ASX Announcement

6th September 2018

Duchenne ACTT Now Conference 2018

Antisense Therapeutics Limited ("ANP" or "the Company") today announced that the Company will attend and present at the Duchenne ACTT Now Conference 2018 being held in Sydney on 6 - 7 September 2018. The conference will address: A – Advocacy, C - Clinical care, T – Trials and T – Therapies in Duchenne Muscular Dystrophy (DMD).

The Conference is held by the Save our Sons Duchenne Foundation and Australasian Neuromuscular Network (ANN) and is aimed at the Duchenne community, those living with Duchenne, their families and all of the professionals involved in caring for those with DMD as well as researchers and global pharmaceutical company representatives.

Save our Sons Duchenne Foundation is Australia's peak body for Duchenne, fighting to find a cure. It was founded in 2008 by Elie and Nancy Eid a few years after their son Emilio's diagnosis.

Australasian Neuromuscular Network has over 300 members linking clinicians, pathologists, counsellors, researchers, allied health professionals and advocacy groups for the benefit of individuals affected by neuromuscular disorders. The ANN's goal is to ensure excellence in clinical management and access to clinical trials and new therapies. Professor Monique Ryan, a clinical investigator in ANP's ATL1102 DMD trial, is a member of the ANN executive committee and the clinical care and clinical trials steering groups.

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Save Our Sons Duchenne Foundation
Website: https://www.saveoursons.org.au/

Australasian Neuromuscular Network
Website: http://www.ann.org.au/

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. https://mdaustralia.org.au.

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