



## **Precision health: Exploring opportunities and challenges to predict, prevent, diagnose, and treat disease more precisely in Aotearoa New Zealand**

### **Public consultation on a topic for a Long-term Insights Briefing**

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**Submitted by:** Rare Disorders NZ

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#### **Declaration of interest**

Rare Disorders NZ works with clinicians, researchers, allied health professionals, academia, government and industry to achieve better outcomes for people with a rare disorder in New Zealand and their whānau. We are funded by grants, donations, fundraising events, Pharma roundtable and a small Te Whatu Ora contract. This submission is in response to Precision Health: Exploring opportunities and challenges to predict, prevent, diagnose and treat disease more precisely in Aotearoa New Zealand, public consultation on a topic for a long-term insights briefing.

#### **Rare Disorders NZ**

Rare Disorders New Zealand is the respected voice of rare disorders in Aotearoa. We are the national peak body organisation, supporting the 300 000 New Zealanders with rare disorders and the people who care for them.

We help those affected by rare disorders navigate the healthcare system, find information and resources, and connect with support groups specific to their condition. We proudly advocate for public health policy and a future healthcare system that works for those with rare disorders – using a strong and unified voice to collaborate with Government, clinicians, researchers, and industry experts, to promote diagnosis, treatment, services, and research.

Our vision is for New Zealand to become a country where people living with a rare disorder are fully recognised and supported with equitable access to health and social care.



**Questions for consultation:**

**1. Do you think precision health is a worthwhile topic to explore in our Long-term Insights Briefing? Why or why not?**

Rare Disorders NZ believes this is absolutely a topic for the long-term insights briefing. It is extremely important and critical, especially for those living with a rare disorder and their family and wider community.

Rare Disorders NZ believes precision health is now part of healthcare worldwide and as we move forward in the next few years will become more and more part of mainstream healthcare. New Zealand and its health system has to be able to be part of precision health research and development, application into health care delivery and look to see where we can be leaders in specific aspects of precision health.

Rare Disorders NZ knows from lived experience and evidence from surveys and research that diagnosis, care and treatment, screening, managing genetics and hereditary issues in families, and wider health matters are so challenging for those living with rare disorders and current outcomes are poor and inequitable. Precision health offers an opportunity to make significant real progress in all these areas for those living with a rare disorder now and in the future.

Rare Disorders NZ believes it is anticipatable that there will be many more rare disease treatments coming on stream in the next 10 years. For New Zealanders and their families to benefit from these advances, rapid and quick diagnoses are going to be necessary. Precision health is the vehicle to achieve this.

Rare Disorders NZ would like to be appropriately involved in the work to develop the insights briefing and help to ensure the policy advocacy, lived experience and expert advice is heard, listened to and utilised well for the benefit of all New Zealanders, and so we reiterate our offer of a joint discussion envisaging session to do just this. We look forward to taking this forward with the team at the Ministry of Health.

**2. What opportunities does precision health create for more effective health care in the future (more than 10 years ahead)?**

Rare Disorders NZ believes precision health provides concrete opportunity to ensure diagnosis is timely and accurate for rare disease and disorders, and then treatments can be targeted and effective.

The current situation is a diagnosis odyssey, taking too long, with too much difficulty and stress for rare disease patients. Without early diagnosis treatment cannot be instigated appropriately often resulting in deterioration and suffering that is not necessary. This leads to treating symptoms and other medical and mental health problems that could be avoided. Rare Disorders NZ believes that precision health whilst may require some upfront costs in some areas would most definitely bring



healthcare system value for money and value-based care if not savings, and not just across health budgets but across wider health and social care welfare budgets.

Rare Disorders NZ believes that precision health provides great opportunity to significantly improve diagnosis, care and treatment, screening, and managing genetics and hereditary issues in families for rare disorders.

Rare Disorders NZ believes it is anticipatable that there will be many more rare disease treatments coming on stream in the next 10 years. For New Zealand families to benefit from these advances, rapid and quick diagnoses are going to be necessary. This is best achieved when coordinated multidisciplinary care is enabled with the best technology. A rare disorders diagnostic programme could be established that has these attributes. Most Australian states have one and they use the precepts of precision medicine to arrive at expeditious diagnoses. This could be an exemplar New Zealand could operationalise at pace.

### **3. What barriers or restrictions do you see in the health system that might hold it back from adapting future precision health advancements?**

Rare Disorders NZ believes there a number of barriers that could eventuate however we believe none of them are insurmountable.

Rare Disorders NZ suggests that the following will need consideration;

- Healthcare professional workforce, current versus future workforce, what are the requirements for education and training in precision health and all its aspects, with particular regard for a focus on rare diseases.
- Graduate curriculum to include precision health across all aspects.
- Switching funding from todays diagnostics, treatment, drugs to new precision health options
- Funding research and translation into clinical practice.
- Use of precision health, specifically genomics in screening for rare diseases.
- Managing a current ill health ethos and approach to a future one of proactive precision health both in screening, prevention, diagnosis and treatments.
- Supporting a sound ethics and ethical approach to precision health that keep the opportunities in practice and that New Zealand ensures it remains in line with other countries in healthcare provision.
- Commercial aspects for commercial companies, ensuring we don't lose the ability to have companies develop and/or provide their research and services in New Zealand.
- Infrastructure requirements, in particular laboratories and life sciences.



**4. What concerns or issues do you have with precision health, or how we may adapt it in Aotearoa in the future?**

Rare Disorders NZ believes the current situation here in New Zealand is one where other countries are already ahead in this area of precision health. We are therefore concerned that New Zealand will be slow to adapt to this new science and application in health care settings. We believe that the MoH should be looking at ways in which New Zealand could leapfrog ahead and not just maintain a just behind position.

Rare Disorders NZ suggests that the following will need consideration;

- Approaching precision health with a positive open mind, developing an insights report that is dynamic, future looking and at the same time practical with immediate steps that can be taken to progress and move forward precision health here in New Zealand. This is about actually making something happen.
- Equity and Te Tiriti, any adoption into health care of precision health will need to have an equity lens in addition to Te Tiriti. Consideration should be given to the wider equity of diverse populations including that of those with Rare Disorders.
- How does New Zealand learn from the experience of other countries? It is possible to do this and we believe this should be done through a lens of learning whilst adapting for the New Zealand context and not dismissing others experiences against the New Zealand context.
- People are already leaving the country to access medicines and treatments overseas, with many never returning as they would have to give up drugs and treatments to return, this will be the case too for precision medicine if this is not supported and implemented in the right way. This in itself is causing inequities for New Zealanders, and will continue to do so.
- Listening to those with lived experience is critical in the development of precision health in New Zealand. The Pae Ora legislation makes it abundantly clear this is both critical and necessary. The voice of lived experience of those living with rare disorders must be heard and listened to in this work.
- Listening to those who are experts from many diverse fields in precision health is important. What at first may seem on the edge of science is being demonstrated to soon becoming more mainstream and its important to be very open minded in the approach to precision health.

**5. Which case study areas do you think the briefing should explore? Why? You can indicate one or more of the following (or identify any other areas of interest to you):**

- a. **Genomics**
- b. **Pharmacogenomics**
- c. **Digital health**
- d. **Computational biology**



Rare Disorders NZ believes all of these are relevant and should be part of the insights report. There is a need to ensure that New Zealand doesn't miss one of these that could be the area that allows the country to progress in an agile way. Rare Disorders NZ suggests the Ministry of Health needs to find ways to prioritise the healthcare system's focus where the most progress can be made for New Zealanders and we ask that Rare Disorders are prioritised within this, to ensure the rare disorders patients and community do not fall further behind.

Rare Disorders NZ would like to highlight the following articles to your attention as they provide examples and evidence of some of the discussions we have narrated above.

- [Genomic Newborn Screening Studies Around the World Begin to Take Baby Steps | GenomeWeb](#)
- [Newborns to get rapid genetic disease diagnosis - BBC News \(ampproject.org\)](#)
- [Harnessing technology to improve babies' well-being - The University of Auckland](#)
- [Base editing: Revolutionary therapy clears girl's incurable cancer - BBC News](#)
- [https://www.linkedin.com/posts/michelle-arrowsmith\\_the-disease-took-zara-then-sara-could-ayla-activity-7002388535873847296-ufSw?utm\\_source=share&utm\\_medium=member\\_ios](https://www.linkedin.com/posts/michelle-arrowsmith_the-disease-took-zara-then-sara-could-ayla-activity-7002388535873847296-ufSw?utm_source=share&utm_medium=member_ios)