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ATL1103 for Acromegaly Granted Orphan Drug Designation in Europe

Antisense Therapeutics Limited (ASX:ANP or “the Company”) is pleased to announce that the European Commission has granted orphan medicinal product designation for the Company’s drug ATL1103 for the treatment of Acromegaly in the European Union (EU).

The approval was based on the recommendation of a positive opinion from the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP). The COMP assessed the scientific documentation for ATL1103 against the criteria for orphan designation, with the COMP stating in their opinion that ATL1103 “...will be of significant benefit to those affected by that condition”.

Orphan designation in the EU enables sponsors to benefit from a number of incentives, including 10 years of market exclusivity once the medicine is on the market. During that exclusivity period, the EMA and the EU Member states shall not accept another application for a marketing authorization, for the same therapeutic indication, in respect of a similar medicinal product. Other benefits relate to assistance in developing clinical protocols, reduced fees, and access to the EU-funded research grants.

ATL1103 for acromegaly was recently granted orphan drug designation by the US Food and Drug Administration (FDA) which provides incentives including 7 years of market exclusivity in the United States.

Mark Diamond, ANP’s Managing Director and CEO said: “This European Orphan designation is another major step forward in the development of ATL1103. The assistance from the EMA in the development of ATL1103 will be invaluable in helping to bring this product to acromegaly patients and will confirm to our potential development partners that the benefits associated with the orphan pathway will be applicable in any transaction”.

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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.