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## ATL1102 for Multiple Sclerosis Phase IIb trial Update

Antisense Therapeutics ("ANP" or the "Company") wishes to advise that the Company has been in recent communications with the US Food and Drug Administration (FDA) in regard to the ATL1102 for Multiple Sclerosis (MS) Phase IIb IND application. The FDA has told ANP that modifications to the proposed clinical trial are needed in order for FDA to clear the IND to proceed. In a recent teleconference with ANP, FDA provided a high-level description of the necessary modifications and will provide actionable details in a formal written response to ANP by late August 2017. During this period of clinical hold ANP will formally submit updates to the IND soon after receipt of FDA's written response and the FDA has 30 calendar days to review and potentially clear the IND.

In parallel, the Company is progressing its grant application with a US Federal Agency, the National Institute of Neurological Disorders and Stroke (NINDS), part of the National Institutes for Health (NIH) where ANP's clinical study synopsis for the Phase IIb study passed through two levels of feasibility assessment. The Company now plans to modify the proposed study design to align with both the anticipated FDA requirements noted above and feedback on the trial received via NINDS interactions. The next step will then be submission to the NINDS Extramural Science Committee (ESC) for review and potential approval to move forward to lodging of the full grant application.

The Company advises that its application to conduct a clinical trial of ATL1102 in patients with Duchenne Muscular Dystrophy (DMD) at the Royal Children's Hospital in Melbourne is ongoing. The Company is currently addressing questions received in line with the hospital's approval process.

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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 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 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)