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Antisense Therapeutics to enter clinical development in Duchenne Muscular Dystrophy

- Company to undertake a clinical trial of its drug ATL1102 in new clinical application, Duchenne Muscular Dystrophy
- Clinical trial is planned to be undertaken at the Royal Children's Hospital (RCH), Melbourne
- World class international advisory board established chaired by Mr William Goolsbee ANP's non-executive director and ex-Chairman of Sarepta Therapeutics
- Funding secured for the trial (subject to receipt of approval to commence the trial)
- Australian Ethical Investment to become largest shareholder in the Company

Antisense Therapeutics ("ANP" or the "Company") announced today the Company's advanced planning to undertake a clinical trial of ATL1102, its immunomodulatory therapy initially in development for the treatment of Multiple Sclerosis (MS), in patients with Duchenne Muscular Dystrophy (DMD). The trial is designed to assess the drug's effects on the inflammation associated with this rare and incurable muscle wasting disease of children.

Mark Diamond, CEO of Antisense Therapeutics said: "Our plan to undertake a clinical trial of ATL1102 in DMD patients is facilitated by the extensive pre-clinical and clinical experience that we have established via ATL1102's development in MS. As DMD is a rare disease with a high unmet medical need, ATL1102 is expected to benefit materially from development incentives, including orphan drug designation that are provided to support rare disease drug development".

DMD and ATL1102

DMD is caused by a mutation in the muscle dystrophin gene leading to severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 to 5,000 males worldwide. A key challenge in the management of DMD patients is to reduce the inflammation that exacerbates the muscle fibre damage. Corticosteroids are the only approved treatments for muscle inflammation, however they do not sufficiently suppress muscle inflammation, are not well tolerated and have serious side effects including adversely affecting growth rate. As a consequence, there is an acknowledged high need for new therapeutic approaches for the treatment of inflammation associated with DMD.

Recently published clinical research on DMD patients has shown that patients who have a greater number of T cells (immune cells) in the blood that express high levels of CD49d (CD49dhiT-cell) are associated with both more severe and rapid disease progression, with an increase in the number of CD49dhi T cells associated with reduced walking capacity[1]. Corticosteroids did not reduce these CD49dhi T cells[1]. ATL1102 has been shown to block CD49d (VLA-4) expression on lymphocytes (including T cells), reduce immune cell numbers (including T cells), and to be highly effective in reducing inflammatory brain lesions in MS patients after only 8 weeks of dosing.

Clinical Development

The clinical trial of ATL1102 is planned to be undertaken at the Royal Children's Hospital (RCH) in Melbourne, with the clinical development of ATL1102 in boys with DMD to be directed by an Advisory Board of international experts in the field. The Advisory Board is chaired by Mr William Goolsbee, a non-executive director of ANP and ex-Chairman of Sarepta Therapeutics (Sarepta are marketers of the antisense drug, eteplirsen, the first and only drug approved for the restoration of muscle dystrophin). Membership of the Advisory Board includes the Australian inventors of eteplirsen, Professor Steve Wilton and Professor Sue Fletcher (from Perth's Western Australian Neuroscience Research Institute and Murdoch University respectively), and Dr Gillian Butler-Browne, Director, Centre of Research in Myology at Sorbonne University in Paris (whose team observed that circulating high CD49d expressing T-cells lead to poor prognosis in DMD patients).

ANP has clincial supplies available to commence the trial shortly after receipt of relevant approvals to commence the trial. Details on the trial will be provided in future news regarding trial approval.

Capital Raising to fund clinical development of ATL1102 in DMD

Institutional Placement to Australian Ethical Investment.

ANP has agreed to place 24,233,911 shares at $0.032 per share to Australian Ethical Investment to raise $775,485, equal to the maximum number of shares that ANP can issue within the 15% placement capacity limit available under the Listing Rule 7.1. The issue of shares to Australian Ethical Investment is conditional on the Company receiving hospital approval any time before 30 September 2017 to commence the clinical trial for ATL1102 in DMD.

The issue price represents the volume weighted average market price for the Company's shares over the 20 days on which sales were recorded prior to 22 June 2017.

Subject to the approval to commence the trial being given, settlement of the Placement will occur on the second business day after ASX announcement by the Company of the receipt of the hospital's approval.

Entitlement Issue

Following the settlement of the placement to Australian Ethical Investment, the Company proposes to undertake a pro-rata Entitlement Issue to shareholders at the same price to raise up to $2,000,000. Subject to approval to commence the trial being granted, Australian Ethical Investment has indicated its intention to take up its pro-rata entitlement and to acquire additional shortfall shares in ANP to increase its holding in the Company to 19.99%.

XEC Partners has been appointed as Lead Manager for the Capital Raising.

Following completion of the Capital Raising, Australian Ethical Investment will emerge as the largest shareholder in the Company.

Mark Diamond, CEO of Antisense Therapeutics said: "We look forward to welcoming as a new shareholder one of the market's most respected ethical investors and a highly regarded fund manager."

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Antisense Therapeutics Limited (ASX: ANP) is an Australian publicly listed biopharmaceutical drug discovery and development company. Its mission is to create, develop and commercialise second generation antisense pharmaceuticals for large unmet markets. The products in ANP's development pipeline are in-licensed from Ionis Pharmaceuticals Inc., 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.

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 defect in the protein or reduction or absence of the dystrophin protein. Children with DMD have dystrophin deficient muscles and are susceptible to contraction induced injury to muscle which triggers the immune system which exacerbates muscle damage (Pinto Mariz, 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, with respiratory, cardiac, cognitive dysfunction also emerging. With no intervention, the mean age of life is approximately 19 years. The management of the inflammation associated with DMD is currently via the use of corticosteroids, which have insufficient efficacy and significant side effects.

1. Pinto-Mariz et al Skeletal Muscle 2015(5:45).