ASX Announcement

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Antisense Therapeutics Announces a Poster Presentation on the Muscular Dystrophy Association Virtual Conference 2020 website

Antisense Therapeutics (ASX:ANP | US OTC:ATHJY), an Australian biopharmaceutical company developing and commercializing antisense pharmaceuticals for unmet need in rare diseases announces a virtual poster presentation on the Muscular Dystrophy Association (MDA) Virtual 2020 Conference website.

For 70 years MDA has been committed to transforming the lives of people living with muscular dystrophy, ALS, and related neuromuscular diseases. As the largest source of funding for neuromuscular disease research outside of the US federal government, MDA has committed more than $1 billion since its inception to accelerate the discovery of therapies and cures.

The poster presentation includes the recently reported data from the Phase II clinical trial of the Company's immunomodulatory therapy, ATL1102 for Duchenne Muscular Dystrophy (DMD) where ATL1102 met the primary endpoint of the study with confirmation of the drug's safety and tolerability. ATL1102 also demonstrated strong effects on secondary endpoints including activity on the targeted CD49d immune cells consistent with the drug's proposed mechanism of action and outcomes on disease progression parameters that exceeded the Company's expectations with improvement or stabilisation across different measures of muscle function and strength. The positive effects on disease progression were further supported by MRI data that suggested a stabilisation in the percentage of fat in muscles and preservation of functional muscle mass.

Following is the poster presentation abstract. The Poster can be viewed on our website at the following link: Poster Presentation - Muscular Dystrophy Association Virtual Conference 2020

Mark Diamond, Chief Executive Officer of Antisense Therapeutics said: "Given the challenging times we are living in, MDA had to reimagine their annual conference and so have recently announced the launch of their 2020 MDA Virtual Conference Hub. Accordingly, we are very pleased that MDA have accepted our Phase II trial data for presentation. The Company is giving the clinical development of ATL1102 our highest priority. As we progress toward late stage clinical development, we intend to further increase international awareness of ATL1102's therapeutic potential in boys suffering this terrible disease. The support of MDA and similarly oriented advocacy groups will greatly enhance attainment of that objective".

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About Antisense Therapeutics 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 MDA For 70 years, the Muscular Dystrophy Association (MDA) has been committed to transforming the lives of people living with muscular dystrophy, ALS, and related neuromuscular diseases. We do this through innovations in science and innovations in care. As the largest source of funding for neuromuscular disease research outside of the federal government, MDA has committed more than $1 billion since our inception to accelerate the discovery of therapies and cures. Research we have supported is directly linked to life-changing therapies across multiple neuromuscular diseases. MDA's MOVR is the first and only data hub that aggregates clinical, genetic, and patient-reported data for multiple neuromuscular diseases to improve health outcomes and accelerate drug development. MDA supports the largest network of multidisciplinary clinics providing best in class care at more than 150 of the nation's top medical institutions. Our Resource Centre serves the community with one-on-one specialized support, and we offer educational conferences, events, and materials for families and healthcare providers. Each year thousands of children and young adults learn vital life skills and gain independence at summer camp and through recreational programs, at no cost to families. During the COVID-19 pandemic, MDA continues to produce virtual events, programming, and advocacy to support our community when in-person events and activities are not possible. MDA's COVID-19 guidelines and virtual events are posted at mda.org/COVID19. For more information, visit mda.org.

Title:

Positive results from a CD49d antisense drug ATL1102 6-month Phase II trial in non-ambulant patients with Duchenne's Muscular Dystrophy

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Introduction

The safety and activity of ATL1102, an antisense drug to the RNA for CD49d, is being evaluated in a 24 week dosing study in non-ambulant patients with Duchenne's Muscular Dystrophy (DMD). DMD immune mediated inflammatory damage is currently treated with corticosteroids but there is a need for safer and more effective treatments. Patients with DMD who have a greater number of immune T cells with high levels of CD49d expression have more severe and rapid disease progression despite corticosteroid use.

Methods

In a single centre, open label Phase II study, nine non-ambulatory male patients with DMD (10-18 years) were treated with 25mg of ATL1102 subcutaneously injected once weekly for 24 weeks and monitored for 8 weeks. Participants receiving glucocorticoid therapy were included if on stable dose for at least 3 months. The primary study endpoints relate to safety and tolerability and secondary endpoints assessed the drug activity via effects on white blood cells and on disease progression via muscle strength and functional analysis (ACTRN12618000970246).

Results

ATL1102 was found to be generally safe and well tolerated. No Serious Adverse Events were reported. There were no participant withdrawals from the study. The most commonly reported adverse events were related to the subcutaneous administration of the drug, mainly injection site erythema and skin discoloration which were generally regarded as mild.

The immune cell data has shown a consistency in the mean reductions in the number of lymphocytes (i.e. CD3+, CD4+, CD8+ and those expressing CD49d) measured from baseline to end of dosing at week 24 with a return to around starting levels post dosing at week 28. This data is supportive of the drug's positive effects on modulating CD49d+ T cells in the blood.

The study endpoints that assessed ATL1102's effect on muscle function showed that 7 of the 9 participants demonstrated either increases or no change in their total PUL2.0 scores from baseline, suggestive of an overall improvement (mean change: 0.9 [95% CI: -1.33, 3.11]). Similarly, an apparent improvement in muscle strength was observed as assessed by MyoGrip (mean change: +0.2 kg [95% CI: -0.25, 0.67]) and MyoPinch (mean change: 0.0 kg [-0.18, 0.19]) compared to the loss of muscle strength reported in the literature in similar patient populations.

The positive effects observed on disease progression parameters are supported by the MRI data which shows that average percentage fat fraction was reduced/stable (Mean (SD): -0.5 (6.6); Median: 1.4) and the remaining muscle (lean muscle) cross sectional area was increased/maintained (Mean (SD): 13.9 mm (SD: 112.5); Median: 5.8 mm). Stabilisation in percentage fat fraction would not be expected in the natural course of disease even under corticosteroid treatment and with the maintenance of the lean muscle area suggests potential preservation of contractile muscle mass.

Conclusions

ATL1102, a novel antisense drug being developed for the treatment of inflammation in DMD, appears to be safe and well tolerated in non-ambulant boys with DMD. The positive effects observed on disease progression parameters support the continued development of ATL1102 for the treatment of DMD.