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## **ATL1103 (COR-004) Higher Dose Study - Dosing Commenced**

- Higher dose study running in parallel with other activities being undertaken by ATL1103 licensing partner, Cortendo AB (now Strongbridge Biopharma plc)
- Costs of the study to be reimbursed by Strongbridge Biopharma

Antisense Therapeutics (ASX:ANP or “the Company”) wishes to advise that dosing has commenced in its ATL1103 higher dose study with two patients (of the planned 4) having received their initial dose of ATL1103 at one of the Australian clinical trial sites.

The ATL1103 higher dose trial is an open-label study of the safety, tolerability, pharmacokinetics and efficacy [effect on serum insulin like growth factor I (sIGF-I)] of ATL1103 in 4 adult patients with acromegaly dosed with ATL1103 up to 300mg twice weekly for 13 weeks.

Dosing of these patients will be completed before the end of the year. The timing of the reporting of results from this study will depend on the enrollment and the medication status of the additional patients required to complete the trial as patients recruited for this study are screened to confirm eligibility during a 28 day period and may need to “washout” any current acromegaly medications for a period of 6 weeks to 4 months, depending on the type of medication, before dosing with ATL1103 may begin.

This ATL1103 higher dose study being undertaken by ANP will run in parallel with other activities to be conducted by its licensing partner, Cortendo AB (now Strongbridge Biopharma plc). These activities include seeking orphan drug designation from the FDA and the EMA, the conduct of Phase 3 enabling chronic toxicology studies and a pre-IND meeting with the FDA in the second half of 2015 to discuss requirements for entry into Phase 3 clinical development.

ANP’s costs associated with this higher dose study are to be reimbursed by Strongbridge Biopharma as part of the ATL1103 licensing agreement announced in May 2015. Strongbridge Biopharma are responsible for the ongoing clinical development of ATL1103 in endocrinology applications and will fund the associated future development, regulatory and drug manufacture costs.

### **Contact Information:**

Website: [www.antisense.com.au](http://www.antisense.com.au)

Managing Director: Mark Diamond +61 (0)3 9827 8999

**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 Isis 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-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 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. In May 14, 2015 Cortendo AB (now Strongbridge Biopharma plc) and Antisense Therapeutics announced that the companies had entered into an exclusive license agreement that provides Strongbridge Biopharma with development and commercialization rights to ATL1103 for endocrinology applications. Under its technology collaboration with ISIS, Antisense Therapeutics' will pay ISIS a percentage (single digit) of the licensing revenue it earns from ATL1103.

**About Strongbridge Biopharma** Following the settlement of its exchange offer to acquire any and all issued ordinary shares of Cortendo AB, Strongbridge Biopharma will become the new parent company of the Cortendo group. The group's strategic focus is to build a biopharmaceutical company focused on the development, in-licensing, acquisition and eventual commercialization of complementary product candidates across multiple franchises that target rare diseases. The group's lead product candidate, COR-003 (levoketoconazole), is a cortisol inhibitor that is currently being studied in the global Phase 3 trial for the treatment of endogenous Cushing's syndrome. COR-003 has received orphan designation from both the European Medicines Agency and the U.S. Food and Drug Administration. The group recently expanded its rare endocrine disease franchise with the completion of transactions for two Phase 2 product candidates: COR-004, a novel second-generation antisense compound, which is in clinical development for acromegaly and designed to block the synthesis of growth hormone receptor (GHR) thereby reducing levels of insulin-like growth factor-1 (IGF-1) in the blood; and COR-005, a next-generation somatostatin analog (SSA) with a unique receptor affinity profile, being investigated for the treatment of acromegaly, with potential additional applications in Cushing's disease and neuroendocrine tumors. The group's intent is to independently commercialize its rare endocrine assets in key global markets.