

7 July 2014

Allowance of Canadian Patent on ATL1103 & Development Update

ATL1103 Canadian Patent Allowance

Antisense Therapeutics Limited (ANP) is pleased to announce that the Canadian Patent Office has allowed patent application 2,517,101 entitled "A Modified Oligonucleotide for inhibition of Growth Hormone Receptor Expression" which covers the Company's growth hormone receptor (GHR) targeting drug ATL1103 and its use until February 2024.

The Canadian patent forms part of the Company's extensive portfolio of intellectual property protecting ATL1103 and its applications in the modulation of Insulin-like Growth Factor-I (IGF-I) in the treatment of the growth disorder acromegaly and other diseases. This portfolio includes patents that have been granted and are now registered in the United States, Japan, New Zealand and Australia. In addition patent applications are in advanced stages of prosecution in Europe. Additionally, International application PCT/AU2013/000095 has been lodged covering the use of ATL1103 in combination with the GHR antagonist Somavert, for the treatment of acromegaly and cancer.

In the US, patent protection covers ATL1103 until January 2025, and in Canada and other jurisdictions to February 2024. In the US, Japan, Europe and Australia claims to ATL1103 can be extended up to a further 5 years. The International PCT application once granted, would cover the use of ATL1103 in combination with Somavert until 2032.

ATL1103 Acromegaly Phase II Clinical Trial Update

Antisense Therapeutics Limited ("ANP") is pleased to provide the following update on the Phase II trial of ATL1103 for the growth disorder, acromegaly.

24 patients have completed the full 13 weeks of dosing in the trial. The opportunity presented for the Company to enrol an additional 2 acromegalic patients into the trial (now 26 in total) who will receive their final dose of ATL1103 by 19 July 2014, which will mark the completion of all dosing in the trial.

Notably, to date there have been no treatment related patient withdrawals from the trial or reports of any treatment related serious adverse events.

The Company expects to receive the results of the primary efficacy endpoint of the trial, being the statistical analysis of the change (percentage reduction) from each patient's baseline (start of the study) serum IGF-I levels to their levels one week after the completion of dosing with ATL1103, by the end of August 2014.

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 24 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. A secondary, but 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. The secondary 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.

Acromegaly is a serious chronic life threatening disease triggered by excess secretion of growth hormone (GH) by benign pituitary tumours. Oversupply of GH over stimulates liver, fat and kidney cells, through their GH receptors, to produce excess levels of (IGF-I) in the blood manifesting in abnormal growth of the face, hands and feet, and enlargement of body organs including liver, kidney and heart. The primary treatments for acromegaly are to surgically remove the pituitary gland and/or drug therapy to normalize GH and serum IGF-I levels. In North America and Europe there are approximately 85,000 acromegaly patients with about half requiring drug therapy. Cost of drug therapy ranges from approximately A\$30,000/annum to over A\$60,000/annum depending on the treatment.

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 and is also in clinical development as a potential stem cell mobilisation agent, 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.

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