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## **ATL1102 Update**

Antisense Therapeutics Limited (ANP or the Company) is currently engaged in and exploring a number of value adding opportunities for ATL1102. These include partnering for further clinical development in multiple sclerosis as well as capitalizing on the recently announced Early Access Program and a promising new initiative in cancer.

### **Partnering**

The Company is continuing its partnering process to find a suitable development partner for ATL1102 in Multiple Sclerosis [relapsing-remitting (RR) and secondary progressive (SP) forms of MS]. ANP has long-patent life protection for both RR and SP-MS and in late 2014 received positive feedback from the US Food and Drug Administration (FDA) on the Company's plan to submit a US Investigational New Drug application for a Phase IIb clinical trial where supportive guidance was obtained from the FDA's Pre-IND assessment of the development strategy for ATL1102, including designs for Phase IIb studies in both RR and SP-MS patients.

In consultation with Destum Partners who are assisting ANP in managing the partnering process for ATL1102 just as they successfully did for ATL1103, the Company will continue to seek to partner ATL1102 but with increasing focus on ATL1102's potential application in treating SP-MS. There is a high unmet medical need in SP-MS with few treatment options available\* and therefore, being a less competitive market place, may provide both increased and broader commercial appeal for ATL1102.

ATL1102's mode of action in targeting the VLA-4 receptor and its therapeutic profile suggests that it has potential to treat SP-MS patients. As support for this, the monoclonal antibody drug Tysabri®, which also targets VLA-4, has been shown in Phase II clinical trials to benefit patients with progressive forms of MS. Tysabri® is currently undergoing Phase III trials in SP-MS with results due this year. Positive results would be an important validating event for ATL1102 potentially adding further value and therefore would be expected to spark more pharmaceutical company interest in ATL1102.

### **Early Access Program**

ANP is currently engaged in establishing an Early Access Program (EAP) for the use of ATL1102 in MS patients not responding to other treatments where ANP can charge for drug supply at prices comparable to existing MS treatments. ANP is currently working to firm up suitable ATL1102 drug product supply and the initial quantities for use in the program while its partner, myTomorrows, is preparing documentation for physician education and the EAP approvals process. The EAP is anticipated to deliver first-hand experience of the use of ATL1102 which the Company hopes will create increasing awareness and interest amongst doctors treating MS and is expected to enhance the drug's overall commercial prospects.

### **Animal Cancer Study**

In a further value adding and promising initiative for ATL1102, the drug has undergone successful testing in a pilot animal cancer study at an American University with a researcher who approached ANP with a request to conduct animal studies with ATL1102 in their established cancer animal model (additional details on the study have been limited at this time to allow for the filing of a new patent application). ANP has provided a small quantity of ATL1102 drug product for the conduct of the pilot

and follow on animal studies which are being run at the University's cost. Results from the follow on studies are anticipated this year which could add further value to ATL1102.

ANP looks forward to providing updates in relation to the further development and commercial prospects for ATL1102 in due course.

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#### **ATL1102 background Information**

ATL1102 is a second generation antisense inhibitor of CD49d, a subunit of VLA-4 (Very Late Antigen-4). In inflammation, white blood cells (leukocytes) move out of the bloodstream into the inflamed tissue, for example, the Central Nervous System (CNS) in MS, and the lung airways in asthma. The inhibition of VLA-4 may prevent white blood cells from entering sites of inflammation, thereby slowing progression of the disease. 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 II clinical trial in RRMS patients and the data has been published (Limmroth, V. et al Neurology, 2014; 83(20): 1780-1788).

#### **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, Novantrone<sup>®</sup>, for use in secondary progressive MS which can only be used for two to three years because of safety limitations.

#### **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 Isis Pharmaceuticals Inc. (ISIS), 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 designed to block GHR production 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.