

24<sup>th</sup> March 2017

## Market and Technology Update

Antisense Therapeutics Limited (ASX:ANP or “the Company”) today provides an update on important developments that ANP regard as relevant to its late stage pipeline of antisense compounds - ATL1102 for Multiple Sclerosis (MS) and ATL1103 for acromegaly.

### **Market**

- **ATL1102 for MS**

- In February 2017, RPI Finance Trust, an affiliate of Royalty Pharma entered into a definitive agreement to acquire rights to the global net sales royalty stream of the MS drug Tysabri® (natalizumab) from Perrigo Company plc [Perrigo receive a royalty on in market sales of Tysabri from Biogen Idec Inc]. The transaction was valued at a total consideration of up to US\$2.85 billion, composed of US\$2.2 billion in cash at closing and up to US\$650 million in potential milestone payments based upon future global net sales of Tysabri in 2018 and 2020.

Relevance to ANP: Tysabri is a monoclonal antibody drug directed to the same target as ATL1102, the VLA-4 receptor. Tysabri is regarded as the current efficacy benchmark for the treatment of RRMS, with global annual sales of over US\$2 Billion. ANP anticipates that ATL1102 could be as potent as Tysabri but potentially safer, less expensive to manufacture, and more conveniently dosed. The Company believes that the above transaction, which is valuing the future sales potential of Tysabri, underscores the continued commercial relevance of ATL1102 for MS.

- Datamonitor Healthcare forecasts that the MS market will expand significantly over the period of 2016–25, with sales in the US, Japan, and five major EU markets (France, Germany, Italy, Spain, and the UK) projected to increase from US\$18 billion to US\$31 billion.

Relevance to ANP: The market for MS drugs has continued to grow, further supported by the introduction of newer and higher priced MS agents, suggesting the sustained market need for improved MS therapies such as potentially ATL1102.

- **ATL1103 for Acromegaly**

- In February 2017, Pfizer Inc. (marketer of acromegaly treatment Somavert® or pegvisomant) said that it had received two subpoenas from the U.S. Attorney’s office in Massachusetts related to charities that help Medicare patients afford co-payments for drugs. Pfizer said that it received subpoenas requesting documents related to the Patient Access Network Foundation and other organizations that provide financial assistance to Medicare patients. Pfizer donated more than US\$10 million to the PAN Foundation in 2016, including for funds related to kidney cancer and acromegaly. As aggressive price increases for certain prescription medications have drawn the ire of politicians and the healthcare industry, concern has grown that donations made by pharmaceutical companies to patient assistance groups may be contributing to the price inflation.

Relevance to ANP: The Company anticipates that ATL1103 will be less expensive to manufacture than pegvisomant, providing greater pricing flexibility to accommodate potential pricing constraints as described above.

## **Technology**

- ANP technology collaboration partner, Ionis (NASDAQ:IONS market capitalization: US\$5 billion) has recently announced significant clinical progress within their pipeline of antisense drugs.
  - Dec. 23, 2016 Ionis announced that the US Food and Drug Administration (FDA) approved SPINRAZA™ (nusinersen) under Priority Review for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients. SPINRAZA is the first and only treatment approved in the U.S. for SMA, a leading genetic cause of death in infants and toddlers that is marked by progressive, debilitating muscle weakness. SPINRAZA was discovered and developed by Ionis and Biogen, and licensed to Biogen who is responsible for future development, manufacturing, and commercialization of SPINRAZA.
  - Feb. 14, 2017 Ionis announced the advancement of IONIS-FXI<sub>Rx</sub> in clinical development under an existing exclusive license agreement with Bayer. Under this agreement, Ionis plans to conduct a Phase 2b study evaluating IONIS- FXI-L<sub>Rx</sub> and will also initiate development of IONIS-FXI-L<sub>Rx</sub>. In conjunction with the decision to advance these programs, Ionis are to receive a US\$75 million payment from Bayer.
  - March 6, 2017 Akcea Therapeutics, a wholly owned subsidiary of Ionis, announced that the pivotal Phase 3 APPROACH study of volanesorsen met its primary endpoint of reducing triglyceride levels in patients with familial chylomicronemia syndrome (FCS). Akcea advised that the APPROACH study will support the regulatory submission for FCS of volanesorsen.

Relevance to ANP: Ionis now have 2 antisense drugs registered with the FDA (Kynamro®, and SPINRAZA) and 12 other drugs in their clinical pipeline (including all the drugs referred to above in clinical development) of the same basic chemistry as ATL1102 and ATL1103. Most of these drugs have been out licensed, including to Big Pharma companies including Bayer, Roche, GSK, and Biogen. Such progress provides important clinical, regulatory, and commercial validation for ANP's product technology.

The Company's Investigational New Drug (IND) application for a Phase IIb trial of ATL1102 in 195 MS patients remains on track for submission to the FDA in April.

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**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. The products in ANP's development pipeline are in-licensed from Ionis Pharmaceuticals Inc., world leaders in antisense drug development and commercialisation. ATL1102 (injection) 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 successfully reduced blood IGF-I levels in Phase II clinical trials in patients with the growth disorder acromegaly.