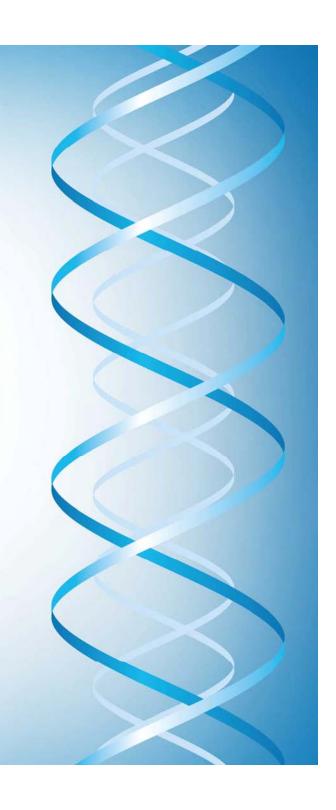


### **AGM Presentation**

1 November 2013

**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 commercialising drugs that can be proven to be safe and effective for use as human therapeutics, and in the endeavour 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 2013, copies of which are available from the company or at www.antisense.com.au.



## **Antisense Therapeutics Limited**

- ANP has exclusive world wide rights to 3 second generation antisense compounds inlicensed from Isis Pharmaceuticals Inc (Isis), world leaders in antisense drug development and commercialization
- Advanced staged product pipeline for diseases where there is a need for improved therapies
  - ATL1103 for acromegaly, cancer and diabetes associated disorders
    - Phase II trial in acromegaly patients underway with interim results end 2013
  - ATL1102 for multiple sclerosis, stem cell mobilisation and asthma
    - Completed successful Phase II clinical trial in MS patients
    - Chronic toxicology study underway with results due early 2014 to support a potential Phase IIb study in MS patients
    - Trial application for human PoC stem cell mobilisation study planned for submission end 2013 with trial results anticipated mid 2014
  - ATL1101 for prostate cancer
    - Positioned to move into clinical development



### **Newsflow/Achievements**

Following are just some of the news items announced by the Company since January 2013:

25/01/2013	ATL1103 Phase II trial - patient enrolment commences
30/01/2013	FDA Approval of KYNAMRO
12/02/2013	ATL1102 US Patent Allowance and Development Progress
19/03/2013	ATL1102 Development and Agreement Update
10/04/2013	ATL1103 Phase II trial - dosing of patients commences
1/05/2013	ATL1102 for Multiple Sclerosis -Toxicology Study to Commence
4/06/2013	ATL1102 for MS - Dosing Starts in Key Toxicology Study
14/06/2013	Positive New Animal Data on ATL1103
2/08/2013	ATL1103 Phase II Trial Update
25/09/2013	ATL1102 for Multiple Sclerosis
27/09/2013	Proposed Consolidation of Securities & Loyalty Option Issue
1/10/2013	ATL1102 for Stem Cell Mobilisation
17/10/2013	ATL1103 Phase II Trial Update - Interim Analysis

# **Conferences/Presentations**

4/02/2013	ANP to Present at BIO CEO and Investor Conference
13/03/2013	Investor Presentation
15/03/2013	ANP to Present at ROTH Capital Partners Conference
22/03/2013	ATL1103 Phase I data presentation at Acromegaly Conference
8/04/2013	Antisense Newsletter
28/08/2013	ANP at Major US Investment Partnering & Industry Conferences
11/09/2013	Rodman & Renshaw Global Investment Conference Presentation
11/10/2013	Bloomberg Brief US Interview
17/10/2013	Article on ANP by Genetic Engineering and Biotechnology News
23/10/2013	ANP to Present at BioTech and Healthcare Investor Roadshow
25/10/2013	Acromegaly Scientific Conference Presentation
28/10/2013	ANP to Present at Australia Biotech Invest 2013

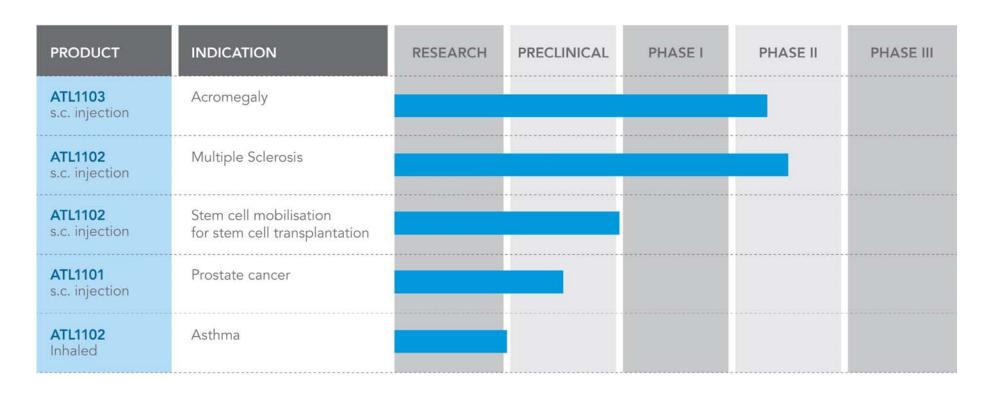
## ANP's technology partner - Isis Pharmaceuticals Inc

### Isis Pharmaceuticals – ANP's technology partner

- Leaders in antisense drug development and commercialisation
- Nasdag listed: Market capitalisation \$3.8 Billion
- 30 drugs in development 5 drugs with launch potential in next 5 years
- Partnerships with Major Pharma Co's; GSK, Astra Zeneca, Genzyme and Biogen Idec
- 5 Licensing transactions announced in 2012
- KYNAMRO™ for cholesterol reduction in high risk individuals partnered with Genzyme
  - 2<sup>nd</sup> generation ASO = same chemistry as compounds in ANP's pipeline
  - Jan 2013 FDA approval = first approved systemically administered antisense drug
- Isis 12 month share price performance: \$39.83 \$7.55 (Current price: \$34.15)



# **Research & Development Pipeline**



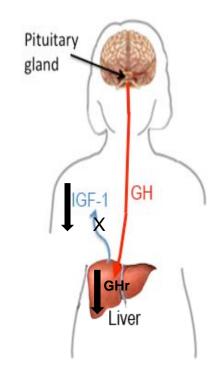
All pipeline drugs are 2nd generation antisense compounds derived via Isis collaboration.



## ATL1103 – Antisense Drug to the Growth Hormone receptor

- ATL1103 is a 2<sup>nd</sup> generation antisense drug to the Growth Hormone receptor (GHr) in development for the growth disorder acromegaly
- Acromegaly is caused by a benign tumour of the pituitary gland that over-produces Growth Hormone (GH) leading to increased blood levels of Insulin-like Growth Factor 1 (sIGF-I)
  - Elevated sIGF-I causes enlargement of organs and the bones of the face, feet and hands. Can also lead to diabetes, hypertension, and cancer
  - Affects ~85 adults per million in the US and Europe (~85,000 adults)
  - Normalising sIGF-I is the treatment goal
  - Orphan drug indication regulatory and IP incentives to develop

ATL1103 reduces liver GHr & blocks GH action on the liver reducing IGF-I in the blood





## **ATL1103 – Treatment of Acromegaly**

- Approx 60% of acromegaly patients are treated by surgical removal of the tumour surgical failures require drug therapy to normalise sIGF-I
- First line drug therapy: somatostatin agonists of somatostatin 2 receptor effective in up to 65% of cases
  - Treatment costs up to A\$30K/annum: Existing sales of somatostatin agonists
     ~\$1Billion/annum
- Pegvisomant (Somavert®) GHr antagonist for first line therapy failures
  - Estimated sales of > \$200 million/annum: Treatment costs of A\$60K/annum or more
  - Use and effectiveness in clinical practice is limited by high cost, inconvenient administration and dosing regimen (e.g. daily injection) negatively impacting its sales potential
- ATL1103 has been shown in animal studies to significantly reduce sIGF-I and has demonstrated a preliminary indication of activity with sIGF-I reduction in a Phase I study in healthy volunteers

ATL1103's target (GHr) is validated by the effects shown by Somavert® however anticipate important advantages over Somavert® including lower cost of therapy, improved safety profile, and a more convenient dosing and administration regimen



### **ATL1103 Project Status**

### Phase II clinical trial underway

- Conducted in Europe (UK, France and Spain)
- 13 week dosing study in 24 acromegaly patients
- Open label, baseline comparison of 2 doses of ATL1103 200mg once and twice per week
- Endpoints: safety, pK tolerability and drug activity including the level of sIGF-I reduction
- Interim analysis of ATL1103's effect on sIGF-I levels results due by end 2013
- Final results anticipated 2'Q'2014
- Next stage of development: Phase III registration studies in acromegaly post Phase II trial success

#### Recent relevant commercial transaction

- In Feb 2013 Roche acquired rights to acromegaly drug Octreolin in Phase III development in a deal worth US\$595m (US\$65million upfront and milestone payments up to US\$530m)
- Provides guide on value of ATL1103 program which, subject to successful Phase II trial outcomes, could move into Phase III studies in 2014



### ATL1102 in MS

#### **Product**

- 2nd generation antisense inhibitor of VLA-4 protein
- VLA-4 is a clinically validated target in MS and inhibition of VLA-4
- ANP successfully completed a Phase II trial of ATL1102 confirming its activity and safety in RRMS patients
  - ATL1102 demonstrated comparable/potentially superior activity to the VLA-4 monoclonal antibody drug Tysabri<sup>®</sup> at the same stage of development
  - Tysabri® is the current efficacy benchmark for RRMS treatment sales of \$1.6 Billion/annum
  - In 2013 Biogen Idec gained all rights to Tysabri from Elan for an upfront of US\$3.25Billion plus payments on future sales

ATL1102 profiles as a highly potent, self administrable drug for the treatment of RRMS (Tysabri® is an iv infusion) and potentially safer and cheaper to manufacture than Tysabri®

### **Project Status**

- Chronic monkey toxicology study underway with results due early 2014 to support a potential Phase IIb study of ATL1102 in MS patients in 2014
- New US patent granted to 2029 and potentially extendable to 2034



## ATL1102 Stem Cell Mobilization (SCM) in Cancer

#### **Product**

- ATL1102 as an acute treatment (1 week dosing) for use with standard GCSF treatment to enhance stem cell mobilisation
- SCM = bone marrow hematopoeitic stem cells (CD34+) are mobilized to the blood for collection before high dose chemotherapy and then re-infused to reestablish immune system post chemotherapy
- Neupogen<sup>®</sup> (Filgrastim) a GCSF agonist is the market leader in SCM: Sales of US\$1.3 Billion/annum
- Mozobil<sup>®</sup> (Plerixafor) used with GCSF to increase SC release. Expensive (\$7,500 per vial) and ~30% of patients still fail to achieve threshold mobilisation level: Sales of ~US\$130 million/annum
- In 2008 estimated that 55,000 patients could benefit from more SC release than with GCSF alone and in 2011 it was reported that globally around 10,000 patients/annum still failing mobilization

### **Project Status**

- ATL1102 increased CD34+ RNA in blood of MS patients at 8 weeks by 1.5 fold v baseline (P< 0.027)</li>
- Systemic animal safety studies completed to support human trials. Clinical safety data established in Phase I volunteer and Phase II MS patient studies
- Orphan drug indication (regulatory and IP incentives). Patent application seeking protection to 2031
- PoC trial planned in volunteers to test the drug's potential to release CD34+ SCs
  - Trial application to be submitted before end of year with results anticipated by mid 2014

# **Consolidation of Securities and Loyalty Option Issue**

- Shareholder approval for a consolidation of its securities on a 10 to 1 basis
- The aim of the consolidation is to reduce the number of shares on issue to a level comparable to our peers
- The consolidation also provides the benefit of:
  - reducing share price volatility
  - reducing negative investor perceptions associated with a low share price
  - positioning ANP to be more attractive to longer term investors in particular local and O/S institutions
  - administrative cost benefits

Key Event	Date
Approval of consolidation at Annual General Meeting	1 November 2013
Last day for trading in pre-consolidation Shares	5 November 2013
Trading in post-consolidation Shares on a deferred settlement basis begins	6 November 2013
Last day for entity to register transfers on a pre-consolidation of basis	12 November 2013
First day for entity to register shares on a post-consolidation basis and to send shareholder notice	13 November 2013
Last day for entity to register shares on a post consolidation basis and to send shareholder notice	19 November 2013
Deferred settlement market ends	19 November 2013
Trading resumes on a T + 3 basis	20 November 2013



# **Loyalty Option Issue**

- Recognise the continuous support of its loyal shareholders
- 1 for 3 non-renounceable entitlement offer
- One new option for every three existing shares held on the 28 November 2013 at \$0.27 (on a post consolidation basis) on or before 31 January 2017 (New Options)
- New options will have an upfront issue price of \$0.012 each (post consolidation)
- Funds from the Loyalty Offer are expected to be used to progress the Company's development pipeline including commencing the ATL1102 Stem Cell Mobilisation Study

Key Event	Date
Announcement of The Loyalty Offer	27 September 2013
Prospectus lodged with ASIC and ASX	20 November 2013
Ex-date for The Loyalty Offer	22 November 2013
Record Date to determine entitlement to participate in The Loyalty Offer	28 November 2013
Prospectus and Entitlement and Acceptance Forms despatched to Eligible Shareholders	2 December 2013
Loyalty Offer Opens	2 December 2013
Loyalty Offer Closes	16 December 2013
Shortfall notice lodged with ASX	19 December 2013
Allotment of options and despatch of holding statements for options	24 December 2013
Official quotation of options on ASX expected to commence	27 December 2013



## **Corporate Initiatives**

- Look to engage experienced US based Corporate Advisory and Consulting Firm (prior to interim ATL1103 Phase II trial results) to help the Company set up and execute licensing plans to optimize outcomes and to best position ANP to extract value from its project assets on a global basis
- Report Phase II interim data on ATL1103 end of 2013 and;
- Report outcomes from ATL1102 chronic toxicology study in early 2014
  - Ramp up ATL1103 and ATL1102 partnering activities on positive outcomes with US corporate advisory firm
- Complete Loyalty Option issue and use funds to commence Proof of Concept ATL1102 stem cell mobilisation study by end 2013 (study results due mid 2014)
- Employ potential future licensing income to fund and continue development of pipeline including ATL1102 stem cell mobilisation project on back of positive results



## **ANP Investment Highlights and Near Term Value Drivers**

- Commercializing a platform technology via collaboration with the leader in antisense therapeutics development, Isis Pharmaceuticals
- Technology validation provided by the first of the class approved for systemic administration (Kynamro<sup>™</sup>) and Isis's technology deals with Big Pharma
- 4 advanced antisense programs with multiple disease applications and significant commercial potential supported by relevant licensing/commercial transactions in the space
  - ATL1103 Phase II acromegaly trial underway with interim results end 2013
  - ATL1102 in MS Chronic toxicology study underway (results due early 2014) to support
    a potential Phase IIb study in MS patients
  - ATL1102 in Stem Cell Mobilisation Trial application for human PoC study planned for submission end 2013 with trial results anticipated mid 2014
  - ATL1101 Positioned to move into clinical trials in prostate cancer

