ASX Announcement

2 September 2019

ATL1102 for DMD commencement of European regulatory interactions

Antisense Therapeutics ("ANP" or the "Company") has received confirmation of two of the three proposed Scientific Advice (SA) meetings for its planned interactions with regulatory authorities on the design and conduct of the next clinical trial of ATL1102 in DMD and on the development path for product registration as previously foreshadowed by the Company. The first meeting is confirmed for end of October with the second meeting scheduled for November and confirmation of the third meeting anticipated within the coming weeks.

The Company had previously received advice from international regulatory consultants that, based on the existing preclinical and clinical data generated in the development of ATL1102, the Company could look to seek approval to conduct a Phase IIb clinical trial of the drug in DMD patients in Europe with this regulatory process to run in parallel with the conduct of the current Phase II study of ATL1102 in DMD patients at the Royal Children's Hospital in Melbourne.

The focus of these Scientific Advice meetings will be on the Phase IIb trial design, dose escalation plans, applicability of the study end-points and the study duration. ANP expects to receive written responses within one month following each meeting.

Once national scientific advice is obtained the Company intends to seek advice from the European Medicines Agency (EMA) with the purpose of receiving the EMA's acceptance of the overall development program for ATL1102 in DMD, in particular the Phase IIb clinical study design and path for product registration.

Mark Diamond, CEO of Antisense Therapeutics said: "We are pleased to confirm this important step of the commencement of regulatory interactions necessary for efficient and timely advancement to the next stage of development of ATL1102 in DMD and we look forward to reporting on the progress of these interactions in due course'.

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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