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ATL1103 Update – proposed International Nonproprietary Name for ATL1103

Antisense Therapeutics today reports that the World Health Organization has published the proposed International Nonproprietary Name (INN) - atesidorsen - for ANP's drug, ATL1103.

http://www.who.int/medicines/publications/druginformation/PL116_2016.pdf?ua=1. A non-proprietary name is also known as a generic name.

The initial application for the INN was made by Cortendo AB (Strongbridge Biopharma) at their cost as an important step towards the commercialisation of the drug. Ownership of the application was subsequently transferred to ANP.

The Company has been advised that there is now a 4 month public comment period. If no objections are received the name is published in the Recommended INN list. Once on the recommended INN list, the Company can use the name without risk of it having to be changed.

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About International Nonproprietary Names (INN) identify pharmaceutical substances or active pharmaceutical ingredients. Each INN is a unique name that is globally recognized and is public property. A non-proprietary name is also known as a generic name. As unique names, INN have to be distinctive in sound and spelling, and should not be liable to confusion with other names in common use. To make INN universally available they are formally placed by WHO in the public domain, hence their designation as "non-proprietary". They can be used without any restriction whatsoever to identify pharmaceutical substances. Another important feature of the INN system is that the names of pharmacologically-related substances demonstrate their relationship by using a common "stem". By the use of common stems the medical practitioner, the pharmacist, or anyone dealing with pharmaceutical products can recognize that the substance belongs to a group of substances having similar pharmacological activity (e.g. mipomersen, etiplirsen, and atesidorsen)
<http://www.who.int/medicines/services/inn/innguidance/en/>

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 has also recently completed a higher dose study in acromegaly patients.

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.