wellington 6241
+64 4 385 1119

enquiries@raredisorders.org.nz

www.raredisorders.org.nz