MEDIA ALERT

New hope for boys with muscular dystrophy

Professionals will come together this Friday and Saturday at the ‘Towards a Brighter Future’ conference where they will call for a national register to be developed for children with rare diseases. This news comes ahead of Rare Diseases Day on Sunday 28 February.

A national register of people with rare diseases will provide experts from around the world with the capacity to further share their knowledge providing the best treatment for patients. It will provide experts with an opportunity to link up with other countries that have national registers to provide an even brighter future for those living with a rare disease.

This news comes following a recent announcement that there is now hope for boys affected by the devastating and lethal neuromuscular disorder Duchenne Muscular Dystrophy (DMD). Progressive muscle weakness means affected boys end up in a wheelchair then have difficulty breathing and swallowing, before dying in their late teens or early twenties.

“For the first time therapies are becoming available that aim to correct the genetic defect that causes muscular dystrophy” said Professor Kathryn North, Head of the Institute for Neuroscience and Muscle Research.

“Major advances in the field are starting to have significant impact on the lives of many children and their families.”

Internationally renowned speakers and local experts from around Australia will present current laboratory and clinical research to 300 national and international participants at the ‘Towards a Brighter Future’ Conference on 26 & 27 February.

Researchers, health professionals and families affected by neuromuscular disorders will come together for the conference, co-hosted by The Duchenne Foundation and the Institute for Neuroscience and Muscle Research (INMR), part of the Kids Research Institute at The Children’s Hospital at Westmead.

Neuromuscular disorders are one of the major causes of ongoing disability in childhood. Duchenne muscular dystrophy (DMD), a genetic disease, is the most common and most severe form of muscular dystrophy, affecting approximately 1 in 3500 live male births.

The multi-disciplinary approach to care and treatment involve a range of health professionals, including clinicians, physiotherapists, occupational therapists, genetic counsellors and psychologists.

“Access Economics has determined that while the incidence of muscular dystrophies is lower than cancer, the burden of cost on society is far greater” said Professor North.

“10 years ago, no information relating to the pathogenesis of disease was known, and the best care that could be offered was managing the patient's physical and emotional wellbeing.”

“Now there is hope. For the first time, experimental medications such as Deflazacort and PTC124 are available to the children of NSW. Our goal is to improve quality of life, improve mobility for longer and increase survival”

The focus of the conference is translational research – seeking scientific answers for questions observed in the clinical setting to ensure children in Australia receive the best possible care.

The conference will highlight the commitment to medical research in Australia that will underpin potential collaborations and participation in international clinical trials programs. This will result in a brighter future for boys with muscular dystrophy.

The Towards a Brighter Future Conference will be held:
VENUE: Eastern Avenue Auditorium University of Sydney
TIME: 8am-5pm 26 and 27 Feb 2010
www.towardsabrighterfuture.org.au