

# Antisense Therapeutics Ltd

ASX:ANP

January 2017

# Forward Looking Statements

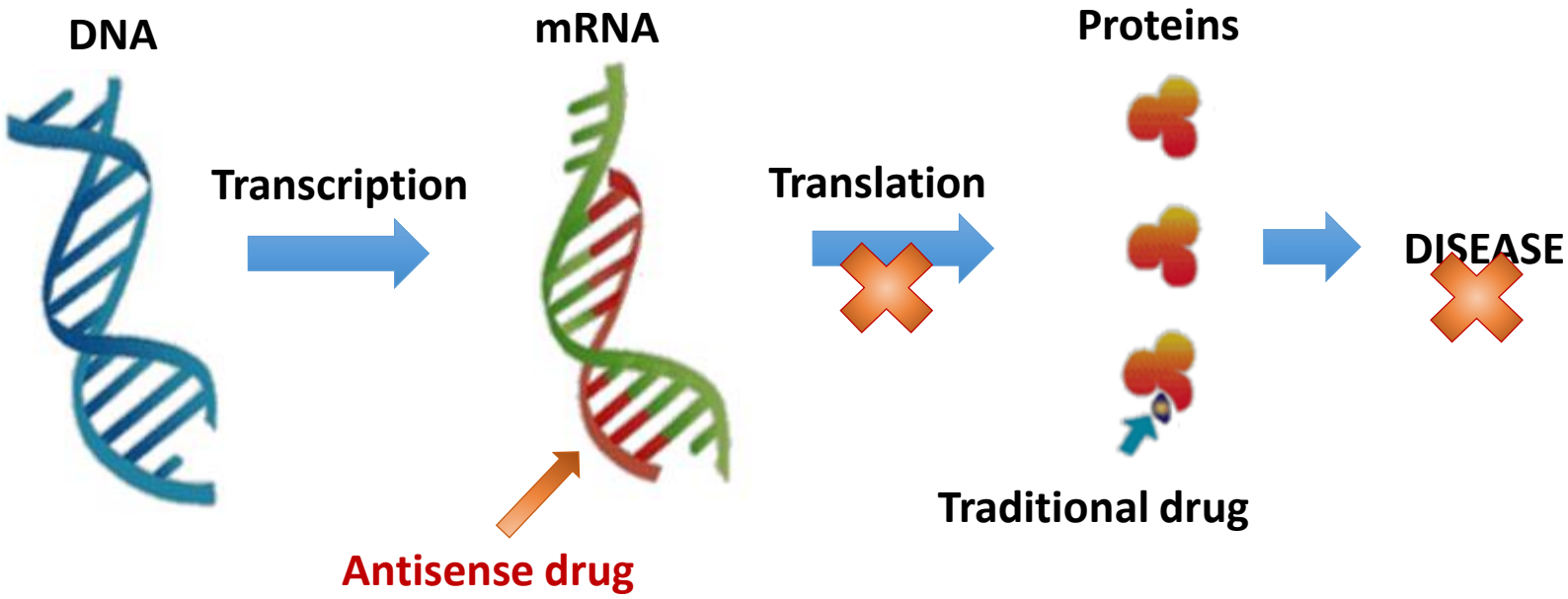
This presentation contains forward-looking statements regarding the Company's business and the therapeutic and commercial potential of its technologies and products in development. Any statement describing the Company's goals, expectations, intentions or beliefs is a forward-looking statement and should 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 2016, copies of which are available from the Company or at www.antisense.com.au.

# Antisense Therapeutics Ltd

- ✓ Advanced stage drug pipeline - 2 compounds with positive Phase II clinical results
- ✓ Compounds licensed from global antisense technology leader, Ionis Pharmaceuticals
- ✓ ATL1103 for acromegaly
  - *Phase II trial in acromegaly patients met primary endpoint with significant (p<0.0001) reduction in sIGF-I, the therapeutic endpoint for treating acromegaly*
  - *Successfully completed higher dose study in acromegaly patients*
  - *In confidential discussions with potential pharmaceutical development partners*
- ✓ ATL1102 for multiple sclerosis
  - *Phase II trial in Relapsing Remitting-MS patients met primary end point with significant (p=0.01) reduction in the cumulative number of new active brain lesions compared to placebo*
  - *Drug significantly reduced B cell numbers - B cell targeting drugs have shown clinical benefit in relapsing and progressive forms MS*
  - *ANP to submit a US IND application in early 2017 for Phase IIb human trial*



# Antisense - what is it and how does it work?




**Small pieces of chemically modified DNA designed to block the production of disease causing proteins**

# Ionis RNA based therapeutics – a hot property

Monday, December 19, 2016

## Ionis Pharmaceuticals Gets $28M From AstraZeneca

IONIS PHARMACEUTICALS

Carlsbad-based biopharmaceutical sdeveloper **Ionis Pharmaceuticals** said Friday that it has received $28M from **AstraZeneca**, folloing the completion of IND studies and licensing of an antisense drug for treating cancer. The company said that it is eligible to receive up to $137M more in additional development and regulatory milestone payments from AstraZenca associated with its drug, along with "low double digit" royalties from sales of the drug.

Market Realist

## FDA Approves Spiranza, Ionis and Biogen Move Higher

By Val Kensington | Dec 28, 2016 8:57 am EST

FierceBiotech

BIOTECH RESEARCH IT CRO MEDICAL DEVICES

Biotech

## Ionis' volanesorsen hits efficacy endpoint in phase 3

by *Nick Paul Taylor* | Dec 20, 2016 5:22am

## Ionis Pharma's Target Lifted To $68 Following More Favorable Spinraza Ramp Estimates



Manikandan Raman , Benzinga Staff Writer | FOLLOW +

December 29, 2016 10:35am | Comments

## The FDA's Christmas Breakthrough



**Matthew Herper,** FORBES STAFF

*I cover science and medicine, and believe this is biology's century.*

FOLLOW ON FORBES

**Ionis Pharmaceuticals (NASDAQ:IONS) Market Capitalisation US$6 Billion**

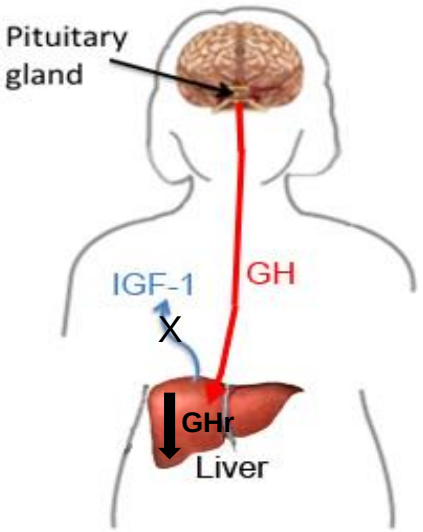


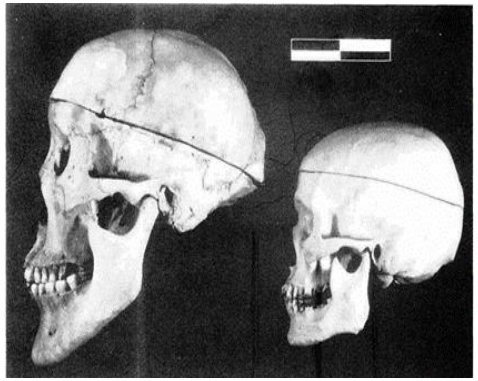
# ATL1103
# Acromegaly

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# ATL1103 for Acromegaly

- Acromegaly disease of abnormal enlargement of organs and bones of the face, feet and hands due to a benign tumor of the pituitary gland that causes excess GH production
- Current first line drug therapies are effective in 45-70% of cases only (Global Sales ~ $1B/annum) with only 1 second line therapy drug Somavert® (Global Sales >$200M/annum) estimated to be capturing just 25% of market due to high cost and poor patient compliance
- ATL1103 reduces expression of Growth Hormone receptor 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 <u>all</u> animal and human studies undertaken to date**
- ATL1103 potential advantages include lower cost of therapy, improved safety profile, and more convenient dosing and administration



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# Project Status: ATL1103 for Acromegaly

- Phase II trial
  - *Primary Efficacy Endpoint Met with significant (p<0.0001) reduction in sIGF-I, the therapeutic endpoint for treating acromegaly*
- Higher dose study
  - *3 patients were dosed for 13 weeks, with one patient at the request of the Principal Investigator receiving an extended dosing period of an additional 12 weeks*
  - *All 3 patients received a therapeutic benefit from the drug with 2 of 3 patients achieving the goal of sIGF-I normalisation at completion of their respective dosing periods*
- Certain toxicology studies to support longer term clinical trials initiated by former partner completed or nearing completion
- Orphan Drug designation granted in US and European Union providing important incentives including market exclusivity, reduced fees, and potential access to grants and tax credits towards trial costs
- ANP is in confidential discussions with potential pharmaceutical partners



# ATL1102
# Multiple Sclerosis

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# ATL1102 for Multiple Sclerosis

- **Multiple Sclerosis** (MS) is a chronic, progressive, and debilitating autoimmune disease that affects central nervous system, brain and spinal cord
- Affects approx 400,000 people in North America and more than 2.5 million worldwide. Global sales for MS drugs in 2015 were US$20 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*
  - *Significant B and T cell reductions uniquely positioning ATL1102 as a selective T and B cell modulator*
- US and EU patents granted to 2029 in RR-MS and progressive forms of MS potentially extendible up to 5 years
- **Phase II data comparable/superior to best in class drugs at same stage of development, with potential for superior administration and safety profile**



# Project Status: ATL1102 for MS

- Phase IIa data published in Neurology
- Engaged in process to attract a pharmaceutical company partner to undertake Phase IIb
- cGMP manufacturing ready for production of Phase IIb clinical trial supplies
- To further support the commercialisation process and continue to add value to the program, ANP intends to submit a US IND application in early 2017 for longer duration Phase IIb human trials of ATL1102
  - *Application for a Phase IIb trial in 195 R-MS patients (RR-MS and SP-MS patients)*
  - *Trial design potentially allows for a future Phase III study to be conducted in either RR-MS or SP-MS*
  - *With the assistance of consulting firm FreeMind, ANP anticipates making an application after IND clearance for an award grant of the type and size (>US$10 million) that could fund the conduct of the Phase IIb trial*
- ANP is also proposing to undertake a smaller investigative study of ATL1102 in relapsing SP-MS patients and is applying for grant funding to conduct this study

# New Growth Opportunities: Criteria

As part of new strategy, an intensified effort to access value adding new development pipeline opportunities to expand and leverage current business

- RNA targeting therapeutics, Antisense, siRNA etc
- Strong IP position
- Attractive worldwide market opportunity with sophisticated healthcare investor interest
- Therapeutic area with unmet medical need - Neuro-inflammatory, autoimmune, endocrinology, oncology
- Strong competitive position
- New preclinical compounds and advancement of existing compounds into new clinical applications

# Corporate Overview

| KEY FINANCIALS | |
|---|---|
| Market Capitalisation | A$6M |
| Cash as at September 2016 | A$3.9M |
| Ordinary shares on issue | 161M |
| Share price (12 month) | $0.03 - $0.07 |

| | |
|---|---|
| **Mr Robert W Moses**<br>Independent Non-Executive Chairman | Formerly Corporate Vice President of CSL Limited. Mr. Moses draws on more than 40 years' experience in the pharmaceutical/biotechnology industry. |
| **Mr Mark Diamond**<br>Managing Director & Chief Executive Officer | Over 26 years' experience in the pharmaceutical and biotechnology industry. Formerly Director, Project Planning/Business Development at Faulding Pharmaceuticals in the USA, in-licensing within Faulding's European operation and International Business Development Manager in Australia. |
| **Dr Graham Mitchell**<br>Independent Non-Executive Director | Joint Chief Scientist for the Victorian Government Department of Environment and Primary Industries. Formerly Director of Research in the R&D Division of CSL Limited. |
| **Dr Gary Pace**<br>Independent Non-Executive Director | Dr Pace has more than 40 years' international experience in the development and commercialization in biotechnology/pharmaceuticals industries. Long-term board level experience with both multi-billion and small cap companies. |
| **Mr William Goolsbee**<br>Independent Non-Executive Director | Founder, Chairman and CEO of Horizon Medical Inc. 1987 – 2002 until acquisition by UBS Private Equity. Founding Director then Chairman of ImmunoTherapy Corporation until acquisition by AVI Biopharma, Inc. (now Sarepta Therapeutics). Former Chairman of privately held BMG Pharma LLC and Metrodora Therapeutics. |

# Corporate Summary

RNA-based technologies continuing to see investor and Big Pharma interest

Developing a highly validated and commercially attractive technology

Progressing two clinically advanced development programs

Looking to add new growth opportunities to complement existing product pipeline



# Antisense Therapeutics


## Mark Diamond, CEO

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