ASX Announcement

29 September 2020

New ATL1102 data to be presented at the 25th International Annual Congress of the World Muscle Society

- **Poster presentation on ATL1102 improvement of PUL2.0 in non-ambulant boys with DMD compared to a natural history control;**
- **Investor webinar presentation 9AM Friday 2 October 2020**

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY] is pleased to advise that new ATL1102 in DMD data is scheduled to be presented in a poster presentation at the World Muscle Society (WMS) Virtual Congress 2020, being held from 28 September 2020 – 2 October 2020, 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 25th WMS Congress is being held in a virtual format due to COVID-19. Over 400 abstracts have been submitted on all aspects of neuromuscular disease. The event will consist of panel sessions, oral lectures and poster presentations through a virtual conference centre, which will then be available on demand for 3 months to registered attendees to continue viewing.

The poster presentation titled: **"ATL1102 treatment improves PUL2.0 in non-ambulant boys with Duchenne muscular dystrophy compared to a natural history control"** G. Tachas; N. Desem; P. Button; G. Coratti; M. Pane; E. Mercuri; will be presented at Poster session 4, 17.30 – 19.30pm BST on Thursday 1 October.

The Company will lodge an announcement on the new data on Friday 2 October 2020 and conduct an investor webinar at 9AM (AEST) that day | UK 12AM | US PDT 4:00PM, EDT 7.00PM (Thursday 1 October).

To access the webinar and for details of how to register online please follow this link:

https://us02web.zoom.us/webinar/register/WN_JPRXpRipQ2uzjHlFxRNqVA

This announcement has been authorised for release by the Board.

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About Antisense Therapeutics Limited (ASX:ANP | US OTC:ATHJY) 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. ATL1102 has also shown to be very effective in reducing inflammatory brain lesions in a patients with MS (Limmroth, V. et al Neurology, 2014; 83(20): 1780-1788) and recently delivered highly promising clinical results in patients with Duchenne muscular dystrophy (DMD) a rare and fatal muscle wasting disease where inflammation in the muscle leads to fibrosis and death of muscle tissue. For more details please refer to the Company's announcements available on the Antisense Therapeutics website https://www.antisense.com.au/asx-announcements/.

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.