ASX Announcement

4 March 2020

ATL1102 Phase II DMD trial data to be presented at Duchenne ACTT Now Conference

- Dr. Ian Woodcock, Principal Investigator of the ATL1102 Phase II DMD trial will present an overview of the positive trial results in his talk on targeting inflammation in Duchenne Muscular Dystrophy

Antisense Therapeutics Limited ("ANP" or "the Company") will attend and present at the Duchenne ACTT Now Conference 2020 being held in Melbourne, at the Royal Children's Hospital 8-10 March 2020.

The Duchenne ACTT Conference organised by the Save Our Sons Duchenne Foundation is aimed at the families and those living with Duchenne Muscular Dystrophy (DMD), and the wider community including clinicians, nurses and allied health, alongside researchers, industry and those involved in the regulatory process for new treatments including representatives from the Health Products Regulation Group, Australian Government Department of Health. With both national and international speakers, the conference will cover many topics including A – Advocacy, C - Clinical care, T – Trials and T – Therapies in DMD. Additional information can be found at Save Our Sons Duchenne Foundation: https://www.saveoursons.org.au

Dr. Ian Woodcock, will be speaking at the conference on targeting inflammation in DMD, where as part of this presentation he will provide an overview of the ATL1102 Phase II trial results following successful completion of dosing in all patients, which affirmed ATL1102's excellent safety profile and positive drug effects on disease progression endpoints as reported by ANP in December 2019.

ANP will also present on the ATL1102 DMD program at a Company Research Update session on the Tuesday along with other international pharmaceutical company sponsors of the conference.

Authorised for release by the CEO.

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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 Children's 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 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 wheelchair bound 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.