ASX Announcement

24 July2019

Phase II clinical trial in DMD on track for dosing completion

- Five patients have completed the 24 week treatment phase
- No Serious Adverse Events reported to date
- Dosing of remaining four patients on track to be completed early November 2019

Antisense Therapeutics ("ANP" or the "Company") is pleased to advise that five patients have completed their 24 weeks of dosing in the Phase II clinical trial of ANP's immunomodulatory therapy, ATL1102 for Duchenne Muscular Dystrophy (DMD). The remaining four patients are at various points within the treatment phase of the study.

No Serious Adverse Events (SAE's) have been reported to date. The Data Safety Monitoring Board have been periodically evaluating the safety related trial data and have on each occasion recommended continuation of the trial with no safety concerns. Dosing of all patients in the trial is to be completed in early November 2019.

The open label six-month dosing trial of ATL1102 in nine non-ambulant patients with DMD aged between 10 and 18 years is being conducted at the neuromuscular centre of the Royal Children's Hospital in Melbourne which operates the largest clinic in the southern hemisphere treating children with DMD.

ATL1102 is being developed as a novel treatment for the inflammation that exacerbates muscle fibre damage in DMD patients, currently treated with corticosteroids. Corticosteroids have a range of serious side effects when used for a prolonged period as required in DMD. Currently there are a limited number of anti-inflammatory drugs in clinical development for DMD.

The primary endpoints of the trial relate to the safety and tolerability of ATL1102. The efficacy of ATL1102 in DMD will also be assessed in terms of its effects on disease processes and progression (e.g. the upper limb strength and function of the boys).

The Company expects to report trial results shortly after the completion of dosing, though as previously advised, as the Phase II DMD clinical trial is an open label study there may be an opportunity for study non statistical read-outs on preliminary data prior to the completion of dosing in all patients. This would require a sufficient number of patients to have completed 24 weeks of dosing and for all patients to have passed at least the mid-point (12 week) dosing mark for the Company to be confident and certain of the robustness of such results for disclosure.

Mark Diamond, CEO of Antisense Therapeutics said: "We are pleased to have more than half of patients in the trial having completed dosing with no SAE's reported to date and to be on track for completion of the treatment phase of the trial in the next quarter".

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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 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.

Busby 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