May 2014



A letter from the CEO

Dear Shareholders,

Antisense Therapeutics has been making solid progress in the clinic with its pipeline of products targeting significant unmet medical needs and I am delighted to provide a summary of recent positive developments in this edition of the Company newsletter.

In January we received approval for a Phase I human proof of concept trial of ATL1102 for stem cell mobilisation (SCM). The mobilisation of stem cells from bone marrow into the blood is an important medical procedure used to improve outcomes for patients undergoing chemotherapy to treat certain cancers. The trial began in March and results are expected mid-year.

ATL1102 is also being developed for the treatment of multiple sclerosis (MS). In April, we released ATL1102 toxicology results to support a potential Phase IIb trial.

We also broadened our patent portfolio with a new patent issued in the US for the use of ATL1102 in non-relapsing forms of MS which further adds commercial value to ATL1102 and supports the Company's partnering plans.

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We have also had positive progress with our lead drug ATL1103 targeting acromegaly with the successful recruitment of 24 patients for a Phase II trial which was reported at the end of March. Dosing of patients is well advanced and we expect trial results in the middle of this year.

In January, Antisense Therapeutics was selected to present at the Biotech Showcase 2014 San Francisco, California. It was a significant opportunity for the Company to present and engage with US-based investors and prospective partners. Antisense also recently updated Australian fund managers and brokers with a roadshow of investor briefings.

I look forward to providing regular updates via the ASX, particularly in relation to our upcoming milestones as we continue to develop our unique and exciting disease targeting therapies that we hope will make a difference to the lives of many people around the world.

Yours sincerely,

Mark Diamond
CEO



Second Generation Antisense Technology

Antisense Therapeutics is one of only two ASX-listed companies working with RNA-based technology. RNA is a biological molecule important for protein synthesis and is the target of RNA-based therapeutics like our antisense drugs. By targeting the RNA, the production of certain disease-causing proteins is blocked (or 'silenced'), thereby stopping or limiting the progression of disease.

We are working with our US partner ISIS Pharmaceuticals (ISIS), a world leader in antisense technology, from whom we have access to the second generation technology which forms the basis of our three pipeline drugs in development. There are over 30 ISIS second generation antisense drugs currently in clinical development through partnerships with some of the world's largest pharmaceutical companies such as GlaxoSmithKline, Pfizer, Genzyme and Biogen Idec.

One drug, KYNAMRO™ is approved by the US Food and Drug Administration (FDA) and being marketed through Genzyme – a partner of Isis - for the treatment of high cholesterol. Isis' successful clinical development of the second generation antisense technology, along with the partnering of the technology with major pharmaceutical companies provides great validation and prospects for the work that we are doing here at Antisense Therapeutics.

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antisense THERAPEUTICS

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Antisense Therapeutics' lead drug ATL1103 is in development as a potential treatment for diseases associated with excessive growth hormone action such as acromegaly and the sight disorder diabetic retinopathy.

Acromegaly is a rare condition that results in excessive release of growth hormone into the blood that causes the abnormal growth of certain bones and tissues of the body.

ATL1103 is designed to block growth hormone receptor expression which then significantly reduces the adverse effects of the growth hormone in the bloodstream, which is known to be important in the treatment of acromegaly patients.

Antisense Therapeutics has successfully completed a Phase I trial of the safety and tolerability of ATL1103 and in December last year announced encouraging interim results from a Phase II trial in acromegaly patients.

With the recruiting of the 24 patients required for the study now complete and dosing well advanced, results of the completed trial are expected in mid-2014. The reporting of the trial results will not only be a significant milestone for the Company, but potentially also for the industry and for patients with this rare and difficult to treat condition.

Acromegaly affects about 85,000 people in North America and Europe, with about half requiring drug therapy. The current cost of drug therapy ranges from approximately AUD $30,000 up to $90,000 per annum per patient.



What is acromegaly?

Acromegaly is a condition where the human body produces too much growth hormone which results in abnormal growth of the bones and tissues of the body.

The excess growth hormone results from a benign tumor of the pituitary gland - a gland located beneath the brain and responsible for the release of various hormones.

Acromegaly causes overgrown bones in the face, hands and feet. Patients are also at increased risk of

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complicating conditions such as high blood pressure, diabetes, heart disease and even cancer which lead to premature death if untreated. Treatment approaches are to surgically remove the pituitary tumour or drug therapy to normalise growth hormone action in the bloodstream.

Did you know?

Acromegaly can occur in both adults and children. In adults, acromegaly affects eight out of every 100,000 adults caused by the excess production of growth hormone after the skeleton and other organs finish growing. In children, acromegaly results in gigantism characterised by excessive skeletal growth and height significantly above average.



ATL1102 for Multiple Sclerosis

Antisense Therapeutics is also focused on developing an effective treatment for multiple sclerosis - one of the most common and debilitating diseases affecting the central nervous system - with its antisense drug ATL1102.

There are two main forms of MS - relapsing-remitting MS (RRMS) and secondary progressive multiple sclerosis (SPMS).

Both forms of MS are characterised by brain lesions, a condition exacerbated by the unusual movement of leukocytes (white blood cells) into the central nervous system, causing inflammation in the brain.

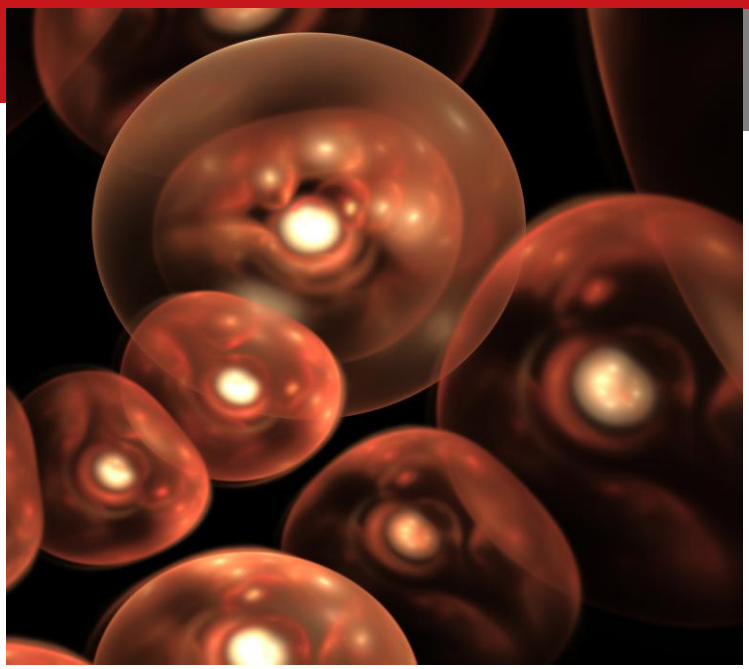
ATL1102 is an antisense compound developed to inhibit inflammation as it prevents the movement of leukocytes into the central nervous system. In a Phase IIa clinical trial in RRMS patients, ATL1102 was found to be highly effective in reducing brain lesions in patients.

Tysabri®, an approved and currently marketed drug, targets the same receptor (VLA-4) as ATL1102. Interestingly, the results from the Phase IIa trial has shown ATL1102 efficacy to be as good as, if not superior to Tysabri® at the same stage of development. Tysabri® is currently regarded as the most potent drug for the treatment of RRMS. Antisense Therapeutics anticipates ATL1102 could be as potent as Tysabri® but potentially safer and cheaper to manufacture and more conveniently administered through a pre-filled syringe. Tysabri® achieved net sales of US$1.6 billion in 2013.

In April, Antisense Therapeutics released new animal toxicology results of ATL1102 which showed the

drug to be well tolerated at the doses tested. The toxicology results provide the opportunity for the Company to discuss our plans for the further development of ATL1102 with the FDA. The Company anticipates final review of the data in June 2014 followed by a pre-IND meeting with the FDA in the third quarter of 2014 to assess the design of a Phase IIb MS trial.

In the same month the Company also broadened its IP position, adding to ATL1102's commercial desirability when we announced a new granted patent in the US for the use of ATL1102 for the reduction of brain lesions in both RRMS and SPMS.



Stem cell mobilisation

Antisense is also investigating ATL1102 as a stem cell mobilisation (SCM) therapy.

SCM is a medical procedure used to improve clinical outcomes for cancer patients undergoing chemotherapy. Certain chemotherapy treatments can have an overall toxic effect on the human body, particularly on the bone marrow which produces the body's supply of stem cells.

Steady production of stem cells from the bone marrow ensures the replacement of old or dead body cells. In the case of cancer patients undergoing treatment, stem cell supply is significantly affected which slows their recovery and their ability to fight disease.

To counteract this, SCM is performed where a drug is administered to release hematopoietic stem cells from the bone marrow into the blood and then the stem cells are collected from the blood of the cancer patient (or from a donor) before the patient receives chemotherapy.

The collected stem cells are then given back to the patient post-chemotherapy to replenish the lost stem cells, which accelerates patient recovery.

SCM is a commercially attractive opportunity with a market potential estimated at several hundred million dollars per annum.

It is proposed that ATL1102 be used in combination with granulocyte colony-stimulating factor (G-CSF), the main agent used in SCM to increase the number of stem cells, so as to improve the outcome for the patients undergoing the cancer therapy.

In January, the Company received approval to conduct a Phase I trial for the use of ATL1102 in SCM. The randomised, open-label trial of 10 healthy volunteers began in March 2014 to assess proof of

concept of ATL1102 when used alone or in combination with G-CSF to effectively release stem cells. The Company expects results to be available in mid-2014.

Nucleus Network, one of Australia's leading Clinical Research Organisations at the Alfred Hospital, was selected as the clinical trial site.

Recent news in the RNA Therapeutics space

RNA-based therapeutics is an exciting area of medical research and attracting the interest of investors and large pharmaceutical companies around the world.

Antisense Therapeutics welcomes discoveries and developments in the RNA therapeutics sector that provide further validation and support for the work being undertaken by Antisense Therapeutics and that have the potential to improve the health outcomes for patients globally.

As developers of the world's first systemically injected antisense drug to reach the market, ISIS and Sanofi's subsidiary Genzyme, have been recognised by the US National Organization for Rare Disorders (NORD) for their achievement.

Isis and Genzyme developed KYNAMRO, a second generation antisense drug for the treatment of homozygous familial hypercholesterolemia, a rare genetic disease affecting about 6000 people around the world that limits the body's ability to clear cholesterol.

NORD awarded Isis and Genzyme its "Partners in Progress Corporate Award for 2014" earlier this month.

Isis CEO Stanley T. Crooke has said "KYNAMRO™ is the first systemic antisense drug to reach the market and is the culmination of two decades of work to create a new, more efficient drug technology platform."

NORD presents awards each year recognising researchers and others who improve the lives of people with rare diseases.

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