ASX Announcement

8 February 2022

Dosing commenced in MCRI collaboration animal study

- Dosing has commenced in a first animal model of an inflammatory muscle disease
- Data from the first phase of the program due 2Q'CY22
- Combination with a dystrophin restoration drug study to commence Q2'CY22
- Combination study results due Q3'CY22

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY | FSE:AWY], today announced that dosing has commenced in an inflammatory muscle disease animal model under the previously advised collaborative research agreement with the Murdoch Children's Research Institute's (MCRI) to investigate the therapeutic potential of ATL1102 in a new muscle disease, where today there are no effective treatments. All animals successfully received their first dose of the antisense CD49d drug or control (oligonucleotide mismatch or saline) treatment.

ATL1102 has been shown to be clinically active in non-ambulant Duchenne muscular dystrophy (DMD) patients while antisense inhibition of CD49d has also demonstrated activity in a muscular dystrophy (*mdx*) mouse model of DMD, reducing both the CD49d target in the muscle and muscle damage. Having achieved positive results in the DMD *mdx* animal model, antisense inhibition of CD49d is now to be assessed in a mouse model of another inflammatory muscle disease, where there are similar immune mediated inflammatory features to the *mdx* model.

While the Company has filed patents to seek protection for the use of ATL1102 in this new indication, the Company has not named the indication as further important intellectual property protection could be generated through the successful conduct of the study program at the MCRI. ANP can, however, advise that the new inflammatory muscle disease indication being studied is a rare muscle disease that effects both children and adults with no effective marketed therapy, no disease modifying agents in advanced development and where ATL1102's observed immunomodulatory activity would be suggestive of potential treatment benefits.

Dosing has now commenced in the first phase (acute setting) of this development program where the antisense inhibition of CD49d target effects in the muscle will be assessed. Results from this first phase of the program are anticipated in 2Q'CY22. The second phase (chronic setting) of the program will study the drug effects over a longer dosing period in the animals where the antisense inhibition of CD49d target effects in reducing muscle damage, as determined by fat content in the muscle, will be assessed. Preventing increase in fat levels in the muscle is a key clinical goal for patients with this inflammatory muscle disease. Notably ANP has previously reported ATL1102's positive effect in stabilizing fat levels in the muscle of DMD patients. Data from the second chronic dosing phase is expected 2H'CY22.

Expanding ATL1102's application into this new indication would allow ANP to leverage established core competencies (for example rare disease experience, scientific partnerships and scientific collaborations e.g. MCRI, KOL's etc.) and the extensive non-clinical and clinical data generated on ATL1102 to deepen the Company's product pipeline with the potential for ANP to move rapidly into the clinic based on positive animal data or out-license.

As previously announced, the collaboration with the MCRI will also assess the potential of antisense inhibition of CD49d effects in the DMD *mdx* model in combination with a dystrophin restoration drug to improve therapeutic outcomes beyond that achieved by the single agent alone. This study is on track to commence 2Q'CY22 with results due Q3'CY22. Sales of the dystrophin restoration drugs in the US in 2021 were in excess of US$600m. Currently the dystrophin restoration drugs are used in combination with steroids and predominantly for the younger ambulant DMD population, but are yet to demonstrate in controlled studies to be effective in further delaying loss of ambulation beyond the use of steroids alone, which underlines the exciting opportunity for a new combination therapy that can deliver real benefits for DMD patients.

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. ATL1102 has also shown to be very effective in reducing inflammatory brain lesions in 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.

About MCRI. The Murdoch Children's Research Institute (MCRI) is the largest child health research institute in Australia committed to making discoveries and developing treatments to improve child and adolescent health in Australia and around the world. They are pioneering new treatments, trialing better vaccines and improving ways of diagnosing and helping sick babies, children and adolescents. It is one of the only research institutes in Australia to offer genetic testing to find answers for families of children with previously undiagnosed conditions.