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Orphan Drug designation from FDA for ATL1103 in Acromegaly

Antisense Therapeutics Limited (ASX:ANP or “the Company”) is pleased to announce that the US Food and Drug Administration (FDA) has granted Orphan Drug designation to the Company’s drug ATL1103 for treatment of Acromegaly.

ATL1103 is an antisense drug designed to block growth hormone receptor expression in advanced clinical development and is a potential treatment for diseases associated with excessive growth hormone action including acromegaly.

Orphan drug designation is granted by the FDA to drugs intended for the safe and effective treatment of rare diseases that affect fewer than 200,000 people in the U.S. The FDA provides incentives for companies to develop products for rare diseases which may include tax credits towards the cost of clinical trials, waiver of US prescription drug filing fees and orphan product exclusivity upon marketing authorisation, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years. Accordingly, potential marketers of orphan drugs generally place a substantial premium on their commercial value.

The process of applying for Orphan Drug designation for ATL1103 in acromegaly to the European Medicines Agency has commenced. Orphan Drug designation in the European Union qualifies the sponsor of the drug for 10 years of marketing exclusivity following marketing authorisation. Other benefits relate to assistance in developing clinical protocols, reduced fees, and access to EU-funded research grants.

Mark Diamond, ANP’s Managing Director and CEO said: “This is an important regulatory and commercial milestone in the further development of ATL1103 and represents another key step forward towards bringing this potentially transformative therapy to patients with a significant unmet need”.

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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 Ionis 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 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.

About ATL1103

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 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 inhibit the production of GHR. In a Phase I study in healthy subjects, ATL1103 demonstrated a preliminary indication of drug activity, including suppression of IGF-1 and the target GHR (via circulating growth hormone binding protein) levels. In a Phase II trial in acromegalic patients, ATL1103 met its primary efficacy endpoint by showing a statistically significant average reduction in IGF-1 levels from baseline ($P < 0.0001$) at week 14 (one week past the last dose) at the twice weekly 200 mg dose tested. Antisense is currently undertaking a higher dose study in acromegaly patients.