

ASX Announcement6 February 2020

Positive results in DMD unlock the broader value creation potential of ATL1102

Following the recently reported positive clinical trial results in the Phase II clinical trial of ATL1102 in Duchenne Muscular Dystrophy (DMD) that affirmed the safety and immunomodulatory activity of the drug on CD49d T cells in the blood with clinical benefits on muscle strength and function, in parallel with progressing plans for the Phase IIb trial in DMD, the Company is now actively exploring clinical development opportunities in other indications where inflammation plays a key role in disease progression.

ATL1102 is an antisense inhibitor of CD49d, a subunit of VLA-4 (Very Late Antigen-4). Antisense inhibition of CD49d expression has demonstrated activity in a number of animal models of inflammatory disease including asthma, arthritis and multiple sclerosis (MS). ATL1102 was previously shown to be highly effective in reducing MS inflammatory brain lesions in a Phase IIa clinical trial in Relapsing Remitting -MS patients. The ATL1102 Phase IIa clinical data has been published in the medical Journal Neurology (Limmroth, V. et al Neurology). Further, the Company has an Investigational New Drug application (IND) with the US Food and Drug Administration (FDA) clearing ATL1102 for use in a Phase IIb clinical trial in MS patients at the same 25mg per week dose that has shown activity in the DMD trial. MS drug sales in 2018 were US\$23 Billion and forecast to grow to US\$39 Billion by 2026.

The Company is consulting with clinical experts on the appropriate next steps for clinical development in MS while also re-engaging with pharmaceutical companies active in the MS space to discuss partnering opportunities. The Company is following up potential sources for non-dilutive grant funding for a Phase IIb clinical trial of ATL1102 in MS patients.

The Company has continued to file new patent applications to protect the use of ATL1102. Recently international patent application PCT/AU 2018/050598 titled 'Methods for treating multiple sclerosis using antisense oligonucleotides' advanced to the national phase in the US, Australia, New Zealand, Canada and Europe. When granted this patent family would provide protection for the use of ATL1102 in MS until 2038, potentially extendible for a further 5 years in the US, Australia and Europe.

In addition to MS, the Company sees exciting potential for ATL1102's use in other neuroinflammatory and muscular dystrophy disorders given the expected antisense platform and CD49d target based advantages in these applications. In 2019 ANP filed a patent applications to support clinical development and commercialisation of ATL1102 in muscular dystrophies in addition to DMD (PCT/AU2018/051353 & US16/404561) and will continue to file new patents to broaden IP protection and add further commercial value to the ATL1102 asset while expanding the Company's product pipeline.

Recent presentations at conferences in the US have included technical updates and development progress of the Company's projects. These presentations have stimulated interest from parties with potential strategic interest in ANP's projects.

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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, arthritis 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. There are limited treatment options for SP-MS patients. The sales for MS treatments in 2018 were US\$23 billion.