12 September 2017

ATL1102 for Multiple Sclerosis and Duchenne Muscular Dystrophy Update

Antisense Therapeutics ("ANP" or the "Company") wishes to advise that it has submitted its formal response to the US Food and Drug Administration (FDA) in regard to the ATL1102 for Multiple Sclerosis (MS) Phase IIb IND application to address the points specified by the FDA in their clinical hold letter. The FDA has 30 calendar days to review and potentially clear the IND with a response expected on or before 30 September 2017.

In parallel with the FDA process above, the Company's application to conduct a clinical trial of ATL1102 in patients with Duchenne Muscular Dystrophy (DMD) at the Royal Children's Hospital (RCH) in Melbourne has progressed with Company receiving notification that all Drug Trial Subcommittee issues have been resolved. The Human Research Ethics Committee (HREC) has advised the Company that given the on-going nature of the current FDA IND process, it will require clearance of the ATL1102 for Multiple Sclerosis (MS) Phase IIb IND by the FDA for the HREC to approve the DMD trial. Should the IND application be cleared, the Company expects the DMD trial approval to be received shortly thereafter.

As previously announced on 26 June 2017, Australian Ethical Investment participation in the proposed Capital Raising had been agreed with the issue of shares conditional on the Company receiving hospital approval by 30 September 2017 to commence the clinical trial for ATL1102 in DMD. The Company will update the market on the details of the Capital Raising prior to or around 30 September 2017.

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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).

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.