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ATL1102 for Multiple Sclerosis and Duchenne Muscular Dystrophy Update

Antisense Therapeutics Limited ("ANP" or "the Company") provides the following update on the progress of its Multiple Sclerosis and Duchenne Muscular Dystrophy programs.

Multiple Sclerosis Program

Following the FDA's partial clearance of the Company's proposed Phase IIb clinical study of ATL1102 for Multiple Sclerosis (MS), ANP's US regulatory advisors lodged a submission on behalf of the Company for a meeting request with the US Food and Drug Administration (FDA) to clarify criteria under which MS patients could receive higher doses of ATL1102 in the Phase IIb study. The FDA has subsequently advised that the most efficient path forward for the Company is to submit a complete written response to the partial hold outlining ANP's proposed changes and inclusion of additional safety related parameters to the Phase IIb clinical study protocol that would potentially allow for the administration of higher doses. The Company anticipates submitting its complete response early in the New Year. The FDA would then have 30 calendar days from submission to respond to the letter.

Duchenne Muscular Dystrophy (DMD) Program

As previously advised, ANP is planning to conduct a clinical trial of ATL1102 in DMD patients at the Royal Children's Hospital (RCH) in Melbourne.

Through consultations with the Principal Investigators of the trial, who are clinical experts in DMD, and their interactions with the RCH, the Company is now proposing an important change to the clinical trial protocol - to extend the dosing duration of the study from 8 weeks (as per the currently submitted dose escalation study protocol) to 6 months.

The extension of the dosing period may allow for ATL1102 to show an improvement in key clinical endpoints that are relevant to DMD disease progression (e.g. the upper limb function of the boys), generally only observable in longer term studies, and of the type that would be required for future product registration. Dose modelling predictions of the use of ATL1102 in a 6 month MS study (Guzy & Bauer)** lend support to the potential activity of this intended dosing regimen for the DMD trial. This would be an initial single dose study of 25mg of ATL1102 per week in wheel chair bound boys weighing between 30 to 60kg.

This new trial protocol is in preparation for submission to the Human Research Ethics Committee of the RCH for review at their next meeting on 5 February 2018. Further details on the trial will be communicated in news regarding trial approval. Notably, the change to a single dose administered over 6 months will have no material impact on the study cost or timeline estimates compared to the 2 month escalating dose study design.

As previously advised ANP is assessing options to access additional capital to fund the value adding activities described above. The Company continues to investigate and apply for non-dilutive grant funding opportunities extending now to the new DMD program. Market interest in new DMD treatments is very high. Accordingly the Company anticipates pharmaceutical company partnering interest as further clinical development advancements are made in our DMD program.

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Antisense Therapeutics Limited (ASX: ANP) is an Australian publicly listed biopharmaceutical drug discovery and development company. Its mission is to create, develop and commercialise second generation antisense pharmaceuticals for large unmet markets. The products in ANP's development pipeline are in-licensed from Ionis Pharmaceuticals Inc., 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.

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)[1].

** Guzy S, Bauer R. Pharmacometrics in drug development: concepts and applications. In: Faltin FW, Kenett RS, Ruggeri F, eds. Statistical Methods in Healthcare. Chichester, UK: John Wiley & Sons; 2012:56–77.

About Multiple Sclerosis (**MS**) MS is a life-long, chronic disease that progressively destroys the central nervous system (CNS). It affects approximately 400,000 people in North America and more than 2 million worldwide. It is a disease that affects more women than men, with onset typically occurring between 20 and 40 years of age. Symptoms of MS may include vision problems, loss of balance, numbness, difficulty walking and paralysis. In Australia MS affects over 20,000 people. *Relapsing-Remitting MS (RR-MS):* People with this type of MS experience clearly defined attacks of worsening neurologic function. These attacks—which are called relapse or exacerbations —are followed by partial or complete recovery periods (remissions), during which no disease progression occurs. Approximately 85% of people are initially diagnosed with relapsing-remitting MS. *Secondary-Progressive MS (SP-MS)* occurs when after an initial period of relapsing-remitting MS, many people develop a secondary-progressive disease course in which the disease worsens more steadily, with or without occasional flare-ups, minor recoveries (remissions), or plateaus. Before the disease-modifying medications became available, approximately 50% of people with relapsing-remitting MS developed this form of the disease within 10 years. The market for drugs treating RR-MS has been valued at more than USD$20 billion. There are limited treatment options for SP-MS patients. The market potential for SP-MS treatments has been estimated at US$7billion.

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 (Pinto Mariz, 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 respiratory, cardiac, cognitive dysfunction also emerging. 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/