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ATL1102 New Stem Cell Mobilization Indication - International Patent Application Lodged

- **Pre-clinical and clinical data from previously conducted studies supports potential for new stem cell mobilization indication for ATL1102**
- **International Patent application lodged**
- **Leading expert supports potential of ATL1102 in stem cell mobilization**
- **New stem cell indication expected to enhance ATL1102's overall commercial value and attractiveness to potential partners**

Antisense Therapeutics Limited ("ANP" or "the Company") is pleased to advise of a potential new application for the Company's drug ATL1102 as a stem cell mobilization agent in stem cell transplantation based on data generated from previously conducted studies on ATL1102. This data has formed the basis of an International Patent Application that has been lodged on ATL1102 entitled "Method of mobilizing stem cell and/or progenitor cells." The application seeks to provide protection for the stem cell mobilization application of ATL1102 until 2031 and if granted, would add to the already comprehensive intellectual property position established on ATL1102 to underpin its commercialization.

Stem cell transplantation is a medical procedure used to improve clinical outcomes for patients undergoing chemotherapy to treat cancer. Blood cells are produced in the bone marrow and arise from special 'parent' cells, called stem cells. The stem cells that form blood and immune cells are known as hematopoietic stem cells. Some chemotherapy has toxic effects on bone marrow, so hematopoietic stem cells are collected from the blood of the patient – or from a donor's blood – before the patient receives chemotherapy. These collected stem cells are then stored and given back later to replace those lost during chemotherapy.

There are normally only a small number of stem cells in the blood, so stem cell mobilization agents are typically used to increase this number before stem cell collection from a donor or patient. These stem cells are identified by protein markers on the surface of the cell (e.g. CD34+) and their corresponding RNA. Granulocyte colony-stimulating factor (G-CSF) is the main agent used for hematopoietic stem cell mobilization. The market for G-CSF agents is reported to be several billion dollars per annum. While G-CSF is successful in mobilizing hematopoietic stem cells, there is an opportunity to improve on the level of stem cell release achieved with the use of G-CSF alone by the addition of complimentary therapies - the role envisaged for ATL1102.

One such complimentary therapy, Mozobil™, has been approved for use in combination with the main agent G-CSF. First marketed in 2009, Mozobil™ sales in 2010 were ~US$100Mill with peak sales potential estimated at US$350Mill per annum. Mozobil™ has orphan drug status in the United States and European Union for the mobilization of hematopoietic stem cells. While Mozobil™ does boost the number of stems cells released beyond G-CSF alone, a clinical need remains for agents that can improve on the level of stem cell release currently being achieved with the G-CSF/Mozobil™ combination. ATL1102 has a novel action (different to both G-CSF and Mozobil™) in targeting the VLA-4 receptor, the blocking of which has been shown to aid in the release of stem cells from the bone marrow. This could see ATL1102 used in place of Mozobil™ should it demonstrate clinical superiority, or together with both G-CSF and Mozobil™ to enhance their effects.

In a review of pre-clinical and clinical data previously generated in relation to ATL1102, the following findings support the potential of ATL1102 in the stem cell mobilization indication:

- A version of ATL1102 designed to work in mice was used over 7, 10 and 14 days in combination with G-CSF given on the last 3 days of treatment, released one of the recognized stem cells (GEMM cells) up to 13 times more ($p<0.01$) compared to saline control at day 14, and 7 times more than with G-CSF used alone ($p=0.05$).

This animal data provides support for a relatively quick pharmacological action of the drug; with stem cell increases being achieved within a week of treatment, as well as the drug's potential to be used in combination with G-CSF to increase the level of stem cell mobilization beyond G-CSF alone.

- In a Phase II study of ATL1102 in relapsing-remitting multiple sclerosis (RR-MS) patients, ATL1102 increased CD34 RNA 1.5 times ($p<0.027$) at 8 weeks (being the earliest time point available for measurement) compared to baseline in total blood RNA of the MS patients, thereby demonstrating relevant biological activity in humans.

As part of its review process, ANP have been consulting with Professor Miles Prince of the Peter McCallum Institute on the potential of ATL1102 as a stem cell mobilization agent. Professor Prince is providing the Company with advice on the clinical aspects of ATL1102 in the stem cell mobilization indication and has described the preliminary data generated by ANP as promising and supportive of the potential of ATL1102 in the stem cell mobilization indication where there is a need for agents that can improve on the level of stem cell release achieved by GCS-F alone.

Mobilization agents are administered over a short duration (1 week). This results in a minimization of the cost and time of running clinical studies when compared to testing drugs that need chronic (long term) administration as they require clinical trials that run for many months to years to achieve sufficient amount of clinical data/experience for registration. Short term treatments reduce the chance of potential adverse drug effects as the level and duration of drug exposure is significantly decreased compared to chronically administered treatments. Notably, the safety profile of ATL1102 in this stem cell mobilization indication is supported by the Company's previously conducted Phase II clinical trial of ATL1102 in MS patients, where the drug was administered for 8 weeks and was viewed as safe and well tolerated.

ANP Managing Director and CEO Mark Diamond added "This new stem cell mobilization opportunity builds additional value in ATL1102 by capitalizing on the significant amount of data generated to date on this compound. Importantly we have human safety data on ATL1102 at the doses that would be used clinically in this indication, which puts this project at an advanced stage."

ANP has already built an extensive ATL1102 Intellectual Property estate including granted US and European composition and use patents along with Isis antisense platform and manufacturing patents. This new patent relating to stem cell mobilization potentially adds further value to the patent portfolio on ATL1102 which may also be supplemented by an orphan drug designation where the regulatory bodies provide 7 years of market exclusivity post drug approval in the US and 10 years in Europe.

As previously advised, the Company continues to seek a new partner to further develop ATL1102 for the multiple sclerosis indication. It is the Company's view that this new indication in stem cell mobilization enhances the commercial value of ATL1102 and its attractiveness to a wider range of potential future partners.

Background Information

Antisense Therapeutics (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. ATL1102 (injection) has successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with multiple sclerosis. ATL1103 is a second-generation antisense drug designed to block GHr production and thereby lower blood IGF-I levels and is in clinical development as a potential treatment for growth and vision disorders. ATL1102 (inhaled) is at the pre-clinical research stage as a potential treatment for asthma. ATL1101 is a second-generation antisense drug at the pre-clinical stage being investigated as a potential treatment for prostate cancer

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