

**ASX:ANP** 



## **Forward Looking Statements**

This presentation contains forward-looking statements regarding the Company's business and the therapeutic and commercial potential of its technologies and products in development. Any statement describing the Company's goals, expectations, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those risks or uncertainties inherent in the process of developing technology and in the process of discovering, developing and commercializing drugs that can be proven to be safe and effective for use as human therapeutics, and in the endeavor of building a business around such products and services. Actual results could differ materially from those discussed in this presentation. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the Antisense Therapeutics Limited Annual Report for the year ended 30 June 2014 and the report for Half Year ending 31 December 2014, copies of which are available from the Company or at www.antisense.com.au.



## **Antisense Therapeutics Ltd (ASX:ANP)**

- Advanced stage product pipeline with multiple disease applications and significant commercial potential
- ✓ Compounds licensed from global antisense technology leader, Isis Pharmaceuticals (Nasdaq: ISIS Market Capitalisation US\$8 Billion)
- ✓ Only Australian Biotech company with two drugs with positive Phase II clinical data
- ✓ Partnered with Cortendo AB to develop and commercialise ATL1103 for acromegaly
  - ✓ Licensing income with upfront payment received plus potential future milestone payments and royalties
  - ✓ Future development costs to be funded by highly focussed, experienced and committed drug development and commercialisation partner
- ✓ 2<sup>nd</sup> drug ATL1102 for multiple sclerosis partnered with myTomorrows to establish an Early Access Program for patients
  - ✓ Set up and running costs born by partner
  - ✓ Potential for cash flow positive sales to commence in 2015
- ✓ Strengthened balance sheet

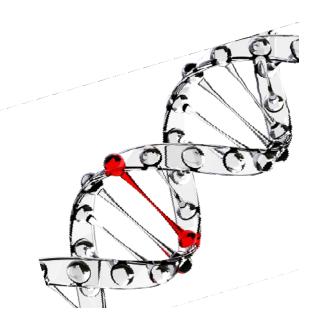


## **Corporate Snapshot**

KEY FINANCIALS	
Market Capitalisation	A\$19M
Cash as at 31 March 2015	A\$1.5M
Ordinary shares on issue	176M
Share price	\$0.11
Post 31 March 2015 - ATL1103 licensing deal	\$6.2M

Top Shareholders	
Cortendo	8.5%
Circadian Technologies	8.1%
Leon Serry & Associated Companies	3.9%
ISIS Pharmaceuticals	3.3%





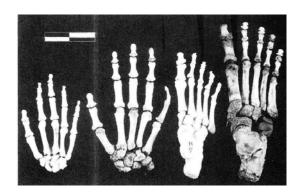


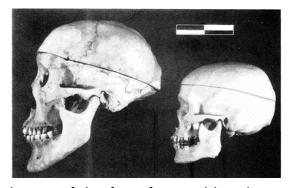
## ATL1103 Acromegaly



## Acromegaly – what is it?

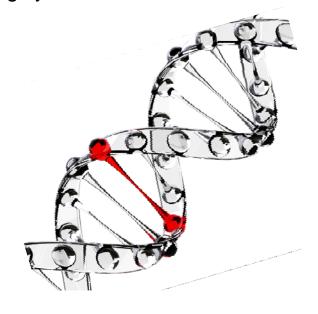
- Acromegaly is the abnormal enlargement of organs and bones of the face, feet and hands
- Due to a benign tumor (adenoma) of the pituitary gland causing over-production of Growth Hormone (GH) and Insulin-like Growth Factor 1 (sIGF-I) leading to diabetes, hypertension, and cancer
- Affects ~85 per million in the US and Europe (~85,000 adults)
- Orphan disease
- Normalising IGF-I blood levels is the treatment goal for acromegaly





Images depict a comparison between bones of the face, feet and hands





## **ATL1103** for Acromegaly

#### Current treatments

- Surgical treatment curative in 80-90% of microadenomas and <50% of macroadenomas</li>
- Approved first line therapies somatostatin agonists [SSA's] effective in 45-70% of cases only (Global Sales ~ \$1B/annum)
- 1 drug used for SSA failures Somavert® (Global Sales >\$200M/annum)
  - Difficult to administer (daily injection that requires preparation by patient)
  - Estimate capturing just 25% of market due to high cost and poor compliance

### ATL1103 Phase II trial successfully completed

• Primary efficacy endpoint met with a statistically significant reduction of sIGF-I levels of 26% at the 400mg/week dose



## ATL1103 for Acromegaly – Project Status

- Executed exclusive license agreement with Cortendo AB (Cortendo) for the development and commercialisation of ATL1103 (COR-004) for endocrinology applications
- Cortendo is a biopharmaceutical company incorporated in Sweden and based in the USA
  - Focused on developing treatments for rare diseases, and is initially developing product candidates for the treatment of endogenous Cushing's syndrome and acromegaly
  - Cortendo intends to independently commercialize its rare disease focused product candidates, if approved, in the U.S., the E.U. and in key global markets
  - Cortendo plans to source new product candidates by acquiring or in-licensing them from other companies or academic institutions
  - Cortendo recently announced capital raising of approx US\$33 million with leading institutional investors such as RA Capital, NEA, Broadfin, HealthCap, Longwood and TVM Capital for continued development of pipeline including ATL1103
- ANP undertaking higher dose study of ATL1103 in acromegaly patients in Australia



# Cortendo and Antisense Therapeutics Licensing Agreement for ATL1103 for Acromegaly

### Terms of the agreement

- Cortendo provided ANP with an upfront payment of US\$5m (A\$6.2m), consisting of US\$3m (A\$3.7m) in cash and a US\$2m (A\$2.5m) investment in ANP equity (@A\$0.1675/share; escrowed for up to 2 years)
- Additional payments, contingent upon achieving specific development (clinical and regulatory) and commercialization milestones, may total up to US\$105m (A\$131m) over the lifetime of the agreement.
- Royalty payments based upon sales performance
- Cortendo are responsible for the ongoing clinical development, regulatory interactions and commercial offering of ATL1103 in endocrinology applications and will fund the associated future development, regulatory and drug manufacture costs
- ANP retains commercialization rights for ATL1103 in endocrinology applications in Aus. and NZ, and worldwide rights for other ATL1103 indications, and may utilize new ATL1103 data generated by Cortendo in pursuing other indications



# Cortendo intends to drive the development of COR-004/ATL1103

### **Intended Next Steps**

- Complete the high-dose study currently underway
- Manufacture product for use in longer-term tox studies to support longer clinical studies and for clinical development
- Initiate longer term tox and repro tox studies
- Begin manufacturing scale up process
- Initiate meetings with US and EU regulators to establish development path and prepare for IND/CTA





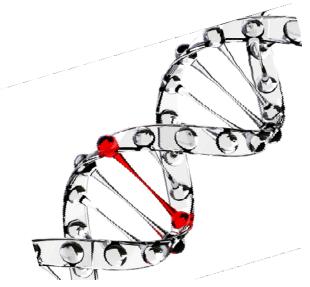
## ATL1102 Multiple Sclerosis



## **ATL1102** for Multiple Sclerosis

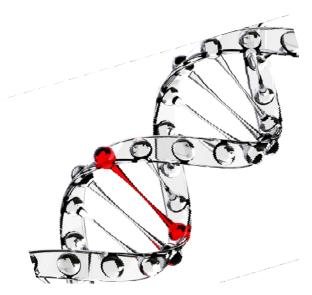
- Multiple Sclerosis (MS) is a chronic, progressive, and debilitating autoimmune disease that affects central nervous system, brain and spinal cord
- Global sales for MS drugs in 2013 were US\$14 Billion
- ATL1102 is an antisense inhibitor of VLA-4 protein a clinically validated target in MS
- Successful Phase II trial completed in patients with Relapsing Remitting-MS
  - Met primary end point after only two months of dosing reducing the cumulative number of new active brain lesions by 54.4% (p=0.01) compared to placebo





### **Project Status: ATL1102 for MS**

- US and EU patent allowances extending patent protection to 2029
- Positive response received from FDA on Pre-IND assessment for a Phase IIb trial
- Phase IIa trial results published in the Journal of the American Academy of Neurology
- Engaged in the process to attract a pharmaceutical company partner to undertake the Phase IIb trial
- Early Access Program now established initially for use of ATL1102 in Europe



## Early Access Program (EAP)

### EAP offers patients access to new non-registered pharmaceuticals

- Products may be provided to patients with a life threatening or debilitating disease where no alternative or appropriate therapy exists
- > 1,000,000 patients annually in Europe alone are left without treatment options with only a small percentage able to access new therapies via clinical trials<sup>1</sup>
- Can charge for drug access in certain markets
- Product must have been in Phase 2 trials or later and have shown evidence of efficacy

### **EAP Pricing**

- Pricing set by the Sponsor (ANP) at comparable price to registered products
- MS treatment costs in Europe range from A\$25,000–\$33,000 per patient per year<sup>2</sup>

<sup>&</sup>lt;sup>1</sup> Estimated by myTomorrows

<sup>&</sup>lt;sup>2</sup> Pricing for Tysabri, Gilenya, & Tecfidera -Toumi M and Jadot G in J. Market Access and Health Policy 2014, 2: 23932

## **EAP in Europe for ATL1102**

#### **MS Market**

- 400,000 people with MS in Europe and more than 1,000,000 world wide
- Significant number of patients do not properly respond to existing therapies suggesting a pool of possible candidates for use of ATL1102 under the EAP conditions

### **EAP Establishment for ATL1102**

- Partnering Agreement signed with EAP specialists myTomorrows
- myTomorrows to perform EAP activities at their cost including data collection and seeking EAP approvals initially in Europe in those markets where drug use is reimbursed
- ANP to provide ATL1102 product for use in the EAP
  - Arrangements being established to source ATL1102 from existing supplies (2 batches) for potential use in the EAP
  - Proposal to initially source one batch of ATL1102 to commence the EAP
  - Subject to this first batch of material being confirmed (through retesting) as of suitable quality for use in the EAP, the quantity of material is expected to be sufficient for one year's treatment for over 150 patients at the 200mg/week dose
  - Material could be available as early as 4'Q'15 for use in the EAP
  - Potential cash flow positive income to commence in 2015

### **myTomorrows**

#### PROVIDING EARLY ACCESS TO DRUGS IN DEVELOPMENT

myTomorrows is creating freedom of choice for physicians and patients with unmet medical needs by offering earlier access to medicines that show promising results during clinical trials, but are not officially registered yet. With the support of their doctors, patients who suffer from cancer, a neurological disorder, a rare disease or a severe depression, can have earlier access to such medicines. myTomorrows identifies innovative drugs, informs physicians and patients and facilitates requests for access to these drugs in development via a world-wide Internet-based platform.

For more information about myTomorrows, please visit the website <a href="https://www.mytomorrows.com">www.mytomorrows.com</a>.



## **Antisense Therapeutics**

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