Save Our Sons

Duchenne Foundation (SOSDF)

Submission to the

NSW Upper House Inquiry into

Health outcomes and access to health and hospital services in rural, regional, and remote New South Wales

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"You have to be quite confident in what you want in a regional area and in a small country town you have to be prepared to advocate for the care we need"

Deb, a mother of a boy with Duchenne from Northern NSW.

Executive Summary

This submission was drafted after extensive consultation with the Duchenne and Becker muscular dystrophy community living in regional, rural, and remote NSW.

Access to good quality and affordable health (in the neuromuscular field) and hospital systems is a critical factor to the life chances and quality of life of families who are struggling with the progressive and fatal nature of Duchenne and Becker muscular dystrophy. Where these families choose to reside should have no bearing on the quality and availability of key neuromuscular and hospital services. **Yet clearly in NSW it does.**

On that basis, the Save Our Sons Duchenne Foundation (SOSDF) welcomes the opportunity to provide a submission to the NSW Upper House Portfolio Committee Number 2 - Health (the 'Committee') Inquiry on Health outcomes and access to health and hospital services in regional, rural and remote NSW.

While the Terms of Reference for this Inquiry are broad and extend far beyond the immediate context of the Duchenne and Becker community, SOSDF and the Duchenne and Becker community nonetheless believes this Inquiry provides an excellent opportunity to raise a number of key concerns 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 process.

In the absence of a cure for Duchenne and Becker muscular dystrophy and with medical and technological advances extending the life expectancy of boys with the fatal condition, it has become critical to ensure that the health burden and costs for Duchenne and Becker families be minimised. These additional health costs have been captured in the landmark McKell report *Living with Duchenne and Becker in*

Australia: Supporting Families Waiting for a Cure commissioned by SOSDF (a full copy of this report is attached to this submission - please also refer also to https://www.saveoursons.org.au/introductory-video-save-our-sons-duchenne-foundation-keynote-report-into-duchenne-and-becker-in-australia/).

1According to this report, which was officially launched by a number of Federal politicians from across the political spectrum at Parliament House 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 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.

Families also 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.

Unfortunately for many families "living in the bush" these disease cost burdens are simply heightened as there may be few or inadequate locally based health, GP, neuromuscular and emergency services (cardiologists, endocrinologists, physical therapists, pulmonologists etc.) and very sparse, or limited knowledge amongst health professionals in rural and remote communities of the Duchenne and Becker conditions. Furthermore, the travel and accommodation costs involved in accessing the appropriate care for boys with Duchenne and Becker (typically involving travel to metropolitan hospitals) is only partially offset by existing schemes/subsidies such as the Isolated Patients Travel and Accommodation Assistance Scheme (IPTAAS).

Opportunities for rural and remote families to participate in all-important clinical trials are also hamstrung by the lack of physical infrastructure in regional and rural NSW and by issues obtaining transport to population centres where trials may be based.

[1] McKell Institute Living with Duchenne and Becker in Australia: Supporting Families Waiting for a Cure Page 44

While the Duchenne and Becker community is acutely aware they constitute a small (rare disease) population subject to an already overstretched public health budget, more can be done to assist those Duchenne and Becker families in the bush to gain access to good quality and affordable health care. Too often, it is simply left to these families to advocate ("become the squeaky wheel") to the health care providers and/or to educate those delivering health services about the care and special needs of those suffering from Duchenne and Becker muscular dystrophy. In the words of Shane, a father of a boy with Duchenne from the South Coast:

"You have to educate for Duchenne and Becker. They just sit there and go it's Multiple Sclerosis. You have to explain the whole emotional journey to people. I just start to cry and break down in front of people".

And this from Alison a mother of a Duchenne boy from Ungarie:

"The Doctors have limited knowledge and we have to appear as teachers……I'm a fighter for Jack. I'm not one to stand by and let things go easily".

Amongst the recommendations subsequently proposed by SOSDF to the NSW Parliamentary Inquiry into rural, regional, and remote health and hospital services are the following:

- the provision of outreach neuromuscular services to regional, rural and remote NSW;
- greater flexibility and interpretation of the IPTAAS (and an expansion and significant increases in the funding available);
- greater training and education of GP's and other allied health professionals based in regional, rural and remote NSW in rare diseases such as Duchenne and Becker muscular dystrophy;
- greater linkages/coordination be encouraged between metropolitan hospitals and regional hospitals in the delivery of health care services to the Duchenne and Becker community;
- that audits be undertaken in regional hospitals and area health services to ensure the provision of basic equipment (e.g., ceiling hoists) is available to adequately care for Duchenne and Becker patients;
- the identification of gaps and knowledge in rural health services be undertaken in relation to the diagnosis and standard of care of Duchenne and

Becker muscular dystrophy and measures be developed and implemented to resolve these issues as a matter of urgency;

- NSW Department of Health to undertake a specific consultation with the Duchenne and Becker community in regional, rural and remote NSW in relation to health needs and requirements and investigate the establishment of ongoing consultative mechanisms;
- an expansion in telehealth services for Duchenne and Becker boys and their parents/carers; and
- improved transition processes between pediatric and adult care; and the
- NSW Government to work with the Federal Government, pharmaceutical companies and other key stakeholder/patient groups to help facilitate the participation of Duchenne and Becker families residing outside metropolitan centres in NSW, in key clinical trials.

SOSDF notes with some interest the NSW Government's recent submission to the Federal Government's Inquiry into approval processes for new drugs and novel medical technologies -**refer attached.** The Executive summary of that document made an important (and welcome) claim which reads:

[2]***NSW has a strong history of paving the pathway for new drugs and novel technologies in Australia. NSW also has key opinion leaders in creating, developing and manufacturing advanced medical devices, diagnostics and therapeutics, particularly in gene therapies, viral vector engineering, cell therapies and phage therapies.***

Time is of the Essence

Numerous parents and carers reminded SOSDF during this consultation that Duchenne and Becker are "time sensitive" and degenerative diseases. That they are killer conditions which with the passing of time, progressively destroy every muscle function in the human body and inevitably culminate in the untimely and premature deaths of too many young people.

Our community firmly believes therefore that everything must be done to expedite improvements in our health and hospital system to ensure that families, irrespective

[2] NSW Government Submission into Federal Government Inquiry into approval processes for new drugs and novel medical technologies. November 2020, page 5.

of their NSW postcodes, are able to access the same standards of care – and within the same timeframe and at the equivalent financial costs to those in metropolitan areas.

Furthermore, that the Government and the health system generally, do more by way of disseminating/coordinating information on the availability of clinical trials and new medicines/treatments to **all** families (irrespective of address) impacted by Duchenne and Becker. Too often it appears it is only through the individual vigilance, advocacy, resourcefulness, assertiveness and research capacity of particular parents and carers, that the whereabouts or prevalence of particular trials or new treatments/medicines becomes known- the lack of information being more acute for those living away from the metropolitan centres.

Hospitals were not seen as talking with each other and there was a perceived disconnect between the health system, pharmaceutical companies, Government agencies and other key stakeholders. This clear lack of collaboration amongst critical players is of great concern.

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 health, educational, employment, social 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 neuromuscular nurses program in some of our major children's hospitals across Australia, (including Westmead in NSW) SOSDF also delivers a telehealth nursing service, scholarship programs, critical equipment and resources (such as wheelchairs, scooters, cough assist machines, and portable ventilators) 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. We are also currently in the process of

recruiting an NDIS Coordinator to assist the Duchenne and Becker community navigate the current NDIS system. For more information on SOSDF and the (cruel) Duchenne and Becker conditions please refer to the attached web link www.saveoursons.org.au.

SOSDF is also responsible for major research projects such as the aforementioned McKell 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 (inclusive of those living in regional/rural and remote NSW) provided a comprehensive summary of issues impacting the Duchenne and Becker community 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;
- issues with the National Disability Insurance Scheme;
- the (un) timely diagnosis of Duchenne and Becker; and
- the importance of coordination of care.

Terms of Reference

Following consideration of all the Terms of Reference for the NSW Upper House 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 **TOR 1 (c)** which states:

That Portfolio Committee No. 2 - Health inquire into and report on health outcomes and access to health and hospital services in rural, regional and remote NSW, and in particular:

1. **c) access to health and hospital services in rural, regional and remote NSW including service availability, barriers to access and quality of services.**

Although focussing on TOR 1 c) our submission also touches other aspects of the terms of Inquiry including but not limited to 1 (a) through to 1 (d), being:

a) health outcomes for people living in rural, regional and remote NSW;

b) a comparison of outcomes for patients living in rural, regional and remote NSW compared to other local health districts across metropolitan NSW;

d) patient experience, wait-times and quality of care in rural, regional and remote NSW and how it compares to metropolitan NSW.

SOSDF Consultation Process

SOSDF determined to consult as widely as possible directly with the Duchenne and Becker community in rural, regional, and remote NSW in the preparation of this submission. Social media posts were initially organised encouraging 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 and some allied health professionals living and working across country NSW.

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 TOR 1 c).

In addition to this consultation, 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 country NSW as well as families living in other rural, regional, and remote areas of Australia. An extensive survey targeting the Duchenne and Becker community had been launched on 4 December 2019 and closed on 23 December 2019. [3]There was a total of 173 responses, a sizeable sample of the estimated population living with Duchenne and Becker in Australia. 77.05% of this sample were parents of children with Duchenne and Becker and grandparents and siblings made up the rest.

Our submission is structured to highlight those issues which were identified as most relevant by our families throughout our consultation process. The submission is not intended to be a comprehensive "catch all" response (or generalisation) and importantly recognises that variations exist in the experience of rural, regional, and remote health and hospital systems across NSW. Further, that while there have been a large number of criticisms and negative responses from our community, there have

[3] McKell Report Pages 14/15.

also been some highly positive experiences of the health and hospital system and those who are currently working within it. We spoke for example to a family living at Jesmond and they were highly complementary about services provided at the John Hunter Hospital in Newcastle. There were others who were prepared to praise the goodwill and efforts of nurses and Doctors who were working under extremely stressful and trying conditions.

Responses have been made in good faith and in a concerted attempt to draw attention to gaps and shortcomings in the health system in the hope that positive change and increased funding and services are resultant. SOSDF 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.

Finally, at **Attachment 3** to this submission we have provided a YouTube video for the Committee to review and consider as it provides invaluable insights into our organisation and importantly, the "lived experience" of those who are suffering from Duchenne and Becker.

Key Issues and Findings:

Lack of Local Accessibility/Knowledge/ Outreach services

"***Now we feel we have to be the experts- a large folder of information is taken with us wherever we go. If we didn't actively seek out care in Wagga Wagga we would simply slip through the cracks (as others have). In Sydney adults can still go to neuromuscular clinics we can't in rural/regional areas".*** Sally, a mother of a Duchenne boy from the Riverina region of NSW.

"***Specialist appointments are all in Canberra and NDIS is saying it's your cost. We have to go to Sydney for surgery because he is still classed as a child. We would like to get him into hydrotherapy but there is none around here",*** Tammie Lee a mother of a Duchenne boy from Bega.

Boys (and rare girls) diagnosed with Duchenne and Becker muscular dystrophy require an array of ongoing and complex medical treatments and services. These treatments change as the boys get older and the disease symptoms become more pronounced and debilitating. **Attachment 2** is a graphic representation of some of the care and neuromuscular requirements of these boys.

Overwhelmingly we heard from families about the lack of these services for Duchenne and Becker in regional, rural and remote areas with families frequently travelling hundreds of miles to major metropolitan hospitals in Sydney, Melbourne and Brisbane to obtain the care and services they required, this simply adding to the already huge stress of caring for children and young men with this disease.

"There was next to no local support -period. We just did it ourselves, it was so much easier doing it ourselves", David and Annterese, parents of a deceased Duchenne boy from North Nowra.

As already highlighted, parents too often felt they had to be educating health service staff in rural, regional, and remote areas as there was little local knowledge or awareness of the condition. This becomes extremely problematic in situations where there are families who are unable to speak up or who lack the time and resources to seek out services or make the system more responsive to their child's needs. At the extreme end of this, SOSDF heard some horror stories of country hospitals ill-equipped or lacking the basic knowledge (and equipment, such as manual hoists) for caring for boys with Duchenne or Becker.

Recounts one father of a Duchenne boy living on the South Coast:

"When Michael was in hospital he was just put in with older people and not others his same age. They didn't have hoists or slings. ER was difficult to access. The rigmarole we were put through and the lack of care and concern was appalling"

States another mother from the Riverina area with a boy with Duchenne:

"The nurses are exceptionally caring and do their best but often lack the familiarity with Duchenne/wheelchairs/hoists etc-now there is a specific room with a ceiling hoist in the paediatric section of a new hospital upgrade-don't think this is true of general ward…Have had to wait long periods of time to access emergency room hoist in non-emergencies as this was the only way to examine Fletch".

One North Coast mother of a Duchenne boy recapped the following story:

"When he was 10, he fell over and went to hospital. I said he needed X rays. The Doctor just rolled his eyes. We ended up going to a regional hospital. He had fractured his back. Had I not advocated for him it would have been a very serious situation…"

By contrast, most families praised the quality of services they received from neuromuscular clinics through Sydney's Children's hospital network (Randwick and Westmead) and the access to more specialised services and personnel.

"***We can relax in the knowledge that his care is being managed appropriately in the city (mostly) if not only for the familiarity and exposure to it".*** Mother of a Duchenne boy from Central West NSW.

Gemma, a mother of a Duchenne boy from Jesmond, had this to say about the John Hunter Hospital in Newcastle:

"We are very lucky with the doctors here they are very knowledgeable. There are excellent pediatricians who follow right through".

Many families advocated the need for outreach services to be provided in their particular community and region, as one important means of overcoming local gaps

in services and provision. SOSDF was advised that the Children's Hospital at Westmead used to have an outreach clinic in Lismore, but this was stopped. In the Riverina, SOSDF also learnt that some families of Duchenne and Becker boys took this lack of outreach into their own hands and raised enough (private) funding to bring the neuromuscular team at the Children's Hospital at Westmead out to Wagga Wagga for visits for a couple of years -where they were able to service up to 14 families from Wagga Wagga and outlying areas with various forms of muscular dystrophy. Unfortunately, it appears this is no longer occurring.

While commending such local efforts, SOSDF does not believe it should be left to local communities to raise the necessary funding to enable and entice specialist hospital teams to visit regional, rural, and remote areas. We say this should be a Government responsibility which is funded and structured in such a manner to ensure that all regions and remote areas are able to access on a systematic basis, quality and specialised neuromuscular care and support -and without the need for families to be constantly uprooting and travelling miles to the metropolitan centres. This should be a core service of the hospital network and health system throughout the State.

Further, and as suggested by some allied health professionals, there needs to be greater coordination between metropolitan and regional hospitals and health services to ensure that knowledge and expertise can be given where gaps are evident in the levels of local knowledge and expertise on Duchenne and Becker in regional and remote health contexts.

Argues Michele, a telehealth nurse working in the area of Duchenne and Becker muscular dystrophy who takes calls from across Australia:

"Most things could be done in regional hospitals, but we just need to make sure resources are available. Someone needs to link in with city hospitals and there should be greater levels of communication between city and regional hospitals"

SOSDF understands from our conversations with these health professionals that there are a number of functions that could possibly be done at regional hospitals

with results sent back to tertiary hospitals for review-should these regional hospitals be resourced and equipped correctly. These include all lung functioning/cardiac monitoring, ECHO's, ECG, cardiac MRI, bone density, bone density infusions, sleep studies, and casting for AFO's orthotics.

Education of local health professionals: Care Management and Coordination/Transition.

The provision of outreach services should be accompanied by much greater efforts to ensure that health practitioners in regional, remote, and rural areas are all equipped with some working knowledge and awareness of Duchenne and Becker muscular dystrophy. Further, that proper care management and coordination is undertaken especially as boys' transition from paediatric to adult care -coordination which could be overseen by metropolitan specialists working in concert with their regional and rural colleagues.

Whilst SOSDF appreciates the vast challenges this may create (and especially given the number of rare diseases and conditions) we nonetheless maintain it is unacceptable for our community to encounter the depths of misunderstanding and "ignorance" it currently receives from the health and hospital system in rural and remote NSW. To quote again from some families:

"***Our endocrinologist called it "Doo-chen-ease" and admitted she hadn't seen it since uni",*** mother of a Duchenne Boy from Wagga Wagga.

"They sit there and go it's MS (multiple sclerosis)", father of a Duchenne boy on the South coast.

"There didn't appear to be a real care plan dealing with boys from season to season. No preparation for winter. No overall road map with no pit stops for therapy", father of a Duchenne boy from the South Coast.

"***They are relying on parents to be medical advocates for their kids***", mother of a

Duchenne boy from the far North Coast.

Issues with the transition of Duchenne and Becker boys from paediatric to adult care is the subject of major research currently being undertaken by SOSDF as many gaps and concerns have been identified over the years. Traditionally it appears the younger boys have received the best and more wholistic approaches to care (through specialised neuromuscular clinics in children's hospitals) whereas the older boys and their families have been largely left to their own wares with little oversight/coordination by medical professionals. This becomes an acute issue in regional, rural and remote areas when it becomes overlaid by issues of distance. Not surprisingly, transition issues were amongst some of the core issues raised by some parents and carers who are living outside metropolitan centres.

"Adult care is vastly different to paediatric. Transition support is essential. Everything needs to be actively sought out -not readily available", Sally a mother of a Duchenne Boy from Wagga.

"Westmead Childrens -they looked after him. But when he became an adult, we just do it ourselves. It's so much easier to do ourselves", a father of a Duchenne boy from Nowra.

Failure/Delays in Disease Diagnosis:

*"**We went round and round in circles",*** father of a Duchenne boy from the South Coast.

Aligned with the above issues was a common complaint from many families about the difficulties they encountered getting an initial diagnosis from the medical system about the Duchenne and Becker condition. While this is not a problem unique to those families living in rural, regional and remote areas as was well documented in the [4]McKell report, it nonetheless appears particularly acute in regional, rural and remote NSW where there is such limited knowledge and general awareness of this

[4] McKell report pages 16/17

condition. This lack of an early diagnosis clearly has negative implications for health and wellbeing, quality of care, family planning, risk reduction, and general career and personal/professional planning.

SOSDF heard repeated stories not only about the delays in diagnosis but also the scant support provided to parents and carers once the diagnosis was finally given. We heard for example the story of Luka, a Lismore mother who took 5.5 years to finally obtain a diagnosis of Becker muscular dystrophy after a genetic test and after an extended period of six-monthly blood tests and repeated journeys to the Gold Coast to see neuromuscular specialists:

"It was very long process. He was diagnosed only in May this year at 5 and a half years of age. There was over two years of investigation. At Lismore hospital these young paediatricians had told us not to even worry. Don't even bother with blood tests but I knew something was wrong. Since Gabriel's diagnosis in May I have had a 10-minute conversation over the phone with his paediatrician in Lismore to inform me he has Becker. Since then I have had two telehealth conversations with the Brisbane Children's hospital which was a very general introduction to what they do there. A very confusing conversation later and I was left not having a clear understanding of what this means for him. I feel that living in a rural area has limited support and information available to me particularly from professionals, and I have had to seek this out myself".

Or there is the case of a father in the Shoalhaven region whose boy with Duchenne now lives in Young. He told SOSDF that it had taken 4 years to finally get a diagnosis for his son and more than two years after blood tests had been carried out.

"The Doctor said he had nil issues he was just going to be a slow boy. When I was not convinced and took it further, I got results within 24 hours and was told he had an incurable disease. I got no support and was given this death sentence and just had to handle it".

SOSDF advocates for the NSW Government to investigate this issue and to develop strategies to overcome current roadblocks in diagnosis. We suspect that one of these roadblocks may go to the levels of coordination and communication within the health sector. Moreover, we seek to ensure that greater care and support is given to those parents and carers who have been given this diagnosis and all that it entails – premature death, uncertainty around child/ren future prospects, muscle deterioration and loss of mobility, financial costs, caring roles and responsibilities, and ongoing medical care.

Telehealth: The Good and the Bad

SOSDF notes with some interest the NSW Government's *"Telehealth Framework and Implementation Strategy 2016-2021".* In particular, we note comments such as the statement that [5]*telehealth has been particularly important in rural and remote areas, where it has had a positive impact on patients and clinicians, through reduced travel time and improved access to specialists for advice.*

Unquestionably, the growth in telehealth services (especially with the advent of COVID-19) has meant much of the isolation and "tyranny of distance" experienced by families in the bush can be addressed to some degree -and by clinicians attempting to service those families. It also means that families are not required to "uproot" on every occasion they seek medical advice/assistance. Finally, we are advised that some of the older boys would prefer telehealth to in-person appointments - for reasons of convenience and self-pride.

However, with that said, telehealth is NO panacea for the difficulties in access to quality health services experienced by the Duchenne and Becker community living in rural, regional, and remote NSW.

Telehealth is not a suitable vehicle, for example, for any of the tests or scans described earlier (lung functioning/cardiac monitoring, ECHO's, ECG, cardiac MRI,

[5] NSW Health Telehealth Framework and Implementation Strategy 2016-2021 Page 7.

bone density, bone density infusions, sleep studies, casting for AFO's orthotics) which are so vital and necessary in the care of Duchenne and Becker boys – and which we maintained could possibly be delivered from regional hospitals in partnership with tertiary city institutions. Nor can they serve any purpose in remote areas where internet connections are poor or intermittent such as "outback areas" or within shipping containers in bushfire ravaged areas on the South Coast of NSW - where one Duchenne family currently resides.

Telehealth is also of limited utility when it is necessary for health practitioners to view and make assessments about the full body (including the legs and feet) of someone suffering from Duchenne or Becker – as this is impossible on small screens.

It would seem however that Telehealth is an important means to allow families to maintain contact with specialists and other health professionals (and especially in between major appointments with city based neuromuscular clinics). Says Patricia, a mother of a Duchenne boy:

"Scans or testing that require medical equipment can't be done via telehealth. Ollie's telehealth appointments have been more of a "lets touch base" to update the neurologist, organise scripts, get referrals. I'm still required to measure Ollie's height and weight and record his blood pressure for their data records (which requires a visit to the local GP). I came across a mum who said one benefit of telehealth was that she was able to link in with a specialist in a different state, something she wasn't able to do prior to covid".

Telehealth also offers the opportunity to provide greater support (particularly for mental health) for parents and carers who are dealing with the daily pressures and stresses of children with Duchenne and Becker. Parents have advised us that there are few support groups "operating in the bush" and that this is especially needed, given they are worn out from the constant demands of caring and advocacy.

Not surprisingly, SOSDF supports the increase in Medicare assistance (up from 10 to 20 consultations) for those people who are currently seeking psychological counselling assistance. While psychological counselling may be important for some parents and carers dealing with Duchenne and Becker, we believe Telehealth opens

up the promise of other health strategies which could be introduced to support Duchenne and Becker families living in regional, rural and remote NSW.

IPTAAS: Inadequate travel and accommodation subsidies

"To go to Sydney, we need to fly. Flight approvals are just too hard, and you get no assistance. The process is just so convoluted and difficult to navigate", a mother of a Duchenne boy from Gulmarrad.

Transport to major cities such as Sydney, Melbourne, or Brisbane to access specialist services and care is a major issue for Duchenne and Becker families -especially if the child/young person is confined to a wheelchair. There are typically huge costs involved not only with the travel to and from cities, but also through foregone wages if parents and carers are required to take time away from their workplace.

While the IPTAAS scheme is critically important in helping to facilitate travel and accommodation for Duchenne and Becker families there are nonetheless a number of shortcomings with the scheme.

Firstly, currently [6]22 cents per kilometre is reimbursed for private car travel. While this is helpful, it does not cover for the wear and tear on vehicles where long distance travel is required. This is also well shy of current ATO rates of [7]72 cents per km for 2020/21 recognised for workplace travel.

Secondly, the subsidies for accommodation in the major cities are woefully inadequate at $20 per night at hotels or $20 per night per person for private stay. Many families are subsequently unable to afford good quality city accommodation which is located close to the particular hospital they are visiting.

[6] NSW Government brochure on IPTAAS
[7] www.ato.gov.au

Thirdly, we understand that air travel subsidies are limited and do not cover the cost for all parents meaning that many air travel costs are still borne directly by families.

Fourthly, the scheme does not provide for any meal or food allowances - meaning large expenses are typically racked up purchasing food especially if families are staying away from home for long periods of time.

Fifthly, and noting that many families will be unable to utilise public transport, taxi reimbursement is inadequate on whichever of the IPTAAS measures you look at. At $20 for a 1 day appointment you could barely get a taxi to take you from your hotel doorstep to the first set of traffic lights (let alone get across town) in Sydney such are the high flag call rates. Sally, a mother of a Duchenne boy from Wagga, recalls one story of a huge taxi expense in Sydney:

"When flying to Sydney Children's Hospital, we were told to take a taxi to Westmead as there was not an accessible bone density facility at SCH and told to claim on IPTAAS. This information was incorrect and cost us approximately $200 each way and IPTAAS reimbursed $22".

Sixthly, there appears to be a lack of information readily available to Duchenne and Becker parents on eligibility for travel and accommodation entitlements under the scheme. SOSDF spoke to a number of families who indicated they had not been aware of IPTAAS support and had just managed travel arrangements through their own means.

Finally, there also appear to be issues in relation to accessing IPTAAS if families already have private health insurance cover. In the words of Alison, a mother of a Duchenne boy from Ungarie:

"There are a lot of out of pocket expenses that IPTAAS doesn't cover if you have private health cover. Our private health covers only a certain number of kilometres and no accommodation is covered. I've had up to $4,000 with out of pocket expenses".

While one could argue that particular families have chosen private health cover and it is an issue for those Funds and not the Government, it should be recognised that these families have also chosen private cover largely because of the special medical needs and assistance required for their boys - and to become less of a burden on the public health system. We believe that Duchenne and Becker families who are already dealing with huge medical/social/care costs should not be "penalised" as a consequence of their choice to take out private health insurance.

In summary, SOSDF recognises the importance of subsidies provided by IPTAAS. They are instrumental in ensuring that those families living in regional, rural and remote NSW are able to access the best health care which is available. However, we do maintain that subsidy levels require urgent review and eligibility criteria expanded and made more flexible, to ensure that greater utilisation of the scheme can be made by the Duchenne and Becker community.

Access to Clinical Trials for Duchenne and Becker families living in regional, rural and remote NSW

"***We were a part of the GSK Exon skipping trial run out of Westmead. We participated for almost 3 years. This involved leaving home just before 6am every Monday, driving 45 mins to the airport, flying to Sydney, 1 hr taxi to the hospital for drug injection, bloods etc then taxi back to the airport to fly home. If we were lucky, we could catch at 2pm flight home, otherwise it was 6pm. Happy to chat to share our story. It can show parents will do anything to get their kids the access to treatment they deserve***", Deb, a mother of a Duchenne boy from Northern NSW.

Clinical trials are a key part of a health system and access to them is something which is actively sought by all Duchenne and Becker families – in pursuit of a cure for the condition. Clinical trials provide families with hope yet, unfortunately, clinical trial options in this country have been hamstrung by factors such as population size, bureaucratic red tape, and gridlock. Please refer to the **attached** [8]SOSDF submission to the Federal Government which details issues around clinical trials in more detail.

[8] SOSDF Submission to the Federal Parliamentary approval of new drugs and novel medical technologies.

For families living in regional, rural, and remote NSW access to clinical trials (which if available are usually always run from major population centres) become all the more problematic. Participation is only possible if families and carers are prepared to go to the extraordinary lengths which are described by Deb in the example above.

SOSDF would urge the NSW Government to collaborate more closely with the Commonwealth Government and health-related agencies, pharmaceutical companies, and key stakeholder groups to bring more clinical trials to NSW (including regional and rural NSW). As we advocated to the Commonwealth Government, we believe there are a range of measures/roles that could be undertaken by Governments at all levels to promote, secure and increase the number of clinical trials conducted on Australian soil. These measures and roles include, but are not limited to, brokering, negotiating, coordination, provision of essential infrastructure and allied services, providing tax and other incentives, and subsidisation.

Furthermore, and acknowledging the difficulties in setting up clinical trials outside metropolitan areas, SOSDF believes the NSW Government could play a more proactive role in ensuring that Duchenne and Becker families living in the regions or remotely, are able to participate in clinical trials (when they become available) on an equivalent basis to their city cousins. Financial assistance beyond that currently provided by IPTAAS subsidies should be considered along with greater coordination and communication efforts between the tertiary centres and regional/rural health systems described earlier. When trials become available, families irrespective of postcodes should be advised, their medical records and histories made available and the logistics of participation worked through.

Consultation with NSW Health

The Duchenne and Becker community in rural, regional, and remote NSW is an isolated one but an extremely resilient and articulate one. Currently, they have no voice other than that which they provide on an individual level (which can be a very loud one) and through peak agencies such as SOSDF. With so little generally known about the condition outside of the major metropolitan neuromuscular clinics, SOSDF proposes that the NSW Government (through NSW Health) establish opportunities and mechanisms for regular consultation to occur between NSW Health and these families. SOSDF would be prepared to play a role in setting this up and helping to facilitate the participation of our community in any consultation processes - to

ensure that the very best health outcomes are delivered and on an equivalent basis, across the board.

SOSDF do acknowledge that our organisation have met with the NSW Minister for Health, the NSW Shadow Minister for Health, and Executives from NSW Health over the past year and will continue to advocate strongly to elected representatives and senior departmental officials on behalf of the Duchenne and Becker community in Australia in order to achieve genuine, meaningful and real outcomes of value for all whom we represent.

Conclusion:

Although this NSW Upper House Inquiry appeared to be resultant from a series of [9]high profile issues in our country hospital system, SOSDF nonetheless believes it provides an invaluable and unique opportunity to constructively progress a number of public health concerns which have been present for Duchenne and Becker families living in regional, rural and remote NSW.

SOSDF is therefore extremely thankful that this Inquiry has been established by Members of the NSW Legislative Council with cross-party support. It demonstrates an important political consensus around the need to move the agenda forward in relation to ensuring access by all NSW residents (irrespective of residential address) to quality health and hospital systems and outcomes.

This submission has been written in good faith and as an attempt to make an important contribution to this process. SOSDF 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.

SOSDF 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 health and well-being of our community are much too important for us not to actively participate in the important work of this Committee.

[9] Sydney Morning Herald "Parliament to Launch inquiry into "appalling" state of NSW country hospitals" September 15, 2020.

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

RECOMMENDATIONS

1) That the NSW Government fund and coordinate the provision of outreach neuromuscular services to regional, rural and remote NSW;

2) That the NSW Government review the current subsidy levels of IPTAAS and ensure greater flexibility and interpretation of the IPTAAS criteria (and that an expansion and significant in the overall funding available to the scheme be delivered);

3) That the NSW Government facilitate greater training and education of GP's and other allied health professionals based in regional, rural and remote NSW in rare diseases such as Duchenne and Becker muscular dystrophy;

4) That the NSW Government encourage, establish and coordinate greater linkages/relations between metropolitan/tertiary hospitals and regional hospitals in the delivery of health care services to the Duchenne and Becker community;

5) That the NSW Government undertake audits in regional hospitals and area health services to ensure the provision of basic equipment (e.g. ceiling hoists) is available to adequately care for Duchenne and Becker patients;

6) That the NSW Government work to identify any gaps and knowledge in rural health services in relation to the diagnosis of Duchenne and Becker muscular dystrophy - and in the process review the support provided to families with a newly diagnosed child;

7) That the NSW Government (through NSW Health) undertake a specific consultation with the Duchenne and Becker community in regional, rural and remote NSW in relation to health needs and requirements. Further, that mechanisms/fora are established to ensure ongoing communication/consultation between NSW Health and the Duchenne and Becker community occurs;

8) That the NSW Government explore an expansion in telehealth services for Duchenne and Becker patients and their parents/carers who are living in the regions or in rural and remote areas;

9) That the NSW Government look to improved transition processes between pediatric and adult care for Duchenne and Becker patients living in regional, rural and remote NSW; and,

10) That the NSW Government work with the Commonwealth Government, pharmaceutical companies and other key stakeholder/patient groups to help facilitate clinical trials located both within and outside Sydney and/or the participation of Duchenne and Becker families residing outside metropolitan centres in NSW, in key clinical trials.

REFERENCES

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

2) NSW Government Submission into Federal Government Inquiry into approval processes for new drugs and novel medical technologies. November 2020

3) NSW Health Telehealth Framework and Implementation Strategy 2016-2021

4) NSW Government brochure on IPTAAS

5) www.ATO.org.au

6) SOSDF Submission to the Federal Parliamentary approval of new drugs and novel medical technologies.

7) Sydney Morning Herald "Parliament to Launch inquiry into "appalling" state of NSW country hospitals" September 15, 2020.

Attachment One:

Consultation Questions -NSW Parliamentary Inquiry

1) **Have you been able to access the appropriate health and hospital services living in rural/regional NSW for your child/ren suffering from Duchenne or Becker?**

2) **What sort of issues or barriers if any, have you experienced for your child/ren suffering with Duchenne or Becker while attempting to access health and hospital services living in rural/regional NSW?**

3) **If you have been unable to access health and hospital services for your child/ren suffering with Duchenne or Becker what have you done to address this?**

4) **If you have had to seek (specialist) services or hospitals in Sydney or another regional centre for your child/ren with Duchenne or Becker, what time, cost and other personal expenses has this incurred for your family? Where do you seek those services from?**

5) **Can you tell us anything about your experiences with health and hospital system/services in your local area? Were there long wait times, were staff well informed/aware of the Duchenne or Becker condition, did you receive a good quality of care?**

6) **If you have utilised health and hospital services in metropolitan areas of Sydney for your child/ren with Duchenne or Becker, how did these services compare to your experience of health and hospitals in rural/regional areas?**

7) **What gaps (if any) in health or hospital services do you think exist for the Duchenne and Becker community living in rural/regional NSW?**

8) **Do you have any specific stories you would like to convey about your family's experiences with the health and hospital system in rural/regional NSW?**

9) **Do you have any recommendations on how the NSW Government could improve or help facilitate enhanced service delivery to families living with Duchenne and Becker in rural/regional NSW?**

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Attachment 2.

Attachment 3.

SOSDF YouTube Documentary

This video is an 8-minute YouTube production 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