

21 November 2017

ATL1102 for Multiple Sclerosis and Duchenne Muscular Dystrophy Status Update

Antisense Therapeutics (“ANP” or the “Company”) wishes to advise that its regulatory advisors have 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 under the Company’s proposed Phase IIb clinical study of ATL1102 for Multiple Sclerosis (MS). Further details will be available following receipt of the FDA’s response.

With regard to the Company’s plans to conduct a clinical trial of ATL1102 in patients with Duchenne Muscular Dystrophy (DMD), ANP is yet to receive a response from the Human Research Ethics Committee at the Royal Children’s Hospital (RCH) in Melbourne on the approval status of its trial application that now incorporates an updated trial protocol with inclusion of a “lead in” lower starting dose arm.

The Company expects to update the market as additional information becomes available.

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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.

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/>