ASX Announcement

30 September 2020

US FDA Grants Rare Pediatric Disease Designation to ATL1102 for the treatment of DMD

- **Potentially eligible for rare pediatric disease priority review voucher (PRV);**
- **PRV may be utilised to expedite marketing authorization review by FDA; or**
- **PRV may be sold to provide additional non-dilutive capital**

Antisense Therapeutics Limited [ASX:ANP | US OTC:ATHJY] is pleased to advise that U.S. Food and Drug Administration (FDA) has granted rare pediatric disease (RPD) designation for ATL1102 for the treatment of DMD, a rare and fatal muscle wasting disease where inflammation in the muscle leads to fibrosis and death of muscle tissue.

As part of advancing US regulatory strategy and recent application to US FDA for an Orphan Drug Designation (ODD), a request for a rare pediatric disease designation was submitted in conjunction with the ODD application. The FDA has granted the designation of ATL1102 as a drug for a rare pediatric disease following submission of data from Phase II clinical trial of ATL1102. The FDA has also determined that DMD meets the definition of a rare pediatric disease based on the information submitted by the company and reliance upon additional supportive information. Therefore, FDA has determined ATL1102 to be eligible for rare pediatric disease designation for treatment of DMD.

Further, under the FDA's Rare Pediatric Disease Priority Review Voucher Program*, a company that receives an approval for a product designated for a rare pediatric disease may qualify for a voucher that can be redeemed to receive an expedited priority marketing authorization review. The award or non-award of a rare pediatric disease priority review voucher is made at the time of marketing approval, should the Company request such a voucher in its marketing application, and subject to it meeting the eligibility criteria for a PRV. Priority review can cut the FDA review process by as much as 4-6 months, shortening the time it takes for the drug to reach the market.

The sponsor of a rare pediatric disease drug receiving a priority review voucher may transfer or sell the voucher without limitation, subject to applicable FDA requirements for filing and use. An intangible benefit of the voucher is the value created for a company if the faster review leads to an expedited approval and therefore provides a commercial advantage via earlier sales of a new drug on the market. In recent years, a secondary market for the vouchers has emerged allowing companies to use the sale of PRVs to recoup expenses undertaken for drug research and development and present them with additional source of non-dilutive capital to support further advancement of clinical development. Since 2009 when the first PRV was awarded the values for these vouchers have ranged between US$68 million and US$350 million.

Mark Diamond, Chief Executive Officer of Antisense Therapeutics said: "We are very encouraged by the granting of the rare pediatric disease designation to ATL1102 by the FDA, which recognizes a great need for new and improved therapies for boys with DMD. We look forward to future interactions with FDA as we refine our strategy for development of ATL1102 in DMD in the US."

This announcement has been authorised for release by the Board.

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Rare Pediatric Disease
A rare pediatric disease is defined by the Federal Food, Drug, and Cosmetic Act to include a serious or life-threatening disease, which primarily affects individuals aged from birth to 18 years and affects fewer than 200,000 people in the U.S.

Priority review voucher
The term "priority review voucher" means a voucher issued by the Secretary of Health and Human Services to the sponsor of a rare pediatric disease product application that entitles the holder of such voucher to priority review of a single human drug application submitted under section 355(b)(1) Federal Food, Drug, and Cosmetic Act or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] after the date of approval of the rare pediatric disease product application. For additional information including eligibility criteria refer to Rare Pediatric Disease Priority Review Vouchers: Guidance for Industry: https://www.fda.gov/media/90014/download

*NOTE: According to the current statutory sunset provisions the RPD Priority Review Voucher Program is currently expected to expire on September 30, 2020. A drug that has been granted RPD designation prior to this date is still eligible to receive the PRV if it receives final FDA approval before September 30, 2022, unless extended by US Congress (under the pending House legislation, this may be extended to 30 September 2026).

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.