Prader-Willi Syndrome Association New Zealand
ADVOCACY, EDUCATION AND SUPPORT

Submission to Pae Ora Legislation Committee

Item of business: **Pae Ora (Healthy Futures) Bill**

Submission name: Joanna Davies, Operations Manager, on behalf of:
The Prader-Willi Syndrome Association NZ

Prader-Willi Syndrome is a rare and complex neurodevelopmental disorder which is thought to have a birth incidence of approximately 1 in 16,000. The Prader-Willi Syndrome Association provides information and support to individuals with Prader-Willi Syndrome, their whānau, caregivers and wider networks. We also advocate on behalf of our members and aim to increase awareness and understanding of this complex disorder.

General Position

The PWSA(NZ) welcomes reform of our health system, mainly because services are too fragmented at present resulting in an incohesive approach to patient care. We also believe reform is necessary due to inequalities in access to relevant expertise, health services and pharmaceuticals.

Whilst we support the general intent of this Bill to address inequity, variation and poor health outcomes for some groups, we believe the fundamental changes proposed to the structure and accountability of our health system fail to take into account the health system experiences of people with rare disorders and include appropriate legislation to improve health outcomes for this patient group. The Pae Ora Bill completely overlooks the rare disorder population, which may constitute up to 300,000 New Zealanders or 5.9% of our national population. *(Co-authored by Orphanet and EURORDIS: Nguengang Wakap, S., Lambert, D.M., Olry, A. et al.* ***Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database****. European Journal of Human Genetics, 2020.)*

By its own admission in the Explanatory Note: General Policy Statement, the Pae Ora Bill declares: *"Successive reviews of the publicly-funded health system…..have found consistently poor outcomes for some groups, in particular Māori, Pacific peoples, and people with disabilities, and significant unwarranted variation in service availability, access, and quality between population groups and areas of New Zealand…….one of the root causes of this inequity and variation was the structure of the health system. It described a system that had become fragmented and complex, leading to unclear roles, duplication, misalignment, and a lack of a common whole-system ethos."* The issues identified above directly impact people with rare disorders such as PWS, yet the rare disorder population group are ignored in this important legislation proposing a major restructure of our health system. New Zealand currently has no official definition of rare disease or disorders, does not capture relevant rare disorder data and has no specify policies addressing the needs of this group. This does not align with international best practice and is not in harmony with the intent of a reform to establish a new health system that aims to improve health outcomes for all, achieve

equity, and to build pae ora (healthy futures) for all New Zealanders (Clause 3a, b and c). People with rare disorders will remain invisible within health legislation and policy.

In our family support role, we sometimes hear of children who have been diagnosed with PWS several years later than they should have been, and have therefore missed out on essential early interventions. We often hear from families who are struggling to find the right advice, medication or support. It is quite common for individuals and families to not receive appropriate healthcare or supports because their needs are not well understood or do not fit the usual 'tick boxes'.

The PWSA(NZ) does not support this Bill without amendments to reference people with rare disorders as a specific population group and to address their needs by legislating for a specific Rare Disorders Health Strategy. A Rare Disorders Strategy would provide a framework to guide the health system in providing a more informed and coordinated approach to managing the care of rare disorder patients, with clearly defined healthcare pathways. A Rare Disorders Strategy could bridge existing gaps in regional health provision by improving accessibility to professional expertise and to relevant supports and services. It could also enable a more integrated care approach involving the health, disability and education sectors.

The PWSA(NZ) does not support clauses in this Bill which exclude Pharmac from the principles in this Bill surrounding equity and engagement with population groups. We would also expect to see a focus on equitable performance within Functions of Pharmac listed in clause 62, especially in light of the recently published interim report by the Pharmac Review panel. A generation of New Zealanders with PWS experienced extremely lengthy resistance to funding an essential treatment for PWS and as a result, they are now experiencing long-term negative health outcomes creating a wider impact on our health and disability systems. The Pharmac Review Interim Report highlighted various issues, including a lack of equity in decision making for rare disorder groups and that a heavy focus on cost savings disadvantages smaller groups needing higher cost medicines. It is therefore essential that the Pae Ora Bill incorporates a response to issues raised in the interim report. A Rare Disorders Health Strategy could outline a specific assessment pathway to ensure equitable access to modern rare disorder medicines.

Example Case

Hannah lives in Wanganui and her son Tommy was born in 2019 and has PWS. This is an extract from a video Hannah recorded for the Asia Pacific PWS Conference held in October 2021. (Day 3: Hear Our Voices - New Beginnings: www.pws.org.nz/news-events/conferences/asia-pacific-pws-conference/presentations-and-slides. Access password = APPWS2021-KL)

"The growth hormone in New Zealand, you're eligible from 6 months. So Tommy started growth hormone at 9 months and I think a big part of that was because we live in a small town. There aren't all the different types of doctors in our town so people come from Auckland for the clinics. So for endocrinology, the people that do the growth hormone, they come every 3 months. And I think the communication between different disciplines isn't that great, or different DHBs. So we had to really push to get that started. It was kind of like there was a piece of that puzzle missing in terms of we didn't have everybody in the same room saying, ok, this is what needs to happen for your child. I guess that is one frustrating part of the system – that you aren't just mum and dad; you are also constantly advocating for your child."

This exemplifies why a specific health strategy for rare disorders is necessary. Research has shown that the benefits of growth hormone therapy increase with earlier treatment and this delay would have reduced potential benefits for Tommy. Specific, coordinated and integrated pathways

for cohesive clinical care would have improved this situation by ensuring that upon diagnosis, Tommy's medical team would have been informed by relevant experts on a course of care and treatment under a rare disorder health strategy. Changes to decision making policy and budgetary constraints at Pharmac would also have ensured that Tommy's treatment could have started even earlier than 6 months if access to GH treatment was expanded on a par with other OECD countries, as advocated for by the PWSA(NZ) for almost 2 decades.

Recommendations

We wish to make the following recommendations regarding amendments to the proposed legislation:

- Explanatory Note: General Policy Statement – opening paragraph to be amended as follows:
  ….have found consistently poor outcomes for some groups, in particular Māori, Pacific peoples, people living with rare disorders, and people with disabilities, and significant unwarranted variation in service availability, access, and quality between population groups and areas of New Zealand

- Clause 4 Interpretation
  - To include a definition of rare disorder as defined by EURORDIS and widely used internationally: rare disorder means a disease or disorder which affects less than 1 in 2000 people in the New Zealand population.

  - To include a definition of 'population group' as referred to in several places in this Bill: population group means Māori, Pacific peoples, people with a disability, or people with a rare disorder

  - To add health strategy means any of the following health strategies:
  (e) the Rare Disorders Health Strategy

- Clause 7 Health System Principles
  7 (1) (a) and (b) outline that the health system should be equitable for all, including population groups, and that there should be engagement with population groups to ensure development and delivery reflects needs and aspirations.
  7 (4) states that the engagement principles of 7 (1) (b) do not apply to Pharmac and we question how their performance can meet the equity requirements of 7 (1) (a) without engaging with population groups who often become experts in their diagnosis and needs?

  We recommend the removal of clause 7 (4).

- Clause 10 Overview of Minister's Role
  People with rare disorders have unique issues, yet common challenges, which need to be recognised through a specific health strategy. We recommend adding that the Minister's role includes issuing the following health strategy:
  (v) Rare Disorders Health Strategy
  We are aware that Rare Disorders NZ have long campaigned for the introduction of a National Framework for Rare Disorders within health system policy and that they have identified 7 key priorities for incorporation in such a strategy. We support their suggestions

for the development of a Rare Disorders Health Strategy and know these priorities have been developed based on gathered data and reflect best practice overseas.

- Clause 14 Functions of Health New Zealand
  14 (3) states "In performing any of its functions in relation to the supply of pharmaceuticals, Health New Zealand must not act inconsistently with the pharmaceutical schedule."
  We believe there are times when Health New Zealand should act inconsistenly with the pharmaceutical schedule if unmet patient need has been identified. Current practice is that Pharmac will decline much needed treatments on the basis of budgetary constraints or for other reasons which are not always transparent. There must be a way to override such decisions when a solid case can be made for funding.

- Clause 29 Overview of important health documents
  29 (1) (b) the Minister to determine the following strategies for improving the health status of New Zealanders….
  Recommendation to add (v) Rare Disorders Health Strategy
  We also question why such important legislation is intended as a guide only? [See 29 (2)]

- Clause 30 Government Policy Statement on Health (GPS)
  This clause states that the Minister must issue a GPS at intervals of no more than 3 years apart and that the purpose of the GPS is to set priorities for the health system. This is concerning because at this stage, there is a major ommision of legislation for a significant population group in this Bill and if unrectified, this population group may have to wait an additional 3 years before priorities could be set to specifically include them in health policy and the development of a strategy can begin. The rare disorders population group have waited long enough, and our health system policy is already behind that of other nations who have specific health and medicines access frameworks in place for rare disorders.

- Clause 47 Process
  47 (1) In preparing the New Zealand Health Plan, Health New Zealand and the Māori Health Authority must engage with —
  (c) individuals and organisations that Health New Zealand and the Māori Health Authority consider appropriate.
  We would like to question who determines which individuals and organsiations are considered appropriate for engagement? There should be a requirement that bodies representing population groups are included.

- Clause 53 Code of Consumer Participation
  It is stated that the HQSC must develop a Code of Consumer Participation and that the code must contain principles for the purpose of supporting consumer participation and enabling the consumer voice to be heard. However, it is not clear which consumer voices will be heard or invited to participate.
  We suggest adding that the code must also contain a requirement that all population groups are supported to participate.

- Clause 62 Functions of Pharmac
  There is no mention of a requirement for Pharmac to perform equitably, especially in light of the Phamac Review Interim Report. There are concerning terms in this proposed legislation, such as:

62 (1) (c) engage as it sees fit

62 (2) Pharmac must perform its functions within the amount of funding provided to it

It should not be the case that patients are denied proven life-saving or life-changing treatments when a review of the pharmeceutcal budget is needed. Pharmac should be granted sufficient budget to fund medicines that are deemed part of normal available treatment plans overseas, and Pharmac need the ability to request an additional allocation of funds as needed.

Pharmac should not be allowed to 'engage as it sees fit', effectively ignoring patient groups or dismissing compelling evidence provided to them through seemingly arbitrary decision making processes.

- Clause 64 Board of Pharmac to ensure advisory committees

  64 (1) (b) a consumer advisory committee to provide input from a consumer or patient point of view.

  This clause does not include any requirement for the CAC to include consumer voices from any particular population group, such as the rare disorder patient group.

  We recommend this clause is amended to add such requirements.

- Clause 77 Resposnsibility to operate in a financially responsible manner

  77 (3) This section does not limit section 51 of the Crown Entities Act 2004.

  The Crown Entities Act 2004 Clause 51 ensures that enities such as Pharmac should prudently manage assets and liabilities, ensure long-term financial viability and act as a successful ongoing concern. We believe there could be intense pressure upon Pharmac to spend within their allocated budget and this may be why Pharmac does not appear to request budget increases when necessary. There needs to be clearly defined circumstances which warrant the request of an increase in funding.

- Clause 81 Committees

  In making appointments to a committee of a board of an organisation, the board must endeavour, where appropriate, to ensure representation of Māori on the committee.

  We would like to question why representation of other population groups are not included in this clause? It is essential in the development of policy and strategy, that all minority population groups have representation.

- Clause 97 Regulations

  97 (1) The Governor-General may, by Order in Council, on the recommendation of the Minister, make regulations— Regional arrangements

  Some of our members are not served well by regional disparities in available services, as exampled in Hannah's story. A lack of access to expertise can also result in late diagnosis and poor health outcomes. People with rare disorders need to come under national service arrangements in order to achieve the best health outcomes.

- The PWSA(NZ) also notes that there is little reference to mental health services in this Bill. This is an area that affects many of our members as there is a predisposition to mental health problems in PWS, particularly during adolescence and early adulthood. However, many families report difficulties in accessing services with long wait times for appointments. They also report that when they do access services, they struggle to obain accurate diagnosis, the mental health issues associated with PWS are not well understood, and treatments are sometimes inappropriate. Diagnosis can be made more difficult due to the

combination of mental health issues alongside intellectual disability, plus social and behavioural dysfunction. Mental health problems can present differently in people with PWS. Response to pharmaceutical treatments and dose can also be unique to PWS and varies in individuals. The use of pharmacogenomics to tailor appropriate individual treatment without the need to trial lots of medications is recommended, although this service is not currently available in New Zealand.

The above issues highlight that not only should there be improved access to mental health services for the general population of New Zealand, but relevant expertise and provision is required for particular groups. This is an example of where a planned pathway within a Rare Disorders Health Strategy would be beneficial.

We recommend amending this Bill to add clause(s) that will address the current mental health crisis and note that relevant mental health service provision be incorporated within Health Strategies as needed.

Summary

The implementation of a specific Rare Disorders Health Strategy would have far reaching advantages for the well being and long term health outcomes of our membership. A cohesive approach to health provision for rare disorders would have wider social impacts and make economic sense.

We request acknowledgement of the common challenges faced by people living with a rare disorder such as PWS, and a commitment to address these challenges through the development of a Rare Disorders Health Strategy. We believe that legislation proposing a health system for the future of Aotearoa New Zealand should not be approved without including legislation for this significant population group.