17 June 2016

ATL1102 for Multiple Sclerosis - Update

Antisense Therapeutics ("ANP" or the "Company") wishes to advise on the status of its ATL1102 for MS project.

Partnering interactions

As highlighted in the 15 February 2016 Company Update, ANP has been interacting with a number of potential pharmaceutical partners. These interactions have included the sharing of supportive data on the drug's potential as a treatment for Secondary Progressive MS (SP-MS) and confirm that there is partnering interest in the SP-MS indication, where there is a high unmet medical need. More than 25% of MS patients have this form of the disease and there are only a few treatment options available*. Thus SP-MS is a less competitive market than Relapsing Remitting MS (RR-MS) where there are over 10 drugs approved, each with sales of over US$1 billion.

ANP has undertaken successful Phase IIa clinical testing of ATL1102 in RR-MS patients where promising data (including an observed reduction in B cell numbers) was generated, suggesting that the drug may be active in SP-MS. The targeting of B cells has recently become a particularly high area of interest in MS treatment. Accordingly, the Company is refocussing its clinical development strategy to include SP-MS and is currently finalising the design of a Phase IIb trial in SP-MS patients (see below IND submission). Despite this refocus of ANP's clinical development efforts, the Company is cognisant that there remains an acknowledged need for potent and better tolerated therapies for treating RR-MS. Hence the Company continues to incorporate RR-MS in its partnering and commercialisation plans.

The Company, via its Corporate Advisors, Destum Partners, is continuing to explore out-licensing opportunities in RR-MS and SP-MS with potential corporate partners including recent interactions held around the BIO International Convention in San Francisco which the Company attended. The Company will report on material developments should partnering discussions move into formal licensing negotiations.

Phase IIb Investigational New Drug (IND) submission

The Company intends to submit an IND application for a Phase IIb trial in SP-MS patients. The IND application is in preparation and ANP expects to submit the IND with the Food and Drug Administration (FDA) before end 3'Q'2016. The Company expects an IND approval for a Phase IIb trial of ATL1102 would further support the drug's commercialisation and partnering efforts. In parallel, the Company is actively pursuing potential non-dilutive funding sources and other development opportunities for ANP to conduct the Phase IIb trial in the event the Company determines this to be the best path forward. In order to potentially help ANP access such grant funding, the Company has executed an agreement with consulting firm FreeMind http://www.freemindconsultants.com which specialises in assisting life science organisations secure non-dilutive funding from US Federal Agencies and Private Foundations.

Early Access Program (EAP) with myTomorrows

ANP has been undertaking drug manufacture of ATL1102 for potential use in the EAP. The drug compound has been manufactured and the formulation of this material into injectable product was completed in March this year. This formulated injectable product is undergoing testing to confirm that it is ready for human clinical use the results of which are expected this month.

As a first step towards activating the EAP, and in light of feedback received from MS clinicians, potential marketers of ATL1102 (as outlined above) and in consultation with myTomorrows, ANP are proposing to undertake a small investigative study of ATL1102 in relapsing SP-MS patients in Germany with

Professor Volker Limmroth (Cologne City Hospital, Department of Neurology, Germany), the Principal Investigator of our original Phase II study. In the Phase IIa trial of ATL1102, patients were administered 200mg three times in the first week and twice weekly 200mg per week for the following 7 weeks (Limmroth, V. et al Neurology, 2014; 83(20): 1780-1788). As previously communicated and as noted in the Neurology publication**, future trials contemplate longer dosing of ATL1102 to be at 200mg per week or lower in order to reduce the frequency of dosing and ameliorate potential side effects. Following recent discussions with the Company's technology collaboration partner Ionis Pharmaceuticals, this continues to be ANP's development strategy for ATL1102. In line with this, patients in this investigative study would be dosed at 200mg/week. Accordingly the study would be expected to generate important and supportive data on the use of ATL1102 in this SP-MS patient population at the 200mg/week dose for the potential treatment of SP-MS patients under the EAP. Timelines and costs for the conduct of this study will be reported to the market once the study details are confirmed. With FreeMind's assistance, the Company is also pursuing potential grant funding for this study.

The Company will continue to report on material developments in commercialisation plans for ATL1102 in MS.

Contact Information:
Website: www.antisense.com.au
Managing Director: Mark Diamond +61 (0) 3 9827 8999

About Antisense Therapeutics Limited
Antisense Therapeutics Limited 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. Antisense Therapeutics has 4 products in its development pipeline that it has in-licensed from Ionis Pharmaceuticals Inc. (formerly Isis Pharmaceuticals Inc.), a world leader in antisense drug development and commercialisation - ATL1102 (injection) which 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 targeting the growth hormone receptor which in a Phase II clinical trial, successfully reduced blood IGF-1 levels in patients with the growth disorder acromegaly, ATL1102 (inhaled) which is at the pre-clinical research stage as a potential treatment for asthma and ATL1101 a second-generation antisense drug at the pre-clinical stage being investigated as a potential treatment for cancer.

About MS
Multiple Sclerosis (MS) is a life-long, chronic disease that progressively destroys the central nervous system (CNS). It affects approximately 400,000 people in North America and more than 2 million worldwide and the current market for MS drugs is estimated at more than USD$14 billion. It is a disease that affects more women than men, with onset typically occurring between 20 and 40 years of age. Symptoms of MS may include vision problems, loss of balance, numbness, difficulty walking and paralysis. In Australia MS affects over 20,000 people. **Relapsing-Remitting MS:** People with this type of MS experience clearly defined attacks of worsening neurologic function. These attacks—which are called relapse or exacerbations —are followed by partial or complete recovery periods (remissions), during which no disease progression occurs. Approximately 85% of people are initially diagnosed with relapsing-remitting MS. **Secondary-progressive MS** occurs when after an initial period of relapsing-remitting MS, many people develop a secondary-progressive disease course in which the disease worsens more steadily, with or without occasional flare-ups, minor recoveries (remissions), or plateaus. Before the disease-modifying medications became available, approximately 50% of people with relapsing-remitting MS developed this form of the disease within 10 years.

* There is only one approved compound in the US.and two in Europe.

Early Access Program

Early Access Programs allow biopharmaceutical companies to provide eligible patients with ethical access to investigational medicines for unmet medical needs within the scope of the existing early access legislation. Access is provided in response to physician requests where other treatments have been unsuccessful and no alternative or appropriate treatment options are available to these patients.

**Limmroth, V. et al Neurology, 2014 "...ATL1102 demonstrated an increasing effect over time with T1 Gd lesion reductions by week 8 and the greatest T1 Gd lesion reductions observed at week 12, 4 weeks after the last dose. This extended duration of activity post dosing of ATL1102 was potentially related to the time course for the formation-turnover of new enhancing lesions and the drug's long (3 weeks) tissue half-life. The extended duration of action supports the proposition of less frequent or lower dosing in longer-term studies than the twice weekly 200-mg dosing employed in the current trial. Pharmacometric modeling suggests 200 mg once weekly, every other week, and every 3 weeks over 6 months has the potential to significantly reduce MRI brain lesions and to minimize side effects including platelet reductions. This dosing schedule could be employed in longer-term clinical trials..."

ATL1102 background Information

ATL1102 is a second generation 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 by the Company to reduce MS lesions in a Phase IIa clinical trial in RRMS patients and the data have been published (Limmroth, V. et al Neurology, 2014; 83(20): 1780-1788).