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ATL1103/atesidorsen Early Access Program – Progress Update

Antisense Therapeutics Limited (“ANP” or “the Company”) is pleased to provide an update on its progress to initiate an ATL1103 (also referred to as atesidorsen) Early Access Program (“EAP”) for the treatment of acromegaly patients within select countries of the European Union, those initially planned being the Netherlands, Germany and France.

The formulation of atesidorsen raw material into injectable product work has recently been completed with the newly formulated injectable product (Drug Product or DP) to now undergo release testing for human use, with results expected next month. Allowing for raw material losses that occur as part of the manufacturing process, sufficient number of vials have been produced to dose 12 acromegaly patients for 1 year at a twice weekly 200mg dose. The DP is then to be appropriately labeled and packaged for supply to patients under the EAP.

In parallel, the Company with its partner, leading Early Access provider myTomorrows (Amsterdam, The Netherlands), is progressing work on the documentation required for the regulatory approvals to supply atesidorsen product under the EAP. Alongside this documentation, the requisite Quality Assurance and Pharmacovigilance programs are being established and associated agreements are being finalised for execution. The regulatory approval and reimbursement processes can commence following product launch by myTomorrows which is after labeled product is in the myTomorrow’s warehouse and is released by myTomorrow’s Quality Person for the EAP and all the regulatory documentation is ready.

The activities necessary to initiate atesidorsen EAP treatments are anticipated to be completed, or in place by end of 3’Q’18, as previously advised, with reimbursement approvals anticipated to come through on a country by country basis after the relevant regulatory approvals.

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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 myTomorrows

At myTomorrows, we believe that everyone should be able to access all suitable treatment options, whenever available, wherever they exist. Working together with medicine manufacturers to devise and execute strategies for early access, we strive to make it easier for doctors and their patients who ran out of viable treatment options to find, get information about and access to medicines in development. 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 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.