

Antisense Therapeutics

ASX:ANP

September 2015

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 2015, copies of which are available from the Company or at www.antisense.com.au.

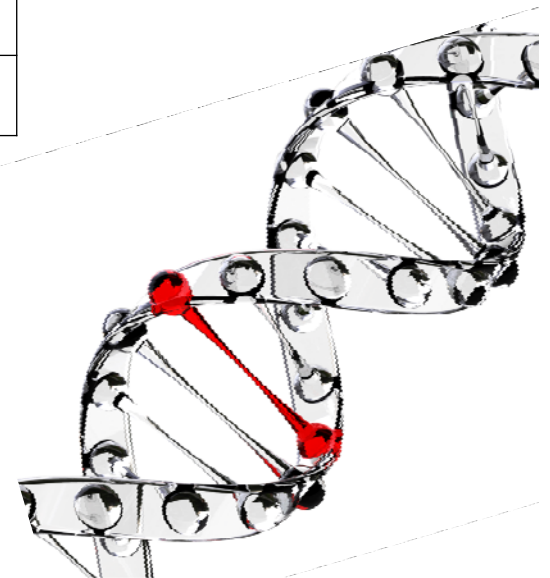
Antisense Therapeutics Ltd (ASX:ANP)

Highlights

- ✓ 2 drugs with positive Phase II clinical data
- ✓ ATL1103 for acromegaly - partnered with Strongbridge Biopharma (formerly Cortendo AB) to further develop and commercialise
 - ✓ US\$5M (A\$6.2M) upfront payment received
 - ✓ Additional licensing payments up to US\$124m (A\$177m), if milestones are successfully achieved, plus royalty payments of up to 15% on net sales
 - ✓ Future development costs to be funded by highly focussed, experienced and committed drug development and commercialisation partner
- ✓ ATL1102 for multiple sclerosis - partnered with myTomorrows to establish an Early Access Program for patients (commencing in Europe)
- ✓ Strengthened balance sheet with ATL1103 upfront licensing payment

Corporate Snapshot

| KEY FINANCIALS | |
|-----------------------------------|--------|
| Market Capitalisation | A\$14M |
| Cash as at 30 June 2015 | A\$6.8 |
| Ordinary shares on issue | 176M |
| Share price | \$0.08 |
| SUBSTANTIAL SHAREHOLDERS | |
| Strongbridge Biopharma (Cortendo) | 8.5% |
| Circadian Technologies | 8.1% |



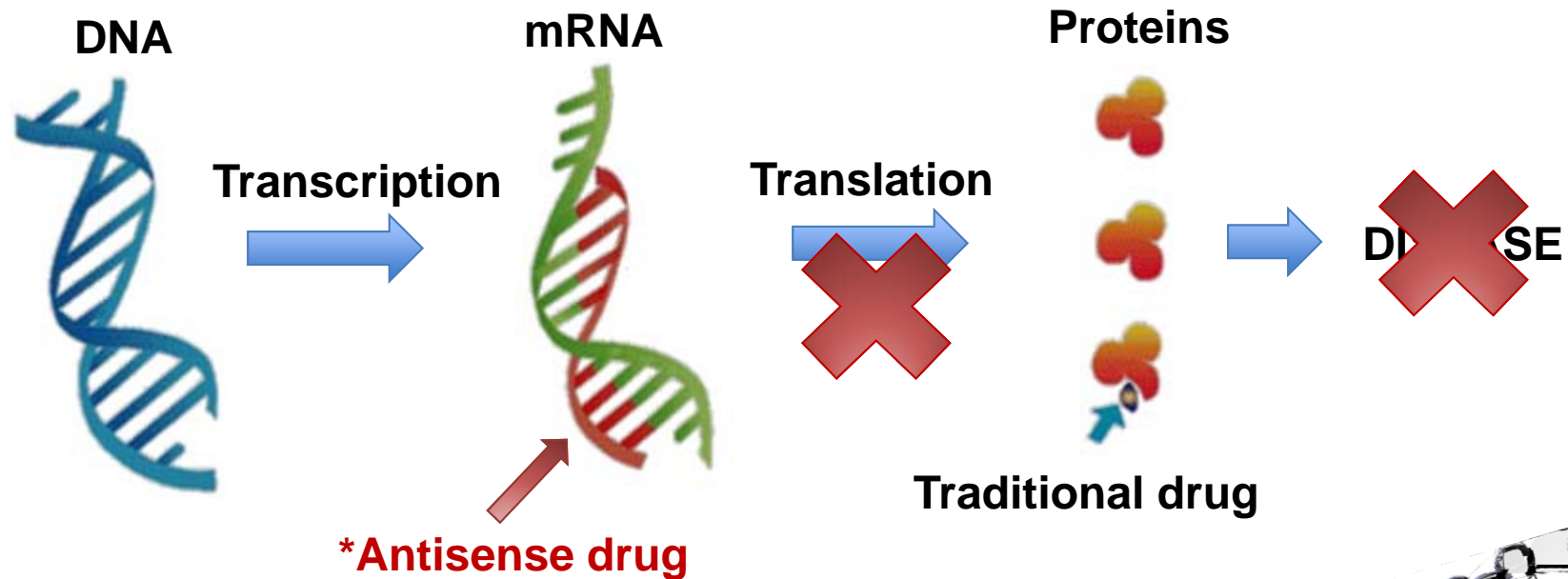
Product Pipeline

- ✓ Developing for commercialisation an advanced stage pipeline of antisense compounds for diseases where there is a need for improved therapies

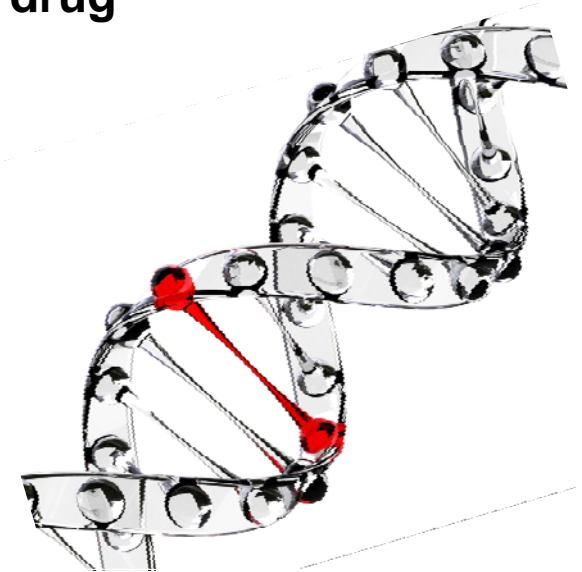
| PRODUCT | INDICATION | RESEARCH | PRECLINICAL | PHASE I | PHASE II | PHASE III |
|---------------------------|--------------------|---|--------------------------------|--|--|-------------------------------|
| ATL1103 s.c. injection | Acromegaly | [Red bar spanning Research, Preclinical, Phase I, and Phase II] | | | | [Grey bar spanning Phase III] |
| ATL1102 s.c. injection | Multiple Sclerosis | [Red bar spanning Research, Preclinical, and Phase I] | | | [Red bar spanning Phase II] | [Grey bar spanning Phase III] |
| ATL1101 s.c. injection | Prostate Cancer | [Red bar spanning Research and Preclinical] | | [Red bar spanning Phase I] | [Grey bar spanning Phase II and Phase III] | |
| ATL1102 inhaled | Asthma | [Red bar spanning Research] | [Red bar spanning Preclinical] | [Grey bar spanning Phase I, Phase II, and Phase III] | | |

All compounds are in-licensed from Isis Pharmaceuticals, world leaders in antisense drug development and commercialisation (NASDAQ: ISIS, Market Capitalisation US\$6 Billion)

Antisense - How does it work?



**Small (12-25 nucleotides) DNA or RNA-like compounds that are chemically modified to create highly targeted drugs*



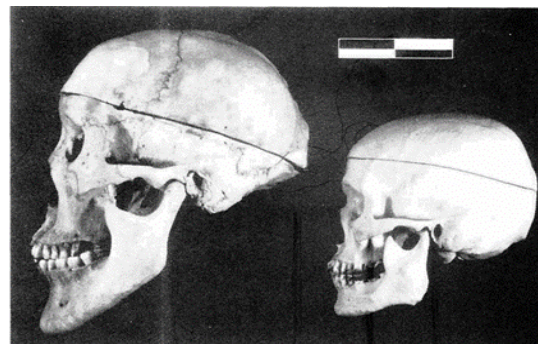
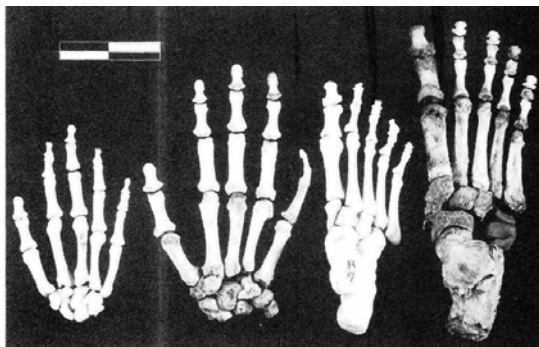


ATL1103

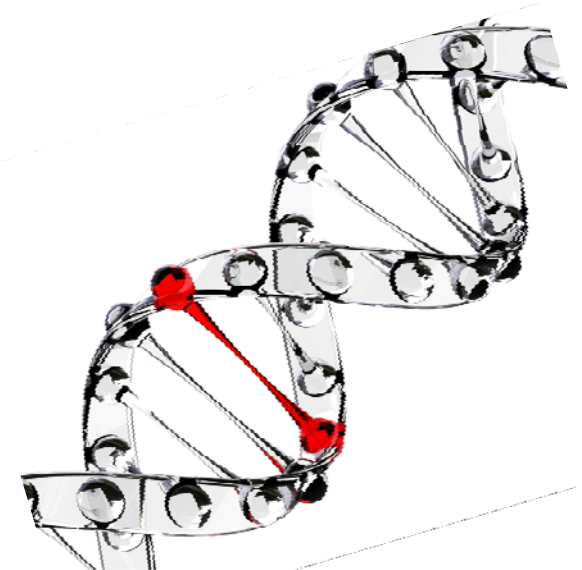
Acromegaly

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 – regulatory assistance and IP protection incentives to develop
- **Normalising IGF-I blood levels is the treatment goal for acromegaly**



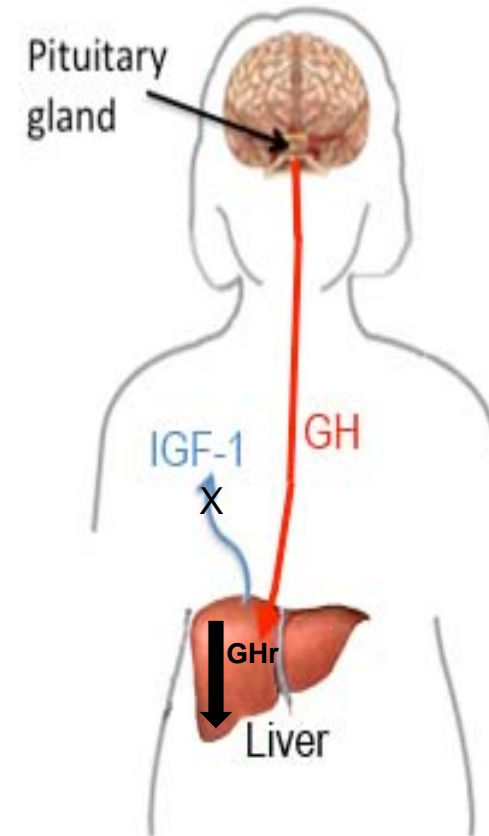
Images depict a comparison between bones of the face, feet and hands



ATL1103 for Acromegaly

ATL1103 targets Growth Hormone receptor (GHR)

- ATL1103 reduces expression of GHR in the liver & blocks GH action on the liver, which reduces sIGF-I
- Normalising sIGF-I is the treatment goal in acromegaly
- ATL1103 has suppressed sIGF-I in all animal and human studies undertaken to date
 - ✓ Repeat mouse studies, 6 week non human primate study, 3 week multiple dose Phase I study in volunteers and 13 week Phase II study in acromegalic patients
- Reducing sIGF-I also has a potential role in the treatment of diabetic retinopathy, nephropathy and certain cancers



ATL1103 for Acromegaly

Current treatments

- Surgical treatment curative in 80-90% of microadenomas and <50% of macroadenomas
- Approved first line therapies - somatostatin agonists [SSA's] e.g. octreotide - effective in 45-70% of cases only (Global Sales ~ \$1B/annum)
- 1 drug used for SSA failures Somavert® (*Global Sales >\$200M/annum*)
 - *Expensive (up to 3 times cost of first line therapies)*
 - *Difficult to administer (daily injection requiring preparation by patient)*
 - *Somavert® estimated to be capturing just 25% of market due to high cost and poor patient compliance*

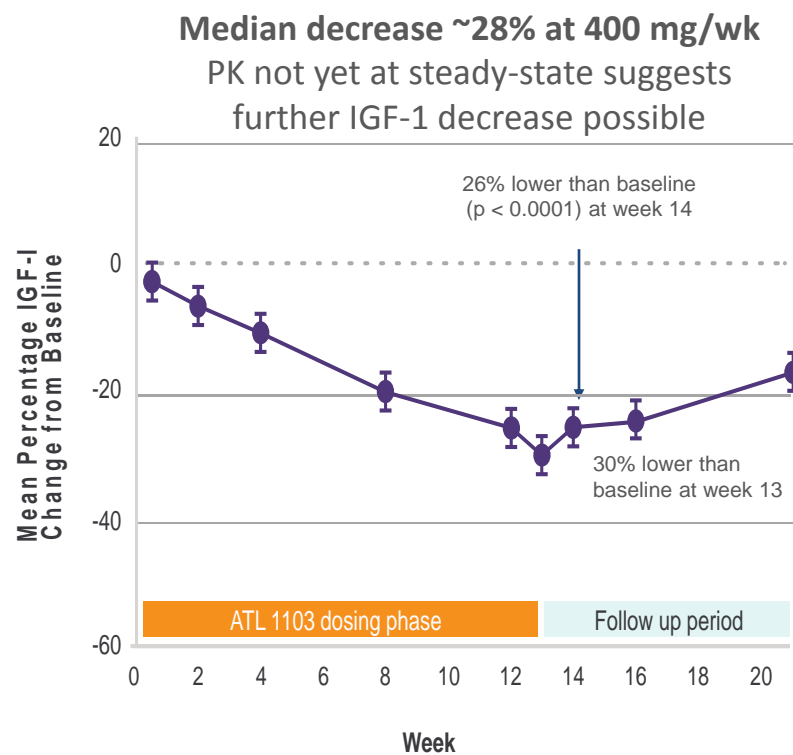
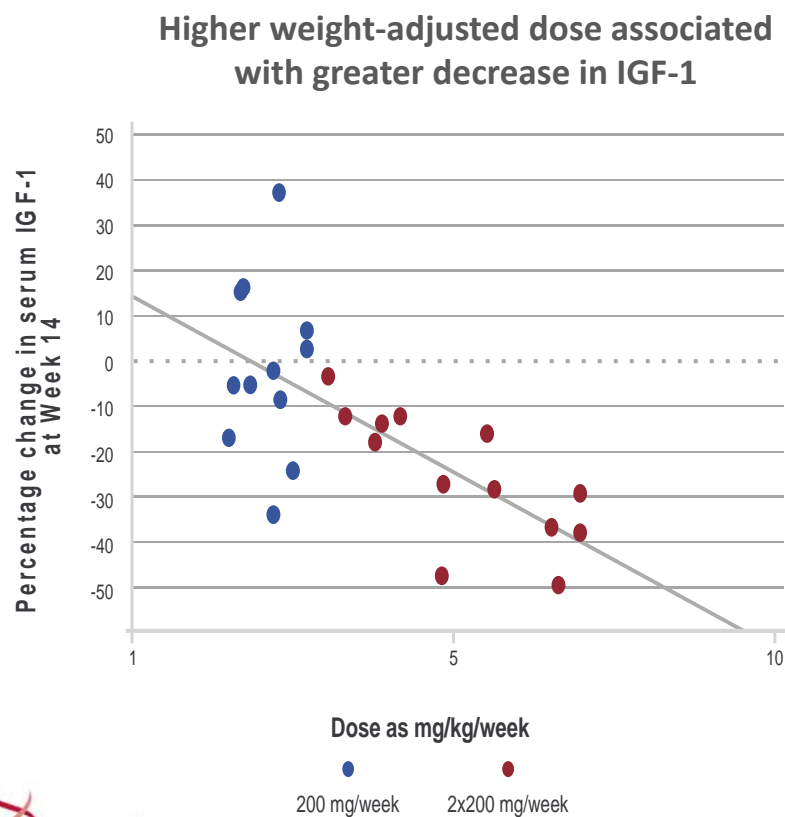
ATL1103 for first line therapy failures

- Potential advantages include lower cost of therapy, improved safety profile, and more convenient dosing and administration



Phase 2: Primary Efficacy Endpoint Met

- Treatment for 13 weeks shows dose-dependent decrease of IGF-I; potential increase of efficacy with longer treatment
- Statistically significant reduction of sIGF-I levels of 26% at the 400mg/week dose



Licensing Agreement for ATL1103 for Acromegaly

Agreement with Strongbridge Biopharma (formerly Cortendo) for the development and commercialisation of ATL1103 (COR-004) for endocrinology applications

- Strongbridge is a biopharmaceutical company focused on developing treatments for rare diseases including acromegaly
- ANP received **upfront payment of US\$5m (A\$6.2m)**
- Additional payments of up to **US\$124m (A\$177m)** if milestones are achieved
- Royalty payments of up to 15% on net sales
- Strongbridge to conduct ongoing clinical development and commercialisation of ATL1103 in endocrinology applications and will fund the future development, regulatory and drug manufacture costs
- ANP retains commercialisation rights for ATL1103 in endocrinology applications in Australia and New Zealand, and worldwide rights for other ATL1103 indications
- ANP may utilize new ATL1103 data generated by Strongbridge in pursuing other indications such as cancer, diabetic retinopathy and nephropathy

ATL1103/COR-004 Licensing Agreement – Milestone Events

- Start of the first Phase III Trial for ATL1103/COR-004 for the first Acromegaly Indication in any jurisdiction outside Australia & New Zealand
- Filing of New Drug Application in the U.S. for ATL1103/COR-004 for the first Acromegaly Indication
- Filing of Marketing Approval Application in the European Union for ATL1103/COR-004 for the first Acromegaly Indication
- US Approval (excluding Pricing Approval) for ATL1103/COR-004 for the first Acromegaly Indication
- EU Approval (excluding Pricing Approval) for ATL1103/COR-004 for the first Acromegaly Indication; and
- Japanese Approval (excluding Pricing Approval) for ATL1103/COR-004 for the first Acromegaly Indication

In addition, ANP is entitled to further payments based on achievement of the above milestones for a 2nd indication in Acromegaly and/or with respect to any other endocrinology indication. There are also commercial milestones based on sales performance targets with respect to the first acromegaly indication. These payments in total would add up to US\$124Million if milestones are successfully achieved.

Strongbridge to drive the development of ATL1103/COR-004

Intended Next Steps

- ANP completing the high-dose study currently underway (costs to be reimbursed by Strongbridge)
- Strongbridge to seek orphan drug designation for ATL1103/COR-004 from the FDA and EMA
- Strongbridge plan to conduct Phase III enabling chronic toxicology studies in two animal species in parallel with seeking a pre-IND meeting with the FDA in 2nd Half 2015 to discuss requirements for entry into Phase III clinical development
- Strongbridge then intend to file an IND for ATL1103/COR-004 in the US and begin multinational development program to support regulatory approval in the US and EU

R&D Acromegaly Company Valuations

Chiasma Inc

- One product at clinical stage of development only - oral octreotide capsules
- Completed Phase 3 in acromegaly; New Drug Application accepted for filing by FDA
- July 2015 successful US NASDAQ IPO raising approx US\$117M
- Current Market Capitalisation US\$670M (A\$970M)

Strongbridge Biopharma

- One product in Phase 3 for cushing syndrome, 2 products with Phase 2 data in acromegaly (including ATL1103/COR-004)
- Traded on the Norwegian Over the Counter Market (NOTC-A)
- Current Market Capitalisation NOK 2.6B (A\$350M)
- Filed Registration Statement for US NASDAQ IPO to raise US\$86M



ATL1103 for Cancer and Diabetic Complications

Rationale for ATL1103 for Cancer and Diabetic Complications

- ✓ ATL1103 is a clinically advanced drug that targets the Growth Hormone receptor (GHR)
- ✓ Inhibits GHR production in the liver thereby reducing IGF-I in the blood (sIGF-I)
- ✓ Reducing GH effects and sIGF-I has a potential role in the treatment of a number of diseases including Acromegaly, Cancer, Diabetic Retinopathy and Nephropathy
- ✓ Development of ATL1103 for Acromegaly/Endocrinology partnered with Strongbridge Biopharma (formerly Cortendo AB), however ANP retains world-wide rights for all other indications (e.g. cancer and diabetic complications)
- ✓ Existing ATL1103 toxicology and clinical data would support and expedite future patient trials in these new indications reducing the time and cost for moving into Phase II
- ✓ Patents granted to 2024/2025 with potential for up to 5 years extension
- ✓ Company reviewing the multiple ATL1103 value adding opportunities and expects to report on plans in the coming months

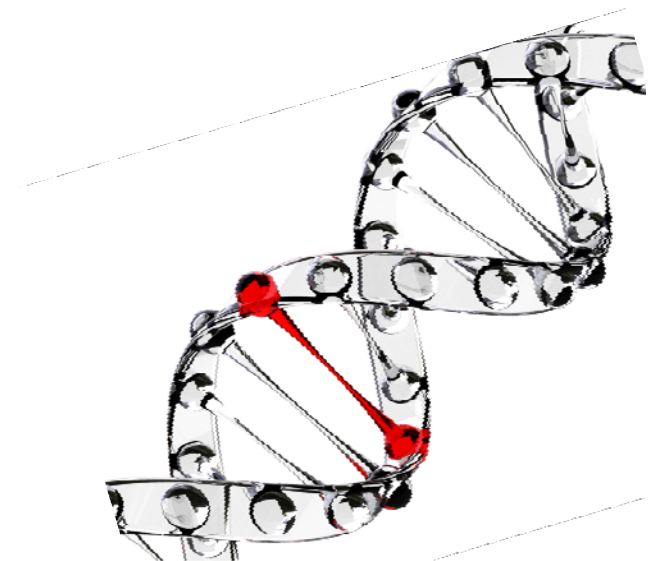


ATL1102

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 process to attract a pharmaceutical company partner to undertake Phase IIb
- Early Access Program (EAP) being established initially for use of ATL1102 in Europe where ANP and its partner myTomorrows may charge for drug access by MS patients not responding to other treatments
 - 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
 - myTomorrows are to perform EAP activities at their cost including data collection and seeking EAP approvals
 - ANP is currently working to firm up suitable ATL1102 drug product supply and the initial quantities for use in the program while myTomorrows is preparing documentation for physician education and the EAP approvals process

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Summary

- ✓ 2 drugs with positive Phase II clinical data in markets with significant unmet need
- ✓ Strong patent position
- ✓ Strengthened balance sheet with reduced development spending commitments
- ✓ Potential to generate revenue from Early Access Program for ATL1102 for multiple sclerosis in the near term
- ✓ Future ATL1103 licensing payments of up to US\$124m (A\$177m) through partnership with Strongbridge Biopharma (formerly Cortendo AB), if milestones are successfully achieved, plus royalty payments of up to 15% on net sales



Antisense Therapeutics

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