SAVE OUR SONS
DUCHENNE FOUNDATION

Save Our Sons Duchenne Foundation (SOSDF) Submission to the Queensland Parliamentary Inquiry into Social Isolation and Loneliness in Queensland

August 2021

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"Loneliness and Isolation are major factors for the Duchenne and Becker community. Duchenne muscular dystrophy is a rare disease and therefore it is challenging to find members of the local community who you can relate to and feel amongst your own. It is also incredibly difficult and isolating when there are very few activities and resources that a person living with DMD can use and you are forced to not attend and take part in various community activities that are easily accessible and catered to for able body people or people not in a wheelchair".

A mother with a boy with Duchenne living on the Gold Coast.

Executive Summary:

This submission was drafted by Save Our Sons Duchenne Foundation (SOSDF) after extensive consultation with members of the Duchenne muscular dystrophy (DMD) and Becker muscular dystrophy (BMD) communities living in Queensland.

Social isolation and loneliness are huge issues impacting the DMD and BMD community in Queensland (and other parts of Australia). They are issues affecting the quality of lives of all young people and their family members, who are living with this condition which can exact an enormous toll on all personal, social and community facets of a person's life. Social isolation and loneliness are compounded in the Queensland context by a range of factors which include but are not limited to:

- the lack of targeted services and service provision in Queensland which are geared towards groups with rare diseases such as DMD and BMD;
- the lack of connection experienced by the DMD and BMD community because of service gaps and opportunities more generally;
- the outdoor nature and culture of the Queensland community which is much harder for the DMD and BMD community to navigate and which continues to demonstrate variations in levels of inclusivity;
- inconsistencies in the education system with some Schools prioritising an inclusive culture and others marginalising it;
- the remote location of some families and the vast distances which are often required to be travelled for families and young boys/men with Duchenne to secure access to health,

recreation and social activities and the inadequacies in some of the transport infrastructure (e.g., airlines) which can be inaccessible or burdensome for wheelchair travellers; and

- lack of accessible facilities and equipment to promote inclusion. For example, beach wheelchairs, powerchair sports where modified wheelchairs are available (and not an additional financial burden on families), wheelchair accessible bars, clubs, gyms, cinemas etc.

Based on the above, the Save Our Sons Duchenne Foundation (SOSDF) welcomes the opportunity to provide a submission to the Community Support and Services committee (the 'committee') which is conducting the Queensland Parliamentary Inquiry into Social Isolation and Loneliness in Queensland (the "Inquiry"). While the terms of reference for this Inquiry are broad and extend far beyond specific rare disease communities such as the DMD and BMD community, SOSDF and the

community we represent, believes this Inquiry provides an excellent opportunity to raise in good faith, several key concerns and recommendations for consideration by Committee members. Further, our community believes such an Inquiry to be long overdue and we remain hopeful that some lasting and far-reaching outcomes can be achieved through this bi-partisan and constructive political process.

There are approximately 30 different genetic conditions that make up the muscular dystrophies and the severity ranges on a spectrum. *The most severe end of the spectrum is known as Duchenne muscular dystrophy lacking completely dystrophin protein. Decreased or truncated dystrophin protein is associated with less severe form is Becker muscular dystrophy. (https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/)*

In the absence of a cure for Duchenne and Becker muscular dystrophy and with clinical trials; pharmaceutical; medical and technological advances extending the life expectancy of boys with the disease, it has become critical to ensure that the social isolation and loneliness burden experienced by this community is addressed and minimised to the extent which is possible. This Inquiry can play an important role in ensuring that these outcomes are achieved and that families struggling with this condition no longer must endure the barriers such as that described below by a mother of a boy with Duchenne from Brisbane:

"Accessibility is an issue. For example, lots of houses, playgrounds, cafes, bars, clubs, some gyms and shops cannot be accessed without going up/downstairs. The majority of people sit in the centre or back seats at the movies which aren't usually accessible to wheelchairs.
Lack of shared activities contributes to feeling isolated e.g., difficulty in participating in mainstream sports. More shared activities would be helpful, not just classes for disabled kids. Also support for clubs/groups to be inclusive such as allowing a few adjustments e.g., Special seating."

With the Paralympics and Olympic games scheduled for Brisbane in 2032 (ironically premier sporting events bringing the world community together) there would now appear to be a priceless opportunity for the Queensland Government to rectify any shortcomings in community infrastructure and facilities - as part of the Olympic construction and building works program. Wheelchair accessibility to all key social and recreational events should be prioritised as part of any Olympic building investment/works - in the process, allowing the State Government to showcase these progressive developments to the rest of Australia and ultimately, the world.

Who we are?

Save Our Sons Duchenne Foundation (SOSDF) was founded in 2008 and is the peak body for those living with Duchenne and Becker muscular dystrophy (around 1,000 young people) across Australia. Our vision is to find a cure for Duchenne and Becker muscular dystrophy whilst

actively working to ensure enhanced quality of life (including quality of health, educational, employment, social and recreational opportunities) for those young people and their families affected by this condition. Advocacy and community engagement work are crucial to achieving this vision along with ongoing fundraising and events management designed to raise funds for essential research, service delivery and the provision of critical resources and equipment to the Duchenne and Becker community.

Save Our Sons Duchenne Foundation is a non-government Australian charity, who do not receive government assistance to represent the DMD/BMD communities as their peak body. We have raised millions of dollars for research and clinical trials, along with the funding of critical neuromuscular and clinical trials nurses' programs in most of our major children's hospitals across Australia, (including Queensland's Children's hospital in Brisbane). SOSDF also delivers a telehealth nursing service, scholarship programs, and prior to NDIS we funded critical equipment and resources (such as wheelchairs and scooters, cough machines) and a number of initiatives and programs such as teachers' information packs, music therapy, scientific conferences which are designed to enhance the quality of life, skills and social development/interaction and knowledge of young people suffering from Duchenne and Becker.

For more information on Save Our Sons Duchenne Foundation and the progressive muscle wasting disease of Duchenne and Becker please refer to the attached web link www.saveoursons.org.au.

McKell Access Economics Institute Report:

Save Our Sons Duchenne Foundation is also responsible for major research projects such as the landmark Mc Kell Institute report "*Living with Duchenne and Becker in Australia: Supporting Families Waiting for a Cure* which was commissioned in early 2020 '(a full copy of this report is **attached** to this submission - please also refer to:

https://saveoursons.org.au/blogs/advocacy/introductory-video-save-our-sons-duchenne-foundation-keynote-report-into-duchenne-and-becker-in-australia

This report which aside from identifying issues around the lack of clinical trials and new medical/treatment options for the Duchenne and Becker community in Australia, provided a comprehensive summary of issues impacting the Duchenne and Becker community, including the astronomical financial, personal and psychological costs involved with supporting a child/ren with Duchenne and Becker.

[1]According to this report, which was launched by a number of parliamentarians in Canberra in September 2020, Duchenne in particular, is associated with significant lifetime health and social care costs. It is estimated that these can total up to $2.25 Million for a child living until their mid-thirties. In addition, informal care costs total up to $630,000 in terms of reduced female participation in the workforce. On average, the financial cost of Duchenne for a child born today are expected to be $1.3 million. However, the costs for a child living to their mid-thirties rise to $2.88 million.

[2]Families who participated in the McKell research typically reported high out of pocket medical costs, ranging to $1800 per month.

The costs (and time invested) of caring for boys with DMD and BMD clearly puts enormous strains on family budgets and will subsequently limit opportunities and the time available for many personal, social and recreational activities -compounding issues of isolation and loneliness. As the McKell research also discovered having a child with a rare disease can also be an extremely isolating and lonely experience with other parents having no real understanding or appreciation of the lived experience of Duchenne and Becker. In the words of one mother with a boy with Duchenne who was interviewed as part of this research project:

[1] McKell Access Economics Institute Report Pages 14, 44
[2] Mc Kell Access Economics Institute Report Page 22

[3]***Having a child diagnosed with a rare condition is often a confusing and lonely experience. Unlike other aspects of parenthood those in your social and support networks are unlikely to have ever experienced what you are going through, and this can add to the isolation felt by families".***

These sentiments were echoed many times over by families who participated in the current Queensland consultation process. In the words of another mother with a boy with Duchenne from the Gold coast:

"My son is 8yrs and has not been invited to a birthday party for 3 years. He has not been invited to a playdate or sleep over with any of his peers ever. We have held these activities and not been offered any invitations in return. As a parent I see very little of other people in a social setting. I am either at work, at medical appointments, quickly doing the school run, at a computer dealing with invoices, the NDIS, specialists, physios, school, Plan Managers, support co-ordinators, researching equipment, appointment setting etc. There is very little time for a coffee. It can feel quite isolating at times and very lonely. I struggle now to find common ground to talk to other parents as I am out of practice because we are immersed in being carers rather than parents to our son."

Save Our Sons Duchenne Foundation Consultation Process:

Save Our Sons Duchenne Foundation was determined to consult as widely as possible with the Duchenne and Becker community across Queensland in the preparation of this submission. Social media posts and newsletter articles were initially distributed to encourage the community's participation and feedback to the Inquiry. Following this, a series of individual zoom consultations of 30-45-minutes duration were held with parents/carers of boys/young men with Duchenne.

[3] McKell Access Economics Institute Report Page 15

Participants including boys/young men with DMD and Becker were also able to email written feedback and responses.

A series of questions were posed to those involved in the consultation, a copy of which appears at **Attachment One** at the conclusion of this submission. These questions attempted to go to those issues we considered most relevant to some of the Terms of Reference (TOR) of the Inquiry.

In addition to this consultation, the SOSDF team held some community meetings in Brisbane in May 2021, with the DMD and BMD community (including young people with the condition). While these meetings preceded the establishment of this particular Inquiry, these meetings confirmed to SOSDF staff, that isolation and loneliness were critical issues amongst community members - with limited opportunities available for quality connection and interaction being articulated by several participants.

Shortly after these meetings, the SOSDF team applied for a Queensland Mental Health Grant to enable SOSDF to organise a further community dinner and self-care workshop in October 2021 for Queensland families grappling with DMD and BMD (A copy of this application is **attached** for the Committee's information).

Finally, and as already highlighted an extensive (and complementary) consultation with the Duchenne and Becker community had already been undertaken by the McKell institute as part of their research on behalf of SOSDF. While this work was across all states, it nonetheless engaged families from across Queensland. An extensive survey targeting the Duchenne and Becker community had been launched on 4 December 2019 and closed on 23 December 2019. [4]There was a total of 173 responses, a sizeable sample of the estimated population living with Duchenne and

[4] Mc Kell Report Pages 14/15.

Becker in Australia. 77.05% of this sample were parents of children with Duchenne and Becker and grandparents and siblings made up the rest.

Structure of Save Our Sons Duchenne Foundation submission:

Our submission is structured to highlight those key issues which were identified by our families throughout our consultation process which led to heightened experiences of isolation and loneliness. The submission is not intended to be a comprehensive "catch all" response (or generalisation) and importantly recognises, that variations exist in the experience of families across Queensland (especially for example, in relation to the levels of inclusion in the education system). Responses have been made in good faith and in a concerted attempt to draw attention to gaps and shortcomings in social and community provision. These responses are made in the hope, that positive change and increased funding and attention to the issues of a highly disadvantaged rare disease community, are forthcoming.

Save Our Sons Duchenne Foundation will subsequently make a series of **recommendations** at the conclusion of this response which in large part, will reflect the outcome of our discussions with the Duchenne and Becker community in Queensland.

Finally, at **attachment 2** of this submission we have attached some videos for the Committee to review and consider as they provide invaluable insights into the "lived experience" of those who are suffering from Duchenne and Becker muscular dystrophy.

Terms of Reference:

Following consideration of all the Terms of Reference for the Inquiry, SOSDF determined to concentrate our energies and resources on those Terms of Reference most relevant to our community. In this instance we have chosen to focus primarily on four of the six terms of reference and to concentrate most heavily on those TORs (TOR 2/3) where we received most information and input. Our response to those 4 terms of reference follows:

1) ***the nature and extent of the impact of social isolation and loneliness in Queensland, including but not limited to:***
    - ***identification of and consultation with vulnerable and disadvantaged individuals or groups at significant risk across the life course***
    - ***the interplay of COVID-19 with this issue***

As already highlighted, social isolation and loneliness are widespread amongst the Duchenne and Becker community in Queensland, which is arguably one of the most disadvantaged groups in the State. Not only are their enormous financial, personal and social costs arising for families with the lived experience of the disease (costs which place an enormous toll on this community's ability to engage in social, leisure and recreational opportunities) but the progressive muscle wasting nature of this disease means that young boys and men suffering from the condition, experience increasing difficulty in engaging with their peers and the broader community -as their physical capacity for engagement and participation, declines over time.

Subsequently, on-line gaming and other web-based interactions with peers often prevails for many, meaning these young boys/men miss out on the robust and more satisfying/nurturing forms of direct personal and social interaction. Says one grandmother of a boy with Duchenne based in Northern Queensland:

"The sole contacts of my grandson are international via the internet".

Notwithstanding that there are an increasing number of boys and young men with DMD and BMD who are achieving remarkable things, (and against huge odds) isolation and loneliness are nonetheless pervasive. Says one young man from Brisbane with Duchenne.

"Loneliness and isolation are huge factors for people with DMD and BMD. Our community experiences higher level of these feelings, especially the older people in the community. Lack of a social circle. As the condition progresses it can be more tiring to go out……DMD and BMD can limit a person to activities that they can do. Also, places need to be accessible and have accessible toilets etc. Also, a lack of connection or opportunities for experiences. Tiredness can have an effect.

Isolation and loneliness are experienced by the DMD and BMD community (parents/carers and sons) across a range of different contexts from schooling, sport and recreation,

through to social groups, employment and community life. The factors behind this will be explored in more detail in the following term of reference, but suffice to say, parents/carers get too little respite and quality "free time" and generally struggle to find sufficient support networks and peers with whom they can interact, find support and debrief. Boys and young men on the other hand, find too many physical barriers and exclusive cultures still in play. One Queensland mother reported to SOSDF, that her son with Duchenne was 12 years old and had been excluded from school sports days.

"Nothing is offered in 1st year in high school. He just sits on the sidelines and watches others participate. Schools need to step up".

An NDIS report of note:

[5]*"Participants with a Neurodegenerative Condition in NDIS, March 2021"* is another significant report for purposes of this Inquiry. This report is produced by the NDIA as part of its annual reporting requirements. [6]This report highlights amongst many other things, the strong desire of the carers/parents of children and young people with muscular dystrophy to spend more time in paid employment (a critical point of social activity and interaction) and to spend more time with their friends.

[7]This report also highlights the low levels of participation by children/young people with muscular dystrophy involved in a community, cultural or religious group in the past 12 months (approximately 30-38% across all ages). Sadly, the report also reveals that approximately 40% of boys/young men with muscular dystrophy do not have any friends outside of their family or paid staff.

These are clearly all facts worth bearing in mind when considering issues of social isolation and loneliness in Queensland.

[5] NDIS "Participants with a Neurogenerative Condition in NDIS" March 2021
[6] NDIS "Participants with a Neurogenerative Condition in NDIS" March 2021 Page 74.
[7] NDIS "Participants with a Neurogenerative Condition in NDIS" March 2021 Page 69.

Impacts of COVID:

For a community that is already extremely isolated with more limited social options and resources, the impacts of COVID (and specifically lockdowns) have generally varied. Overall, however, COVID has compounded the isolation and loneliness experienced by this community. Boys and young men with DMD and BMD are likely to be more at risk of having more severe symptom's if they contract a COVID-19 infection especially if they have heart or respiratory issues. On that basis, many boys and families have been required to curtail social and recreational activities during the COVID period.

A young man with Duchenne highlighted this problem during our consultation

"People with DMD and BMD are very vulnerable when it comes to COVID. The events that they may have may be too dangerous now".

Another mother of a young man with Duchenne from Central Queensland highlighted how COVID had really exacerbated the isolation and loneliness experienced by many young men with this condition:

"Yes, many are lonely and have no friends, many only have families as their friends and some will not admit it. They may have people to talk to but they need someone to actually come and hang out with you and spend time with you and this is not happening and Covid is also a huge contribution to this. Firstly, for their safety and secondly people who are normal and do not have a disability or an illness can go out and live life where others are stuck at home and nobody cares for them. As I say they have friends in the same boat, but it is not the same especially when the young men would also like a young lady to be interested and maybe a relationship out of that".

Snap lockdowns have also been more problematic for families in terms of accessing treating specialists, therapies, carers and medications. Home-schooling has also been particularly challenging especially where a boy may also have a comorbid behavioural disability/learning difficulty such as autism which is common amongst boys with this condition.

As with the wider community, lost travelling opportunities were also a toll of the virus -an issue compounded in the DMD and BMD community because "time is of the essence" (disease

progression, loss of mobility and muscle usage) for these boys/young men with this life limiting condition.

Stated one mother of a Duchenne boy from South-eastern Queensland when asked if COVID had played a role in increasing isolation and loneliness:

"***Absolutely-since COVID things have been worse. He missed out on loads of therapy last year which affected his application when applying for NDIS funding, he was given less money, because of course we did not have the opportunity to utilise the funding.***

Travel restrictions have meant that our boy could not visit his auntie in New Zealand. Also, time is not on side for these boys, we wanted to take our boy overseas while he could still travel. We booked a trip to Europe which was cancelled indefinitely".

The inability for carers and other support persons to visit families during COVID lockdowns was another issue increasing a sense of isolation and loneliness amongst the DMD and BMD community.

2) *the causes and drivers of social isolation and loneliness, including those unique to Queensland*

Our consultation identified many factors resulting in increased isolation and loneliness for the DMD and BMD community in Queensland, some of which appeared to be compounded in the local State context. For example, remote locations, fewer support services than other states etc.

Save Our Sons Duchenne Foundation will just concentrate on a few of the key findings below:

a) Lack of support services: "Connecting the Unconnected".

The DMD and BMD community consistently highlighted the lack of support services and options available to both parents/carers and young people in Queensland which were dealing with this rare condition.

Where once there appeared to be more services offering regular parent support groups, counselling, camps for young people etc., many families lamented what they claimed was the lack

of current programs and services or a refocussing of service delivery by some organisations - towards more specific, targeted and critical NDIS provision.

These sentiments are captured in the following statement from a Queensland mother of a boy with Duchenne living in a remote part of Queensland:

"Our major city is Brisbane-we have very little in terms of social groups and anything that is run is in Brisbane only -which leaves people in other parts of Queensland isolated.

When we see events that happen in NSW and Victoria such as the walks, BBQs, conferences etc it makes me sad, and we feel left out. My boy doesn't get affected as he can't read and does not see the emails regarding "whats going on in the community" but I do, and I live and breathe Duchenne through my son. He gets very upset about having weak muscles and not being able to take part in sports and things at school. He hates that he is different, and he feels useless.

Perhaps if I had more support -he would feel more connected"

"The lived experience" of DMD and BMD for both parents/carers and the boys/young men places an enormous toll on the mental health and well-being of all involved. Parents/carers typically grieve throughout all different stages/life cycles of the disease -from diagnosis, loss of mobility, schooling and post schooling transitions, through to declining health and death. And all too often, parents/carers are doing this on their own or without sufficient support and backup (especially where marriage and other significant partnerships/relationships have all too often broken down).

Unfortunately, self-care, social/community interaction and quality respite become "luxuries" for many community members meaning that mental health is often compromised - and this despite the incredible resilience, fortitude and strengths displayed by many of these families. The needs for accessible and specialised counselling/psychological services, support groups and opportunities for social connection and capacity building are therefore paramount. Sadly, these remain largely unmet needs in Queensland.

Programs designed to meet the mental health and well-being (personal/social/recreational/health) needs of boys and young men with DMD and BMD are also critical as they contend with the debilitating progressive advance of the disease and the barriers and exclusions which are put in place by mainstream social institutions.

(**SOSDF brought some families together at community meetings in Brisbane earlier this year. The feedback we received helped motivate us to participate in this Inquiry**)

This is perhaps no more apparent than the lack of opportunities for DMD and BMD boys/young men to participate on camps which was one issue consistently raised by this community. Camps were seen as providing key opportunities for participants to experience new surrounds, directly interact with peers and build ongoing social relationships. They were also seen as invaluable skill development opportunities with participants pushed to learn and "master" a range of new activities. Camps were also seen as opportunities for parents/carers to have some much needed respite from their onerous carer responsibilities.

As exclusion from school camps and activities appears to be an ongoing reality for many DMD and BMD boys, camps and activities run by the non-government/charity sector become more critical. Yet unfortunately, there appears to be a dearth of options available. Furthermore, funding for participation on camps becomes an issue when local options are few and far between. Save Our Sons Duchenne Foundation staff are looking for opportunities to fill some of the isolation and gaps in community connectedness, however COVID has severely impacted on our ability to hold large fundraising events and we have no government funding to offer scholarships into services like camps in other states.

One Queensland mother we spoke to took the extraordinary step of sending her son on a camp in NSW and utilising core NDIS funding for this purpose:

"The cost of attending this camp was huge but we wanted our son to have the chance to do some activities that he cannot usually do such as abseiling as the camp had adapted equipment and adequate support to enable this. To begin with I had to apply for NDIS funding the previous year in advance. This came at a cost too as the NDIS scheme did not give us much extra funding towards it, so we had to pool it from other areas in our NDIS plan. In addition my husband and I both had to take leave, and then of course there was the expense of travel and accommodation. In short, it ended up being an expensive do".

Another mother of a boy with Duchenne bemoaned the fact that:

"My boy is 12 and we have never encountered a camp"

Lack of community self-organising:

Finally, the lack of a sense of community/connection amongst DMD and BMD families in Queensland was highlighted as a major factor in loneliness and isolation (partly as the outcome of a lack of support services). One family went to great lengths to discuss the lack of self-organising that occurred amongst Queensland families in great contrast to what they had experienced previously in Victoria where it was not uncommon, for 40-50 community members to regularly get together, share information and experiences and catch-up on a social level. These catchups being so vital for mental health and connection. This is not happening in Queensland.

Said one dad with a Duchenne boy who had relocated from Victoria to Queensland:

"The community is not strong up here. It is much stronger in other states. No-one is bringing the community together to be strong, no-one is connecting people especially when children are diagnosed. Social media networks are not strong up here….the feeling that you are not alone is really important. It's harder to know what you can do up here. In Victoria, information is shared".

b) Inconsistencies across the Education system: "It's largely about the Principal"

One of the most frequently sited reasons for the isolation and loneliness experienced by boys/young people with DMD and BMD was the exclusion some experienced in the Queensland education system.

While some parents were full of praise for the efforts of schools to ensure schools were inclusive and provided accessible facilities, others were scathing and highly critical arguing that schools did little to educate staff and students about disabilities:

Said one mother of a Duchenne boy from North Brisbane.

"Kids at school are not being educated about difference. It's all about education and making disability mainstream. If people have knowledge, then maybe kids won't point and giggle when my son runs. We need to start with the kids and work our way up. At the moment schools are simply not stepping up".

Another mother from central Queensland with a boy with Duchenne relayed this heartbreaking story of her son's experiences at school:

"Imagine being able to fly and then losing your wings? That is what it is like for these boys. They watch their peers progress and grow, strengthen their skills and live their dreams. They watch their friends flourish and know they never will. My son had friends at school in prep, year one and two. They have ditched him in the playground now to play football etc, so he is left with the girls now. The boys help him in the classroom but out on the playground, they want to run around and not look after him".

And this from a mother of a Duchenne boy from the Gold Coast:

"I think a focussed program on inclusion by Education Queensland that encourages staff AND students about disability inclusion is needed. Proper courses presented by disabled members of the community. Too many prominent people in my son's life do not have a fundamental grasp of what inclusion means and only put in a basic level of effort in catering to the disability sector".

In late 2020, SOSDF prepared a collective submission to the [8]Federal Education Department's Review of the *Disability Standards in Education 2020.* A copy of this submission is **attached** for the Committee's attention. Many of the issues we identified through that consultation process are applicable to the Queensland context – in short, factors identified as part of this current SOSDF consultation process are not unique to Queensland but appear to be prevalent across our primary and secondary school systems in all state jurisdictions. We note for example, some of the common threads between the Queensland Inquiry consultation and the broader education consultation. Namely, the lack of knowledge/awareness of DMD and BMD in the various education systems and the critical role that School Principals can play in determining how inclusive a school culture can be - and the prioritisation which is given to reasonable adjustments, learning support and wheelchair accessible facilities.

On the flip side, one dad with a boy with Duchenne from Brisbane could not speak highly enough of the efforts which were made by the School Principal in making his son's school inclusive and accessible:

"Its 100% the Principal. The school is very inclusive. All buses are wheelchair friendly, we have wheelchair accessible toilets and ramps and we even have wheelchair sports days".

c) The great outdoor Queensland culture: "Beautiful One Day, Perfect the Next"

As highlighted previously, Queensland's emphasis on an outdoor lifestyle and beach activity, is particularly challenging and alienating for the DMD/BMD community where physical disability can be so restrictive. This is particularly so when many basic facilities and equipment are missing - wheelchair ramps, beach wheelchairs, sporting options and clubs with adaptive equipment etc.

Says one dad with a son with Duchenne from Brisbane:

[8] 2020 Review of the Disability Standards for Education 2005 - Department of Education, Skills and Employment, Australian Government (dese.gov.au)

"Its all about getting outdoors, so it makes them feel like they can't participate in the community"

Save Our Sons Duchenne Foundation notes as an example, the lack of inclusive activities listed in the holiday guide below produced by the City of the Gold Coast council.

https://new.goldcoast.qld.gov.au/Things-to-do/Active-Healthy-program/Active-Healthy-holiday-programs.

On a positive note, this council does appear to be making some steps forward in ensuring greater accessibility to beaches (Beach Access Program) and other facilities for people in wheelchairs. For example, through the provision of beach wheelchairs and beach matting, mobility maps, mobility equipment hire and recharge points.

It is worth noting however, that many young men with Duchenne and Becker have no upper arm strength and cannot self-propel. On that basis they require power wheelchairs for independent mobility -these are not readily available for hire and nor can they manage some of the beach and other outdoor terrains in question.

If some outdoor activities are not negotiable for these families, then additional efforts need to be made by political, community and business leaders to ensure there are other options and alternatives (rather than home based internet activity) which are fully inclusive and accessible to this community.

d) Transport Issues: "Accentuated in the BIG Queensland context".

Transportation issues are compounded in Queensland because of the remoteness of some families and the vast distances involved in travelling to essential services such as the Queensland Children's hospital in Brisbane -where neuromuscular clinic services are available.

Despite most airlines and airports having disability action plans, wheelchair accessibility on regional and domestic airlines remains problematic and a major hurdle to be navigated by families needing to travel large distances and in reasonable timeframes. SOSDF has heard several stories of wheelchair damages incurred and/or other problems encountered at airports for those travelling in wheelchairs. This of course works to reinforce the isolation and loneliness felt by many families as travel becomes a major (and costly) exercise in itself.

Wheelchair modifications to vehicles are essential for many families especially in remote areas with poor local transport infrastructure and options. Yet such modifications have proven to be extremely problematic and costly for some families.

As explained by one mother of two Duchenne boys from North Queensland:
"Vehicle modification is a big issue. It took me a year of fundraising to raise enough money for the vehicle. We went months without mobility while buying the new car".

Other families talked about the ongoing bureaucratic delays they experienced with NDIS in obtaining sufficient funding to modify their vehicle. Such delays simply isolate families who may be dependent on such vehicles to undertake daily activities (let alone go on longer trips and holidays).

Save our Dons Duchenne Foundation heard little comment about bus and train accessibility issues from our community. However, after reviewing some of the Queensland Government's travel information websites we did note the following (including several very positive developments);
-[9]some but not all long-distance trains are wheelchair accessible;

[9] https://www.qld.gov.au/disability/out-and-about/travel-transport/rail-travel

-all new trains and train stations and buses/bus stops must comply with Disability standards for accessible public transport and upgrades to new stops are currently underway;
-an excellent station access guide is available at https://www.queenslandrail.com.au/forcustomers/access/station-access-guide which suggests most city and suburban railway stations are wheelchair accessible with accessible toilet and other facilities available ((although there remain some notable exceptions);
-most buses on the Translink network have low floors or ramp systems. Stations along busways—roads dedicated to buses—have lifts, ramps and pathways;
-it is less clear about wheelchair accessibility on private buses or accessibility with bus operators in regional Queensland who are outside the Translink area of Southeast Queensland.

e) **Lack of community awareness and knowledge of Duchenne: "There is none".**

The last factor SOSDF will highlight in relation to the issue of social isolation and loneliness is the lack of knowledge and community awareness of Duchenne and Becker muscular dystrophy. Not uniquely a Queensland factor, it is nonetheless pervasive in the broader community and impacts all interactions between the DMD and BMD community with educational, health, recreation, cultural/arts and social institutions and providers.

This lack of community awareness is very isolating for community members who are forced to advocate, educate and explain at all levels to ensure their child receives the equivalent access to social goods and services as the able bodied. This also translates to friendships and other relations and highlights again, the need for support groups and other connections between people who share the lived experience of this disease.

As explained by one mother of a boy with Duchenne:

"Friends in the community just don't get it. We are all so isolated, and unless we come across people with same issues on Facebook, we just miss out".

Greater community awareness of rare diseases such as Duchenne and Becker muscular dystrophy will facilitate a more inclusive and embracing culture and subsequently ensure, that much of the social isolation and loneliness experienced by this community is broken down.

3) the protective factors known to mitigate social isolation and loneliness

"Providing services and social groups for people with DMD and BMD. Having things to do and meeting new people. Finding meetups and going to events. Having a social circle. Shifting people's perspective towards disability. Need to have a more inclusive society….programs like "Just Like You" by Variety Children's charity can be helpful, especially in schools".
(A young Queensland man with Duchenne).

As should be clear from this submission the key protective factors which SOSDF believes will mitigate social isolation and loneliness for the Duchenne and Becker community will include but not be limited to:

-more opportunities for connection with other people who share the "lived experience" of DMD and BMD;
-more opportunities for greater connection with the broader community in relation to social/recreational/leisure activities and opportunities;
-public investment in services and infrastructure which facilitates greater levels of involvement and interaction of the DMD and BMD community in social, cultural and community life;
-provision of accessible and specialised services which are delivering mental health and well-being support and which are informed by the "lived experience" of rare diseases such as Duchenne and Becker muscular dystrophy;
-a more inclusive culture which embraces and engages with young people with DMD/BMD throughout all stages and aspects of their lives – in schooling, employment, recreation/social, health etc;

-community infrastructure and sporting/recreational/social organisation which are fully accessible and inclusive of the DMD and BMD community;
-greater public awareness and understanding of rare disease conditions such as DMD and BMD and the need to embrace this community in all aspects of community and social life;
-public acknowledgement and recognition of the resilience, fortitude and strengths of this community and the need for society to learn from, value and gain knowledge/insights from the lived experiences of those members of the community dealing with DMD and BMD;
-removing access barriers that may still exist on aircraft and our road, rail and bus systems and networks; and
-streamlining and making more cost efficient, the process for vehicle modifications to enable wheelchair accessibility and day to day mobility for families.

1) ***how current investment by the Queensland Government, other levels of government, the non-government, corporate and other sectors may be leveraged to prevent, mitigate and address the drivers and impacts of social isolation and loneliness across Queensland, including:***

   services and programs such as health and mental health, transport, housing, education, employment and training, sport and recreation, community services and facilities, digital inclusion, volunteering, the arts and culture, community

Save Our Sons Duchenne Foundation seeks only to make some very brief comments in relation to this term of reference as much of the ground has already been covered in the above. Simply, we say that more investment needs to be leveraged at all levels of government and the private sectors to ensure that more services and programs (such as those detailed in the TOR) are delivered and provided in such a way, as to ensure greater inclusion and consideration of the needs of rare disease communities such as the DMD and BMD community.

Save Our Sons Duchenne Foundation believes the Queensland State Government can play a key role in coordinating the different tiers of Government in this effort while providing the brokerage that may be necessary to deliver a range projects and programs which are designed to mitigate and address the drivers of social isolation and loneliness. For example, mental health and well-being initiatives which are cognisant of and adapted to the various phases of the Duchenne and Becker disease life cycle, public awareness campaigns which raise awareness of inclusion issues (and which are underpinned by a philosophy that people are only disabled to the extent our society excludes them), public works programs which increase wheelchair accessibility and facilities etc.

As providers of many services and programs, the State Government is ideally placed to ensure that its agencies are delivering services with inclusion issues, policies and practices centre-stage. These agencies are also best placed to consult with disadvantaged rare disease communities and their representative organisations about the types of programs and infrastructure which should be delivered to ensure that issues of isolation and social exclusion are mitigated. The Committee should note that SOSDF is prepared to help facilitate such a consultation process with the Queensland DMD and BMD community.

Finally, audits of existing infrastructure, programs and projects should also be undertaken to ensure inclusion and accessibility issues have been adequately addressed. With the hosting of an upcoming Olympic/Paralympic games it becomes incumbent on the Queensland Government to undertake such activity and to leverage investment and commitment from all Government levels and the private sector towards this effort.

Conclusion:

Although this Inquiry is broadly based and targets the entire community of Queensland, Save Our Sons Duchenne Foundation nonetheless believes it provides an invaluable and unique opportunity to constructively progress some concerns which have been present for Duchenne and Becker families living in Queensland.

Save Our Sons Duchenne Foundation is therefore extremely thankful that this Inquiry has been established by Members of the Queensland parliament with cross-party support. It demonstrates an important political consensus around the need to move the agenda forward in relation to ensuring issues of social isolation and loneliness are addressed and strategies implemented. This submission has been written in good faith and as an attempt to make an important contribution to this process. Save Our Sons Duchenne Foundation has endeavored to raise those issues as fairly and as accurately as they were articulated to us by members of the Duchenne and Becker community.

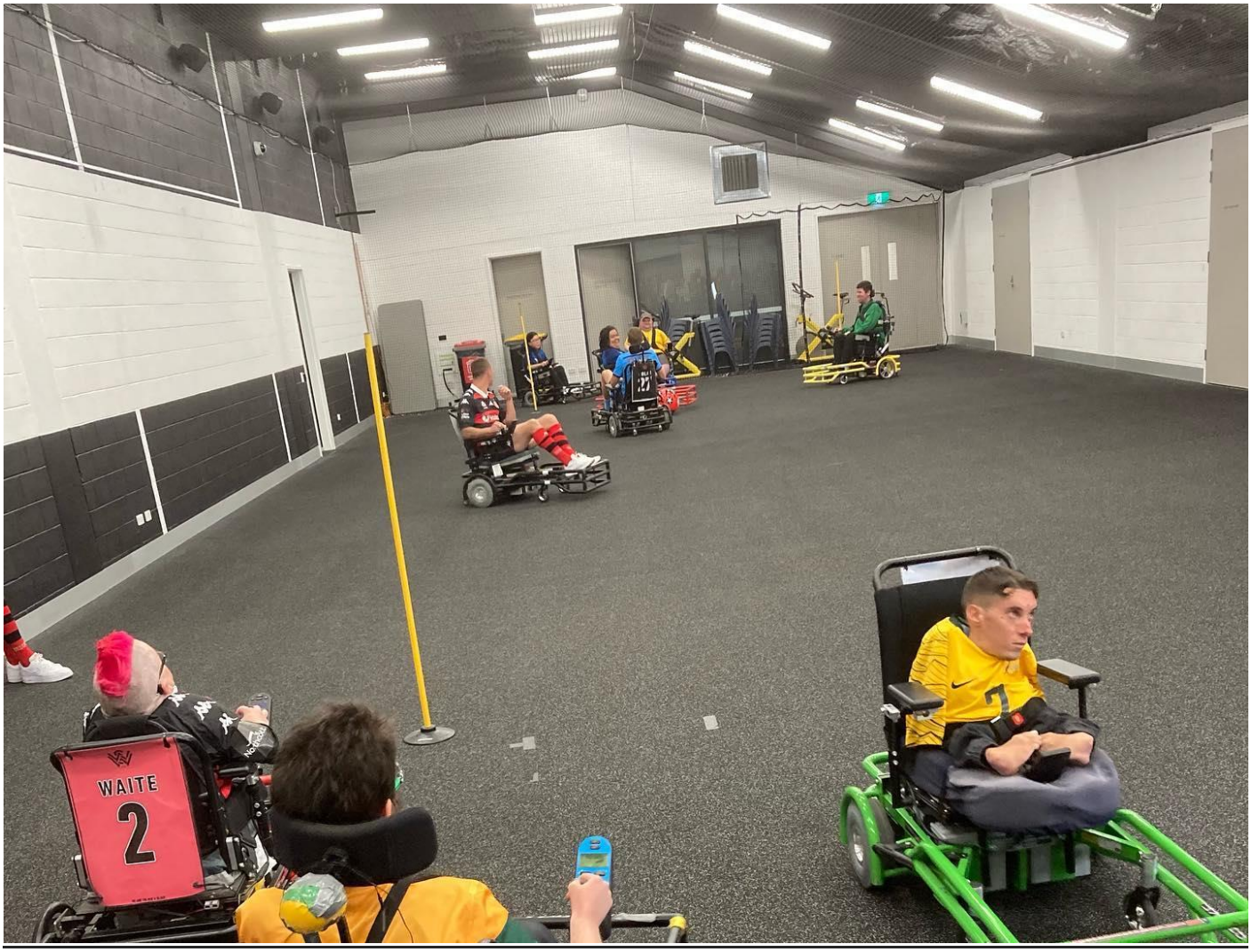
Save Our Sons Duchenne Foundation makes no apology for attempting to capitalise on the bi-partisan political momentum which has now been built up in relation to the issues which are the subject of the Inquiry. The general happiness and well-being of our community are much too important for us not to actively participate in the important work of this Committee.

Our organisation, along with the wider Duchenne and Becker community, would therefore welcome any further opportunities (e.g., public hearings) to participate and provide further feedback to the Committee.

Save Our Sons Duchenne Foundation conclude this submission, with the following heartfelt statement from a Queensland mother of a boy with Duchenne. We believe this statement neatly summarises the tasks ahead for all parties who are concerned to address issues of social isolation and loneliness:

"I think those boys AND girls living with DMD or BMD experience much greater levels of loneliness and isolation than their peers. In mainstream school there desperately needs to be a greater focus on inclusion and belonging for students with a physical disability. Many areas of life are set up for able bodied people and do not factor in the challenges people with muscular

dystrophy face every day trying to fit in. In society there is not enough inclusion being displayed for people with a disability. That seriously needs to improve".



(Launch of the FIPFA Powerchair Football World Cup in Sydney 29/4/21. Powerchair football is a very popular sport amongst the DMD community)

RECOMMENDATIONS:

1. That the Queensland Government investigate and deliver funding for a suite of programs (including programs addressing mental health, well-being and respite) which are targeting rare disease communities such as the Duchenne and Becker muscular dystrophy community in Queensland;

2. That any new programs and services which are targeting rare disease communities such as DMD and BMD be designed to improve opportunities for connection and social interaction between families and young boys/young people along with providing opportunities to participate in skill development and new experiences.

3. That the provision of social and recreational opportunities for boys/young people with DMD and BMD (for e.g., camps) be explored by the Queensland Government in consultation with the Duchenne and Becker community;

4. That further reviews/audits of inclusion practices and policies within the Queensland Education Department be undertaken to ensure consistency and inclusion is practiced and celebrated at all schooling levels and across the entire education system. Further, that these inclusion practices are consistent with the recommendations of the Federal Government's review of the *Disability Standards in Education 2020.*

5. That continuing work (including audits) be undertaken to ensure accessibility of all transport systems (including air, road, rail, buses and ferries) across the Queensland transport system and networks;

6. That potential barriers to inclusion of rare disease communities such as DMD and BMD, in sporting, recreation, employment and leisure facilities/clubs/bars/gyms etc be investigated

and addressed by the Queensland Government -in consultation with the disability and rare disease sector;

7. That a specific consultation be organised between Queensland Government agencies and the Duchenne and Becker muscular community to investigate and develop strategies to address issues of social isolation and loneliness identified by this community in Queensland;

8. That the 2032 Brisbane Olympic/Paralympic games be recognised (and utilised) as an invaluable opportunity by the Queensland Government to address any impediments and barriers (e.g., physical infrastructure) to the full participation of people with disabilities and rare diseases in social and community life.

9. That the Queensland Government embed within the building/construction works for the upcoming Olympic/Paralympic games, improvements to infrastructures and facilities to ensure maximum accessibility for people with disabilities.

10. That the Queensland Government commit to regularly evaluate and report back to the broader and rare disease communities, the outcomes arising from this Inquiry.

REFERENCES:

1) McKell Institute "Living with Duchenne and Becker in Australia: Supporting Families Waiting for a Cure" Angela Jackson/Equity Economics

Tony Zappia MP; Emma McBride MP; David Smith MP; Hon Warren Snowdon MP; Chris Hayes MP; Hon Chris Bowen MP; Peta Murphy MP; Dr Mike Freelander MP; Hon Tony Burke MP

(Official Launch of the McKell Report September 2020).

2) NDIS "Participants with a Neurogenerative Condition in NDIS" March 2021

3) https://new.goldcoast.qld.gov.au/Things-to-do/Active-Healthy-program/Active-Healthy-holiday-programs.

4) https://www.qld.gov.au/disability/out-and-about/travel-transport/rail-travel

5) 2020 Review of the Disability Standards for Education 2005 - Department of Education, Skills and Employment, Australian Government (dese.gov.au)

6) *https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy*

ATTACHMENT ONE:

Consultation Questions:
Queensland Parliamentary Inquiry into Isolation and Loneliness in Queensland

1) Do you think loneliness and isolation are big factors for the Duchenne (DMD) and Becker (BMD) community in Queensland and if so, do you think the DMD and BMD community experiences higher levels of loneliness and isolation than the rest of the community? Why?

2) What do you believe are the factors which most contribute to isolation and loneliness experienced by parents/carers of those young boys and men with DMD or BMD?

3) Is there anything unique to Queensland which you believe contributes to issues of loneliness and isolation for DMD and BMD community?

4) Do you believe young boys and men with DMD and/or BMD experience greater levels of loneliness and isolation than their peers? What are the factors you see as being most important here?

5) From your experience what are the most important factors to prevent social isolation and loneliness being experienced by members of the DMD and BMD community?

6) Has COVID played a role in increasing isolation and loneliness amongst members of the DMD and BMD community in Queensland? How?

7) What suggestions/recommendations do you have for the Queensland Government to address issues of isolation and loneliness for the DMD and BMD community?

8) Do you have any suggestions on specific services, facilities and programs the Queensland Government could fund or enhance to address loneliness and isolation for DMD and BMD families in Queensland?

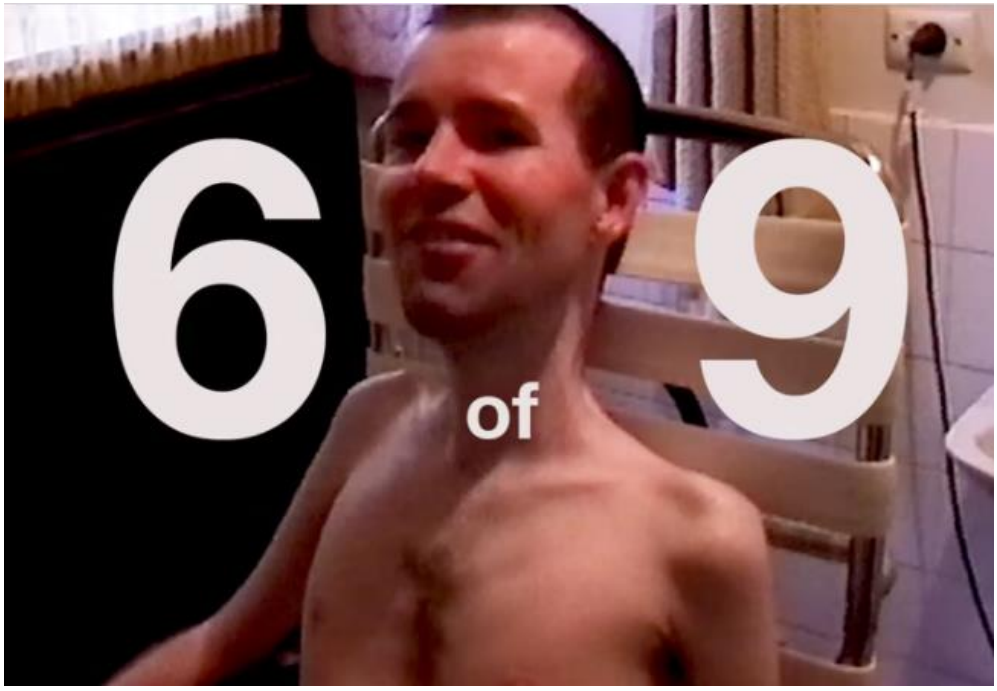
ATTACHMENT TWO

1) **Save Our Sons Duchenne Foundation YouTube Documentary**

This 8-minute video is available on YouTube and produced by Save our Sons Duchenne Foundation which gives a brief overview of Duchenne muscular dystrophy and the work of Save Our Sons in finding a cure to this condition.

https://www.youtube.com/watch?v=GcI7od9fqxs

2) **6 of 9 Documentary**



The following 45-minute documentary was made as a lasting gift for his family by Martin Dix a Melbourne born and raised film maker residing in Los Angeles. It is the story of Martin's brother Kieran who suffered from Duchenne muscular dystrophy and passed away some years ago. When COVID 19 struck in the US, Martin finally found the opportunity to edit over 40 hours of archival footage of his brother Kieran's life – footage which had been left stored away for many years. What he finally produced is a moving documentary which documents both the lived experience of Duchenne for those who suffer directly from it, but also the huge emotional and personal impacts for those who care and love someone with the disease – in this case, Martin's seven other brothers and his mum and dad. Save Our Sons Duchenne Foundation feels honoured that Martin wanted our organisation to use this film as part of our advocacy work and on that basis, we are privileged to be sharing this with members the Community Support and Services Committee.

https://vimeo.com/427928501?fbclid=IwAR10sETVXNJLt7on1Og2FlDjo8GFKIbIB1ahyxuSrMbt_9y2-X9WIjxgR9s

Lance Dale
Advocacy Officer
Save Our Sons Duchenne Foundation
10 August 2021.

SAVE OUR SONS
DUCHENNE FOUNDATION

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Living with Duchenne & Becker in Australia

SUPPORTING FAMILIES *waiting for* A CURE

SAVE OUR SONS

DUCHENNE FOUNDATION

ANGELA JACKSON / EQUITY ECONOMICS

APRIL 2020

ABOUT THE MCKELL INSTITUTE

The McKell Institute is an independent, not-for-profit, public policy institute dedicated to developing practical policy ideas and contributing to public debate.

For more information phone (02) 9113 0944 or visit
www.mckellinstitute.org.au

ANGELA JACKSON

Angela Jackson is an experienced public sector economist with almost 20 years' experience working as an economist on public policy issues.

Angela was Senior Advisor and Deputy Chief of Staff to the Australian Minister for Finance and Deregulation from November 2007 to September 2010.

Currently completing a PhD in health economics, Angela has specialised knowledge of the health sector alongside an intimate understanding of government.

Angela also serves as a non-Executive Board Member of Melbourne Health, which runs Royal Melbourne Hospital, and is a member of the Victorian Advisory Board for the National Heart Foundation.

Angela was the author of *Disability &Rare Disease: Towards Person Centred Care for Australians with Rare Diseases* published by the McKell Institute in October 2019.

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Living with Duchenne & Becker in Australia

SUPPORTING FAMILIES *waiting for* A CURE

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APRIL 2020

CONTENTS

FOREWORD

In 2019, the McKell Institute released Disability & Rare Diseases: Towards Person Centered Care for Australians with Rare Diseases. That project highlighted the gaps in care for Australians living with rare disease including those with Duchenne muscular dystrophy, and the less common Becker muscular dystrophy, the conditions which are the focus of this report.

Duchenne and Becker are genetic, muscle-wasting conditions that affect around 1000 Australians. While there is hope for a cure, this report highlights the challenges facing many Australians – and their families – living with the condition today.

This report details the issues and challenges facing those living with Duchenne and Becker and their families as they wait for a cure. It explores the gaps in the existing healthcare system and the costs facing families whose family members live with Duchenne and Becker, undertaking a cost of disease analysis, and surveying 150 families and individuals experiencing the impacts of the disease.

The survey results paint a picture of a healthcare system that often fails to meet the needs of those living with Duchenne and Becker. While this report highlights the fact that Australia's system compares well to international benchmarks, it makes clear there is more to be done.

20 per cent of survey respondents had a delay of more than three years between noticing symptoms and receiving a diagnosis, and a clear variation in the quality care was identified across state and territories. Alarmingly, the survey also highlights the shortfalls of the National Disability Insurance Scheme (NDIS) for those with Duchenne and Becker. 16.6 percent of respondents told of worse treatment under the NDIS, while a further 31.1 per cent said the NDIS had neither improved nor worsened their situation.

Of course, Duchenne and Becker is first and foremost a healthcare challenge. But inadequate treatment mechanisms impose a significant economic burden on families and the economy, too. Children born with Duchenne or Becker have a life expectancy of less than 30 years. This report estimates that for families, the costs of lifetime social care and health care can total around $2.25 million, with the reduced workforce participation of families due to caring costing over $600,000.

The NDIS is a landmark, $22 billion per year reform. But it is clear that more needs to be done for Australians with rare diseases such as Duchenne and Becker. To that effect, this report makes 12 actionable recommendations, ranging from ideas aimed at achieving earlier treatment and diagnosis, to improving the delivery of care, funding future therapies, and improving access to clinical trials.

While most Australians enjoy access to world-leading care, our health system always needs improving – especially for those living with rare conditions. This report makes an important contribution towards that aim of achieving a better healthcare system for all Australians.

SAM CROSBY
CEO, MCKELL INSTITUTE

EXECUTIVE SUMMARY

Duchenne muscular dystrophy is a rare disease,[1] but the most common of the muscle-wasting diseases affecting children.[2,3] Children with Duchenne cannot produce a protein needed for muscle strength and function, and over time this leads to muscle damage.[3] Children with the less common Becker muscular dystrophy have an error on the same gene, but experience less severe symptoms as their bodies can still produce some of the protein.[4,5]

Receiving a diagnosis of Duchenne or Becker places a timer on when a child will start to lose physical functioning, and eventually die.[2] Advances in treatment have significantly improved the life expectancy and function of children born with Duchenne,[6,7] but most children with the disease do not live past their 30th birthday.[6,8]

The symptoms of Becker generally don't present until children are older, and often only in adulthood.[4] Damage is slower in Becker because while those with the condition produce some of the muscle protecting protein, this is at insufficient levels to stop damage.[4]

Available therapies have helped slow the progression of Duchenne and Becker, but have not provided a cure.[2,9] There is however hope. Gene therapies in the final stages of development could cure Duchenne and stop the timer for many children – offering the hope of a healthy and long life for children with Duchenne and Becker today and into the future.[10]

For many, when a cure becomes available the disease will have already progressed to the point where they have lost the ability to walk and breathe independently. Even with a cure these children will need ongoing care and support for the rest of their lives.

This paper aims to highlight some of the issues and challenges facing families of children and adults with Duchenne and Becker as they wait for a cure. We aim to better understand how Duchenne and Becker impacts families, how we can ensure that new treatments benefit Australian children sooner and better support those with the condition today.

A cost of disease study undertaken for this report shows that Duchenne is associated with significant lifetime health and social care costs. We estimate that these can total up to $2.25 million for a child living until their mid-thirties. In addition, informal care costs total up to $630,000 in terms of reduced female participation in the workforce. The cost of any gene therapy for Duchenne needs to be seen in the context of these lifetime costs.

Surveying over 150 people and their families with Duchenne and Becker muscular dystrophy we provide a comprehensive picture of the experience of people living with these conditions in Australia today.

The survey highlighted that many Australians are waiting too long for a diagnosis and continue to endure a diagnostic odyssey before being able to access treatment for the condition and receive genetic counselling on their future reproductive choices.[11]

20 per cent of respondents had a delay of more than 3 years between first noticing symptoms and receiving a formal diagnosis.

While Australia compares favourably to international benchmarks,[9,12,13] there are large variations across the state and territories indicating more could be done. Newborn screening for Duchenne and Becker would help end this odyssey and ensure that future genetic treatments are administered before significant loss of function occurs.

The survey also highlighted ongoing issues with the support provided to children with Duchenne and Becker through the National Disability Insurance Scheme (NDIS), that were highlighted in the 2019 McKell Institute report: *Disability and Rare Disease – Towards Person Centred Care for Australians with Rare Diseases*.[14]

The National Disability Insurance Scheme (NDIS) aimed to transform the lives of people with a disability, with an unprecedented boost in funding for services and supports. In this context, a remarkable 16.6 per cent of respondents to the survey said that the $22 billion NDIS had worsened their situation, and a further 31.1 per cent said that the scheme had neither improved nor worsened their situation. Delays in receiving equipment was often raised by those indicating that the NDIS had worsened their situation.

There is an urgent need for the NDIS to ensure that it can quickly meet the changing needs of people with a disability. These issues were reflected in the recent Tune Review of the NDIS, where it was highlighted that the lack of flexibility created issues for participants with changing needs.[15]

The NDIS's ongoing inability to adjust to changes in the needs of clients is significantly impacting children with Duchenne and Becker that have constantly changing needs due to the progressive nature of the disease. We recommend that the Australian Government accept and implement the recommendations of the Tune Review to address these issues, and that the NDIA immediately provide additional support to families of children with Duchenne and Becker to overcome bottle necks.

Meanwhile genomics gets set to revolutionise health care, with over 750 treatments currently under development.[16] From 2025 it is expected that up to 20 new treatments will become available every year[17] – each with the capacity to transform lives but also associated with high additional costs for health systems.

The National Health Genomics Policy Framework provides the roadmap for our health system in dealing with the new treatments in the pipeline, however there is concern that the health system remains unprepared for the tsunami that is about to hit.[16]

This lack of preparedness will undermine the financial sustainability of our health system, but also potentially delay or deny Australians access to life-changing treatments.[16]

We recommend that the Australian Government prioritise the development of clear funding mechanisms for new gene therapies as part of the review of the National Health Genomics Policy Framework in 2020. This will provide industry and those hoping to benefit from the new treatments with greater certainty and understanding of the Government's proposed approach.

There are also ongoing issues with the approval of clinical trials in Australia, that are undermining efforts to ensure Australian children have access to the next phase of clinical trials for the new gene therapies. While national reforms are underway, our review of international practice highlights that they do not go far enough in streamlining and centralising approval processes, nor do they address issues specific to gene therapies. More can be achieved to ensure that Australian children with Duchenne have the opportunity to participate in these clinical trials.

Our review of regulatory regimes in the United Kingdom, Europe and Canada found that **Australia is the only jurisdiction that requires licensing of genetically modified o ganisms for use in clinical trials or approval by a separate gene technology regulator.** This adds to approval times and hinders the ability of Australian children to gain access to important clinical trials.

We make a number of recommendations that will ensure Australia's health system is world leading, and competitive in attracting clinical trials, including a central one-stop platform, a single application form, a national body to oversee clinical trials and national uniform legislation. In addition, we call for the Government to move to streamline applications for undertaking clinical trials with genetically modified organisms, to ensure Australian children do not miss out on participating in these clinical trials.

These are exciting times for Australians with rare genetic conditions, including with Duchenne and Becker. Advances in medical science mean that there is now the real prospect of a cure. As families continue to wait however, the Government can take action today to provide these families with the support they need and ensure the earliest possible access for their children to these life-changing treatments.

THE McKell Institute

RECOMMENDATIONS

Getting Treatment Earlier

RECOMMENDATION 1
The Departments of Health and Primary Care Networks in New South Wales and Tasmania review the diagnostic processes and pathways for Duchenne diagnosis with the aim of reducing the national variation in time to diagnosis.

RECOMMENDATION 2
The Government fund a trial and evaluate the cost-effectiveness of pre-conception and newborn screening for Duchenne.

Getting the Care that is Needed

RECOMMENDATION 3
Urgent review of delays in access to equipment to ensure that NDIS participants receive approved equipment in a timely manner.

RECOMMENDATION 4
The NDIA establish a specialist team focused on ensuring children with Duchenne and Becker are not facing avoidable delays in receiving equipment.

RECOMMENDATION 5
The Australian Government provide funding to establish up to two Centres of Excellence for Duchenne and Becker in Australia.

RECOMMENDATION 6
The State and Territory Governments to provide funding certainty for neuromuscular nurses to provide care coordination for all patients with Duchenne and Becker.

Funding Future Therapies

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.

Improving Access to Clinical Trials

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.

INTRODUCTION

Background

Around 1000 Australians are currently living with Duchenne and Becker muscular dystrophy in Australia, the most common muscle-wasting disease affecting children.[i]

Duchenne and Becker are rare diseases that present many challenges to families affected by the condition.[18] A lack of understanding or knowledge from medical professionals, and uncertainty around what the condition will mean for individual children makes facing a new diagnosis even more difficult.[19]

Children with Duchenne and Becker are born with a fault, or mutation, in the longest gene in the body.[2,9,20] This fault stops their body producing a protein (Duchenne) or reduces the amount of protein produced (Becker), dystrophin, which is vital for muscle strength and function.[4,20,21] Without the protein all the bodies' muscles, including the heart, progressively weaken over time.[4,21]

Boys are predominately affected by Duchenne and Becker because they only have one of the genes that produce the protein.[2,4,21] Girls have two of the relevant genes and as long as one of them does not have the fault they can still produce the protein and do not develop Duchenne, but can pass the condition on to their children.[4,21]

Duchenne progresses through childhood and into early adulthood.[2] Becker often doesn't start to impact physical functioning until later childhood or early adulthood.[4] While other children gain more physical abilities as they age, children with Duchenne progressively lose function. After taking away their ability to walk at around 13 to 14 years of age, Duchenne robs children and adults of their ability to breathe independently, to talk, and undermines heart function, eventually causing premature death.[2,8]

Because Duchenne impacts so many parts of the body, those affected require large teams of specialists to oversee their medical care.[22] In addition, from early adolescence through to the end of life children with Duchenne require significant social care support, from both formal and informal sources.[23]

The progressive nature of Duchenne means that the needs of those affected are constantly changing, and the treatments they require to maximise their functioning is always shifting. This makes securing the necessary medical and social care supports in a timely manner critically important.

Diagnosis

The first symptoms of Duchene typically emerge after a child's first year of life, but diagnosis does not typically occur until around 5 years of age.[12] Symptoms are varied and can range from frequent falling, difficulty running or climbing stairs and the inability to get up off the floor.[21] Speech delays can also be common, alongside comorbidities including autism, intellectual disability and ADHD.[21]

CASE STUDY

7 year-old Harrison lives in Perth and was diagnosed at the age of 3.

There is no history of Duchenne in our family, but my wife was a carrier of the gene and passed it on to our first son Harrison.

When he was around one, we noticed that he was not developing at the same rate as other children so we started down the process of consulting professionals which ultimately led to diagnosis. This took over two years and by that stage we had had our second son, Jack who was also at risk of having Duchenne. We were lucky that he was not impacted.

Having a son with a rare disease means we often know more about his condition than his Doctors and it's a lot of work to keep up to date with developments in treatments so that he continues to get the best care possible. I think I may have read every single article about Duchenne on the internet!

With treatment including his fantastic physio team Harrison has improved over time, but we have reached the plateau now where the gains are over. Stairs are starting to become harder and we know that without a corrective therapy his physical capabilities will start to deteriorate in the future.

My wife and I try our best to make our family life as normal as possible, so that both our sons experience a childhood just like other kids. It is hard, and behind closed doors the journey we are on can go from being full of hope to full of despair.

We hope that a cure will be found and that our son will live his best possible life. We despair that as much as we will continue to fight for him, accessing a cure may be too late for his journey. So we try and make sure he is happy regardless of what happens to him physically.

We are just trying to keep his body in the best possible shape until help arrives in the form of a corrective therapy.

It is this hope that keeps us going, and why our son taking part in a meaningful clinical trial in Australia would mean the world to our family.

Diagnosis can take years and involve multiple medical professionals, as knowledge of the condition is not high and misdiagnosis common.[13,24]

Earlier diagnosis is important for a number of reasons.[9]

Because the condition is genetic there can be multiple cases within the same family. Delays in diagnosis mean that families have multiple children with the condition before they receive the diagnosis for the eldest child.[12] This compounds the effect on families and could be avoided with earlier screening for the condition.[12]

Importantly the earlier children start treatment the better the long-term prognosis. Treatments currently include physiotherapy and steroid treatments, that can help maximise muscle functioning and reduce the damage to muscles.[9]

i This is based on both population wide estimates and reported number of patients at major clinics around Australia.

COST OF DISEASE ESTIMATES

The medical, formal and informal care costs of a child with Duchenne rise over their life as their physical functioning slowly declines.

In order to better understand these costs we have calculated the cost of Duchenne over a person's life (please see Appendix 1 for full methodology). We have taken the perspective of a child born today to provide a clear picture of future potential costs in the absence of a curative treatment.

Whereas decisions to fund new therapies can often focus solely on the improvements in quality of life and incremental increase in health care costs, in the case of Duchenne a wider perspective provides a more comprehensive picture of the true costs of the disease.

Using previously published research and reported costs from the survey we are able to estimate the cost of a child born with Duchenne today in Australia over their expected life in terms of additional health, formal and informal caring costs.[22]

The analysis shows that the expected lifetime medical costs of Duchenne currently average $300,000, but for a person that survives up to their mid-thirties can reach $590,000. In addition, the expected lifetime social care costs average $700,000, but for a person surviving into to their mid-thirties cost up to $1.67 million.

In addition, we can estimate the impact on maternal labour supply of having a child with Duchenne. It is estimated that lost hours worked can be expected to cost families $339,000 on average with the cost for a child that survives until their mid-thirties rising to $631,488.

The financial cost of Duchenne therefore over the lifetime of a child born today can be expected to be $1.3 million with the cost for a child living to their mid-thirties of $2.88 million.

Treatments for Duchenne

The medical management of all muscular dystrophies has been transformed over the past twenty years, significantly improving life expectancy.[6,8] This has been driven by the widespread use of corticosteroids, alongside the optimisation of physiotherapy and cardiorespiratory care.

Gene therapy treatments are now being developed which promise to stop the progression of Duchenne, however early treatment will be critical as even with these ground-breaking treatments it is not possible to reverse damage already done to muscles.[5,10]

There is also uncertainty over the funding of new gene therapy treatments when they do become available. Australia's current funding of pharmaceuticals is geared towards ongoing treatments rather that one-off curative treatments, and it is not clear how the health system will meet the substantial costs associated with gene therapies.[16]

Clinical Trials in Australia

Any parent of a child with a rare or non-rare disease that is life threatening or life shortening wants their child to be able to access new treatments as soon as possible. The opportunity to participate in clinical trials is critical for Australian children living with Duchenne to enable early access to investigational treatments not otherwise available in Australia that may extend or improve the quality of their lives, as well as the development of urgently-needed new therapies.

While a number of clinical trials have been conducted in Australia for Duchenne and Becker treatments, delays and regulatory complexity in the approval process for gene therapy trials may threaten access for Australian children.

The National Disability Insurance Scheme

Launched in July 2013 the National Disability Insurance Scheme will be fully implemented by mid-2021. The scheme will cover 475,000 Australians when fully implemented and cost over $22 billion a year.[25]

The NDIS has replaced a number of State-based schemes providing support to children and adults with Duchenne and Becker. It provides individualised support which is agreed through face-to-face meetings with an NDIS planner and local area coordinators.[25]

While this greater flexibility is a positive, issues have been identified with the NDIS not being responsive to individuals with changing needs and failing to provide integrated care across the health and disability systems.

In the 2019 McKell Institute report *Disability and Rare Disease – Towards Person Centred Care for Australians with Rare Diseases*, we highlighted that the NDIS is struggling to deal with some clients that have rare diseases, such as Duchenne and Becker.[14] Of particular concern was the fragmentation of care and delays in access to necessary equipment.

For children with Duchenne these issues are particularly relevant due to the underlying medical nature of the condition and the changing needs of the condition.

Current Action

There is significant community and political support for families and those affected by Duchenne and Becker.

Since it was established in 2008, Save Our Sons has raised over $20 million through generous community support. This has allowed the Foundation to fund a number of specialist nurses in clinics for Duchenne across Australia, help Australian children access clinical trials, for important research into the conditions, and to provide quality-of-life enhancing equipment.

However, more is required and with the potential for a cure, there is a need to make sure the system allows Australian children timely access to treatments that offer hope for families impacted by the condition.

LIVING WITH DUCHENNE IN AUSTRALIA

Having a child diagnosed with a rare condition is often a confusing and lonely experience.[18] Unlike other aspects of parenthood those in your social and support networks are unlikely to have ever experienced what you are going through, and this can add to the isolation felt by families.

Medical professionals can lack knowledge of the condition, placing a significant burden on parents to become 'experts' in their child's condition.

The additional needs of children increases the caregiving role and impacts a family's ability to work, and therefore their financial security.[18] Other children in the family may also be affected by the limits that having a sibling with a rare condition places on the activities a family can undertake and the financial resources available.

We undertook a survey of families with children with Duchenne and Becker and those living with Duchenne and Becker in Australia to better understand their experience of living with the condition and how it is affecting their lives. Below we present the findings of the survey before discussing the key issues raised by families and those living with Duchenne and Becker in Australia.

The survey was launched on Survey Monkey on 4 December 2019 and was promoted heavily on social media and through direct communication with families registered with the SOS Duchenne Foundation. Closing on 23 December 2019 there were a total of 173 responses, a sizeable sample of the estimated total population living with Duchenne and Becker in Australia.

CASE STUDY

Sam lives in Queensland and is the father of 13 year-old Lila

Duchenne is a rare disease in boys, but in girls it is even rarer. We noticed Lila was not developing normally at around 2 years of age, and a number of tests were done. If she had been a boy we would have been diagnosed then, but because she was a girl they thought it couldn't be Duchenne and didn't do the final tests.

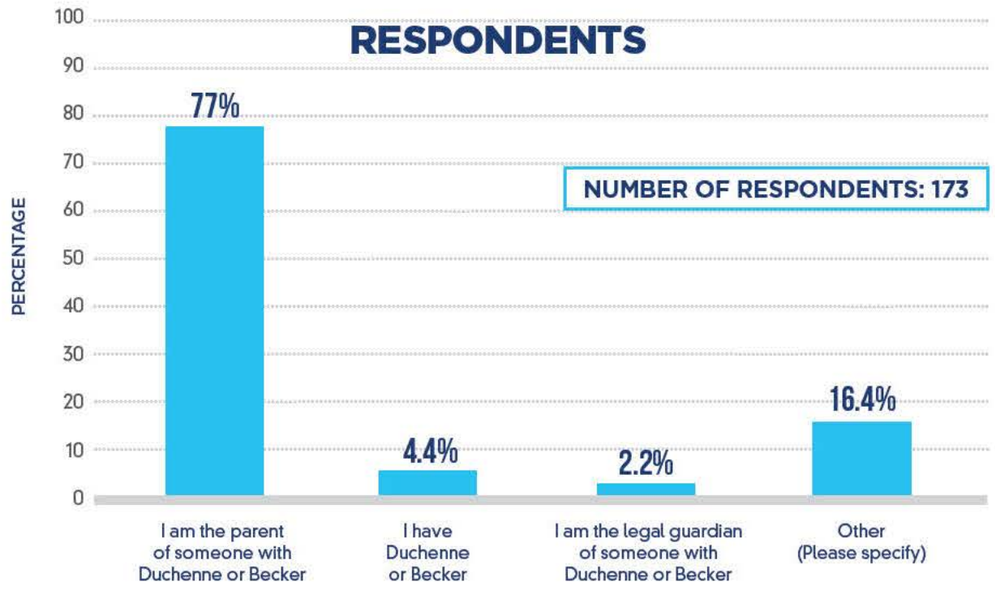
Four years later, and another round of tests and we finally got the diagnosis. This is why if newborn screening is introduced it will be for girls as well as boys, because girls have such a hard time getting a diagnosis. It would also make sure girls know they have the gene, so they don't inadvertently pass it on to their sons.

The doctors looking after Lila have been great, but there is so little research on girls with the condition and so people don't know how it will progress and how will impact Lila. We really hope that more research will be done on girls with the condition so other families have more information and better care for their girls.

We have four other kids, and its hard on them. Lila is very often the focus, because her needs are so great and we try to only do things as a family that she can join in with. This means there is a lot we cannot do.

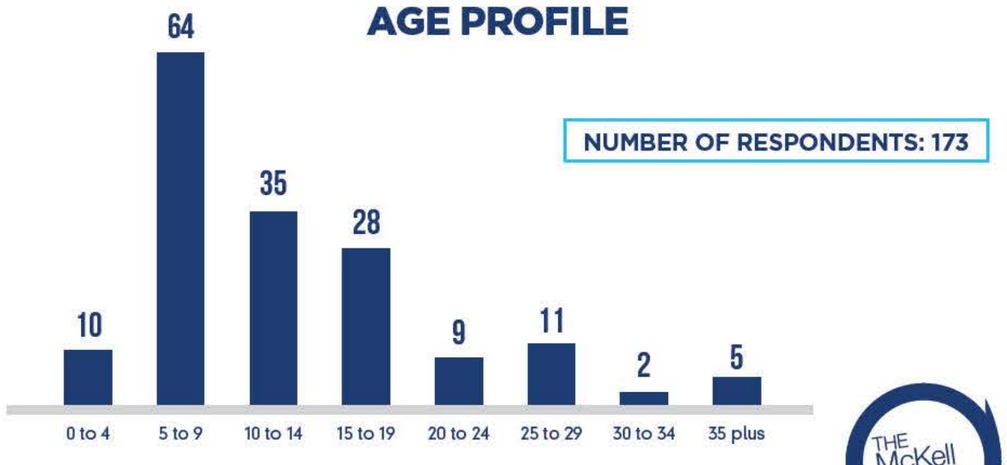
Survey Results

The majority of respondents to the survey were parents of children with Duchenne and Becker, representing 77.05 per cent of the sample. Within the Other Category, the largest groups were Grandparents (23.3 per cent) and Siblings (30 per cent).



Age

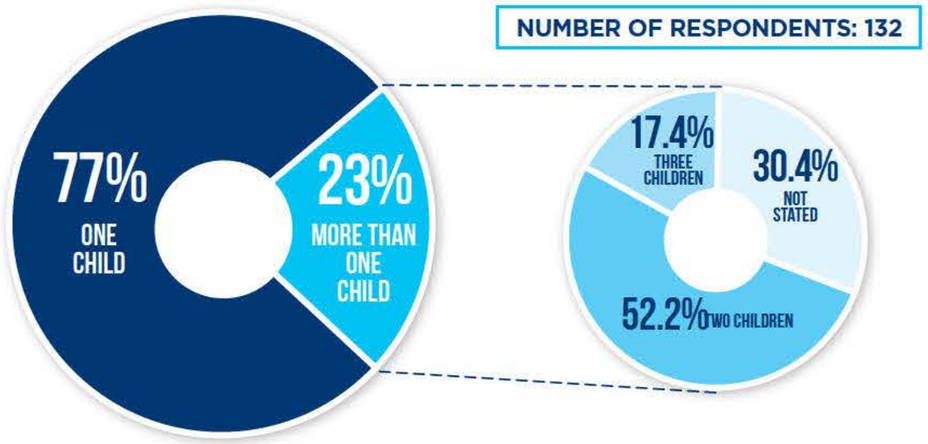
The average age of the person affected by Duchenne and Becker was 13 years, with age ranging from 1 year to 50 years.



Family

We asked whether there were other children in the family with Duchenne or Becker, and if so how many children. **23.5 per cent of parents responded that they had more than one child with the condition.** Of those the majority had two children with Duchenne or Becker.

NUMBER OF CHILDREN WITH DUCHENNE OR BECKER IN FAMILY



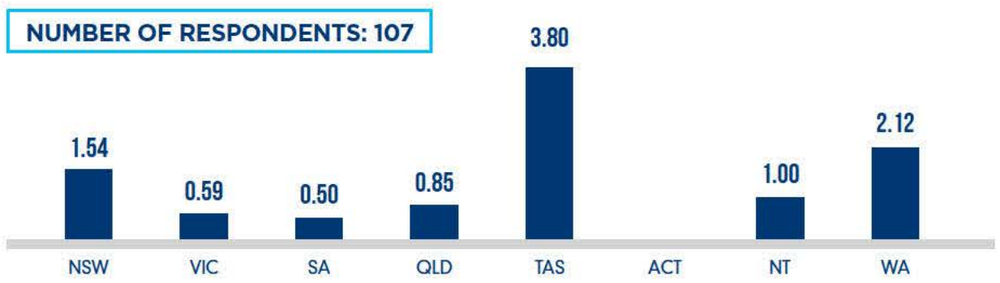
Delays in Diagnosis

Responders were asked at what age first symptoms became apparent and then at what age the child received a formal diagnosis for Duchenne or Becker. The average age of diagnosis was 4.39 years, with a range from 1 to 20.

The average delay in diagnosis was 1.09 years, but we observed variation across state and territories. The longest delay was in Tasmania at 3.8 years. Of interest there was a notable difference between the two largest states, with **the average delay to diagnosis in NSW was 1.54 years versus an average delay in Victoria of 0.59 years.**

The difference between states illustrates that there are gains to be made in reducing the delay between first symptoms and diagnosis, which will become more critical when gene therapy becomes available.

DELAY TO DIAGNOSIS



Getting a Diagnosis

Respondents were asked about the process of diagnosis.

Families saw on average three professionals before they received a diagnosis, with 30.4 per cent seeing four or more health professionals to get a diagnosis. Many respondents indicated that either there was insufficient information or support provided around the diagnosis when asked to comment on what could be improved about the process.

Numerous respondents highlighted the role of the Save Our Sons Duchenne Foundation in providing information when they first received the diagnosis.

"We knew nothing. The process of diagnosis was hard for us because we were not informed at all and we felt so lost and alone. We didn't know what to do." **SURVEY RESPONDENT**

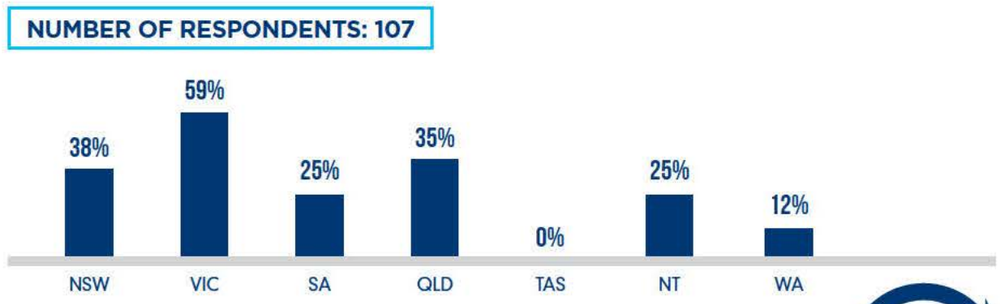
"If GP's knew the signs and listened to my concerns we would of had an earlier diagnosis, earlier intervention and perhaps a better chance of being ambulatory longer."
MOTHER FROM WESTERN AUSTRALIA

"I THINK ALL CHILDREN SHOULD BE TESTED IF THERE ARE SIGNS OF MUSCLE WEAKNESS, BECAUSE MY DAUGHTER WAS A FEMALE SHE WASN'T TESTED FOR YEARS."
MOTHER FROM QUEENSLAND

Access to Clinical Trials

36 per cent of respondents reported having accessed clinical trials. The majority of these were in Victoria, with almost 60 per cent of respondents from Victoria indicating they had participated in a clinical trial. This compared to 38 per cent of respondents from New South Wales. No respondents indicated that they were involved in a trial for gene therapy.

CLINICAL TRIALS



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Treatment

The survey asked respondents a number of questions about their treatment for Duchenne. The gold standard of care includes the use of steroids and regular contact with a Cardiologist, Neurologist and Respiratory Physician.

We found that there were large differences across regional, rural and city areas with the number of respondents accessing gold standard care.

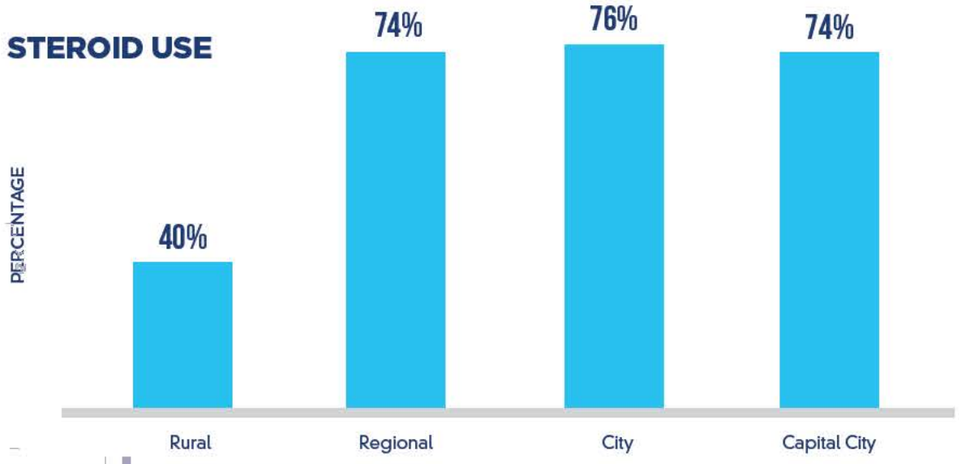
"Overall we are very happy with the care and treatment options of our son since his diagnosis. We feel closely connected to our wonderful team and know we are in the best hands despite the prognosis. We have benefitted from access to local trials and kept informed about ongoing treatments and trials coming up. Information is only a phone call or email away with knowledgeable, kind and caring staff. It helps having a great team involved at times when you feel isolated or unsure." **MOTHER FROM NEW SOUTH WALES**

Steroid Use

While steroid use will not be suitable for every child with Duchenne, it is regarded as the first line treatment to slow the progression of the condition. 72 per cent of respondents reported the use of steroids.

There was a very low level of use in rural areas, with just 40 per cent of respondents reporting the use of steroids. This compared to between 74–76 per cent in regional and city areas.

The main reason given for not being on steroids was a belief that they would not be beneficial and concerns about side effects. A number of children were too young to commence steroid use, and parents reported that they would commence once they were older.

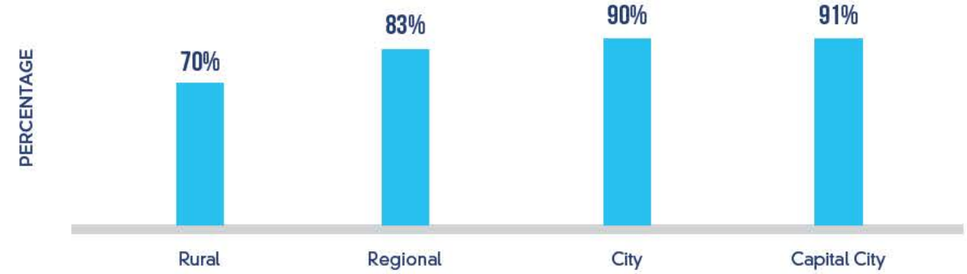


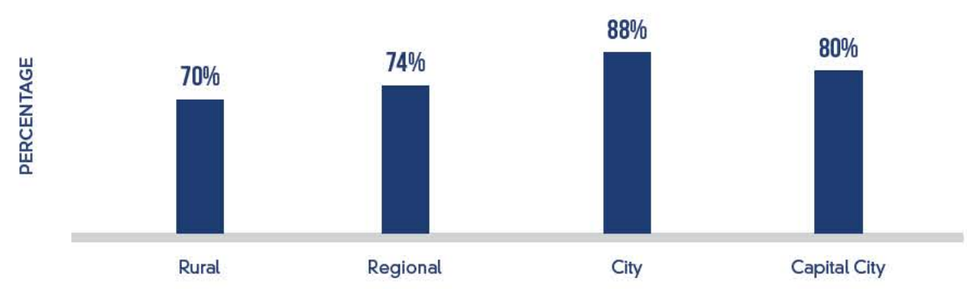
NUMBER OF RESPONDENTS: 109

Neurologist, Cardiologist and Respiratory Physician

There are a number of Specialists involved in the care of people with Duchenne, but three specialists – Neurologists, Cardiologists and Respiratory Physicians are considered necessary for gold standard care.

66.4 per cent of survey respondents reported having seen all three of these specialities over the past year. There were some differences across regional and rural areas and access to the specialists, with those living in rural areas less likely to see each of the specialists.





NUMBER OF RESPONDENTS: 109

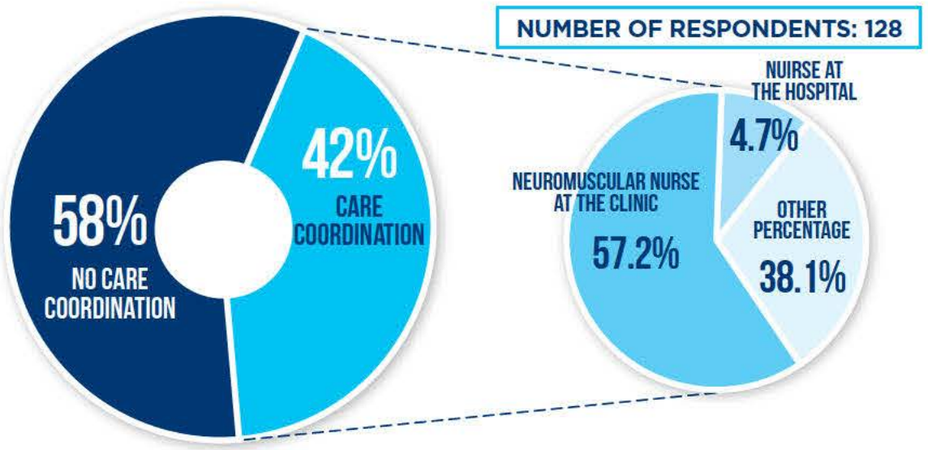
Care Coordination

With respondents reporting seeing an average of 9.65 health professionals over the past year, coordination of care is critical. However only 39.4 per cent of people reported having someone help with their care coordination. Of those the majority were seen by Neuromuscular nurses at clinics which are currently funded by the Save Our Sons Duchenne Foundation, funders of this report.

"I found that once we left the Children's Hospital because he was an adult, we were left on our own. We were linked in with the respiratory team, and a cardiologist. We had and still do have a regular neurologist. Our GP doesn't know anything about Duchenne. So we have nobody overlooking his whole health. We really are out on our own, trying to figure out his health care. It's a disgrace!"
MOTHER FROM REGIONAL SOUTH AUSTRALIA

"We need to have coordinated services in the adult hospitals we end up going multiple times sometimes in a week and have to travel an hour each way to get there. Why is this achievable for children but not adults? What changes the day you turn 18? If anything it gets harder."
MOTHER FROM REGIONAL WESTERN AUSTRALIA

CARE COORDINATION



"WE LIVE IN PERTH WA. UNFORTUNATELY CLINICAL TRIALS ARE NOT COMING TO PERTH AT THIS POINT IN TIME. TRAVELLING TO SYDNEY OR MELBOURNE IS NOT FINANCIALLY AN OPTION."

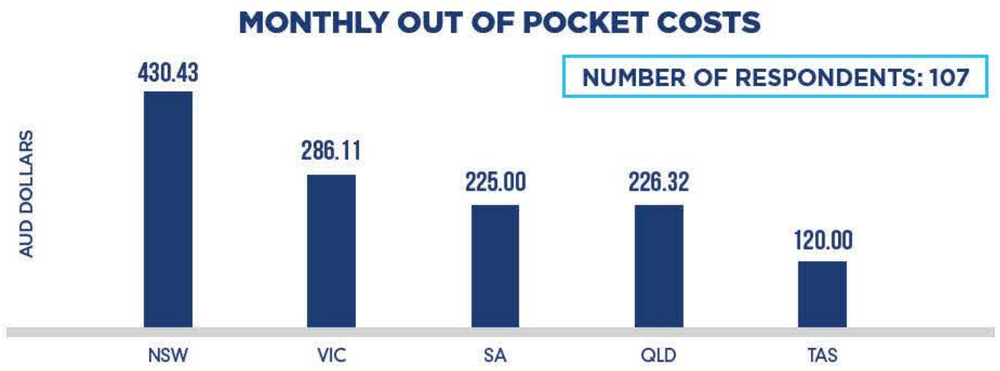
MOTHER FROM WESTERN AUSTRALIA

THE McKell Institute

Cost of Supporting a Child with Duchenne

Families reported high out of pocket medical costs, ranging to $1800 per month. Out of pocket costs were much higher in NSW than in other states and territories.

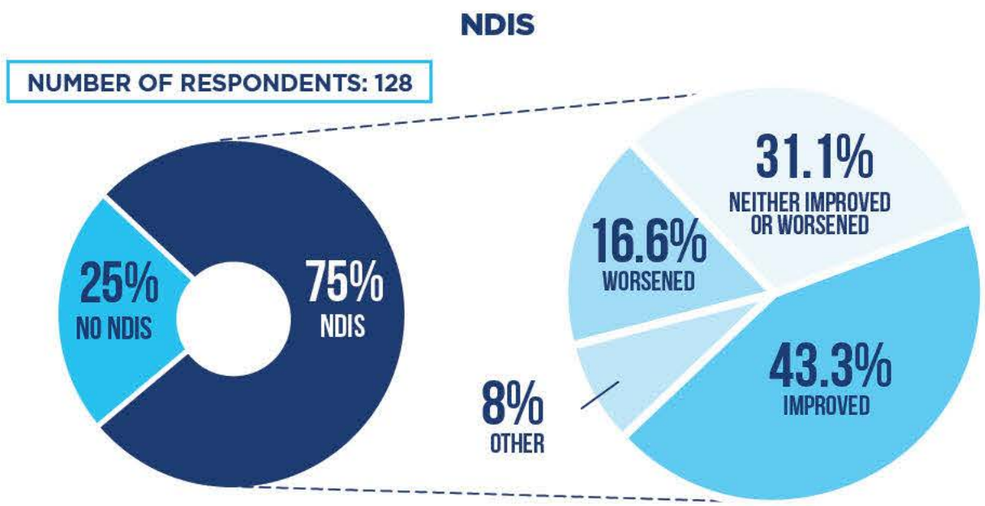
Out of pocket costs in NSW were $430.43 per month on average, compared to $250 per month on average across the other states and territories.



The National Disability Insurance Scheme

Of respondents to the survey 75 per cent receive services and supports under the NDIS.

43 per cent of respondents receiving services and supports from the NDIS indicated that services had improved, and 16 per cent reported that services had worsened.



> "NDIS IS A NIGHTMARE, NO SERVICES IN MY AREA, CAN'T GET RESPITE WORKERS OR PERSONAL CARERS WHEN WE REQUIRE IT, SEVERE SHORTAGE OF OT'S SO CAN'T GET THINGS DONE WITH NDIS"
>
> MOTHER FROM VICTORIA

"Receiving equipment and support is still just as slow under NDIS and amount of paperwork and hoops to jump through is bigger. I'm still waiting for a manual wheelchair after six months even through NDIS only took a few weeks to approve."
MOTHER FROM QUEENSLAND

"We are currently waiting to be enrolled into the NDIS which is a slow frustrating process. Financially looking at renovations for an accessible bathroom & accessible vehicle is both overwhelming & daunting!! We need to find a cure soon as the cost of living with this condition is both financially and emotionally draining for our son and our entire family."
MOTHER FROM WESTERN AUSTRALIA

"NDIS is shocking, cause families unnecessary stress, as they don't understand the condition."
MOTHER FROM VICTORIA

"The constant delays and underfunding had a very detrimental impact on Ali's condition and that these are the things that NDIS don't understand – this condition is degenerative and time is not a luxury for children with Duchenne. "
MOTHER FROM NSW

Key Issues

The survey and stakeholder consultations highlighted a number of key issues facing families living with Duchenne and Becker in Australia.

These include timely diagnosis, the quality and timeliness of care, access to clinical trials and access to new treatments.

Addressing these issues would help support families with a child with Duchenne and ensure that children have the best possible prognosis.

Timely Diagnosis

Timely diagnosis is critical for children with Duchenne and Becker as it ensures that they receive the optimum care and allows genetic counselling to assist in their reproductive planning.[12,13,24]

The average age of diagnosis in the survey was 4.38 years and the average delay between first symptoms and diagnosis was 1.10 years. Both compare favourably to the available international evidence[ii] (see Table below) however variation across state and territories indicates that under current arrangements more could be done in some states and territories.

	AVERAGE AGE OF DIAGNOSIS	AVERAGE DELAY
Previous Papers		
United States[13]	4.9 years	2.4 years
United Kingdom[26]	4.3 years	1.6 years
Europe[26]	4.3 years	1.3 years
SOS Duchenne Survey		
NSW	4.69 years	1.54 years
VIC	3.56 years	0.59 years
SA	2.75 years	0.50 years
QLD	4.50 years	0.85 years
TAS	6.00 years	3.80 years
NT	2.50 years	1.00 years

RECOMMENDATION 1

The Departments of Health and Primary Care Networks in New South Wales and Tasmania review the diagnostic processes and pathways for Duchenne diagnosis with the aim of reducing the national variation in time to diagnosis.

ii Note these figures are for Duchenne only and do not include estimates for Becker.

Many families still experience a 'diagnostic odyssey' with 1 in 3 seeing more than 3 health professionals to receive a diagnosis and 1 in 5 having delays of three years between first seeking medical treatment and receiving a diagnosis.

With the availability of potentially curative gene therapies the importance of earlier diagnosis will become critical. By the stage most children are currently diagnosed, they have already suffered irreversible muscle damage.

Newborn screening would help end this odyssey, and provide much earlier and more accurate diagnosis of the condition.[27] It involves a similar multi-step process to that currently used in the National Screening Programme for cystic fibrosis. First a blood test identifies babies at risk of the condition. Those babies identified then have a diagnosis either confirmed or refuted through a DNA test.

A number of pilot studies internationally have demonstrated the efficacy of newborn screening,[11,28] but no studies have yet been undertaken in Australia.

The current Mackenzie's Mission pilot is undertaking pre-conception screening of 700 autosomal recessive and X-linked conditions, including Duchenne.[29]

However, such a programme if instituted nationally would not necessarily pick up every case of Duchenne, as the error can occur for the first time in a child and not be inherited from a parent. In order to pick up these cases as early as possible newborn screening for Duchenne would still be necessary.

RECOMMENDATION 2

The Government fund a trial and evaluate the cost-effectiveness of pre-conception and newborn screening for Duchenne.

Quality and Timeliness of Care

Duchenne and Becker are progressive diseases, and a child's needs can change quickly. The NDIS is not geared towards participants with changing needs, which means it can often fail to provide children with the equipment and services they need in a timely manner.[15]

When children with Duchenne and Becker do not receive equipment or treatment in a timely manner, it can lead to quicker progressions of the disease. This robs children of more time with the ability to undertake certain activities, before that ability is lost forever.

Analysis of those who responded that the NDIS had made their situation worse in the Save Our Sons Duchenne Foundation Survey showed that it was a lack of responsiveness that drove much of the negative experiences of the NDIS.

> NDIS is a great system but we had waited for 9 months without hearing anything about our minor modification and equipment replacement applications. Took two months of unanswered phone calls and emails to LAC and calls to NDIA to get anything to happen. Claims should be triaged as urgent, non-urgent major, non-urgent minor with rough turnaround times (within life of plan) indicated.
>
> **MOTHER FROM QUEENSLAND**

> There needs to be faster approaches to obtaining equipment needed for a degenerative condition like this. Waiting over 12 months for equipment to help prevent contractures etc, is ridiculous. A treatment has been approved in the USA. We need it here and now. How do we do this?!
>
> **MOTHER FROM WESTERN AUSTRALIA**

These findings are consistent with the findings of the McKell Institute report *Disability and Rare Disease: Towards Person Centred Care for Australians with Rare Diseases* and also the recently released Tune Report into the NDIS.[14,15]

The 2019 Tune report also made a number of recommendations to improve the administration of the NDIS, and notes that the delays in equipment are of particular concern.[15]

Addressing these issues is a priority for children with Duchenne and Becker, and the Government should prioritise these reforms in its forthcoming response to the Tune Report. In the interim we also recommend that the NDIA establish a specialist team focused on ensuring that children with Duchenne and Becker are not facing avoidable delays in equipment.

RECOMMENDATION 3
Urgent review of delays in access to equipment to ensure that NDIS participants receive approved equipment in a timely manner.

RECOMMENDATION 4
The NDIA establish a specialist team focused on ensuring children with Duchenne and Becker are not facing avoidable delays in receiving equipment.

CASE STUDY

Chris, 28 year old living with Duchenne in Victoria

My name is Chris and I live in Warrnambool in Victoria. I am 28 years of age.

While Duchenne makes doing a lot of things difficult, I am focused on doing what I can do find a cure. I work hard to raise money and spread awareness through our

Muscular Dystrophy Awareness Warrnambool foundation. Our aim is to help find a cure for future generations.

I am the eldest in my family. Two years after I was born, but before I was diagnosed my mum had my little brother, Aaron. He died of Duchenne two years ago.

While I know it's been tough for my parents, they have always encouraged us to do what we wanted and found a way to make life as normal as possible. Because of this I don't really feel that Duchenne had a big impact on us growing up. Having a positive attitude is really important to me.

I was able to fully participate in school from Prep all the way up to year 12, and completed a three year business traineeship. This is even though physically I declined through this period.

From around 5 to 11 we just needed buggies or a manual wheelchair for long distances, but started using a manual wheelchair from 11 years of age.

My muscle strength has gradually decreased over time, and for the last few years I have not been able to feed myself or go to the toilet without the help of mum and dad.

Some things would have made life easier, including having a standing wheelchair sooner and some respiratory support that could have reduced the impact of cold and flus while I was growing up.

My Mum and Dad have been everything. They have always done everything for my brother and I. They have had a lot of physical injuries from having to help us, but they wouldn't have it any other way.

Specialist Centres

Most children with Duchenne and Becker receive care through major public hospitals around Australia. However, differences in patient's numbers and resourcing constraints lead to difference in the care received.

A number of respondents from outside Victoria and New South Wales commented that there were issues with specialist teams not being up to date with the latest treatments, requiring families to become experts in the disease.

> "WE SPENT 18 MONTHS TRYING TO DETERMINE THE CAUSE OF DEVELOPMENT DELAYS IN WA. ONCE WE MOVED TO SYDNEY WE FOUND MUCH BETTER HEALTH SERVICES AND WERE DIAGNOSED WITHIN 3 MONTHS. INFORMATION AFTER DIAGNOSIS AND GENETIC SCREENING IN NSW WAS GOOD."
>
> MOTHER FROM WESTERN AUSTRALIA

CASE STUDY

Bailey is 13 years old from Western Australia and was diagnosed with Duchenne at the age of 10

Since diagnosis nearly 3 years ago, our family has been turned inside out and upside down, twice (our then 3 year old was also given the same diagnosis). The amount of TIME we lost in those early years of searching for answers we will never get back. I used to say to my son (pre-diagnosis) that "mummy is trying to find a way to help fix your silly muscles" only to discover I was the reason he has the condition, and that I had unknowingly passed it on to two of my sons. It was soul destroying.

We lost our ability to make better, more educated choices for him, he lost years of invaluable support. We have all suffered with our mental health yet getting psychological support in our NDIS plan is like drawing blood from a stone! There is no support for siblings who also lose out big time.

Early screening through the Newborn screening test, early intervention from child health nurses, GP's. If we had of had that kind of information we could have had the ability to make informed decisions about our son's care, his schooling, and get in some good family adventures whilst he was more mobile.

Our deletion is 'friendly', apparently. Just exon 56 is missing. I'm hopeful exon skipping may be an option but here in WA, I'm not holding my breath, and financially relocating to the east coast would be extremely difficult. (This has been hard to write, as for my own sanity I have to actively choose not to look too far back. It's still extremely raw). But Bailey's story deserves to be told as he's an extraordinary kid, despite his DMD.

THE McKell Institute

In Europe and the United Kingdom centres of excellence are a feature of rare disease policy, and sit at the centre of a system that can better respond to the health and disability care needs of people with rare diseases including Duchenne and Becker.

Given the nature of Duchenne and Becker, and rapidly evolving treatments, there is an acute need for the establishment of specialist centres that have the capacity to acquire and maintain knowledge and expertise through both research and patient interaction.

Through providing a central point of contact for people with rare diseases, their families, and health and disability professionals, everyone can have access to the same information on a rare disease.

RECOMMENDATION 5
The Australian Government provide funding to establish up to two Centres of Excellence for Duchenne and Becker in Australia.

Care Coordination

Gold standard care for Duchenne and Becker requires care coordination, due to the number of specialist required to provide patients with health care.

Respondents saw on average 9.65 medical professionals in the past twelve months, however less than half had any help with care coordination. This places significant burden on families and undermines quality clinical care.

Of those that did have care coordination, the majority had a neuromuscular nurse who are largely funded by the Save Our Sons Duchenne Foundation. The reliance on private funding for these roles on an ongoing basis presents some risk to care of children with Duchenne and Becker.

RECOMMENDATION 6
The State and Territory Governments to provide funding certainty for neuromuscular nurses to provide care coordination for all patients with Duchenne and Becker.

Access to New Treatments

A cure for Duchenne is just one of the 750 gene therapies working through the pipeline. By 2025 the US Federal Drug Administration predict that between 10-20 gene therapies will be added to the market each year.[17]

A tsunami is coming and it is uncertain if our health system and funding mechanisms are ready. The current reliance on existing mechanisms and approaches is not taking into account the specific issue raised by gene therapy for our health system, and without a coordinated strategy Australians will not fully benefit from this revolution in health care.

From ensuring that Australians are accessing the early clinical trials for new treatments, to strengthening diagnostic processes, establishing comprehensive patient registries that facilitate treatment and tracking of outcomes and providing certainty in funding mechanisms – there remains significant policy work to be undertaken in Australia. This relates to Duchenne and Becker but also to other diseases that finally have the hope of a cure through new gene therapies.

While the National Health Genomics Framework offers a roadmap to addresses many of these issues, the timeframes on implementation risks creating delays in access to new treatments. The revolution is occurring now, and our health system needs to be reformed to make sure Australians can benefit.

In particular, while the prospect of gene therapies offer new hope to families that a cure may be soon available for Duchenne and Becker, the cost of such therapies will be prohibitive for most families. They will rely on public funding through Australia's Pharmaceutical Benefits Scheme (PBS).

The PBS has demonstrated its flexibility in pursuing novel funding agreements that may be suitable for gene therapies, however it remains unclear what approaches will be used for gene therapies.

Gene therapies are estimated to cost upwards of $2 million per patient, and will place significant upfront cost burdens on health systems. While our analysis has shown there will be significant savings for the health and social care system, who pays for these new treatments will be an important issue.

We recommend that the Australian Government prioritise the development of clear funding mechanisms for new gene therapies as part of the review of the National Health Genomics Policy Framework in 2020. This will provide industry and those hoping to benefit from the new treatments with greater certainty and understanding of the Government's proposed approach.

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.

Access to Clinical Trials

The importance of accessing clinical trials was brought up in a number of free text responses, with many respondents indicating their willingness to participate and previous failed attempts to be enrolled in clinical trials.

There are currently no trials of the new generation of gene therapies being undertaken in Australia, and there are concerns that Australian children will miss out on the next phase of trials due to be undertaken in 2020.

Pharmaceutical companies claim that it is due to the regulatory burden. Government claims that these problems have been addressed and that Australia is a competitive place to undertake clinical trials.

These are not easy issues to navigate, and in the next section we undertake a review of the approval process for clinical trials in Australia, finding that despite reform efforts there remains a number of areas of concern.

CLINICAL TRIALS

Access to clinical trials is of critical importance to families of children with Duchenne, as they represent the chance to gain early access to new treatments. With the development of potential cures for Duchenne through gene therapy this urgency will become greater.

Clinical trials are also important to the Australian economy, contributing more than $1 billion annually in direct expenditure and investment, as well as broader flow-on benefits.[30]

There are concerns that Australia is not as competitive at attracting clinical trials as it could be, and that its regulatory processes are too cumbersome. In particular there are additional layers of regulatory approval for gene therapies that further slow down processes and undermine the ability of Australians to be included on clinical trials.

There have been recent efforts to streamline processes and provide a more consistent approach across the state and territories, however we recommend that Australia should go further in streamlining and centralising processes. We find this is consistent with international best practice.

Below we outline current approval processes in Australia and compare these to overseas jurisdictions. From this analysis it is possible to make a number of recommendations on possible reforms to the Australian process for clinical trial approval.

Clinical Trials in Australia

Australia has strengths as a clinical trial destination, many of which have been achieved through significant reform efforts over the past decade. These include:[30,31,32]

- efficient regulatory timeframes under the Therapeutic Goods Administration's Clinical Trials Notification Scheme;
- reduced duplication in ethics reviews through the National Mutual Acceptance scheme;
- high quality research and data outputs;
- good reputations of research teams and key opinion leaders;
- established referral networks and national patient databases;
- standardised costing to assist with budget negotiations;
- research and development tax incentives; and
- an ethnically diverse English-speaking population.

However, barriers that contribute to delays in trial start-up times and may work against selection of Australia as a clinical trial site include:[30,33]

- lengthy and variable timeframes for local site governance approvals;
- the lack of a truly nationalised system for ethics approval, resulting in the need for multiple ethics submissions;
- long and separate process for genetically modified organisms; and
- difficulties meeting patient recruitment targets.

Although Australia is a relatively expensive clinical trial location (particularly compared with South-East Asian and Latin American countries), pharmaceutical companies report that they balance cost considerations against data quality, trial start up times and patient recruitment capacity (though companies note that data quality is increasingly seen as a minimum requirement rather than an advantage).

CASE STUDY

Michele's Son David has Becker Muscular Dystrophy and lives in Western Australia

I carried the gene that gave our son David Becker muscular dystrophy without ever being aware. And our daughter carries the gene as well. But only David is affected.

From around three months of age we noticed that he was not developing as expected. David also has an intellectual disability.

There is a massive impact on the whole family unit from what activities we can do as a family. My daughter had to have tutoring in early school years due to most of my time taken up caring for my son who was experiencing delayed development and needed constant watching for his safety. She missed out on a lot of attention as a result.

David has become progressively more and more dependent on support as time progresses rather than becoming more independent with age like a typical kid. These constant caring needs places a massive stress emotional and financially on the whole family unit.

There was no referral or information provided to us about who we could contact for support when David was diagnosed. I luckily found Muscular Dystrophy WA online who then put me in touch with the Save Our Sons Duchenne Foundation. These ladies were amazing once I was connected with them, providing information and about standards of care and lived experiences.

It often feels like we have to fight every step of the way to get David the care he needs, which is exhausting. Here in Western Australia we often find the Neuromuscular clinic is not up to date with current trends and standards of care that are advocated for in other states of Australia and Worldwide.

A cure for Duchenne and Becker Muscular Dystrophy is now a goal within sight, and this is what we should all be aiming for – while it would be great if David could be on a clinical trial, the most important thing is that we get the cure sooner rather than later. Then all kids would benefit.

Australia's cost disadvantage will only negatively influence trial site decision-making if Australia is not seen as offering an advantage in these other areas, underscoring the importance of reform efforts to address identified barriers[30, 33].

Pharmaceutical companies have reported that difficulty meeting patient recruitment targets in Australian clinical trials has a significant impact on future decisions about whether to include Australia as a trial site.

Factors contributing to poor patient recruitment include Australia's small patient pool sizes and inaccurate estimates of potential patient populations; whereas establishment of national patient databases is highlighted as a significant enabling factor for patient trial participation[30, 33].

The establishment of the Australian Duchenne Registry, a collaboration between Save Our Sons Duchenne Foundation, the Office of Population Health and Genomics in Western Australia, and the Murdoch Children's Research Institute in Melbourne, is an important initiative that will facilitate Duchenne patient recruitment and improve the attractiveness of Australia as a location for Duchenne clinical trials.

The Registry will enable accurate estimates of patient pools, facilitate speedy recruitment of Duchenne patients and carriers, and assist pharmaceutical companies with clinical trial planning.

However, delays and variability in timeframes for ethics and research governance approvals may shorten the time available for patient recruitment and are likely to impede the selection of Australia as a site for Duchenne trials. For clinical trials of novel gene therapies for Duchenne and other diseases, time to trial start up is particularly likely to be a barrier due to extremely lengthy timeframes for the issue of genetically modified organism (GMO) licences by the Office of the Gene Technology Regulator.

Concerted action is needed to address these timeframes in order to prevent Australian children with Duchenne from missing out on accessing potentially life-saving new treatments through clinical trials, and to avoid delaying the development of novel gene therapies and other treatments that will save lives.

Current Reform Efforts in Australia

Substantial work is underway to streamline clinical trial processes, with the aim of reducing time to trial start up and ensuring Australia remains a preferred clinical trial destination.

The Australian Government's Encouraging More Clinical Trials in Australia initiative has provided $7 million nationally to support state and territory governments to redesign clinical trial systems in accordance with the revitalised Council of Australian Governments Health Council clinical trials agenda.

Through the Clinical Trials Project Reference Group, Australian jurisdictions have agreed to collaborate on measures to address priority action areas. These include:[32]

- establishing central points of contact in each jurisdiction to coordinate clinical trial management and improve system navigation for trial sponsors and participants;
- developing and capitalising on networks, partnerships and infrastructure to drive coordinated change across the clinical trials sector;
- data collection to inform systems improvement and enhance sector knowledge and performance;
- embedding clinical trials into core hospital governance arrangements; and
- creating a clinical trials governance framework.

Work has commenced across these action areas.

The Framework is due for imminent release and will be piloted in health services in 2020 ahead of full implementation in 2021.[34] This is a first step towards national accreditation of health services undertaking clinical trials, and a nationally consistent approach to clinical trial governance.[35]

While these are important steps forward, issues of fragmentation and inefficiency in Australia's clinical trial processes remain. Urgent work and investment by Australian Governments is needed to ensure Australia is a preferred destination for Duchenne clinical trials, and Australian children have timely and equitable access to Duchenne treatments.

International Comparisons

There is global competition to attract clinical trials given the benefits for healthcare systems and economies. Factors influencing a country's competitiveness as a clinical trial destination include its reputation for quality and reliability of research and data, trial start-up times and regulatory burden, cost, and patient pools and patient recruitment.[30]

We investigate the clinical trial approval processes and timeframes in New Zealand, the United Kingdom (England and Wales), the United States and Canada to enable comparison with Australia.

SUMMARY OF CLINICAL TRIAL APPLICATION AND APPROVAL REQUIREMENTS AND PROCESSES BY COUNTRY

COUNTRY	SINGLE OR MULTIPLE ETHICS APPLICATIONS IN MULTI-SITE TRIALS?	SINGLE OR MULTIPLE RESEARCH GOVERNANCE APPLICATIONS IN MULTI-SITE TRIALS?	SEPARATE OR COMBINED ETHICS AND RESEARCH GOVERNANCE PROCESSES?	CENTRALISED OR DECENTRALISED ETHICS REVIEW?	CENTRALISED OR DECENTRALISED GOVERNANCE REVIEW PROCESS?	SEPARATE GENE THERAPY APPROVAL PROCESS?
AUSTRALIA	There is a single national Human Research Ethics Application Form. A single HREC application may be possible in NMA jurisdictions, but trials with sites in different jurisdictions may require multiple HREC applications.	Multiple. A site-specific authorisation is required for each trial site.	Separate.	Partially centralised. HREC reviews are state-based but a single HREC approval may be accepted across NMA jurisdictions.	Decentralised. Site-specific authorisation is required for each trial site.	Yes. A GMO licence must be issued by the OGTR to use GMOs in clinical trials.
NZ	A single HDEC application covers all trial locations.	Multiple. Each locality requires a separate locality authorisation.	Separate.	Centralised. Only one HDEC review is required nationally.	Decentralised. Each locality is responsible for providing local authorisation.	No. Regulatory applications are reviewed by GTAC as part of the clinical trial regulatory process.
USA	Multiple. Ethics applications to each trial site IRB are required.	Multiple. Research governance is addressed in ethics applications to each trial site IRB.	Combined.	Decentralised. Each site IRB is individually responsible for ethics review.	Decentralised. Each site IRB is individually responsible for research governance review.	No. However, sponsors must submit a more in-depth IND to the FDA (including a Chemistry, Manufacturing and Control component), and Institutional Biosafety Committee approval is required at each trial site.
UK	A single ethics and governance application to the HRA covers all trial sites.	A single ethics and governance application to the HRA covers all trial sites.	Combined.	Centralised ethics reviews are conducted under the HRA.	Centralised research governance reviews are conducted under the HRA.	No. Ethics reviews of trials involving gene therapy are by GTAC as part of the HRA ethics review process.
CANADA	Multiple. Ethics applications to each trial site are required.	Multiple. Research governance is addressed in ethics applications to each trial site.	Combined.	Decentralised. Ethics reviews are conducted at individual trial sites.	Decentralised. Governance reviews are conducted at individual trial sites.	No. Sponsors are required to submit the same CTA with additional content relating to manufacturing and controls. This is reviewed by the Biologics and Genetic Therapies Directorate within Health Canada.

In all the jurisdictions, as in Australia, approval of a clinical trial involves approval by a health products regulatory authority, as well as ethics and research governance approval, and specific approval of the use of GMOs or gene therapies.

New Zealand

REGULATORY APPROVAL (INCLUDING GMO APPROVAL)

To undertake a clinical trial in New Zealand of a new or unregistered medicine or technology, the trial sponsor must submit an application to Medsafe (the New Zealand Medicines and Medical Devices Regulatory Authority) for approval by the Director-General of Health.

Medsafe then forwards the application to the relevant Health Research Council. This is either the Standing Committee on Therapeutic Trials, or if the trial involves a new or genetically modified organism, the Gene Technology Advisory Committee (GTAC). These committees provide recommendations to the Director-General of Health, who approves, provisionally approves or rejects the application based on the proposed trial's compliance with the Good Clinical Practice requirements, as well as scientific validity. The sponsor will receive notification of the outcome within 45 days of confirmation of the application.[36]

ETHICS REVIEW

In addition, all clinical trials require ethics review by a Health and Disability Ethics Committee (HDEC). Higher-risk trials will undergo a full review pathway and be reviewed at an HDEC meeting, while lower risk trials can be reviewed between meetings. Final decisions from the HDEC are received within 35 days for higher-risk trials, and 15 days for lower-risk trials. Only one HDEC review is required for any number of trial sites across New Zealand, and trials can commence as soon as confirmation of approval is received.

RESEARCH GOVERNANCE

Locality authorisation is the New Zealand equivalent of Australia's research governance and site assessment authorisation. Sponsors apply for locality authorisation through the Online Forms website.[37] However, each locality may have different requirements for sponsors.

The Medsafe, HDEC and locality authorisation processes can all occur in parallel. The processes can take as little as 40 days from application to full approval if sponsors are organised with documentation.[38]

United Kingdom (England and Wales)

REGULATORY APPROVAL

In the United Kingdom (England and Wales), clinical trials involving unapproved medical products are required to obtain a Clinical Trial Authorisation from the Medicines and Healthcare Products Regulatory Authority (MHRA). This application is submitted through the Common European Submission Portal, which allows a single application to be within reach of all relevant agencies. In regular cases, the MHRA's assessment is completed within 30 days of the application. However, for lower risk trials, the trial can go ahead 14 days after the MHRA acknowledges receiving the application, providing no objections are raised.[39]

ETHICS AND RESEARCH GOVERNANCE APPROVALS, INCLUDING GENE THERAPY APPROVALS

Since 2016, United Kingdom ethics reviews and local research governance reviews have been combined into one process under the Health Research Authority (HRA).[40] Clinical trial sponsors are required to submit only one application for HRA approval, which involves an assessment of governance and legal compliance, as well as a Research Ethics Committee review.

Review of trials involving gene therapy is part of the HRA approval process. Applications to the HRA are considered by the Gene Therapy Advisory Committee (GTAC), the UK national Research Ethics Committee for gene therapy research.

The benchmark for HRA approval is 60 days.[41] However, according to 2016 data, the mean time for HRA approval was approximately 90 days.[40] For gene therapy trials, the benchmark for GTAC approval is 90 days.[42]

United States

REGULATORY APPROVAL

For a clinical trial to be conducted in the United States, sponsors must submit an Investigational New Drug (IND) Application to the Food and Drug Administration (FDA). A sponsor must then wait 30 calendar days before commencing any trial, during which the FDA assesses the IND to ensure scientific validity and safety, and may discuss objections with the sponsor or issue a clinical hold if the trial poses unreasonable risk.[43] For clinical trials involving gene or cell therapy, the IND must include specific information about manufacturing specifications, testing and collection procedures.[43,44]

ETHICS AND RESEARCH GOVERNANCE APPROVAL

All clinical trials regulated by the FDA require institutional ethics committee approval by an Institutional Review Board (IRB) before a trial may commence. This may occur in parallel with FDA approval. As it is institutionally based, an IRB also assesses site-specific aspects of the trial. It is possible for a trial to be approved by a central IRB, and the judgment accepted by other trial sites. However, individual ethics applications may still need to be submitted to each trial site.[45]

Different IRBs also have different average timeframes for reviews, which depend on whether the trial requires a full board review. However, there is a 30-day national benchmark for processing of IRB applications.[45].

If the FDA has no objections to the IND within 30 days, and IRB approval is obtained, the trial may commence. This means that clinical trials in the United States may be approved in only 30 calendar days if the 30-day benchmark for IRB review is met.

GENE THERAPY/GMO APPROVAL

In addition to review by the FDA and IRB, clinical trials involving gene therapy or GMOs must be reviewed by an Institutional Biosafety Committees (IBC) at each trial site, as well as comply with FDA regulations applying to gene therapy in clinical trials. Each institution must establish an IBC to review proposed clinical trials and sponsors must apply directly to the IBC for each trial site.[46] In the IND submitted to the FDA, there is an additional Chemistry, Manufacturing, and Control (CMC) section of the application for clinical trials involving gene or cell therapy.[44]

Canada

REGULATORY APPROVAL (INCLUDING GENE THERAPY APPROVAL)

To undertake a clinical trial involving an unapproved medicine or pharmaceutical in Canada, a Clinical Trial Application (CTA) must be submitted to Health Canada.[39] CTAs involving un-marketed pharmaceuticals must only include summarised information about the drug and are sent to the Therapeutic Products Directorate, while CTAs involving biologicals, radiopharmaceuticals or gene therapy must include additional information with respect to manufacturing and release controls and are sent to the Biologics and Genetic Therapies Directorate.[47,48] All CTAs are subject to a 30-day default review period following receipt of the application by Health Canada.[48,49]

ETHICS AND RESEARCH GOVERNANCE APPROVAL

Canada has a decentralised process for ethics review of clinical trials. Trial sponsors are required to apply to the Institutional Ethics Committee of each participating clinical trial site for ethics and research governance approval, and this may occur in parallel with the CTA submission. Requirements may differ across provinces, so different sites may have different application processes and components.[45] Each institution individually reviews legal and contract issues and other research governance matters, including insurance and indemnity arrangements. The time taken for each Institutional Ethics Committee to reach its conclusion varies according to the institution and the frequency of meetings. However, Canada has a 30-day benchmark for processing of ethics review applications.[50]

If a CTA and ethics review application are submitted in parallel and the ethics review benchmark of 30 days is met, clinical trials in Canada may be approved in as little as 30 days.

How Does Australia Compare with Other Jurisdictions?

Australia's CTN Scheme is the most efficient regulatory process of the jurisdictions studied and a key strength of Australia's clinical trial system. The CTN Scheme is unique among the jurisdictions in requiring regulatory notification rather than approval and allowing trials to commence as soon as the CTN is submitted, provided ethics and site authorisations have been obtained. This means that the CTN process need not add any time to the total clinical trial approval timeframe.

TABLE 2 SUMMARY OF CLINICAL TRIAL APPROVAL TIMEFRAMES BY COUNTRY

COUNTRY	REGULATORY APPROVAL TIMEFRAMES	GMO/GENE THERAPY APPROVAL TIMEFRAMES	ETHICS AND RESEARCH GOVERNANCE APPROVAL AVERAGE TIMEFRAMES AND BENCHMARKS
AUSTRALIA	> Trial can commence immediately upon CTN notification. > 30-50 working days for CTX approvals.	> 90 working days for DNIR licence. > 150 working days for DIR licence.	> 60-day benchmark for HREC review. > No benchmark for SSA timeframe. Timeframes vary between trial sites. > Average total timeframe for HREC review and SSA of 150 to 160 days (2014-17 data). > Average timeframe for HREC review of 25-26 days when time spent waiting for information from applicants is discounted (2014-17 data). > Average timeframe for HREC review of 78-87 days when time spent waiting for information from applicants is included (2014-2017 data). > Average timeframe for SSA authorisation following HREC approval of 147 days (2016-17 data).
NZ	> 45 days.	> 45 days (as part of regulatory approval timeframe).	> An HDEC decision must be made within 35 days for high-risk trials and 15 days for low-risk trials. > No benchmark for locality authorisation. Timeframes vary between trial sites. > Average timeframes not available.
USA	> 30 days.	> Decentralised institutional approvals differ in timeframe.	> 30-day national benchmark. > Average timeframe not available.
UK (ENGLAND AND WALES)	> 30 days for regular trials. > 14 days for lower risk trials.	> 90-day national benchmark (as part of ethics approval).	> 60-day national benchmark. > Average timeframe of approximately 90 days (2016 data).
CANADA	> 30 days.	> 30 days (as part of CTA review).	> 30-day national benchmark. > Average timeframe not available.

Any time gain from the CTN Scheme in Australia may be eroded, however, by variable and lengthy HREC review and SSA timeframes, and by extended GMO licence approval timeframes for clinical trials involving gene therapies.

Australia's ethics approval timeframes are faster than international benchmarks (30 days in Canada and the United States, 15-35 days in New Zealand and 60 days in the United Kingdom) when time spent waiting for information from applicants is discounted. From 2014 to 2017, the mean timeframe for ethics review ranged from approximately 25 to 26 days. However, when this waiting time is included, the mean timeframe increases to between approximately 78 and 87 days and falls behind international benchmarks.

This suggests a need for improvement in the planning and performance of trial applicants, and for provision of more comprehensive information at the time of the initial application.

Australia's mean ethics review timeframes are substantially slower than the required timeframes for ethics review in New Zealand, the only other jurisdiction studied with a separate ethics review process.

In New Zealand, a single ethics review is accepted for any number of sites across the country, and reviews occur within only 35 days for high-risk trials and 15 days for lower-risk trials. The speed of ethics reviews in New Zealand suggests that further efficiencies could be gained in Australia if a truly national system for ethics review were introduced. As discussed, for multi-site trials across more than one jurisdiction in Australia, multiple applications are likely to be required which may have different information requirements for different trial sites, leading to duplication and inefficiency.

Australia's research governance processes contribute significantly to clinical trial approval timeframes. Australia is not unique in having decentralised and fragmented research governance processes.

Of the jurisdictions studied, only the United Kingdom has a centralised national process of research governance review. However, a key difference between Australia and Canada, the United States and the United Kingdom is that ethics review and research governance/SSA are separate processes in Australia, whereas in the other jurisdictions ethics and governance review functions are combined.

This appears to at least partly explain slower timeframes in Australia, where the average time for completion of the two processes ranges from 150 to 160 days (2014-17).

Only a very small proportion of trials (8-17 per cent in 2014-17) complete ethics and SSA processes within 60 days, most take 60-120 days (approximately 30-50 per cent in 2014-17), and the remainder take 120-180 days or longer.[50]

In comparison, the mean time for ethics and research governance approval in the United Kingdom is approximately 90 days according to 2016 data. Comparative data from United States and Canada is not available, but the 30-day benchmarks in these jurisdictions for ethics and research governance indicates that Australia is well behind.

Although separation of the processes in Australia has enabled streamlining of ethics reviews under the NMA, an unintended consequence has been that HREC review and SSA most often happen in sequence rather than in parallel. This appears to be one of the main contributors to delays in clinical trial approval timeframes in Australia.

In contrast, in the United Kingdom, the introduction of a single application and centralised process for ethics and governance review under the HRA has significantly reduced approval timeframes. Previously, there was a dual-application system and review of legal compliance was undertaken locally at each NHS organisation.

According to 2016 data, the mean time from HRA submission until HRA approval was approximately 90 days, with 53 days between HRA approval and recruiting the first patient. Within the HRA assessment, there was a mean of only 20 days between the ethics approval and the HRA approval. In the previous system which relied on sequential ethics then research governance/site specific approvals, there was a mean of 176 days between ethics approval and the first patient being recruited.[40]

For clinical trials of gene therapy for Duchenne, Australia's GMO licence approval process is far lengthier than any of the jurisdictions studied and is likely to be a major impediment to selection of Australia as a site for gene therapy trials. Australia is the only jurisdiction that requires licensing of GMOs for use in clinical trials or approval by a separate gene technology regulator.

In Canada and New Zealand, use of gene therapies in clinical trials is reviewed by a directorate or committee within the relevant regulatory authority as part of the central approval process for clinical trials. This means that gene therapy clinical trials are approved within the same regulatory approval timeframes of 30 days in Canada and 45 days in New Zealand.

KEY ISSUES & RECOMMENDATIONS

Despite significant efforts to improve the clinical trials environment in Australia, the selection of Australia as a site for Duchenne trials is likely to be impeded by lengthy and variable ethics and research governance timeframes; the lack of a truly national and harmonised ethics review system; and extended timeframes for licensing of the use of GMOs in clinical trials of gene therapies.

Implementation of the new National Research Governance Framework and national clinical trial accreditation of health services will go some way to improving research governance processes and reducing timeframes. It is hoped that this will lead to greater clarity and understanding of relevant roles and functions, adoption of a single national SSA form and increased use of standard contracts, as well as improving institutions' strategic planning and increasing their focus on meeting national approval timeframe benchmarks.

However, further action is needed to streamline and harmonise ethics and research governance approval processes and requirements, and to reform the process for approving the use of GMOs in clinical trials.

The following recommendations are made to ensure that Australians are able to access clinical trials:

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

Australian governments should collaborate to develop a national 'one-stop' clinical trials portal similar to the Common European Submission Portal. This should be supported by a single IT platform, provide a central gateway for submission of all clinical trial application documents, and allow a single application to be within reach of all relevant agencies. This would eliminate the need for multiple applications, help to promote transparency and increase inter-institutional trust and acceptance of HREC reviews, and promote standardisation of requirements.

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

A single national online application form for ethics and research governance/SSA should also be developed. The form should consolidate information requirements for HREC review and SSAs and should be divided into modules for different areas. A single national form would ensure parallel approval processes, encourage pre-submission planning, and drive applicants to provide comprehensive information and documentation at the application stage. It would also reduce duplication in information requirements and eliminate the need for multiple different applications.

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

The Australian government should establish a national clinical trial coordinating agency to support a centralised and nationally consistent approach. The agency would be responsible for upfront assessment and triaging of applications to relevant bodies, and act as a central point of contact for trial sponsors and applicants. This would help applicants navigate approval processes and reduce inefficiencies such as delays in providing requisite information.

RECOMMENDATION 11
Introduction of national legislation to harmonise regulatory requirements.

Australian governments should collaborate to introduce uniform legislation setting consistent national requirements for clinical trials, including in relation to privacy of personal and health information, data protection, and capacity to consent to trial participation, as well as a uniform national policy framework. The uniform legislation would supersede state/territory legislation to the extent that it applies to clinical trials. This would support centralisation and streamlining of ethics review processes by encouraging mutual recognition of HREC reviews due to standardisation of requirements, and removing the need for multiple applications with different information requirements in different states and territories. It would also help to improve clarity and understanding of regulatory obligations and compliance.

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

The Australian Government should undertake an urgent review of the process for approving the use of GMOs in clinical trials. The review should consider options for introducing a specific clinical trial approval process in recognition of the need for timely approvals and that use of GMOs in clinical trials is likely to be more contained and lower risk than more widespread use of GMOs. Options should include:

- review by a gene therapy directorate or committee within the TGA as part of the central regulatory approval process for clinical trials, following the approach in Canada and New Zealand;
- review by a specific clinical trials division of the OGTR; or
- review by a separate, specially constituted agency or committee for approving the use of GMOs in clinical trials.

The review should also consider the use of risk assessment to fast-track approvals of lower risk use of GMOs or previously approved use of GMOS in clinical trials. Additionally, it should set benchmarks for approval timeframes that are competitive with international timeframes.

THE McKell Institute

CONCLUSION

There is hope. Before long there will treatments that effectively cure Duchenne, but as families wait there is work to be done to ensure they receive the care and support they need and provide the earliest possible access to new treatments.

Feedback from families highlights the importance of early diagnosis with twenty per cent of families waiting over three years. Delays impact long term outcomes and lead to families making reproductive choices without full information. A pilot study on the use of newborn screening will provide evidence of its efficacy and in the future ensure any genetic treatments can be delivered before long-term muscle damage occurs.

The NDIS continues to not provide adequate flexibility for participants with changing needs, leading to delays in equipment and supports. For children with Duchenne months do matter, and the system needs to be reformed to ensure every Australian with a disability benefits from the scheme.

As the prospect of gene therapy gets closer, Australian children risk missing out on pivotal clinical trials due to ongoing perceptions of a cumbersome regulatory system. Reforms to clinical trial approval processes should be expedited and expanded to include the approval of genetically modified organisms.

As part of a broader gene therapy strategy, Australia needs to prepare for the tsunami of new therapies that will test our health systems capacity. This will ensure that the hope which is filling families and children with Duchenne and Becker turns into reality. A future where Duchenne or Becker no longer means a shortened life.

APPENDIX A

COST OF DISEASE ESTIMATES

Health Care Costs

Health care costs associated with Duchenne are well established. A 2016 Study by Teoh et al outline the health and social care costs by age of a child with Duchenne.

We include the health care costs from this study, updating the figures to account for health inflation.

As Duchenne progresses the need for medical intervention grows, and the costs increase. In the later stages of the disease medical costs tend to fall.

AGE	AVERAGE HEALTH CARE COSTS (2014 AUSTRALIAN DOLLARS)
0-4 years	$5,672
5-14 years	$7,587
15-24 years	$15,808
25-34 years	$3,861

Total direct health care costs over the lifetime of an individual with Duchenne are estimated at between $0.3 to $0.6 million.

Social Care Costs

As Duchenne progresses the need for social care increases substantially, due to the loss of physical function. While families often provide much of this support through informal care, formal care supports are heavily relied upon alongside aids and equipment.

From the Save Our Sons Duchenne survey we know that the majority of children with Duchenne rely on supports from the NDIS, and that these costs increase as the children age.

Unlike the direct health care costs, these were found to be highest amongst the young adults aged 25-34, and indicate that this stage of the disease the costs become more care rather than medical related.

AGE	AVERAGE HEALTH CARE COSTS (2014 AUSTRALIAN DOLLARS)
0-4 years	$16,703
5-14 years	$20,812
15-24 years	$68,888
25-34 years	$72,290

Informal Caring Costs

A number of studies have highlighted the impact of having a child with a disability on maternal labour supply.[51,52,53,54,55] In the case of Duchenne, because the disability is progressive the impacts increase with age especially when compared to mothers of children without a disability.

We asked survey respondents about their hours worked, both before and after having children and found that mothers in the survey worked 10 hours less per week than similar mothers in the 2016 census.

The loss of productivity was then calculated using average hourly female wages in current dollars of $36.80 per hour from 6302.0 Average Weekly Earnings Australian, May 2019.

Expected Costs

In order to calculate expected costs we used estimates of the life expectancy of children born with Duchenne. In the absence of a long-term registry in Australia, these have been taken from recent international studies that are likely to underestimate current life expectancy.[6,8]

PROBABILITY OF SURVIVAL AT DIFFERENT AGES

AGE	PROBABILITY OF SURVIVAL
0-4 years	1.0
5-14 years	0.9
15-24 years	0.5
25-34 years	0.25

APPENDIX B

CLINICAL TRIAL APPROVAL IN AUSTRALIA

Clinical Trial Approval Processes in Australia

Approval of a clinical trial in Australia involves the following processes:

- Notification to or approval by the Therapeutic Goods Administration (TGA) under the Clinical Trial Notification (CTN) or Clinical Trial Exemption (CTX) Scheme if the trial uses an unapproved therapeutic good.
- Ethics and scientific review by a Human Research Ethics Committee (HREC) to ensure the trial is in accordance with the National Statement on Ethical Conduct in Human Research (2007) (the National Statement).[56]
- Research governance review by each institution at which a trial will take place, including a site-specific assessment (SSA) of the institution's capacity to undertake the trial and ensuring necessary contractual and insurance arrangements are in place.

In addition, clinical trials involving a genetically modified organism must generally obtain a licence from the Office of the Gene Technology Regulator (OGTR). This is a requirement for clinical trials of gene therapies for Duchenne.

TGA Notification or Approval under the CTN or CTX Scheme

Australian clinical trials involving unapproved therapeutic goods must either be notified to the TGA under the CTN Scheme or approved by the TGA under the CTX Scheme[iii].

The CTN Scheme is generally used for later phase (III and IV) and bioavailability/ bioequivalence trials of medicines but may also be used for earlier phase (I and II) trials if there is adequate preclinical trial information available, especially regarding safety.[57] Most clinical trials in Australia are notified to the TGA under the CTN Scheme.

Under the CTN Scheme, a clinical trial applicant is only required to notify the TGA of the trial and the TGA does not review or evaluate data. The target timeframe for processing of online CTNs is 5–7 working days. However, as soon as the CTN has been submitted, the TGA is deemed to have been notified and the clinical trial may commence, so long as necessary ethics approvals and site authorisations have been provided. The CTN Scheme is recognised as a major enabler of clinical trials in Australia and one of the most efficient regulatory processes for clinical trials internationally.[30]

The CTX scheme is generally used for high-risk or novel treatments where there is no or limited knowledge about safety.[33] The CTX Scheme involves evaluation by the TGA of information about the clinical trial, including scientific data.

There is a 30-50 working day period for the evaluation of a CTX application, meaning that the wait for approval for a clinical trial under the CTX Scheme can be up to three months.[iv] CTX review can occur in parallel with HREC approval and site authorisation, but a trial may only proceed once these approvals are obtained. At the time of writing, the CTX Scheme is under review and may be subject to change.

Ethics and Scientific Review by a Human Research Ethics Committee

All clinical trials in Australia must be reviewed by an HREC according to the National Statement.[v] [56,58] The HREC reviews the scientific validity, ethical acceptability, and the risk versus potential harm of the trial proposal.[58]

Each Australian State and Territory has separate ethics and scientific review requirements and processes. To help streamline these processes, the Australian Capital Territory, New South Wales, Queensland, South Australia, Victoria and Western Australia have agreed to National Mutual Acceptance (NMA), under which each jurisdiction mutually accepts single scientific and ethical reviews of multi-site clinical trials across jurisdictions. This may avoid the need for the trial sponsor to apply to multiple HRECs and allow a project with ethical approval obtained in any of the NMA jurisdictions to be expanded to sites in other jurisdictions.[59]

This has made considerable progress towards streamlining HREC reviews. However, a single national ethics approval process has not yet been established and there continues to be fragmentation between states and territories. Northern Territory and Tasmania have not yet signed up to the NMA, there are some exceptions to the NMA in participating jurisdictions,[vi] and the NMA applies to public health organisations only and not private organisations.

There is also variability in application requirements across the NMA jurisdictions. Although a national Human Research Ethics Application form has been developed for NMA jurisdictions, there are four different IT platforms and application portals across the jurisdictions, requiring applications to be submitted differently depending upon the jurisdiction in which they are lodged.[vii] In addition, trials involving one or more sites in Victoria and Western Australia require a specific module or forms with additional information requirements due to differing legislative requirements in relation to matters such as privacy of personal and health information, data protection and capacity to consent.[59,60]

This means that multi-site trials, or recruitment of participants from multiple jurisdictions, may still require multiple HREC applications and reviews. This leads to duplications and inefficiencies, which may impact approval timeframes and costs.

Despite this, the NMA scheme has contributed to shorter ethics approval timelines, which have been noted by pharmaceutical companies as one of Australia's competitive advantages.[30,33]

From 2014-2017, when days spent waiting for applicants' responses to requests for further information were discounted, between 89 and 94 per cent of HREC approvals met a benchmark timeline of 60 days, with the mean timeframe ranging from approximately 25 to 26 days. This is highly competitive with international benchmarks for time to process ethics applications, which include 145 days in China, 60 days in England and Wales, 35 days in New Zealand and 30 days in the United States and Canada. However, when total time was measured and wait times were not discounted, only 45 to 49 per cent of HREC approvals from 2014-2017 met the 60-day benchmark, and the mean time for approval increased to between approximately 78 and 87 days.[50]

This indicates delays may largely result from deficiencies in initial information provided by applicants, as well as inefficiencies in communications between applicants and ethics committee investigators.[50] It has been reported anecdotally that in most cases approval is not granted on first review, indicating that improving the quality of information the applicant provides to the HREC in the first instance may help to further reduce timeframes.[61]

iii CTN notification or CTX exemption is required for any product not on the Australian Register of Therapeutic Goods (including any new formulation or route of administration of a product) and use of a product beyond the conditions of its marketing approval. Clinical trials of products on the Australian Register of Therapeutic Goods and used within the conditions of their marketing approval are not subject to CTN or CTX requirements but must still be approved by a HREC.

iv The applicant may be the trial sponsor, lead investigator, trial coordinator or a Contract Research Organisation engaged by the trial sponsor.

v Therapeutic Goods Regulations 1990, r 12AD.

vi Phase 0 (first time in human or patient) and Phase I trials are excluded from single ethics review under NMA in South Australia and the Australian Capital Territory.

vii Applications in New South Wales and the Australian Capital Territory use the REGIS website (https://regis.health.nsw.gov.au/), applications in South Australia use the online forms website (https://au.ethicsform.org/SignIn.aspx), applications in Queensland and Victoria use the ERM website (https://au.forms.ethicalreviewmanager.com/Account/Login), while in Western Australia, applications need to be submitted either to the Research Governance Service for Western Australian Health HRECs, or via Online Forms (https://au.ethicsform.org/SignIn.aspx) for non-WA Health HRECs participating in the NMA.

Research Governance Approval, including Site-specific Assessment

Research governance refers to the processes by which each institution undertaking a clinical trial ensures it is accountable for the research. Research governance addresses the safety and quality of research, as well as financial management, risk management, and legal and regulatory compliance.

As part of research governance, institutions undertaking a clinical trial complete a site-specific assessment (SSA) to assess the suitability of the trial for the site. This involves considering trial budgets, physical resources, staff, insurance and indemnity requirements, and contractual arrangements. Both ethics approval and SSA are required before a trial can commence at a site.[62]

It has been reported that the SSA process can be lengthy and may vary widely from site to site and study to study.[30] Standard contract templates for clinical trial agreements and indemnities have been developed. However, it has been suggested that some sites nevertheless apply inconsistent requirements due to inadequate understanding of essential and non-essential steps, leading to lengthy contract discussions.[63]

The major cause of delays and variability in ethics and SSA approval times, however, appears to be that the two processes are often conducted sequentially. Most jurisdictions have a policy of encouraging submission of site assessment documents before or at the same time as submission of ethics applications so that the SSA and HREC approval processes can run in parallel. This supports speedy approvals in some cases, with examples of site authorisation granted in less than two weeks after ethics approval,[33] and even in as little as two days.[61] However, it has been reported that parallel submission rarely happens in practice, contributing to delays in SSAs of 3-6 months after ethics approval.[33,50] From 2014-2017, only around 30 per cent of SSAs occurred within 60 days of HREC approval. In 2016-17, the average time for SSA following ethics approval was 147 days.[50]

When considering the entire timeframe for the ethics and SSA processes, only between 8 and 17 per cent of trials from 2014-2017 completed the processes within 60 days. The proportion of trials completing the processes within 120 days ranged from 31.5 per cent (2014-2015) to 42 per cent (2015-2016) and 51 per cent (2016-2017), with the remainder of trials taking between 120 and 180 days to receive necessary approvals. The average time for completion of the two processes ranged from 150 to 160 days.[50]

Reasons suggested for the delay in submission of SSA/site assessment applications until after ethics approval include:

- a lack of understanding by research governance officers of the roles of SSA and ethics reviews, leading to the view that they ask for similar things and reluctance to devote resources to SSAs until ethics approval is finalised;[33]
- industry delays in providing key documents;
- protracted negotiations on trial budget; and
- clinical loads, lack of resources and/or lack of funding leading to delays in submitting applications.[50]

GMO licence approval

For clinical trials of gene therapies for Duchenne, a licence to use GMOs must also be obtained from the Office of the Gene Technology Regulator (OGTR) before the trial can proceed. If the GMO is expected to be shed or excreted from trial participants and consequently released into the environment, a Dealing involving Intentional Release (DIR) licence is required. If the GMO is expected to be contained in the bodies of trial participants and not shed or excreted, a Dealing Not involving Intentional Release (DNIR) licence is required.

To apply for a GMO licence, an institution must be accredited under the *Gene Technology Act 2000 (Cth)*. GMO licence applications must be endorsed by an institution's Institutional Biosafety Committee before being submitted to the OGTR.[64]

A GMO licence application can be submitted concurrently with HREC and TGA applications. However, the licence approval timelines are extremely lengthy. The OGTR has 90 working days (about 4.5 months) to decide DNIR licence applications and generally 150 working days (about 8 months) to decide DIR licence applications, though if the OGTR seeks information from the applicant, any days it waits for the information do not count towards these timeframes. A longer timeframe may apply for DIR licence applications if the OGTR finds that the GMO may pose a significant risk to people or the environment or the applicant has not proposed appropriate limits and controls.[64]

FOOTNOTES

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THE McKell Institute

SAVE OUR SONS

DUCHENNE FOUNDATION

THE McKell Institute

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Form Submitted 21 Jun 2021, 10:19am AEST

Eligibility

*** indicates a required field**

Before getting started

Before completing this application form, you should have read the **2021 QMHW Community Grant Program Guidelines**.

You will need:

- Your ABN or ACNC number
    - If you do not have an ABN number you will need to complete and submit a Statement by Supplier form - ato.gov.au/forms/statement-by-a-supplier-not-quoting-an-abn/
- A digital copy of your organisations or auspicing organisations Public Liability Certificate of Currency showing the insured coverage amount as $20 million or more to submit to in the application
- Clear details of your event, estimated number of participants, and target audience.
- A budget that relates to how you will spend the grant amount you are seeking.

Only one application per event will be accepted. Whilst we greatly encourage partnerships, two or more organisations **cannot** apply for the same event or activity.

If you are planning to host multiple events or activities, you will need to complete an application for each event you are seeking funding to support. If successful, each event will require a separate acquittal.

Incomplete applications and/or applications received after the closing date will not be considered.

Applications will close at 5pm on Wednesday 30 June 2021.

Incomplete applications and/or applications received after the closing date will not be considered.

If you have any questions please email info@qldmentalhealthweek.org.au or call 07 3105 8308.

Confirmation of eligibility

This section of the application form is designed to help you, and us, understand if you are eligible for this grant. It's crucial that you complete this section before any others to ensure you do not waste your time applying for an unsuitable grant.

I confirm that the applicant ...

- has read and understands the program guidelines
- is located in (and/or supplies services to) Queensland
- is a not-for-profit organisation, company, local council, or school (state and private schools may apply via their P&C)
- is incorporated, or is auspiced by an incorporated organisation for the purposes of this application
- has the appropriate type and level of insurance for the activities that are the subject of this grant
- *is not* any of the following: an individual; political party; religious organisation (excluding schools and organisations that deliver community services); State, Territory or Australian Government agency; organisation who receives any form of funding from tobacco or alcohol companies or foundations; or an organisation seeking capital funding

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Please select below: *
◉ Yes ○ No
You must confirm that all statements above are true and correct.

Contact details

*** indicates a required field**

Privacy notice

We pledge to respect and uphold your rights to privacy protection under the Australian Privacy Principles (APPs) as established under the *Privacy Act 1988* and amended by the *Privacy Amendment (Enhancing Privacy Protection) Act 2012.*

CheckUP collects personal information to enable CheckUP to contact an organisation, and to assess the merits of an application.

CheckUP will collect and store information you provide to enable the implementation of this grants program. Any information you provide will be stored and accessed only by authorised personnel and is subject to the requirements of the Privacy Act 1988.

Applicants must ensure that people whose personal details are supplied with applications are aware of how this information will be used.

By disclosing information about your organisation and/or your sponsoring organisation, you give permission for your contact details to be:

- disclosed to the Queensland Government, including the Queensland Mental Health Commission and Queensland Members of Parliament.
- used by CheckUP for promotion to the general public to access public events. This may include your email address and phone number provided by you at the time of completing an online event registration.
- added to the Queensland Mental Health Week eNews mailing list.
- contacted by CheckUP for future promotions.

CheckUP does not sell or offer your personal details to third party sources other than the above mentioned.

Your organisation

Organisation name *
Save our Sons Inc
Please use your organisation's full name. Check your spelling and make sure you provide the same name that is listed in official documentation such as with the ABR, ACNC or ATO.

What type of organisation are you? *
○ Educational institution (includes pre-schools, schools, universities & higher education providers)
○ Mental health organisation
◉ Peak body
○ Professional association
○ Community group
○ Research body
○ General not-for-profit (i.e. none of the sub-types listed above)
○ For-profit company

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○ Local Government
○ Other:
Please choose the option that best applies to your organisation.

Primary address
3/570 New Canterbury Road
HURLSTONE PARK NSW 2193 Australia

Postal address
3/570 New Canterbury Road
HURLSTONE PARK NSW 2193 Australia

Website address
http://saveoursons.org.au
Must be a URL

Primary contact person *
Mr Lance Dale
This is the person we will correspond with about this grant

Position held in organisation *
Advocacy Officer
e.g. Manager, Board Member, Event Coordinator

Primary phone number *
[redacted]
Must be an Australian phone number.

Primary contact person's email address *
[redacted]
This is the address we will use to correspond with you about this grant.

Secondary contact person
Save Our Sons Duchenne Foundation
If two colleagues or different organisations are organising the event together, this is where you can list the other person/organisation.

rson's email address
[redacted]

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Organisation details

*** indicates a required field**

ABN

Applicant ABN

Information from the Australian Business Register	
ABN	
Entity name	
ABN status	
Entity type	
Goods & Services Tax (GST)	
DGR Endorsed	
ATO Charity Type	
ACNC Registration	No
Tax Concessions	
Main business location	

Must be an ABN.

If you do not have an ABN, please submit a completed ATO Statement by a Supplier Form with your application, otherwise 48.5% of any approved grant may be withheld. Download the form from the ATO.
No files have been uploaded

Public Liability Insurance

Insurance value *
$20,000,000.00
Must be a dollar amount.
Must be at least $20,000,000.

Attach your Public Liability Certificate *

Filename: GIO Not for Profit Protect Policy Schedule GPM004376511.pdf
File size: 89.0 kB
Please upload your or the auspicing organisations public liability insurance certificate

Auspice information

*** indicates a required field**

Applications via auspice

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If your organisation or group is not incorporated or covered by sufficient insurance, you could approach a sponsoring organisation known as auspicing. This may include your local council, or another organisation.

All auspicing organisations must be incorporated or limited by guarantee and hold a minimum of $20 million in Public Liability Insurance.

More information is included in the 2021 QMHW Community Events Grant Guide.

Is your organisation auspiced by another organisation for the purposes of this grant? *

○ Yes ◉ No

Event details

*** indicates a required field**

Event title *

Duchenne Connects

Your event name should be short but descriptive.

Anticipated start date

10/10/2021

If unknown, provide your best guess or leave blank. Remember the event must occur during QMHW (9-17 October).

Anticipated end date

10/10/2021

If unknown, provide your best guess or leave blank

Location of event/initiative *

Brisbane

E.g. Town, city, regional area, or online

Postcode of event/initiative

4000

Must be a postcode

Please provide a short summary of the event/activity *

The event aims to bring together isolated parents/family members from Brisbane and regional Queensland who are caring for children, boys and young men suffering from Duchenne and Becker muscular dystrophy. A dinner/social event in a good quality and accessible venue will be planned and a guest speaker from one of the key mental health services in Brisbane will be present to speak about self -care and well-being strategies. The speaker will also advise on particular mental health and well-being services and resources which are available in the Brisbane and regional areas.

We are confident that parents/carers will connect with each other post event and organise their own future events/activities-which are designed to help with mental health and well-being and provide important support and social opportunities. As so much time is typically

expended in their carer role there is very little opportunity for parents and carers to engage in these opportunities and to participate in self care activities. This dinner event will aim to break that mould and open up new insights, knowledge and challenges.

Save Our Sons Duchenne Foundation (SOSDF) is a national peak body for Duchenne and Becker muscular dystrophy in Australia. Over the years we have observed and experienced the considerable mental health impacts/anguish of caring for children, young boys and young men with Duchenne and Becker muscular dystrophy. It is an "all consuming 24/7 occupation". We are also aware that these parents/carers have too little time available due to balancing work, carer and other responsibilities to expend on their own self care and mental health.

This proposal will bring families together to talk openly about mental health issues and actively discuss and promote a range of self care and well-being strategies. SOSDF has the capacity to reach many families in Brisbane and regional Queensland and would be actively promoting the importance of this event to them.

Currently there are no organisations working to bring the Duchenne and Becker community together in a social setting/event which has an important (and embedded) information provision dimension. Isolation is subsequently a huge factor impacting these families who often feel very alone while coping with this most complex, debilitating and progressive of rare diseases.

SOSDF staff will attend the event to help facilitate the proceedings and ensure that further opportunities for social connection/interaction and self care are fostered amongst the Duchenne and Becker community as a consequence of this "seeding" event.

Must be no more than 400 words.
Be descriptive, but succinct. Here are a few prompts: Why do you want to host this event? Who is your target audience? What activities will occur? Where is your event going to take place?

How will your event help support the aims of QMHW? Detail how the event or activity will maximise community awareness of, and engagement in mental health and wellbeing; promote education and understanding of mental illness; and foster inclusion of those living with a mental illness, their families, carers and support people. *

The event will be held on World Mental Health Day 2021 during Queensland mental health week- a perfect and poignant time for such an overdue activity.

The dinner event will openly discuss mental health, well-being and self care strategies and will raise awareness amongst the Duchenne and Becker community who are present of mental health and well-being issues-consistent with the overall objectives of QMHW. Importantly, the guest speaker will be openly canvassing the importance of self care and facilitating discussion on self care strategies. Resources and information kits will be provided to all attendees.

As highlighted in the above, mental health and well-being are too often sacrificed and compromised by parents/carers who are struggling with the complexities of the Duchenne and Becker disease. As a consequence, many are suffering poor mental health, anxiety, depression and grief and are failing to take good enough care of themselves. This event will be highlighting these issues as well as affirming the critical and important role played by these parents and carers in the lives of their boys.

Aside from the formal component of the evening, the event will be designed to build connection amongst families and reduce the isolation experienced by many Duchenne and Becker parents/carers. It will be the catalyst for further social gatherings and events and will provide SOSDF with a pilot activity which can be implemented in other states of Australia.

Stigma and discrimination leading to exclusion is something encountered by our families

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throughout their journey with Duchenne and Becker muscular dystrophy. This event will openly discuss these issues and promote self care and resilience strategies to help families work through and address these issues. As a collective coming together, families will also be able to share common experiences and concerns and strategies for dealing with these issues.

SOSDF has prioritised the mental health needs of our community and will be working to pull together resources and contacts for not only mental health services but also, social and recreational opportunities for families living with Duchenne and Becker. These resources will help facilitate self care strategies and will draw on the outcomes of this event.
Must be no more than 400 words.

Target audience

Who are the primary beneficiaries of this project/program? *
Family and relationships > Caregivers > Carers of children with additional needs
No more than 5 choices may be selected.
Please choose the group/s that are at the very heart of this event/initiative. You can start typing to use the search functionality.

Approximately how many people will this event involve? *
30
Must be a number.
I.e. Estimated attendees/participants.

Is your event open to the public? *
○ Yes
◉ No

Community support

Evidence of community support is generally highly regarded as events with internal/external buy-in tend to be more successful, so please utilise the below comment box to add any details to support your application that you have not mentioned in previous answers.

Examples of support may include:

- Partnerships with other organisations to work together on the event
- A commitment from a committee to help organise the event
- Volunteers who have put up their hands to help already
- High participation numbers or positive feedback from QMHW events held in previous years
- Interest from vendors or stall holders to participate
- If you are a P&C, this could simply be that you know school staff are supportive of the event and have committed to organising it

What support do you have for your event/initiative?
SOSDF will be working with key family contacts in the Brisbane and wider area to help pull the event together. We will also be utilising our relations with other agencies and contacts (eg, Muscular Dystrophy Australia), to help develop and promote the event.

SOSDF will be working with families to determine a suitable venue and content for the event. These families will also play a critical role in helping SOSDF to promote the event/ invite attendees.

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We will also be actively consulting with mental health agencies in the Queensland area to determine suitable speaker and content for the evening.

SOSDF staff time will be allocated in the organisation of the event and it will be built into relevant staff workplan/s. SOSDF will utilise our own resources on promoting the event and following up on the event outcomes -including feedback, future leads and ideas for subsequent events and opportunities etc.

SOSDF regularly consults our community and we believe there is a strong interest in such an event. We believe our community is seeking more opportunities for coming together and connection. These opportunities provide an invaluable chance for community members to share stories, insights and to self organise. They can play a key role in improving mental health outcomes.
Must be no more than 400 words.

Funding

*** indicates a required field**

Grant amounts

There are three different amount of funding available. These are:

- $500
- $1000
- $3000.

All amounts exclude GST.

Please note that half the funding has been preliminary earmarked for the provision of $500 grants.

Grant amount requested *

$3,000.00
Must be a dollar amount.
Which of the three grant amounts are you requesting in this application?

If you are applying for a higher grant amount ($1000 or $3000), are you open to receiving a smaller grant if you are unsuccessful in obtaining the larger figure.

◉ Yes
○ No

Total event cost

You may want to consider when determining your event plan and budget how you can have more than one funding source. I.e. Can your organisation commit to matching the grant funding dollar for dollar to increase the impact of your event? Can you request sponsorship of goods or come to an in-kind agreement with another organisation? Instead of putting some of the grant towards catering, can you hold a fundraising BBQ?

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Total event/initiative cost
$4,000.00
What is the approximate total budgeted cost (dollars) of your event? This figure may be higher than the grant amount if your organisation is contributing funds or you are looking at multiple funding sources.

Add any additional information you think we should be aware of in terms of your funding sources and budget
SOSDF will be providing in kind assistance by way of staff time in helping to organise and set up the event which will become a component of existing staff workplan/s. While we are seeking funding to also include one staff airfare to and from Brisbane, SOSDF will likely fund the attendance of other staff members at this event.

Part of the budget for this project will be to help accommodate families who live outside of Brisbane in remote and regional areas. If we discover there are more families outside Brisbane wanting to attend, SOSDF will consider helping with some expenses/and or will reconfigure the budget in consultation with funding body.

Budget (GST exclusive)

Please outline your proposed used of the grant in the expenditure table below. Quotes are not required.

This is very important:

- Your budget **MUST** balance (total grant amount requested = total expenditure amount).
- Please **do not add commas** to figures – e.g. type 1000 not 1,000 – this will ensure your figures for each table total correctly.

Examples of expenses could include venue hire, catering, QMHW merchandise, guest speakers, entertainment, musicians, and artists, temporary instructors or speakers engaged specifically for your event, transport.

Remember, funding cannot be used to cover fundraising or the general operating costs of an organisation (e.g. staff wages).

Expenditure	$
Venue Hire and Catering	$1,000.00
Accommodation & Travel	$1,750.00
One flight SYD to BNE return	$250.00

Budget total

Total expenditure amount

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$3,000.00
This number/amount is calculated.

Bank details

If you are successful in receiving a grant the bank details you enter will be used to generate an invoice so your organisation can receive payment.

Applicant bank account
[redacted]
Must be a valid Australian bank account format.

Bank
St George

Certification and feedback

*** indicates a required field**

Certification

This section must be completed by an appropriately authorised person on behalf of the applicant organisation (may be different to the contact person listed earlier in this application form).

I am authorised to sign this application on behalf of the organisation.

I certify that to the best of my knowledge the statements made within this application are true and correct.

I acknowledge that my organisation may be deemed ineligible if any of the information in this application is incomplete, inaccurate, out of date, or misleading in any way.

I have read, and my organisation will abide by, the QMHW Community Grant Program Guide and I understand that if the applicant organisation is approved for this grant, we will be required to accept the terms and conditions of the grant as outlined in the letter of approval.

I agree * ◉ Yes ○ No

Name of authorised person *
Mr Lance Dale
Must be a senior staff member, board member or appropriately authorised volunteer (as per your organisation's delegation of authority policy).

Position *
Advocacy Officer
Position held in applicant organisation (e.g. CEO, Treasurer)

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Contact email * [redacted]

Date * 10/06/2021
Must be a date

Applicant feedback

You are nearing the end of the application process. Before you review your application and click the **SUBMIT** button please take a few moments to provide some feedback.

Please indicate how you found the online application process: *
○ Very easy ◉ Easy ○ Neutral ○ Difficult ○ Very difficult

How many minutes in total did it take you to complete this application? *
120
Estimate in minutes i.e. 1 hour = 60

Please provide us with your suggestions about any improvements and/or additions to the application process/form that you think we need to consider.

8 September 2020

Save Our Sons Duchenne Foundation Submission to the Federal Department of Education, Skills and Employment.

2020 Review of the Disability Standards for Education 2005

CONTENTS

"You Just Have to Be Ahead of the Game"

(Mary from Victoria and mother of boy with Duchenne).

Executive Summary:

This submission was drafted in close consultation with the Duchenne and Becker community in Australia.

That education is of equal importance to the life chances and quality of life of young people suffering from Duchenne and Becker muscular dystrophy as it is to those who are not afflicted by this terrible condition, is uncontestable. On that basis alone, it is critical that mechanisms like the *Disability Standards for Education 2005* (the Standards) are fully operationalised and able to deliver on the high order objectives which are designed to ensure equivalence in educational access and opportunity for all young people whether disabled or not.

Via our own extensive research and consultation processes, SOSDF has formed the view that these Standards are continuing to play an important role in our education system. However, and importantly, it does appear that these Standards are only "given real life" and application through the tiresome advocacy efforts and the sheer determination of parents and carers who are seeking a "fair go" at school for their children with Duchenne and Becker.

From our consultation, it has become apparent that the role of the Standards turns too much on:

- **i)** the particular School Principal and School leadership and whether they are committed or not to principles of inclusion;
- **ii)** the capacity and energy of parents and carers to get organized, "make noise" and regularly provide clear guidance/instruction and checklists to Principals and teachers on the needs of their particular child/ren;
- **iii)** whether good lines of communication (and opportunities for participation) have been established between school administrations and individual parents and carers, and finally;
- **iv)** the amount of funding and resources specific Governments (and their school systems) are prepared to provide toward the provision of reasonable adjustments, teacher numbers, teacher training, teachers' aides/learning supports within schools.

Introduction to SOSDF:

The Save Our Sons Duchenne Foundation (SOSDF) welcomes the opportunity to provide a submission to the Federal Government's review of the *Disability Standards for Education 2005.* We believe the current 5-year cycle of review processes to be

absolutely necessary in ensuring that these Standards continue to be relevant, contemporary and actively utilised in the equalisation of education opportunities between young people with a disability and those without disability.

Who we are?

SOSDF was founded in 2008 and is the peak body for those living with Duchenne and Becker muscular dystrophy (around 1,000 young people) across Australia. Our vision is to find a cure for Duchenne and Becker muscular dystrophy whilst actively working to ensure enhanced quality of life (including quality of educational opportunities) for those young people and their families affected by this condition. Advocacy and community engagement work are crucial to achieving this vision along with ongoing fundraising and events management designed to raise funds for essential research, service delivery and the provision of critical resources and equipment to the Duchenne and Becker community.

Along with the funding of a critical nurses program in some of our major children's hospitals across Australia, SOSDF also delivers a telehealth nursing service, scholarship programs, critical equipment and resources (such as wheelchairs and scooters) and a number of initiatives and programs such as music therapy which are designed to enhance the quality of life, skills and social development of young people suffering from Duchenne and Becker. For more information on SOSDF and the (cruel) Duchenne and Becker conditions please refer to the attached web link www.saveoursons.org.au.

More recently SOSDF released the landmark McKell Institute report (refer https://www.saveoursons.org.au/introductory-video-save-our-sons-duchenne-foundation-keynote-report-into-duchenne-and-becker-in-australia/). This report provided a comprehensive summary of issues impacting the Duchenne and Becker community across Australia including but not limited to:

- the astronomical financial, personal and psychological costs involved with supporting a child/ren with Duchenne and Becker;
- lost wages/income as a consequence of carer responsibilities;
- bureaucratic and regulatory impediments associated with clinical trials and research more generally;
- issues with the National Disability Insurance Scheme;
- the (un) timely diagnosis of Duchenne and Becker; and
- the importance of coordination of care.

This report has now been effectively utilised to draw national political attention to the concerns of the Duchenne and Becker community with a number of issues now placed firmly on the political agenda.

As has been made clear in this keynote report and from widespread consultation with the Duchenne and Becker community over many years, Duchenne and Becker carers and families already contend with enormous additional care responsibilities and issues. Many of these issues involve access and full participation in the educational system and educational opportunities with education critical to the life chances of young people with Duchenne and Becker. Subsequently, SOSDF has recently prioritised consultation with members of our community for purposes of the current review of the Disability Standards process.

SOSDF Consultation Process:

SOSDF determined to consult as widely as possible with the Duchenne and Becker community in the preparation of this submission. Social media posts were initially organised encouraging the community's participation and comment in the review. Following this, a series of individual zoom consultations of 30-45-minutes duration were held with parents/carers and some allied health professionals across Australia. Finally, an on-line zoom discussion was held with parents/carers who indicated an interest in participation.

A series of questions were posed to those involved in the consultation, a copy of which is attached at the conclusion of this submission. These questions attempted to go to those key issues which were identified as part of the main focus of the Department's review.

There was limited consultation with young people with Duchenne and Becker with almost all consultation involving parents, carers and allied health professionals. This was largely due to practical issues in facilitating zoom discussions with the boys and their ages. That said, however, we are confident that the views expressed through our consultation process sufficiently reflect/encompass and were informed by the educational experiences of young people with Duchenne and Becker.

Our submission is structured along the lines of some of the core questions we posed with some **recommendations** to follow in the conclusion.

Consultation Outcomes:

1. **What has been your experience with accessing education? What was the process like to enrol in school or other education and were you happy with the outcome?**

Overall, it appeared that most families were able to access their school of choice and there were few roadblocks/barriers in place. However, it appeared that most families were well organised and prepared and knew what they were seeking before approaching particular institutions – for example, schools were chosen on the basis of layout, and "gut feelings" after preparatory conversations with the school Principal. One parent remarked that due to the (inaccessible) physical aspects of their local public primary school they determined to send their child to a Catholic School situated 50 minutes away.

It was generally a given amongst those we spoke to (and a recurring theme throughout our consultation process) that those who could advocate and speak up strongest generally secured the access and resources (reasonable adjustments) which were required to facilitate their child's participation at a particular school. To quote from the words of Juliana, an Occupational Therapist in WA who works with a number of Duchenne and Becker boys:

"Families who are able to fight/advocate are a lot more successful".

SOSDF suspects there are many (overwhelmed) families who are dealing with Duchenne and Becker who simply do not have the resources, energy or capacity to advocate (to the level necessary) for their child/ren in the education system. This was confirmed by Mary a mother of a boy with Duchenne in Victoria.

"Lots of parents are muzzled. Most go by what the school says. Speaking to other parents they don't push"

This is concerning as access to an educational institution and any provision of reasonable adjustments, appear to be heavily dependent on the ability of families to advocate. Furthermore, if barriers exist at enrolment, then it is probably indicative of the lack of support a child with Duchenne or Becker is likely to receive at that particular school moving forward. In the words of Juliana again:

*"**Families don't get to pick where they go. If barriers exist at enrolment, then why fight all the time -families are already so energy and time poor".***

Access and the provision of support/adjustments also appear to be very contingent on the particular Principal at the school and the lines of communication (or not) which have been established by families with the Principal and head teaching staff- another recurring theme throughout the consultation process. Ross, a father from NSW, stated:

"If the Principal is on board then there are no dramas".

Christine a mother from Queensland also commented that:

"An open line of communication to the Principal is so important and not feeling like you are constantly pestering them".

Generally, it appeared there were few differences between accessing/enrolment in the public system and the Independent/Catholic School sectors. While most parents/carers talked highly of the Catholic system there were some notable exceptions including one father from NSW who commented:

"Support was very minimal. They kept sending my boy home, they couldn't deal with him. There was no plan in place and they just weren't on board. We took him out of the Catholic school and sent him to a public school and we can't praise them enough".

And then there was the story of one mum from Victoria who has been struggling for years to get a wheelchair ramp into her sons' Catholic primary school:

"We are butting heads all the time and they just keep putting things off".

Access issues amongst the selection of families we consulted appeared to become more acute with the transition to high school and the slow progression of the Duchenne and Becker conditions amongst their boys. The choice of school (the facilities, learning supports available, the approach of the Principal and teachers, the school culture) become more critical factors with the general decline in mobility and movement.

2. **Has your education provider/s made reasonable adjustments to ensure you or your child can participate in education?**

Overall, most parents/carers appeared satisfied with the reasonable adjustments implemented by their schools to facilitate the participation of their child/ren. A number of parents indicated that whatever they wanted they generally got (this appeared to be especially so in relation to the Catholic Education Office in some primary schools). That said, many parents/carers indicated it was still a hard struggle to secure these reasonable adjustments. Donna, a mother from Victoria, said:

"It wasn't easy. Had to be a strong advocate. You couldn't rely on the school. My son is a good communicator and that helps because we needed to identify what we needed".

Physical (reasonable) adjustments typically included but were not limited to:

- the creation of wheelchair ramps;
- handrails;
- steps at drinking taps;
- bidets in toilets;
- special chairs; and
- the provision of scooters.

Despite this positive feedback, the SOSDF consultation nonetheless identified a number of "war stories" and inconsistencies between schools with the general sentiment again being, that some schools "go over and beyond" what is required whereas others do the bare minimum - with inclusion of those with a disability an afterthought. Furthermore, that much again turns on your ability to advocate and create good lines of communication with the school leadership.

SOSDF heard, for example, from a father in NSW whose son has been unable to access the senior part of his school playground despite the fact his son is now in senior years at High School. Further, that his son had never attended a school excursion and was only ever asked about participation once he reached Year 10.

Attendance at school excursions, school camps, extra-curricular activities and school sporting activities and carnivals evinced a strong reaction from a number of parents and carers and suggested much more needed to be done by educational authorities by way of reasonable adjustments, to ensure those young people with Duchenne and Becker could participate on an equivalent basis to other students. Too often it appeared that, boys with Duchenne were left on the "sidelines" to amuse themselves

during school sporting activity or denied opportunities to attend excursions/camps unless a parent/carer was able to accompany their child/ren.

On the flip side of this, SOSDF heard examples of how schools included boys with Duchenne and Becker in excursions but failed to make the appropriate adjustments and modifications to ensure the child's attendance was properly accommodated. Patricia, a mother from NSW, told us how her school failed to take proper account of some steps on a field trip to Botany Bay resulting in an accident when teachers were forced to carry her son in his wheelchair. This example also reinforced the critical importance of parental advocacy as Patricia was then able to address issues relating to her son's participation in all future excursions:

"Now, after making a verbal complaint with the new Principal, all excursions are thought out and planned with my son in mind, and a risk assessment is carried out weeks in advance with my input. The assumption that I will be available to assist my son on the day or with transport often determines whether or not he will be able to attend an excursion".

This same mother also relayed the following story to us which highlighted the lack of forward thinking in much school building design and works:

"Demountable classrooms have been built to accommodate the increase in school numbers. A few years ago, the demountable Italian classroom that is part of the curriculum for my son was built with 10 steps to enter the front door. I asked the staff how they expected my son to get into the classroom, I was told "he'll be right, he can just wiggle up and hold the rail". I asked what the requirements were to get a ramp installed and whether the school needed any supporting documents from medical professionals to assist with getting this done but I was told that a ramp would cost $20,000 and it would be a long process to get approved and built. I was advised to wait and see how my son coped. After 3 school terms of advocating for the ramp, it was finally built. I felt like I was a burden to the school.".

Patricia's reflection above also highlights the concern that many parents/carers of Duchenne and Becker have expressed -namely, that they feel they (and their child/ren) become burdensome on the education system because they are constantly required to advocate to schools to get particular measures/strategies in place. Much guilt appears to be associated with this.

The pooling of funding for reasonable adjustments in some school systems also meant that individual families had to fight to guarantee that their child/ren got the specific adjustments they required.

Finally, there were issues with the delivery facilities such as disabled car parking. Christine a mother from Queensland discussed how she was caught up in a bureaucratic "buck passing" exercise between her local school and the local Council over the provision of a disabled car parking facility at the front of her busy school. As the School failed to take responsibility for this issue, Christine was forced to ensure another adult always travelled with her with each school trip to assist with the safe drop off her son in a wheelchair.

3. **Have you or your child been appropriately supported during your/their education? This includes being able to access supports, including specialist resources.**

There was a very mixed response to this question from participants in our consultation. There were issues raised about the difficulties getting private therapists into particular schools and a number of issues going to the lack of (Duchenne and Becker) awareness/expertise in relation to teachers' aides and teachers more broadly.

Issues were raised about the lack of speech therapy in special needs schools and inadequate resources provided to young people (such as laptop computers).

Questions were also raised as to whether young people with Duchenne and Becker were getting equal access to the training opportunities, work experience and TAFE pathways delivered by high schools.

Issues were also raised by those who were knowledgeable of the Disability Standards for Education about the failure of the Standards to cover before-and-after school care provided at schools -meaning many working parents and carers from the Duchenne and Becker community are disadvantaged if reasonable adjustments are required to facilitate the participation of their child/ren in these out-of-school hour services.

Teachers having to manage large student cohorts often meant that the needs of particular boys with Duchenne and Becker were overlooked especially in the absence of sufficient teacher aides.

Some parents and carers believed schools would ignore their recommendations and suggestions prioritising the views of health professionals over and above their lived experiences of Duchenne and Becker. A mother from Victoria, complained that:

"School doesn't take my knowledge on board. They only want to listen to a health professional"

Finally, issues with the quality and quantity of information provided by schools to parents and carers for activities such as school camps was not always forthcoming. As one mum stated:

"The school is just not forthcoming with information about camps. We feel we need to go on the camps just so we know that he will be safe".

On a positive note, a number of parents explained that they were given access to the teaching cohort (usually on a once-off teaching term basis) to provide presentations on Duchene and Becker - to raise awareness and provide key information on the needs of young persons with Duchenne or Becker. These information sessions appeared to be critically important to ensuring that particular schools were more inclusive and cognisant of the needs of the Duchenne and Becker community.

4. **If You or Your Child experienced harassment or victimisation in an education setting what happened? What steps did your/their education provider take to address this? Were you satisfied with the outcome?**

Aside from some limited teasing, very limited harassment, or victimisation by other school students towards their child/ren was reported by participants in our consultation. Interestingly, some teasing and bullying of the siblings of children with Duchenne and Becker was raised. The prevalence of this sort of unacceptable bullying behaviour may be higher than what was conveyed throughout this consultation.

Where some issues existed, parents and carers reported that the school would usually be adept at taking prompt action to stamp it out. Bullying/teasing were also rationalised by parents and carers as other students simply not understanding the condition affecting their child/ren -again pointing to the importance of raising awareness of Duchenne and Becker amongst the school community.

However, on the downside there were some concerning examples where parents and carers alleged that teachers had harassed or antagonised their child/ren. Stories were told of teachers talking down to their child because "*they look younger and are in a wheelchair*" (Dean, a father in NSW) and/or baiting their child/ren to set them up for failure. According to one mother who preferred not to be identified:

"The teacher my son had in year 3 would constantly antagonise him, set him up to fail and make comments such as "xyz won't say hello to me and doesn't have social skills or X hasn't listened to my instructions again' in front of others which in turn would upset my child and make him lose focus which the teacher would loudly reprimand him for. The teacher did not read (or perhaps understand) any of the literature I provided about the cognitive and behavioural functions associated with Duchenne e.g., information is processed in smaller chunks with Duchenne boys so some key messages will need repeating. Now the Principal and I meet at the end of the year and determine which teacher would best suit my son's academic needs for the next year".

Subsequently, with increased teaching awareness and training on issues such as Duchenne and Becker muscular dystrophy, SOSDF believes that the Disability Standards as they relate to bullying/harassment will progress further, in meeting their objectives.

5. **Has COVID-19 impacted on your child's experience in participating in Education?**

Consistent with the findings of the far reaching report undertaken by Children and Young People with Disability Australia (CYDA) *"More Than Isolated: the experience of children and young people with disability and their families during the COVID-19 pandemic"*, the Duchenne and Becker community experienced major issues participating in education as a consequence of COVID-19. This was especially so in relation to some of the key findings identified in the report (page 3), namely:

- Uncertainty about education including school closures and challenges with learning from home, and that progress gained by young people with disability could be lost during this period;
- Inability to obtain essential supplies e.g., groceries which were necessary for children and young people with a disability because of their conditions; and
- Cancellation of support workers.

More specific to the Duchenne and Becker community were:

- the higher health risks arising from COVID-19 as a consequence of their condition;
- limited access to teacher aides, educational supports and therapists during lock-down periods;
- additional learning needs which meant remote learning delivery was so much harder for parents and carers; and
- inflexible requirements to stay home (because of the higher health risks) when the rest of the school community had the option to stay/return to school.

According to Mary from Victoria:

"At least one day he needed to go to school. We need a break. We need a medical reason to say he can go to school. You just have to be an essential worker."

6. **Are you aware of the Disability Standards for Education? If yes, how did you become aware of the Standards?**

Almost universally the parents and carers of boys with Duchenne or Becker were unaware (or at best vaguely aware) of the existence of the Disability Standards for Education. This finding simply reflects the results of previous 5-year reviews (refer

Department's discussion paper) and the need for the Department, to do much more to promote awareness and understanding of their existence.

That said, it is clear to SOSDF that the Standards are positively impacting provided there are parental advocates who are pushing the school administrations and seeking to exercise the rights of their child/ren with Duchenne or Becker - to ensure equivalence in educational access and opportunities. So much would appear to turn on this in the absence of schools actively promoting and "marketing" the Standards to their respective school communities.

SOSDF is fortunate to have within its community, parents who are teachers and school administrators. We were heartened to learn that at least in some schools a module on the Disability Standards has been developed and is delivered as a teaching training requirement every couple of years. At those schools, negotiations with parents with child/ren with a disability is also encouraged to identify specific needs and requirements. Donna from Victoria, a teacher and mother with a boy with Duchenne maintains:

"The Disability Standards support inclusion. I can't see how we can tweak more. What's lacking is the communication side."

Finally, SOSDF notes that at the recent webinar organised by the Department of Education, Skills and Employment to kick-start consultation on the review, the overwhelming majority of participants to an on-line poll believed there should be mandatory training on the Standards for **all** educators. SOSDF firmly believes that mandatory training would certainly reduce the inconsistency between schools and teaching staff in the application of the Standards and would heighten awareness more generally of the need for inclusive pedagogical practices.

Aligned with this view was the position advanced in this consultation that whilst most teachers have good intentions, there was a lack of training regarding technology and the customisation of each child's needs to ensure they succeed i.e. "one size does not fit all", each child is different so customised processes and learning plans need to be developed. This is particularly so for those young people suffering from Duchenne and Becker muscular dystrophy.

7. **Do barriers still exist for students with a disability to access and participate in education and training? If so, how do you think the Standards could be improved to help address these barriers?**

Most, if not all participants, were of the view that barriers remained for their child/ren in accessing and participating in education and training.

Some barriers which were nominated included:

- lack of knowledge by the broader school community of particular conditions such as Duchenne and Becker;
- insufficient training/awareness of teacher aides/learning support staff;
- "hit and miss" nature of teachers -some were seen as responsive, others were not;
- physical infrastructure and facilities of schools;
- parents and carers who were not aware of their rights;
- failure to make school excursions, sports carnivals inclusive; and
- funding or resources for specific conditions such as dyslexia, ADHD.

Then there is also the "mushroom effect" with school administrations keeping parent and carers of child/ren with Duchenne and Becker in the dark. To quote from a mother from Victoria again:

"**Parents are not aware of what they can access. If you don't ask, you don't know. Lots of shit shovelling is required".**

In relation to teachers' aides, insufficient numbers were cited as a major barrier to an inclusive education by a number of parents and carers. A mother from Victoria, has three boys with Duchenne, one of whom also has ADHD. She commented in relation to teachers' aides:

"In terms of his academic studies they will give him one (a teacher's aide) but then take them away. They don't provide an aide consistently and he needs someone constantly".

SOSDF therefore notes with some alarm a series of recent articles in the Sydney Morning Herald "*Schools Must Prepare for 50 per cent rise in students with disabilities: report*" (https://www.smh.com.au/national/nsw/schools-must-prepare-for-50-per-cent-rise-in-students-with-disabilities-report-20200902-p55rrm.html) and "*Schools forced to address deficiencies in health system, professor warns*" https://www.smh.com.au/education/schools-forced-to-address-deficiencies-in-health-system-professor-warns-20200903-p55s6x.html?btis

These articles highlighted the growth in the number of students with disabilities and the need for twice as many specialist teachers and thousands more support classrooms. These articles go on to say that mental health experts are calling for a major investment in disability support staff, training, and resources for schools.

"*Under a status quo scenario, the specialist teaching workforce would need to increase from 12,000 to between 19,000 and 23,000 in 2027, the BCG report said. However, such teachers were in short supply;* ***only 56 per cent of learning and support roles in mainstream schools were filled permanently****" (SMH 3/9/20 Page 3).*

Finally, these articles also highlighted the huge increasing workload on an over-stretched teaching workforce as the profession grapples with new, competing and complex demands. As Professor Ian Hickie explained to a Teachers Federation Inquiry into the changing role of educators over the past 15 years. *"[Teachers have] become pastoral, they've become parental; social workers, psychologists, neuroscientists,"*. (SMH 4/9/20 page 13).

With such increasing demand on our educational system and our teachers and our aides/learning support staff, the totally inadequate resourcing and staffing of schools all-round, what chance do boys with Duchenne and Becker really have in navigating/participating on a level playing field in our current education system?

8. ***What are your views on moving to a completely inclusive education system where there are no longer any special needs classes and units in mainstream education?***

As part of our consultation process, SOSDF was interested to test the views of the Duchenne and Becker community on this question given the recent report by the Australian Coalition for Inclusive Education titled "*Driving change: A Roadmap for achieving inclusive Education in Australia".*

While SOSDF is broadly supportive of the alliance and the push to a totally inclusive education system, (with no segregated specialist schools, support units etc) we acknowledge and respect the fact, that amongst our community there are very mixed views going to this.

Our consultation demonstrated that all parents and carers want the mainstream education system to be as inclusive as possible – reflecting the importance of mechanisms such as the Disability Standards. Most parents, carers and young people clearly do not want to be part of segregated and separate education systems and schools.

Parents and carers however emphasised the importance of **choice** and the ability to be able to decide (without coercion or the imposition of access/enrolment barriers) which school their child/ren would attend. Donna, a mother from Victoria, commented:

"Don't know that I'm fully supportive of a completely inclusive system. There needs to be choice. The mainstream sector is too confronting for many of our community particularly in high school years. We need to respect the wishes of the child and their choice".

A number of parents and carers indicated that much depended on the level and nature of the disability, and there was also a clear distinction drawn between

physical disability (where the mainstream was largely advocated) and intellectual disability where the provision of specialist schools was seen to be more necessary.

Juliana, the occupational therapist from WA, said:

"With the right set up physical needs are much easier to meet. All of my DMD clients go to mainstream schools and that is what they want. For those with intellectual disabilities however it is more complex and special schools are required".

Full inclusion in the mainstream education system is clearly more challenging as the boys advanced through high school and their conditions deteriorated with age.

RECOMMENDATIONS:

1. **That the Department move to ensure that the *Disability Standards for Education* are applied to before-and-after school care programs run within the various School systems;**

2. **That greater efforts and endeavours are made by the Department to ensure all parents and carers are made fully aware of the existence, role, function and application of the *Disabilities Standards in Education;***

3. **That the Department mandate training on the *Disability Standards for Education* for ALL teachers and educators (on an annual basis) and that such training become core to ongoing teacher professional development – and not an afterthought and simply provided at the margins.**

4. **That greater teacher and teacher aide training be provided into various disabilities and conditions such as Duchenne and Becker to ensure the needs of young people suffering from these conditions are recognised, "customised" and accommodated in the education system -and not overlooked as a consequence of wider student cohort management issues;**

5. **That greater involvement and participation of parents and carers with child/ren suffering from a disability be facilitated in the design and layout of any new school buildings and facilities;**

6. **That regular and more formal consultations be scheduled between School Principals/staff and families with children with Duchenne and Becker, to ensure that specific learning and adjustment needs are being met and across the education system -without continued reliance on the capacity of individual parents and carers to advocate;**

7. **That the Department consider a specific consultation with the Duchenne and Becker community to better understand needs, requirements and aspirations of the community as it relates to the education system;**

8. **That the levels of teacher aide and educational support be increased to the Duchenne and Becker community during any future lockdown arising from the COVID-19 or other health pandemics;**

9. **That a review be undertaken by the Department into the inclusion/exclusion of young people with a disability (inclusive of Duchenne and Becker) in school excursions, school sporting activities/carnivals and extra-curricular activities delivered by schools;**

10. **That a representative of the Duchenne and Becker community be invited on to any steering committees, working parties, consultative committees or forums which are charged with overseeing the operation and implementation of the *Disability Standards for Education.***

11. **That there be a significant injection of funding into our education system to increase teaching numbers and resources to ensure that the needs of all young people with a disability are fully met and that educational opportunities are truly equal for all. Furthermore, to increase teaching numbers to ensure teachers and School administrations are not overworked and overwhelmed by the increasing and complex needs, demands and requirements of students and their families.**

Lance Dale **and** **Patricia McPhail**

Advocacy Officer/SOSDF **Community Engagement Officer/SOSDF**

References:

1. Australian Coalition for Inclusive Education "*Driving Change: A Roadmap for achieving inclusive Education in Australia".*
2. Sydney Morning Herald 3/9/2020 "*Schools Must Prepare for 50 per cent rise in students with disabilities: report"*
3. Sydney Morning Herald 4/9/20 "*Schools forced to address deficiencies in health system, professor warns"*

4. Children and Young People with Disability Australia (CYDA) *"More Than Isolated: the experience of children and young people with disability and their families during the COVID-19 pandemic"*
5. Federal Department of Education, Skills and Employment *"2020 Review of the Disabilities Standards for Education 2005: Discussion Paper"*.
6. McKell Institute *"Living with Duchenne and Becker in Australia: Supporting Families Waiting for a Cure"* April 2020.

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Attachment One

Questions which framed the consultation discussions

1. **What has been your experience when accessing education? What was the process like to enrol in school or other education and were you happy with the outcome?**

2. **Has your education provider/s made reasonable adjustments to ensure you or your child can participate in education?**

3. **Have you or your child been appropriately supported during your/their education? This includes being able to access supports, including specialist resources**

4. **If you or your child experienced harassment or victimization in an education setting what happened? What steps did your/their education provider take to address this? Were you satisfied with the outcome?**

5. **Has COVID-19 impacted on your child's experience in participating in education?**

6. **Are you aware of the Disability Standards for Education? If yes, how did you become aware of the standards?**

7. **Do you feel like you understand you or your child's rights when it comes to being able to access and participate in education? If not, what can be done to improve awareness?**

8. **Do you think the standards help students with disability to access and participate in education and training on the same basis as students without disability? Why or Why not?**

9. **Do barriers still exist for students with disability to access and participate in education and training. If so, how do you think the Standards could be improved to help address these barriers?**

10. **What are your views on moving to a completely inclusive education system where there are no longer any special schools or special needs classes and units in mainstream school?**

11. **What would be required to ensure all young people with a disability are able to participate equally along with young people without a disability in mainstream education?**

12. **Would you be prepared to provide us with examples regarding you or your child's experience of the education system which we could utilise in our submission to the Federal Government? This information would be de-identified.**


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