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ATL1102 for MS – Phase II trial results published in the leading Medical Journal *Neurology*

Antisense Therapeutics (the “Company” or “ANP”) is pleased to report the publication of previously generated Phase IIa clinical trial data on ATL1102 in the medical journal *Neurology*.

The article titled “CD49d antisense drug ATL1102 reduces disease activity in patients with relapsing-remitting MS”, is currently available online and will be included in the print edition Volume 83, November 11, 2014.

The article highlights the successful outcomes of the Phase IIa clinical trial of ATL1102 in Multiple Sclerosis (MS) patients where in the randomised, double-blind, placebo-controlled study in 77 patients with relapsing-remitting multiple sclerosis (RRMS), ATL1102 met its primary end point after only two months of dosing, showing a significant reduction, by 54.4% ($p=0.01$) in the cumulative number of new active brain lesions in patients taking ATL1102 compared to placebo.

The efficacy outcomes from this study were viewed to be as good as, or superior to, those achieved with MS drug Tysabri® at a similar stage in its clinical development. Tysabri® (natalizumab) is a monoclonal antibody drug targeting the VLA-4 receptor (same target as ATL1102). In 2013, Tysabri® generated sales in excess of US\$1.6 billion. It is regarded as the current efficacy benchmark for the treatment of RRMS. ANP anticipates that ATL1102 could be as potent as Tysabri® but potentially safer, cheaper to manufacture, and more conveniently dosed.

Principal Investigator of the ATL1102 Phase IIa study and lead author of the *Neurology* publication, Volker Limmroth (MD PhD Professor of Neurology, Chairman Department of Neurology and Palliative Care Medicine Cologne City Hospitals, University of Cologne) said:

“There are a number of unresolved issues with current MS drugs including the occurrence of neutralising antibodies to the antibody, protein and peptide MS drugs as well as long-term safety concerns with the more recently approved drugs. There is a clear need for more effective and safe drugs for the significant population of MS patients who have relapses and non-stable disease.

The ATL1102 Phase IIa trial provides evidence for the first time that antisense oligonucleotides may be used as a therapeutic approach in neuroimmunologic disorders such as MS. ATL1102 was shown to be highly effective in reducing brain lesions in RRMS patients with a quick onset of action and a clinical safety profile that strongly supports its ongoing development as a treatment for this disease.”

The full article can be downloaded from <http://www.neurology.org/content/early/recent>

Antisense Therapeutics CEO and Managing Director Mark Diamond said:

“The Journal of the American Academy of Neurology is the most widely read and highly cited peer-reviewed Neurology Journal. Having our ATL1102 Phase II data published in such a high quality scientific journal is extremely beneficial for the future development and partnering plans for the drug and our current FDA interactions. It also provides further independent validation to the quality of our data and considerable development progress made by the Company in advancing ATL1102.”

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. VLA-4 is a clinically validated target in the treatment of MS. Antisense inhibition of VLA-4 has demonstrated positive effects in a number of animal models of inflammatory disease including MS with the MS animal data having been published in a peer reviewed scientific journal. ATL1102 was previously shown by the Company to be highly effective in reducing MS lesions in a Phase II clinical trial in RRMS patients with the primary study results reported in 2008. The efficacy outcomes from this study were viewed to be as good as, or superior to, those achieved with Tysabri® (natalizumab) the monoclonal antibody drug to the VLA-4 receptor (same target as ATL1102), at a similar stage in its clinical development. Tysabri® can cause a potential lethal viral brain infection known as progressive multi focal leukoencephalopathy (PML) The company anticipates that ATL1102 could be as potent as Tysabri® (2013 sales - US\$1.67 billion) but potentially safer (possibly not causing PML), cheaper to manufacturer, and more conveniently (self) administered. Following the successful Phase IIa trial, ATL1102 was then tested, amongst several other animal safety studies, in a chronic primate toxicology study. An unexpected adverse finding was noted in that study. ANP subsequently completed a new chronic primate toxicology to support a potential Phase IIb clinical trial. As recently reported, the new animal toxicology results on ATL1102 have provided ANP with an opportunity to resume its plans for the further clinical development of ATL1102. The Company is now seeking US Food and Drug Administration's (FDA) guidance and agreement on the intended content of the planned IND Submission. The FDA has confirmed that its goal date for responding to the Company's is October 17, 2014.

Contact Information:

Website: www.antisense.com.au

Managing Director: Mark Diamond +61 (0)3 9827 8999

Australian Investor/Media: Annabel Murphy+61 (0)2 9237 2800; amurphy@buchanwe.com.au

USA Investor/Media: Joshua Drumm +(1) 212 375 2664; jdrumm@tiberend.com

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. ANP has 4 products in its development pipeline that it has in-licensed from Isis Pharmaceuticals Inc., world leaders 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 a second-generation antisense drug designed to block GHR production which in a Phase II clinical trial, successfully reduced blood IGF-I 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.