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# Antisense Therapeutics executes agreement to implement ATL1103 / atesidorsen Early Access Program for Patients with Acromegaly

Antisense Therapeutics Limited ("ANP" or "the Company") is pleased to announce that it has executed a global agreement with innovative early access provider myTomorrows (Amsterdam, The Netherlands) to implement an Early Access Program (EAP) for ATL1103, also referred to as atesidorsen, for the treatment of Acromegaly. This program will initially be established in selected countries within the European Union (EU).

Subject to myTomorrows receiving the requisite regulatory approvals and support for the ATL1103 EAP program, ANP expects to provide ATL1103 to treatment centers in the EU. myTomorrows has undertaken certain product assessments that have included discussions with a number of key acromegaly experts in Europe (including some of the investigators from the ATL1103 Phase II clinical trial) to identify the unmet medical need in the relevant patient group, where myTomorrows received good support for the concept of using ATL1103 in patients not controlled on current acromegaly treatments.

ATL1103 is an antisense drug designed to block growth hormone receptor (GHr) expression thereby reducing levels of IGF-I in the blood. In a Phase II trial in acromegalic patients, ATL1103 met its primary efficacy endpoint by showing a statistically significant reduction in sIGF-1 levels from baseline ($P<0.0001$) at the twice weekly 200 mg dose tested. ANP has also completed a successful higher dose study in acromegaly patients. ATL1103 has Orphan Drug designation in the US and Europe.

ANP currently has sufficient supplies of ATL1103 raw material to potentially treat approximately 15 patients for 1 year. Under the EAP, the Company can set pricing for the drug. The next step for ANP would be to arrange for the ATL1103 raw material to be formulated into injectable product for potential use in the EAP.

Under the EAP agreement, myTomorrows will perform at their cost the EAP activities including relevant data collection and the seeking of the EAP treatment approvals. myTomorrows is to receive a share of EAP related revenue less associated pass through costs including those to Ionis Pharmaceuticals from whom ANP in-licensed ATL1103.

Separate to this EAP agreement, ANP is seeking a partner for the on-going clinical development and potential commercialisation of ATL1103. In the event of future licensing revenue and sales of ATL1103, myTomorrows will also be entitled to a percentage of such sales and licensing revenue received by ANP as compensation for the services provided, but only in those countries where an EAP had been established.

ANP is of the view that an ATL1103 EAP would, next to patient treatment, also further stimulate Key Opinion Leader interest and support within a major pharmaceutical (Europe) market, produce key safety data (without associated clinical trial costs), generate income and facilitate increased partnering interest for the continued development of the drug.

Antisense Therapeutics' CEO and Managing Director Mark Diamond said

"Antisense Therapeutics is pleased to establish this Early Access Program for ATL1103 that may help the lives of those suffering from acromegaly by providing an alternate treatment option. We look

forward to working with treating physicians and myTomorrows to make ATL1103 available to the European medical community".

myTomorrows' Chief Operating Officer, Robert Kraal, said

"Acromegaly is a seriously debilitating disease where failing to provide adequate treatment leaves patients with severe, lifelong implications. With limited satisfactory treatment options available, the Early Access Program for ATL1103 / atesidorsen aligns well with myTomorrows mission and the intention of Early Access legislation, to provide treatment when no registered alternatives exist. We look forward to working with ANP to engage physicians and their patients facing an unmet medical need, and help them find and get access to a potential new treatment."

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

**About Antisense Therapeutics Limited**
Antisense Therapeutics Limited 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. Antisense Therapeutics has 4 products in its development pipeline that it has in-licensed from Isis Pharmaceuticals Inc. (ISIS), a world leader in antisense drug development and commercialisation - ATL1102 (injection) which has successfully completed a Phase II efficacy and safety trial in patients with relapsing-remitting multiple sclerosis (RRMS), ATL1103 drug designed to block GHr production which in a Phase II clinical trial reduced blood IGF-1 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 myTomorrows**
At myTomorrows, we believe that everyone should be able to access all treatment options whenever these exist. Working together with medicine manufacturers to devise and execute strategies for early access, we strive to make it easier for physicians and their patients who have exhausted all approved treatment options to find, get information about and access to pre-approval medicines. myTomorrows has developed a Knowledge Base to combine vast amounts of medical and clinical data to present an actionable overview of the full clinical development pipeline.

**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. In Australia, patient treatment via an Early Access Program would typically fall under the Therapeutic Goods Administration's Special Access Scheme, Personal Import Scheme or Authorised Prescribers pathway.

**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 2$^{nd}$ 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. Antisense has also recently completed a successful higher dose study in acromegaly patients. ATL1103 has Orphan Drug designation in the US and Europe.