ASX Announcement

20 September 2021

New ATL1102 proteomics data to be presented in a Late Breaking News Abstract at the World Muscle Society 2021 Virtual Congress

- **E-poster presentation titled "ATL1102 treatment in non-ambulant boys with DMD modulates latent TGF-beta-binding protein 4, and thrombospondin-1, two disease genetic modifiers of ambulant DMD, and CXCL16"**
- **The new data to be announced on Friday 24 September 2021**

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY | FSE:AWY] today announced that new ATL1102 in DMD data is scheduled to be presented in a poster presentation at the 26th World Muscle Society (WMS) Virtual Annual Congress 2021, being held from 20 September 2021 – 24 September 2021, British Summer Time (BST).

World Muscle Society is an international multidisciplinary scientific society dedicated to the advancement and dissemination of knowledge in the neuromuscular field for the benefit of patients https://www.worldmusclesociety.org. This year the 26th WMS Congress is being held in a virtual format. The event will consist of scientific sessions, e-poster presentations, virtual exhibition and virtual networking through a virtual congress venue. Scientific content and e-posters will be available on demand for 3 months to registered attendees to continue viewing.

The Late Breaking Abstracts aspect of the congress is specifically designed to present the most relevant, exciting, unpublished and recent news regarding every aspect of neuromuscular disorders.

The e-poster presentation titled: **"ATL1102 treatment in non-ambulant boys with DMD modulates latent TGF-beta-binding protein 4, and thrombospondin-1, two disease genetic modifier of ambulant DMD, and CXCL16"** G. Tachas; C. Mueller; R.K DeLisle; I.R Woodcock; M.M Ryan; N. Desem; will be presented on Thursday 23 September 2021 16.30 – 18.30pm BST.

The Company will lodge an ASX announcement on the new data on Friday 24 September 2021 AEST.

This announcement has been authorised for release by the Board.

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About Antisense Therapeutics Limited [ASX: ANP | US OTC: ATHJY | FSE: AWY] is an Australian publicly listed biotechnology company, developing and commercializing antisense pharmaceuticals for large unmet markets in rare diseases. The products are in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ: IONS), an established leader in antisense drug development. The Company is developing ATL1102, an antisense inhibitor of the CD49d receptor, for Duchenne muscular dystrophy (DMD) patients and recently reported highly promising Phase II trial results. ATL1102 has also successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with relapsing-remitting multiple sclerosis (RRMS). The Company has a second drug, ATL1103 designed to block GHr production that 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 patients with RR-MS. 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 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 a substantial 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 as summarized in a publication co-authored by the Director of the FDA CDER (Rosenberg et al, 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 for patients on corticosteroids with a mean age of 13, with respiratory, cardiac, cognitive dysfunction also emerging. Patients with a greater number of immune T cells expressing high levels of CD49d have more severe and progressive disease and are non-ambulant by the age of 10 despite being on corticosteroid treatment (Pinto Mariz et al, 2015). 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.

Rosenberg AS, Puig M, Nagaraju K, *et al*. Immune-mediated pathology in Duchenne muscular dystrophy. *Sci Transl Med* 2015, 7: 299rv4.

Bushby et al for the DMD Care Consideration Working Group/ *Diagnosis and management of Duchenne muscular dystrophy, part 1* Lancet Neurol. **2010** Jan;9(1):77-93 *and part 2* Lancet Neurol. **2010** Feb;9(2):177-89 .

Pinto-Mariz F, Carvalho LR, Araújo AQC, *et al*. CD49d is a disease progression biomarker and a potential target for immunotherapy in Duchenne muscular dystrophy. *Skeletal Muscle* 2015, 5: 45-55.