Rare disorder strategy: Manatū Hauora / RDNZ / RD Support Group Leads hui

Location: Online

Date: 7 September 2023

Summary of discussion

Manatū Hauora (MoH) gave an introduction and update on the progress of the strategy, sharing that the latest document circulated to the reference group was a framework to guide the development of the strategy. The floor was then opened to support group leads to share their thoughts, first on the definition of rare disorders and then on the wider document. The purpose of the hui was for MoH to hear directly from Support Group Leads (SGL) and as such, MoH's commentary was kept succinct.

Definition

- A SGL asked why the definition was dividing rare and ultra-rare and raised a concern that this was othering. An alternate view was expressed by another SGL that it wasn't seen as divisive and shows the extent of rare disorders.

- Another SGL asked if the definition is talking about incidence or prevalence.

- MoH commented that the definition is not meant to be exclusionary and that MoH is looking towards international definitions.

Rare Disorders NZ explanatory note: We support the definition used by MoH. You can find RDNZ's proposal and reasoning for this definition on our website here: www.raredisorders.org.nz/about-rare-disorders/rare-disorders-in-new-zealand/rare-disorders-strategy-progress/rdnzs-proposal-for-a-definition-of-a-rare-disorder/.

- *In our proposal you can see the WHO/Rare Diseases International's approach to the issue of using incidence vs prevalence where they state "while the frequency of most rare diseases can be described by prevalence (the number of cases within a specific population at a given moment or over a specified period), some rare diseases, such as rare cancers and rare infectious diseases, can be more precisely described by incidence (the rate of new cases within a specific population over a particular period)". We would expect the same approach in New Zealand.*

- *In our proposal you will also see that Australia also uses the term ultra-rare disease for the purposes of the Life Saving Drugs Program which pays for specific essential medicines to treat patients with ultra-rare and life-threatening diseases. Ultra-rare is defined as a disease with a prevalence of 1:50,000 people or less in the Australian population. This is different to the Orphan drug designation which uses a definition of a medicine intended to treat a condition that affects fewer than 5 in 10,000 individuals in Australia. Orphan drug designations allow for a waiver of application and evaluation fee for registration in the Australian Register of Therapeutic Goods (ARTG).*

The rest of the document

- There was discussion about the interface of the Rare Disorder Strategy with other Strategies and mention of inclusion of the NZ Health Research Strategy given the reliance that the Rare Disorder population has on research for health outcomes.

  MoH commented that strategies and work programmes other than the Pae Ora strategies will be incorporated as appropriate.

- It was brought up that the word evidence has negative connotations for some when fighting for a diagnosis and they would like to see this replaced with the word information. Another SGL added that evidence of lived experience has been disregarded in the past and been ignored as not published evidence.

  A SGL suggested using evidence informed rather than evidence based.

  MoH noted this and explained their view of evidence includes lived experience.

- It was pointed out that the term Pae Ora/Health Futures can be offensive to people who due to the nature of their disorder are not going to have a healthy future and they would like to see more of a focus on equitable outcomes.

- It was asked that the section about supporting health practitioners to provide quality care include *patient centred* quality care.

- An SGL emphasised the need for the strategy to carry a sense of urgency and asked that the strategy addresses disorders where there isn't the benefit of time, stating they don't see this group represented in the framework document.

- A SGL noted that in relation to the advances in data and digital section, coding systems are broken for rare and people with rare disorders are not captured in data. They said we need to see consistency with coding and systemic change, including an increase in funding to allow this.

- A SGL would like to see more cross-jurisdictional and joined work programmes, a multi-disciplinary team approach that recognises the holistic approach needed for people with rare disorders. They emphasised especially that care should not just be symptom based but preventative and with a long-term outlook. They supported the idea of a rare disorder centre of excellence and would like to see it written down in the strategy as something to work towards.

- A SGL noted that there is little mention of quality of life in the document.

- A SGL would like to see a review of how economics are reviewed when it comes to accessing therapies and that a new model would consider long term costs and complications of not having access to the therapy.

- A SGL disagreed with the statement that "In New Zealand, we have good assessment and prioritisation of individual products".

- A SGL emphasised the need for an interdisciplinary, consumer-centred approach and recommended looking to the Five I's Framework designed by the Health Consumer Advocacy Alliance. They noted that an overarching approach of how to manage rare disorders is needed, and that it is important that it is not siloed.

- A SGL pointed out that rare disorders are not completely health based and that multi-disciplinary teams and coordinated care over the person's lifespan is needed, emphasising that this requires cross ministry cooperation.

- It was brought up that clinicians need a place to go to find information about rare disorders and that a centre of expertise would be a practical, overarching, and reasonable goal to achieve this. It was emphasised that the strategy and the work that follows need to include practical, manageable measures that are tagged to who will be accountable for actioning them.

- A SGL spoke as a parent of a child with a rare disorder. They shared that it affects the entire family. They asked if the strategy supports the idea of setting up disorder specific clinics making the process much more straightforward and coordinated for families, noting that ultimately it would be cost saving and have a whole of lifetime benefit and that fragmented care is not sensible.

  MoH commented that this type of work sits below the level of the strategy and is for Te Whatu Ora to consider (as the operational arm), and that this type of query is too specific for the strategy.

- A SGL talked about virtual clinical trial network networks and how that sort of model works well for rare disorders. They would like to see a rare disorder voice in planning for this. They also raised that clinical trials are very important for people with rare disorders and said that they deserve their own specific mention.

  MoH shared that they are linked in with the clinical trial team within MoH.

- A SGL made a comparison between people covered by ACC and those born with a rare disorder and the difference in the care they qualify for, with post-operative in home care as an example.

- A SGL noted they see the strategy as part one and that the next part will be development. They asked about timeframes and budget.

  MoH shared that MoH develop and write the strategy and implementation is the responsibility of Te Whatu Ora. In terms of timeframes, they are planning to release the strategy in the first quarter of 2024.

  There is currently no budget allocated for implementation. It is a Government decision to allocate budget. MoH are envisioning a model that utilises resource already in the health system.

  Following the release of the strategy, it is expected that Te Whatu Ora will update Te Pae Tata (the Interim New Zealand Health Plan) and the rare disorder strategy is expected to feed into this, rather than there being a rare disorder action plan.

- A SGL asked how people with a lived experience of rare disorders be involved in the implementation of the strategy, stressing the importance of including the lived experience. Another support group lead asked whether MoH would be employing someone with a lived experience, noting that this is vital to co-design.

  MoH said that this is a decision for Te Whatu Ora. They explained that MoH holds a strategic and regulatory role while Te Whatu Ora holds the role of implementation.

- A SGL noted that many people with rare disorders are not eligible for support services from Whaikaha (Ministry of Disabled People). They also flagged the inequity of different districts having different streams of funding and noted they tautoko (support) the earlier discussion of the importance of urgency.

- A SGL asked that given rare disorders are not purely medical, will they need to do this with every Ministry, or will there be inter-ministerial consultation?

  MoH shared that they can work across agencies within government and that the strategy gives a focal point for bringing agencies together.

- A SGL referenced earlier conversations about different agencies and funding and said that the focus shouldn't be on where the funding comes from or who is paying for it, but instead the focus should be on the need of the person.

- A SGL noted that in terms of research and evaluation, they would like to see more rare disorder lived experience feature in medical and allied health education. Another SGL shared that they have been providing lived experience to Otago medical school students and that others should contact the universities. Another SGL talked about the need to run seminars on lived experiences and involve them in the education of specialists.

- A SGL pointed out that the Te Tiriti section could include a pathway for monitoring/auditing.

- A SGL asked for further information about the meaning of unrecognised and undiagnosed conditions and whether this captures people who may have common disorders but are undiagnosed.

  MoH noted this and said they will come back with a response.

- A SGL reiterated earlier points of the need for a centre of excellence or hub for rare disorders.