

## ASX Announcement

17 February 2023

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### **Clinical Trial Presentation at the Duchenne Parent Project aps XX International Conference on Duchenne and Becker Muscular Dystrophy**

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY | FSE:AWY] today announced that an overview of its ATL1102 Phase IIb Duchenne muscular dystrophy trial will be presented at the XX International Conference organised by Duchenne Parent Project aps Italy taking place from 17 to 19 February 2023 in Rome.

During the conference in each session there will be Italian clinicians who will present the overviews with updates of the different clinical studies currently being conducted within a therapeutic strategy including the ATL1102 trial update. The update for the Phase IIb study will be included in the session entitled "Improving muscle health, opposing inflammation and fibrosis" on Saturday 18 February 2023 at 11:30am (CET). The overview slides can be seen [here](#).

Details for the event can be found via the following links.

<https://parentproject.it/2023/02/06/dal-17-al-19-febbraio-a-roma-la-conferenza-internazionale-di-parent-project/>

Ms Annabell Leske, Antisense Therapeutics' Clinical Operations Manager will be meeting with groups in Europe who are providing the clinical trial support services (e.g. central laboratory analysis) for the Phase IIb trial. Annabell will also be meeting with clinical trial investigators as part of the preparation for the Phase IIb trial initiation and attending the Duchenne conference in Italy to meet with the clinicians and other members of the Italian DMD community and to answer questions following the ATL1102 Phase IIb trial update presentation.

#### **About Duchenne Parent Projects aps:**

Duchenne Parent Project aps (Italy) is one of the 50 member organisations of the World Duchenne Organisation. They are an association of patients and parents with children with Duchenne and Becker muscular dystrophy. Since 1996 they have been working to improve the treatment, quality of life and long-term prospects of children and young people through research, education, training and awareness. <https://parentproject.it/>

#### **For more information please contact:**

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*This announcement has been authorised for release by the CEO.*

**About Antisense Therapeutics Limited** [ASX: ANP | US OTC: ATHJY | FSE: AWY] is an Australian publicly listed biotechnology company, developing and commercializing antisense pharmaceuticals for large unmet markets in rare diseases. The products are in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ: IONS), an established leader in antisense drug development. The Company is developing ATL1102, an antisense inhibitor of the CD49d receptor, for Duchenne muscular dystrophy (DMD) patients and reported highly promising Phase II trial results. ATL1102 has also successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with relapsing-remitting multiple sclerosis (RRMS). The Company has a second drug, ATL1103 designed to block GHR production that successfully reduced blood IGF-I levels in Phase II clinical trials in patients with the growth disorder acromegaly.

**About ATL1102** ATL1102 is an antisense inhibitor of CD49d, a subunit of VLA-4 (Very Late Antigen-4). Antisense inhibition of VLA-4 expression has demonstrated activity in a number of animal models of inflammatory disease including asthma and MS with the MS animal data having been published in a peer reviewed scientific journal. ATL1102 was shown to be highly effective in reducing MS lesions in a Phase IIa clinical trial in patients with RRMS. The ATL1102 Phase IIa clinical data has been published in the medical Journal *Neurology* (Limmroth, V. et al *Neurology*, 2014; 83(20): 1780-1788).

**About DMD** Duchenne Muscular Dystrophy (DMD) is an X-linked disease that affects 1 in 3600 to 6000 live male births (Bushby *et al*, 2010). DMD occurs as a result of mutations in the dystrophin gene which causes a substantial reduction in or absence of the dystrophin protein. Children with DMD have dystrophin deficient muscles and are susceptible to contraction induced injury to muscle that triggers the immune system which exacerbates muscle damage as summarized in a publication co-authored by the Director of the FDA CDER (Rosenberg et al, 2015). Ongoing deterioration in muscle strength affects lower limbs leading to impaired mobility, and also affects upper limbs, leading to further loss of function and self-care ability. The need for wheelchair use can occur in early teenage years for patients on corticosteroids with a mean age of 13, with respiratory, cardiac, cognitive dysfunction also emerging. Patients with a greater number of immune T cells expressing high levels of CD49d have more severe and progressive disease and are non-ambulant by the age of 10 despite being on corticosteroid treatment (Pinto Mariz et al, 2015). With no intervention, the mean age of life is approximately 19 years. The management of the inflammation associated with DMD is currently addressed via the use of corticosteroids, however they are acknowledged as providing insufficient efficacy and are associated with significant side effects. As a consequence, there is an acknowledged high need for new therapeutic approaches for the treatment of inflammation associated with DMD.

*Rosenberg AS, Puig M, Nagaraju K, et al. Immune-mediated pathology in Duchenne muscular dystrophy. Sci Transl Med 2015, 7: 299rv4.*

*Bushby et al for the DMD Care Consideration Working Group/ Diagnosis and management of Duchenne muscular dystrophy, part 1 Lancet Neurol. 2010 Jan;9(1):77-93 and part 2 Lancet Neurol. 2010 Feb;9(2):177-89 .*

*Pinto-Mariz F, Carvalho LR, Araújo AQC, et al. CD49d is a disease progression biomarker and a potential target for immunotherapy in Duchenne muscular dystrophy. Skeletal Muscle 2015, 5: 45-55.*