ASX Announcement

18 March 2020

ATL1102 Phase II DMD trial database lock and final results on track

- **New data on additional disease progression parameters pending final analysis**

Antisense Therapeutics Limited ("ANP" or "the Company") is pleased to advise that following the recent completion of the monitoring phase of the ATL1102 Phase II Duchenne Muscular Dystrophy (DMD) trial, the locking of the clinical trial database for final analysis is on track for the end of March 2020 with study results to be reported in April.

The Company has been conducting an open label six-month dosing trial of ATL1102 in nine non-ambulant patients with DMD at the neuromuscular centre of the Royal Children's Hospital in Melbourne.

In December 2019, following completion of dosing in all participants, ANP reported positive clinical trial results that affirmed the drug's excellent safety profile and positive effects on disease progression endpoints.

Notably, the Company expects to report on new trial data relating to the drug's effects (efficacy) on additional disease progression endpoints once final data analysis is completed in the coming weeks.

COVID-19
As the trial is essentially completed, COVID-19 factors that are causing significant challenges for the community at large are presently not adversely impacting on these final activities. The Company is positioned to accommodate measures that are prudent for us to take to safeguard the health of our staff, patients and the broader community and our staff can work from home if self-isolation measures are required.

Authorised for release by the Board.

For more information please contact:

Antisense Therapeutics
Mark Diamond
Managing Director & CEO
+61 (0)3 9827 8999
www.antisense.com.au

Investment Enquiries
Gennadi Koutchin
XEC Partners
gkoutchin@xecpartners.com.au
1300 932 037

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 Children's 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 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 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 wheelchair bound 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.