23 October 2013

# ANP to Present at Canary BioTech and Healthcare Investor Roadshow

Antisense Therapeutics Ltd. (ASX:ANP) today announced that CEO and Managing Director, Mark Diamond, has been invited to present at the Canary Biotech and Healthcare Investor Roadshow in Melbourne on Thursday the 24 October 2013. Attendees at the Investor Roadshow include fund managers, stockbrokers, retail investors as well as biotech and healthcare analysts and industry leaders.

Please see attached presentation.

**For more information on the Canary Biotech and Healthcare Investor Roadshow please go to:**

http://www.canarynetworks.com.au/upcomingforums.php

**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. ANP has 4 products in its development pipeline that it has in-licensed from Isis Pharmaceuticals Inc., world leaders in antisense drug development and commercialisation - ATL1102 (injection) which has successfully completed a Phase II efficacy and safety trial, significantly reducing the number of brain lesions in patients with multiple sclerosis, ATL1103 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 other GH-IGF-I disorders, ATL1102 (inhaled) which is at the pre-clinical research stage as a potential treatment for asthma and ATL1101 a second-generation antisense drug at the pre-clinical stage being investigated as a potential treatment for cancer

**Contact Information**: Website: www.antisense.com.au
Managing Director: Mark Diamond +61 (3) 9827 8999
Investor Relations: Simon Watkin +61 (0) 413 153 272

antisense THERAPEUTICS

# Canary Biotech and Healthcare Investor Roadshow

**24 October 2013**

**ASX:ANP**
**OTC:ATYHJ**

# Antisense Therapeutics Limited

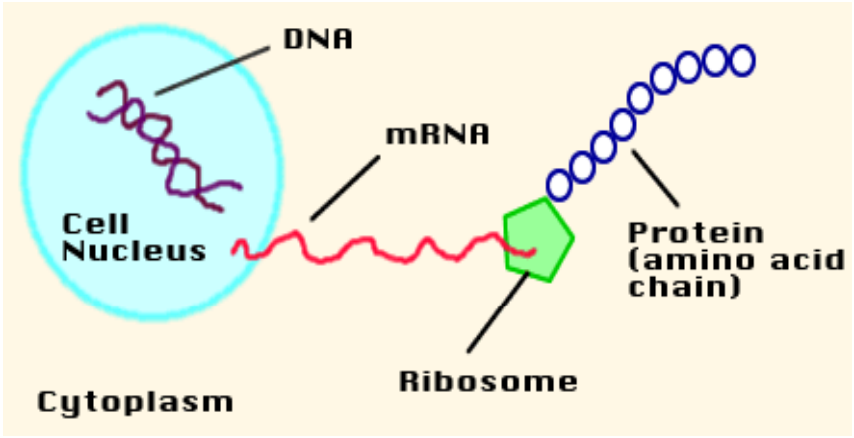
- ANP has exclusive world-wide rights to 3 second generation antisense compounds in-licensed from Isis Pharmaceuticals Inc (Isis), world leaders in antisense drug development and commercialization
- Advanced staged development pipeline for diseases where there is a need for improved therapies
  - ***ATL1103 for abnormal growth (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*

antisense THERAPEUTICS

# Antisense Technology

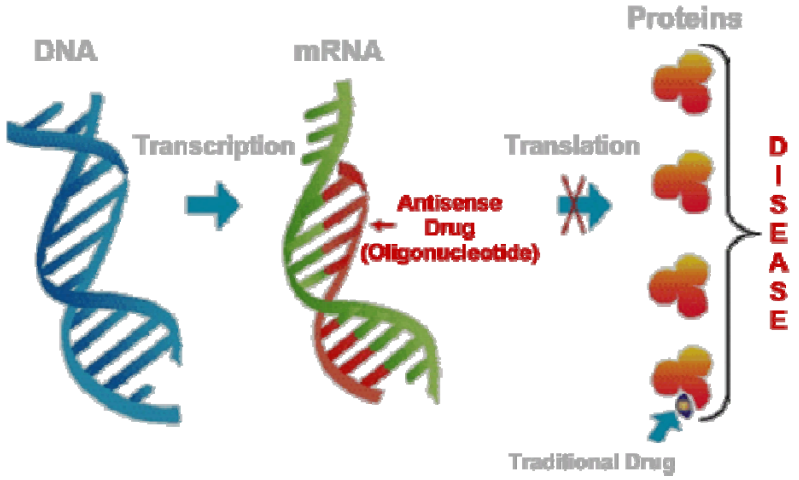
**How proteins are made**

- Proteins are the body's "work horses" (e.g. antibodies, hormones, enzymes)
- Most human diseases caused by inappropriate production of certain proteins
- The genes (in the nucleus of a cell) contain the instructions for making proteins
- A copy of these instructions is made (messenger RNA) which binds to ribosome and a protein is produced




**How antisense drugs work**

- Designed to **block the production of disease causing proteins** by binding to the specific messenger RNA sequence
- Platform technology = same chemistry used to produce multiple drugs for different diseases
- Highly targeted and potent therapeutics
- Isis 2nd generation antisense technology = modifications to increase potency and stability




antisense THERAPEUTICS

# ANP's technology partner - Isis Pharmaceuticals Inc

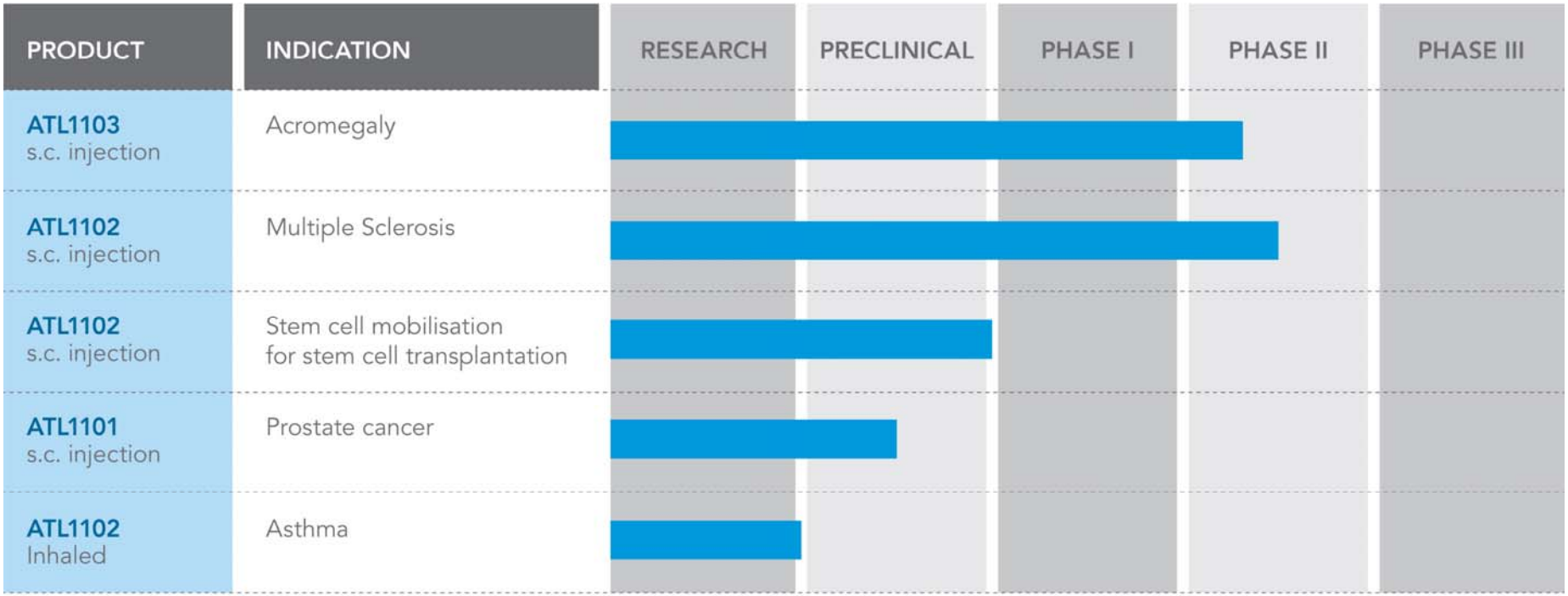
**Isis Pharmaceuticals – ANP's technology partner**

- Leaders in antisense drug development and commercialisation
- Nasdaq 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
  - *2nd 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)

***ANP has world wide exclusive rights to 3 second generation antisense drugs from Isis for all disease applications***

antisense THERAPEUTICS

# Research & Development Pipeline



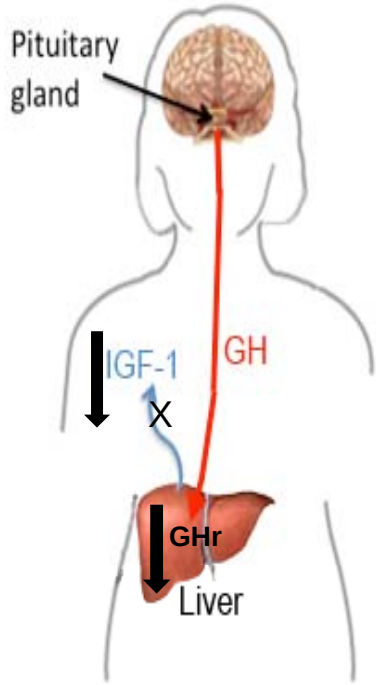

All pipeline drugs are 2nd generation antisense compounds derived via Isis collaboration.

antisense
THERAPEUTICS

# ATL1103 – Antisense Drug to the Growth Hormone receptor

- ATL1103 is a 2nd 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**




Adapted from neurosurgery.ucla.edu/body.cfm?id=101

antisense THERAPEUTICS

# 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***

antisense THERAPEUTICS

# 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

antisense THERAPEUTICS

# 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® 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

antisense THERAPEUTICS

# 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 ® (Filgrastim) a GCSF agonist is the market leader in SCM: Sales of US$1.3 Billion/annum
- Mozobil ® (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)
- 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*

antisense THERAPEUTICS

# ATL1101 for Prostate Cancer

**Product**

- ATL1101 is a 2nd generation inhibitor of IGF-IR (emerging target in oncology)
- IGF-IR inhibition blocks key cell survival/proliferation pathways
- Major Pharma Co's have mAb programs targeting IGF-IR confirming the attractiveness of the target in cancer drug development
- ATL1101 only antisense or 'gene silencing' approach to the target in development

**Project Status**

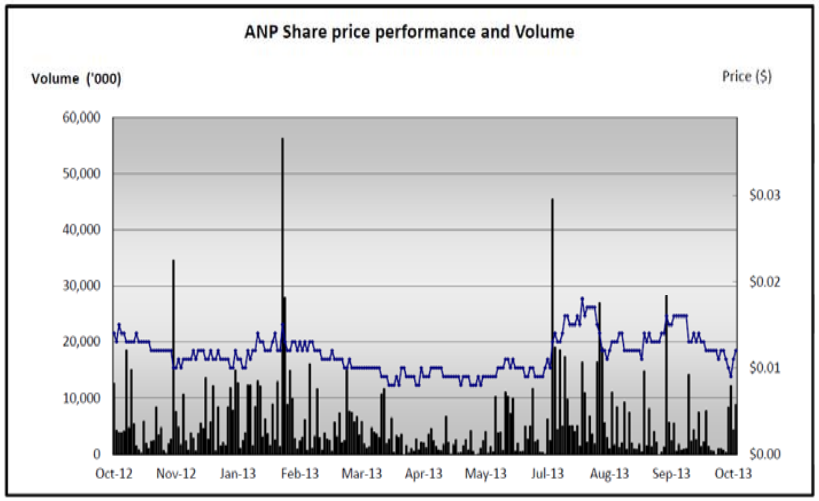
- Significant suppression of key tumour signalling pathways and prostate cancer tumour growth demonstrated in animal studies
- Select toxicology studies completed
- Drug potentially positioned to move into clinical development in prostate cancer patients
- ANP currently assessing development and partnering options for the drug

antisense THERAPEUTICS

# Antisense Therapeutics Limited

## Board of Directors

- **Mark Diamond** – MD and CEO 11 years (Ex Faulding – Project Planning/International Business Development); 25 years experience in pharma/biotech industry
- **Bob Moses** – Chairman (Ex Vice President CSL); 35 years experience in pharma/biotech industry
- **Dr. Chris Belyea** – (Ex CEO Metabolic Pharmaceuticals and Licensing & Projects Manager Circadian)
- **Dr. Graham Mitchell** – Foursight Associates (Ex Director of Research CSL)




**Market Capitalisation** - A$17 million

**Cash as at 30 June 2013** - A$4 million

**Average daily trading (12 months)** – approx A$70K

antisense THERAPEUTICS

# ANP Investment Highlights

- 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™) 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*

antisense THERAPEUTICS