

ASX Announcement4 March 2021

FDA Rare Disease Day 2021

Antisense Therapeutics Limited ("ANP" or "the Company") is pleased to advise that Dr Gil Price, ANP's US-based Consultant Medical Director will be representing the Company at the US Food and Drug Administration's (FDA) Rare Disease Day virtual event to be held on Friday 5 March 2021, 9:00 AM-4:00 PM ET (<https://tinyurl.com/FDA-Rare-Disease-Day>).

The conference is organised by the FDA Office of Orphan Products Development, whose mission is to advance the evaluation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. In the United States rare diseases are diseases that affect less than 200,000 people. While these diseases are individually rare, collectively there are more than 7,000 rare diseases affecting an estimated 30 million people in the United States. Many of these rare diseases are serious or life-threatening and many affect children.

The purpose of this meeting is for the FDA to highlight strategies to support rare disease product development. The event will be attended by patients, patient advocates, researchers, and medical product developers who will share their perspectives on and experiences in rare disease product development and illustrate the types of challenges faced and strategies used to address them.

Additional discussion topics will include the importance of patient engagement and natural history studies in rare disease product development, strategies to support rare disease product development during the COVID-19 pandemic, and current perspectives on rare disease product development from leadership across FDA's medical product centres.

Mark Diamond CEO of Antisense Therapeutics said: "The FDA Rare Disease Day presents an opportunity for us to gain additional insights from the rare disease community and the FDA staff working across the agency ahead of our own meeting with the FDA next month to discuss ATL1102 development in DMD in the US".

Authorised for release by the CEO.

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About Antisense Therapeutics Limited (ASX:ANP | US OTC:ATHJY) is an Australian publicly listed biotechnology company, developing and commercializing antisense pharmaceuticals for large unmet markets in rare diseases. The products are in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ: IONS), an established leader in antisense drug development. The Company is developing ATL1102, an antisense inhibitor of the CD49d receptor, for Duchenne muscular dystrophy (DMD) patients and recently reported highly promising Phase II trial results. ATL1102 has also successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with relapsing-remitting multiple sclerosis (RRMS). The Company has a second drug, ATL1103 designed to block GHR production that successfully reduced blood IGF-I levels in Phase II clinical trials in patients with the growth disorder acromegaly.

About ATL1102 ATL1102 is an antisense inhibitor of CD49d, a subunit of VLA-4 (Very Late Antigen-4). Antisense inhibition of VLA-4 expression has demonstrated activity in a number of animal models of inflammatory disease. ATL1102 has also shown to be very effective in reducing inflammatory brain lesions in a patients with MS (Limmroth, V. et al Neurology, 2014; 83(20): 1780-1788) and recently delivered highly promising clinical results in patients with Duchenne muscular dystrophy (DMD) a rare and fatal muscle wasting disease where inflammation in the muscle leads to fibrosis and death of muscle tissue.