26 August 2019

Acromegaly Early Access Program – Status Update

As previously advised, Antisense Therapeutics Limited ("ANP" or "the Company") executed a global agreement with early access provider myTomorrows to implement an ATL1103 (also referred to as atesidorsen) Early Access Program ("EAP") for the treatment of acromegaly patients within select countries of the European Union. Labelled and packaged ATL1103 drug product is currently being stored in the United Kingdom for shipment to myTomorrows in the Netherlands for potential EAP distribution. However, to date the Company has been unable to obtain myTomorrows' clearance for importation.

Further to myTomorrows requests, the Company has generated additional product data and documentation in order for the ATL1103 drug product to be supplied in accordance with the required regulatory and quality standards and for product to be imported and released by myTomorrows for use in the EAP.

Following a review by an external Quality Person (QP), requested by myTomorrows, of the manufacturing documentation including this newly generated data, the QP advised that due to the ATL1103 material intended for use in the EAP being supplied by a different manufacturer to the one used for the manufacture of material previously used in the Phase II clinical trail of ATL1103, it would first need to be approved by a European Health authority for use in a new clinical trial, for the material to be cleared for the EAP.

The Company had not expected this clinical trial approval prerequisite for ATL1103 EAP initiation, with this new requirement coming on top of the additional data the Company had been asked by myTomorrows to collect and generate to show the comparability of the current batch of ATL1103 material to the earlier batch used in clinical trials. A new clinical trial would require a substantial financial commitment from the Company to proceed with the next phase of clinical development for ATL1103.

ANP's current development focus is directed towards the clinical development of ATL1102 in DMD. ANP believes, though, that circumstances could present in the future where the Company has the capacity and justification to continue to invest in the further clinical development of ATL1103, including activation of an EAP. Until that time, the Company will not apply further resources to the EAP process and will continue to direct its focus and funds on the ATL1102 for DMD program and the ongoing Phase II clinical trial, where dosing of all patients is to be completed in early November 2019.

Separately, interest continues from expert acromegaly clinicians in Europe in the use of ATL1103 for patients uncontrolled on existing therapy and in testing ATL1103 in combination with pegvisomant in order to exploit its potential synergistic clinical benefits in line with the data exemplified in our granted IP. The Company is exploring opportunities to provide ATL1103 drug product to such experts to generate supportive clinical data.

ANP is also continuing to pursue the potential out-licensing of ATL1103 to support and fund its ongoing clinical development and is presently entertaining preliminary interest from some regionally based pharmaceutical companies.

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This announcement is issued exclusively by Antisense Therapeutics Ltd for ASX listing rule purposes.

About Antisense Therapeutics Limited (ASX: ANP) is an Australian publicly listed biopharmaceutical company, developing and commercialising antisense pharmaceuticals for large unmet markets. The products are in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ:IONS), world leaders in antisense drug development and commercialisation. ATL1102 (injection) 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 successfully reduced blood IGF-I levels in Phase II clinical trials in patients with the growth disorder acromegaly. The Company is planning to conduct a Phase II clinical trial of ATL1102 in DMD patients at the Royal Childrens Hospital, Melbourne.

About Early Access Programs
Early Access Programs allow biopharmaceutical companies to provide eligible patients with ethical access to investigational medicines for unmet medical needs within the scope of the existing early access legislation. Access is provided in response to physician requests where other treatments have been unsuccessful and no alternative or appropriate treatment options are available to these patients.

About Acromegaly
Acromegaly is a serious chronic life-threatening disease triggered by excess secretion of growth hormone (GH) by a benign tumour of the pituitary. Oversupply of GH produces excess levels of Insulin-Like Growth Factor-I (IGF-I) in the blood causing the abnormal growth of the bones of the face, hands and feet, and enlargement of body organs. In North America and Europe there are approximately 85,000 acromegaly patients with around one half of these requiring life-long drug therapy. A significant number of patients fail to be adequately treated with current medicines due to efficacy, safety or tolerance related issues. The current average cost for 2nd line acromegaly treatment in Europe is approximately A$80K per patient per annum.

About ATL1103 / atesidorsen
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-1 (IGF-1) in the blood and is a potential treatment for diseases associated with excessive growth hormone and IGF-1 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-1 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-1 levels retarded the progression of the disease and improve vision in patients. Scientific papers have been published on the suppression of blood IGF-1 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 sIGF-1 levels from baseline ($P<0.0001$) at week 14 (one week past the last dose) at the twice weekly 200 mg dose tested (Trainer PJ et al., Eur. J. Endocrinology May 22, 2018, doi: 10.1530/EJE-18-0138). Antisense has also recently completed a successful higher dose study in acromegaly patients. ATL1103 has Orphan Drug designation in the US and Europe.