28 August 2014

ATL1103 Acromegaly Phase II Trial Results

Antisense Therapeutics Limited ("ANP") is pleased to confirm that primary efficacy results for the Phase II trial of ATL1103 for the growth disorder, acromegaly are on track to be received by the Company by the close of business Friday 29 August UK time.

Following receipt of the data, the Company will complete its internal analysis and any necessary follow-up discussions with the UK contract research organisation and ANP's technology partner Isis Pharmaceuticals Inc. The Company expects to make an ASX announcement on the outcome of the trial in the week commencing 1 September 2014.

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Background Information

ATL1103 Phase II trial is a randomised, open-label, parallel group study of the safety, tolerability, pharmacokinetics and efficacy of two subcutaneous dosing regimens of ATL1103 in 26 adult patients with acromegaly dosed with ATL1103 for 13 weeks (3 months) with two months of follow up. Two ATL1103 dosing regimens are being tested (a) 200 mg 3 times in the first week then once weekly thereafter (200 mg/week) or (b) 200 mg 3 times in the first week then twice weekly thereafter (400 mg/week). The primary endpoints or main purposes of the trial as listed on the trial protocol are (i) to evaluate the safety and tolerability of ATL1103 in patients with acromegaly, and (ii) to evaluate the single dose and multiple dose pharmacokinetic profiles of ATL1103 via the subcutaneous route in patients with acromegaly. Another important endpoint that is also on the trial protocol is the evaluation of ATL1103's effect on serum insulin like growth factor I (IGF-I) levels in patients. This efficacy endpoint is the average percentage reduction in serum IGF-I levels at the end of treatment compared to baseline levels for each of the two dosing regimens used in the Phase II study.

ATL1103 is a second generation antisense drug designed to block growth hormone receptor (GHr) expression thereby reducing levels of the hormone insulin-like growth factor-I (IGF-I) in the blood and is a potential treatment for diseases associated with excessive growth hormone and IGF-I action. These diseases include acromegaly, an abnormal growth disorder of organs, face, hands and feet, diabetic retinopathy, a common disease of the eye and a major cause of blindness, diabetic nephropathy, a common disease of the kidney and major cause of kidney failure, and some forms of cancer. Acromegalic patients are known to have significantly higher blood IGF-I levels than healthy individuals. Reduction of these levels to normal is accepted by clinical authorities as the primary marker of an effective drug treatment for the disease. GHr is a clinically validated target in the treatment of acromegaly. In the case of diabetic retinopathy, published clinical studies have shown that treatments producing a reduction in IGF-I levels retarded the progression of the disease and improve vision in patients. Scientific papers have been published on the suppression of blood IGF-I levels in mice (Tachas et al., 2006, J Endocrinol 189, 147-54) and inhibition of retinopathy in a mouse retinopathy model (Wilkinson-Berka et al., 2007, Molecular Vision 13, 1529- 38;) using an antisense drug to the GHr. ANP have also reported that ATL1103 suppressed circulating levels of IGF-I in primates. In a Phase I study in normal volunteers, ATL1103 was assessed as being safe and well tolerated, while also demonstrating a preliminary indication of drug activity including suppression of IGF-I and the target GHR (growth hormone binding protein) levels. ATL1103 commercialisation is covered by patents to at least 2024, with the potential for extensions up to 2029 in some countries and 2030 in the US.

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 5 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 multiple sclerosis, ATL1103 a second-generation antisense drug designed to block GHr production and thereby lower blood IGF-I levels and is in clinical development as a potential treatment for growth and other GH-IGF-I disorders, 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.