ASX Announcement

28 June 2021

Antisense Therapeutics and Murdoch Children's Research Institute enter into new R&D collaboration to further explore ATL1102 use in multiple muscle diseases

- Strong strategic fit leveraging substantial ATL1102 pre-clinical & clinical data package
- Additional positive DMD animal (*mdx*) model data allows for further studies including in combination with dystrophin restoration drugs
- Ex-vivo patient sample and in-vivo mouse model studies to be conducted to assess ATL1102's utility in other muscle diseases
- Grant application submitted for further work in mouse models of muscle disease and to investigate VLA-4 expression on immune cells in patient blood samples in other childhood muscle disease
- DMD patient blood (plasma) sample analysis being completed to inform on the broader immunomodulatory effects of ATL1102

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY | FSE:AWY], is pleased to advise that the Company has entered into a new Research and Development collaboration with the Murdoch Children's Research Institute's (MCRI) scientific researchers, Dr Peter Houweling and Associate Professor Shireen Lamande, to further investigate the potential of ATL1102 to deliver breakthrough treatment for the control of immune mediated inflammatory muscle damage in muscle diseases where there is an acknowledged need for more effective and safer treatments.

ATL1102 has been shown to be clinically active in Multiple Sclerosis and Duchenne muscular dystrophy (DMD) patients while antisense inhibition of CD49d has also previously demonstrated activity in multiple disease animal models. The MCRI researchers and ANP have additionally undertaken experimental work that showed antisense inhibition of CD49d in the X chromosome-linked muscular dystrophy (*mdx*) mouse model of DMD reduces both the CD49d target in the muscle and muscle damage. This data is expected to be submitted for publication in 2021.

Having achieved positive results in the *mdx* animal model now allows for the further study of antisense inhibition of CD49d effects in the *mdx* model in combination with other DMD treatments including the dystrophin restoration drugs to assess the potential of the combination to improve therapeutic outcomes. This work is to be conducted in the 2nd half of 2021 and is funded through ANP's existing cash reserves.

In addition, antisense inhibition of CD49d will be assessed in another animal model of muscle disease where there are similar immune mediated inflammatory features to the *mdx* model, where it has demonstrated positive effects.

ANP is also planning for ATL1102 to be assessed in ANP's ex-vivo cell expression and modeling systems by studying patient blood samples taken from children afflicted by a range of muscle diseases to explore ATL1102's potential activity in these conditions, where there is a clear need for effective therapies. Subject to participant recruitment this work is to be initiated in the 2H'CY21.

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The MCRI have applied for a grant to assist with further investigations of ATL1102 as described above. The Company also expects to benefit from the non-grant related program being conducted in Australia as it should be eligible for the Australian Government R&D Tax incentive of 43.5% rebate of costs.

ANP is presently withholding details on the new disease indications to allow for additional patent protection for the use of ATL1102 in these indications to be filed upon experimental success. Where applicable ANP would also look to seek Orphan Drug designation for additional market protection.

The Company has continued to file new patent applications to protect the use of ATL1102 in new immune-mediated inflammatory muscle indications with the submission last year of International patent application PCT/AU2020/050445 to be progressed with patent applications in the National phase.

As previously advised, the broader immunomodulatory effects of ATL1102 are being investigated by ANP through the analysis of blood (plasma) samples retained from the Company's Phase II trial of ATL1102 in DMD patients. ANP is presently completing this plasma analysis and is expecting this new data to provide insights on the mode of action and broader biological activity of ATL1102. ANP is planning to file for additional patent protection with this new data ahead of its proposed presentation at an appropriate scientific conference in 2H'CY21.

Dr Peter Houweling, Murdoch Children's Research, Musculoskeletal and Neuromuscular Research Group said, "We are pleased to be continuing our active research relationship with Antisense Therapeutics and expanding our collaborative efforts beyond the initial success in DMD to be developing new breakthrough approaches to treat a variety of devastating muscle diseases where there are few effective treatments."

Dr George Tachas, Director of Drug Discovery and Patents at Antisense Therapeutics said, "This collaboration is looking to build on ATL1102's established research and clinical success to date to potentially broaden both ATL1102's clinical utility in DMD and its application into other disease indications that fit with the Company's expertise and focus in immune mediated inflammatory disease and in the process broaden our development pipeline to build additional and substantial shareholder value."

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 a 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, trialling 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.