ASX Announcement

24 August 2021

ANP to participate in PPMD development of new Community-Led Duchenne Guidance for FDA

- ANP to serve as a member of the Pharmaceutical Advisory Board for the DMD Guidance document

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY | FSE:AWY], (the Company) is pleased to advise that the Company's US-based Medical Director, Gil Price, MD, will serve as a member of the Pharmaceutical Advisory Board (PAB) for the development of the New Duchenne Guidance by Parent Project Muscular Dystrophy (PPMD) for the US FDA.

Working closely with the Steering Committee and Working Group Chairs, comprised of individuals representing the patient advocate, caregiver, clinician, researcher, academic, and pharmaceutical industry, the PAB will focus on ensuring perspectives from companies with an interest Duchenne community are represented throughout the guidance.

PPMD successfully developed the first-ever patient group initiated draft guidance for companies developing treatments for Duchenne. Submitted to the FDA in June 2014, the work was a key resource informing companies and FDA about the evolving drug development landscape for Duchenne muscular dystrophy (DMD), as well as the patient focused views of benefit expectations and risk tolerance of the community. PPMD initiative has since become a landmark not only in the Duchenne community, but across rare disease communities exemplifying the value patients and caregivers can bring to drug development.

PPMD has now begun the process for modernizing the landmark Community-Led Guidance of 2014 document to ensure it reflects many advancements in knowledge, understanding, care, clinical trials and approvals over the recent years. Similar to the 2014, PPMD has formed a Steering Committee and Working Groups of over 80 stakeholders. This will help drive even more innovation as well as carve a path toward the ultimate goal of accessible therapies for all patients.

PPMD is the largest most comprehensive non-profit organization in the United States focused on finding a cure for DMD - their mission is to end DMD (www.parentprojectmd.org). PPMD accelerates research, raises voices to impact policy, demands optimal care for every single family, and strives to ensure access to approved therapies. PPMD invest deeply in treatments for people affected by Duchenne and in research that will benefit future generations. PPMD advocate in Washington, DC, and have secured hundreds of millions of dollars in funding.

Antisense Therapeutics US-based Medical Director Dr Gil Price said, "We greatly appreciate the opportunity to contribute to this important document and so look forward to improving the lives of young men".

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.