ASX Announcement

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First patient dosed in Phase II clinical trial in Duchenne Muscular Dystrophy

Antisense Therapeutics ("ANP" or the "Company") today announced dosing of the first patient in a Phase II clinical trial of its immunomodulatory therapy, ATL1102, in patients with Duchenne Muscular Dystrophy (DMD).

Commencement of the trial represents an important development milestone for the Company and for patients seeking better and safer treatments.

ATL1102 is an inhibitor of CD49d expression on certain immune cells (T cells). DMD patients 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 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, and do not appear to be as effective in patients with a greater number T cells that express high levels of CD49d. ATL1102 was previously shown to be a highly active drug with potent effects in reducing inflammatory processes in Multiple Sclerosis patients.

The 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. The Investigators for the study are Dr Ian Woodcock, a Neuromuscular Fellow at the RCH and Professor Monique Ryan, Director of the Neurology Department at RCH.

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. Further details on the trial are available here on the Australian New Zealand Clinical Trials Registry.

Currently there are a limited number of anti-inflammatory drugs in clinical development for DMD and ATL1102 is the only CD49d targeting drug undergoing clinical trials in DMD patients.

Mark Diamond, CEO of Antisense Therapeutics said: "We are very pleased to report that patient recruitment and dosing are now underway being a key inflection point in the Company's progress. We look forward to trial data becoming available and providing further market updates on trial progress. Achievement of this important milestone positions us as a clinical stage company in DMD committed to the pursuit of an effective treatment for a devastating disease that is desperately under-served by existing therapies".

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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. 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