21 September 2020

Committee Secretariat
PO Box 6021
Parliament House
CANBERRA
Canberra ACT 2600
Health.Reps@aph.gov.au

Dear Committee Secretariat,

Re: House of Representatives Standing Committee on Health, Aged Care and Sport inquiry into approval processes for new drugs and novel medical technologies in Australia

Thank you for the opportunity to make a submission to the *Inquiry into approval processes for new drugs and novel medical technologies in Australia*. New drugs and novel medical technologies are urgently needed to treat Duchenne Muscular Dystrophy (DMD) which currently has no cure and results in progressive muscle loss and a dramatically shortened lifespan.

The current gold standard treatment is corticosteroid medication which aims to slow the progression of DMD by a handful of years. However, this medication comes with a range of side-effects including weight gain, headache, osteoporosis with an increased risk of fractures, high blood pressure, and psychological effects including mood swings and behaviour changes. In particular, these psychological side effects often have a significant detrimental impact on the quality of life of the person with DMD because these behaviour changes alter how other people perceive and treat them. Yet, to not take the corticosteroids results in a more rapid deterioration. Children with DMD need better options.

Currently none of the new generation gene therapy clinical trials are conducted in Australia. These trials (including, but not limited to, micro-dystrophin, exon-skipping, and the emerging CRISPR/Cas9 treatment possibilities) offer the most hope for greatly improving the outcome of DMD. It is likely that these treatments will have the greatest benefit when started early in the disease process, before there is significant muscle damage. All children in Australia with DMD should have an opportunity to access these trials. DMD progresses quickly, even with corticosteroid treatment; there just isn't time to wait for overseas trials to finish before children in Australia can access these treatments. Even if families had the financial means to travel overseas there would still be significant barriers to participating in overseas trials (e.g. funding multiple return visits, accessing overseas health services, funding any emergency care needed to treat unexpected treatment side-effects). These gene therapy trials should be available in Australia and to a broad cohort of people with DMD, not just boys aged 4 – 7 years which is the target group for most of these studies at the exclusion of all other ages. Additional groups could be treated as separate sub-studies to ensure the data is analysed separately (e.g. a group for boys aged 4 – 7, another for boys aged 8 – 12, another for non-ambulatory). Importantly, these trials should include a group for girls to participate. There are no clinical trial options currently available to girls with DMD (worldwide) and although the prevalence of DMD in girls is vastly lower than in boys, they are equally deserving of access to clinical trials. With such few numbers the study group for girls may be statistically underpowered but there would still be a lot of useful information to be learned from a series of case studies.

Further, with so much natural history data available for DMD, I question whether a placebo group is still required, particularly after phase 2 of clinical trials if it is needed at all. Placebo groups are the gold standard for clinical trial research; however, this leaves many children with DMD being left untreated for a year or more – a very long time for a disease that progresses so quickly. Using natural history data or standard corticosteroid treatment as the 'control group' comparison would still enable researchers to determine the efficacy of the trial therapy and, importantly, would also ensure each child with DMD has access to a treatment as soon as possible. I also question whether a muscle biopsy is a necessary outcome measure. There is a lot of useful information available from non-invasive mobility and strength measures that would indicate whether the treatment is improving the outcome for people with DMD. A muscle biopsy does provide direct evidence of dystrophin expression; however, if this is crucial, I would hope this could be limited to the first 1 or 2 phases of the trials, with only non-invasive outcome measures used in subsequent phases.

For more specific recommendations on how to address these issues please refer to the Save Our Sons Duchenne Foundation keynote report prepared by The McKell Institute and recently launched in Federal Parliament: 'Living with Duchenne and Becker in Australia: Supporting Families waiting for a Cure'. This report makes several key recommendations, six of which are relevant to the inquiry's four Terms of Reference topics with one related to funding future therapies (Recommendation 7) and five related to improving access to clinical trials (Recommendations 8 – 12). These recommendations are summarised below, and greater detail can be found in the keynote report:

"Recommendation 7: The Australian Government include clear funding mechanisms for gene therapies as part of its 2020 review of the National Health Genomics Policy Framework.

Recommendation 8: Australian Government to establish a national 'one-stop' clinical trials portal.

Recommendation 9: Australian Government to develop a single national ethics review and site-specific assessment application form.

Recommendation 10: Australian Government to establish a national clinical trial coordinating agency.

Recommendation 11: Introduction of national legislation to harmonise regulatory requirements.

Recommendation 12: As part of the National Gene Therapy Strategy review the approval process for the use of genetically modified organisms in clinical trials."

(Save Our Sons Duchenne Foundation, The McKell Institute (2020). *Living with Duchenne and Becker in Australia: Supporting Families waiting for a Cure, pg 8.*)

DMD currently has no cure, it progresses quickly, and there are no gene therapy clinical trials currently available in Australia. The current process for new drugs and novel medical technologies in Australia is inadequate and does not address the urgency and severity of DMD. Clinical trials bring hope, but our sons and daughters with DMD do not have time to wait. More needs to be done to bring these trials quickly and safely to Australia. We need these trials in Australia, open to a broad cohort of people with DMD, and ideally using non-invasive outcome measures and natural history data instead of placebo groups. Clinical trial information also needs to be easily accessible to all families with DMD so that no-one is disadvantaged by their limited experience with, or knowledge of, the health system.

Sincerely,

Julia Burlison