

A Treatment for Duchenne muscular dystrophy – No child left behind

Educationally, Australia has adopted the “no child left behind” strategies of the US educational system in order to find a solution to the declining educational standards of Australians. What about physically, psychologically and emotionally? Is it OK to leave some children behind the rest of the world in those stakes? So what do we know about what underpins functionality in any of these human capabilities? Genetics of course. Is it acceptable to leave our children behind in the genetic stakes?

As you are aware, a proposal to sequence the gene (a mutation to the dystrophin gene causes DMD) and archive all children in Australia with Duchenne muscular dystrophy, is about to be considered by our health leaders. It is a proposal to leave no child in Australia behind from the start of 2010. And we are not talking merely about improving the degree of physical impairment for each child affected by the most common catastrophic childhood disorder only – we are talking about enhancing the cognitive, social and emotional potential of those children too. More importantly it's about our society hanging onto that potential and not losing it, and in the bargain saving the social cost of early medical interventions, hospitalizations and familial grief as those children blossom rather than wither. It is a proposal that puts all Australians in all states on equal footing and it puts Australian children on equal footing with many other technologically advanced nations – equal footing for the chance of strength through participation in actual human trials.

The [attached media release](#) embargoed for release August 31st 2009 (last week) demonstrates that our national registry proposal is “spot on” and in synch with what is happening on the world medical stage. This announcement explains that a Phase II Stage III clinical trial is due to commence early in 2010. It states that one company recruiting candidates will continue to rely on the efficiency of the TREAT-NMD global database to identify children and by default approve trial centres adjacent to them. More recently [AVI-Biopharma](#) and [Santhura](#) have issued similar press releases seeking large scale recruitment. Both children and doctors in these countries will be able to develop in themselves or in their knowledge of how to care for others – but if an Australian national registry is not up and running, our children WILL be left behind. You see, our registry proposal includes the melding of the national registry with this important global registry.

Consider these statements contained in the media release:

Prosensa recently completed a phase I/II clinical trial for PRO-051, its lead compound for the treatment of Duchenne Muscular Dystrophy (DMD), and the company anticipates starting a phase II/III clinical study early next year. PRO-051 is directed to a specific mutation in the dystrophin gene that occurs in approximately 13% of the DMD patient population. In order to set up the pivotal study, patients with a genetic mutation amenable to exon skipping by PRO-051 need to be selected.

TREAT-NMD has developed a global database that contains precise genetic and clinical information from patients with DMD, including age, ambulation status and medication use. Using the TREAT-NMD Global Database for DMD, Prosensa has identified around 300 patients from 21 countries who meet the inclusion criteria for the upcoming trial. The TREAT-NMD database holds up-to-date information about all these patients that will allow them to be contacted for trial recruitment purposes through the national registries. These patients were matched to 50 potential trial sites and selected patients and sites will be contacted for participation in the upcoming clinical trial.

If we are serious about leaving no child behind, in adopting a new human rights declaration, and in restoring our reputation as one of the most egalitarian societies in the world, we will adopt the proposal of the Duchenne national registry working party and actualize a centralized database for persons affected by Duchenne. If we care about our medical and scientific standing in the world, we will make Australia the 22nd country to feed data into the global registry. Our scientists are already sharing their intellectual property with overseas clinicians and scientists engaged in translational research– it would be anathema if their own countrymen were not permitted to reap the rewards of their contributions. It would be a greater indictment if our health leaders did not assist the youngest and most powerless in society despite the fact that in the 0-30yr age bracket, the incidence of Duchenne is at least three times the incidence of prostate, testicular or breast cancer! How will we bear the shame of not only being insular on the world stage but also ageist in our health priorities?

Let 2010 be the year that no child is left behind medically in Australia for the promise of treatment. Australia is a multi-racial society and Duchenne, as a genetic disorder affects all races equally unlike Cystic Fibrosis or Tay Sachs disease. Duchenne reinforces the health disparity with indigenous Australians because indigenous boys with Duchenne always present severely and with much less longevity. In addition, the incidence of DMD is not waning with genetic counselling – the rate of spontaneous mutation therefore must be increasing. Our children now occupy the sanatoriums previously so well appointed for polio, for a reason – we are the most common genetic disorder resulting in severe physical impairment.

We are asking that you consider the facts outlined in this [media release](#) and Australia's role on the world stage - that you and your government endorse the recommendations of Australia's top ranking geneticists and leave no child behind.