

Phase II clinical trial in Duchenne Muscular Dystrophy – Update

- Trial is now over 50% enrolled
- No Serious Adverse Events reported to date
- Patient enrolment is on track for dosing completion in 3Q'2019
- Trial details included on prominent US based DMD advocacy group website

Antisense Therapeutics ("ANP" or the "Company") is pleased to advise that 5 patients are now enrolled in the 9 patient Phase II clinical trial of its immunomodulatory therapy, ATL1102 for Duchenne Muscular Dystrophy (DMD).

Four patients are currently being dosed with ATL1102 in the trial with a 5th screened patient having met the eligibility criteria for the trial and with their dosing scheduled to commence early February. No serious adverse safety related events have been reported to date.

The open label six-month dosing trial of ATL1102 in 9 non-ambulant patients with DMD aged between 10 and 18 years is being conducted at the neuromuscular centre of the Royal Children's Hospital in Melbourne (RCH), which operates the largest clinic in the southern hemisphere treating children with DMD.

ATL1102 is an inhibitor of CD49d expression on certain immune cells (T cells). It has been reported in scientific literature* that patients with DMD who have a greater number of T cells with high levels of CD49d on their surface have more severe and rapid disease progression. ATL1102 is being developed as a novel treatment for the inflammation that exacerbates muscle fibre damage in DMD patients, currently treated with corticosteroids. Corticosteroids have a range of serious side effects when used for a prolonged period as required in DMD.

The primary endpoints of the trial relate to the safety and tolerability of ATL1102. The efficacy of ATL1102 will also be assessed in terms of its effects on markers of inflammation, muscle damage and disease progression.

The trial is now over 50% enrolled and is on track for all 9 patients to have been enrolled and their dosing completed in 3'Q'19 with trial results to follow. Being an open label study, there is a possibility for earlier study read outs on preliminary data in a sufficient number of patients.

Parent Project Muscular Dystrophy - ATL1102 for DMD trial listing

US based DMD advocacy group Parent Project Muscular Dystrophy (PPMD), whose advocacy efforts have secured hundreds of millions of dollars in funding and helped win two FDA approvals, have recently incorporated details on the ATL1102 for DMD trial on their website:

<https://www.parentprojectmd.org/faqs/atl1102-cd49d-antisense-oligonucleotide>

PPMD's advocacy efforts are focused on advancing care and treatments for DMD by leveraging US federal resources, building partnerships, and creating regulatory procedures and infrastructure. Since their advocacy efforts began in 2000, PPMD has invested over US\$50 million into DMD research and therapy development and helped to leverage over US\$800 million in federal funding into muscular dystrophy research, with over US\$500 million specifically for DMD.

Mark Diamond, CEO of Antisense Therapeutics said: "We are very pleased to see the ATL1102 listing by PPMD, the most prominent US DMD advocacy group, as it significantly increases visibility and awareness of ANP's DMD clinical development activities in the US, the world's largest pharmaceutical market."

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About Antisense Therapeutics Limited (ASX: ANP) is an Australian publicly listed biopharmaceutical company, developing and commercialising antisense pharmaceuticals for large unmet markets. The products are in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ:IONS), world leaders in antisense drug development and commercialisation. ATL1102 (injection) has successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with relapsing-remitting multiple sclerosis (RRMS). ATL1103 drug designed to block GHr production successfully reduced blood IGF-I levels in Phase II clinical trials in patients with the growth disorder acromegaly. The Company is conducting a Phase II clinical trial of ATL1102 in DMD patients at the Royal Childrens Hospital, Melbourne.

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 RR-MS patients. 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 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 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 wheelchair bound by the age of 10 (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.

*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