

# Antisense Therapeutics

ATL1102 for Multiple Sclerosis  
Early Access Program

# Forward Looking Statements

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## **THIS PRESENTATION IS FOR THE AUSTRALIAN MARKET ONLY**

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# Early Access Program (EAP)

## **EAP offers patients access to new non-registered pharmaceuticals**

- Products may be provided to patients with a life threatening or debilitating disease where no alternative or appropriate therapy exists
- > 1,000,000 patients annually in Europe alone are left without treatment options with only a small percentage able to access new therapies via clinical trials<sup>1</sup>
- Can charge for drug access in certain markets
- Product must have been in Phase 2 trials or later and have shown evidence of efficacy

## **EAP Pricing**

- Pricing set by the Sponsor (ANP) at comparable price to registered products
- MS treatment costs in Europe range from A\$25,000–\$33,000 per patient per year<sup>2</sup>

<sup>1</sup> Estimated by myTomorrows

<sup>2</sup> Pricing for Tysabri, Gilenya, & Tecfidera -Toumi M and Jadot G in J. Market Access and Health Policy 2014, 2: 23932

# EAP in Europe for ATL1102

## MS Market

- 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

## EAP Establishment for ATL1102

- Partnering Agreement signed with EAP specialists myTomorrows
- myTomorrows to perform EAP activities at their cost including data collection and seeking EAP approvals initially in Europe in those markets where drug use is reimbursed
- ANP to provide ATL1102 product for use in the EAP
  - Arrangements being established to source ATL1102 from existing 3<sup>rd</sup> party supplies (2 batches) for potential use in the EAP
  - Proposal to initially source one batch of ATL1102 to commence the EAP
  - Subject to this first batch of material being confirmed (through retesting) as of suitable quality for use in the EAP, the quantity of material is expected to be sufficient for one year's treatment for over 150 patients at the 200mg/week dose
  - Material could be available as early as 4'Q'15 for use in the EAP
  - Possible cash flow positive income

# myTomorrows

## **PROVIDING EARLY ACCESS TO DRUGS IN DEVELOPMENT**

myTomorrows is creating freedom of choice for physicians and patients with unmet medical needs by offering earlier access to medicines that show promising results during clinical trials, but are not officially registered yet. With the support of their doctors, patients who suffer from cancer, a neurological disorder, a rare disease or a severe depression, can have earlier access to such medicines. myTomorrows identifies innovative drugs, informs physicians and patients and facilitates requests for access to these drugs in development via a world-wide Internet-based platform.

For more information about myTomorrows, please visit the website [www.mytomorrows.com](http://www.mytomorrows.com).

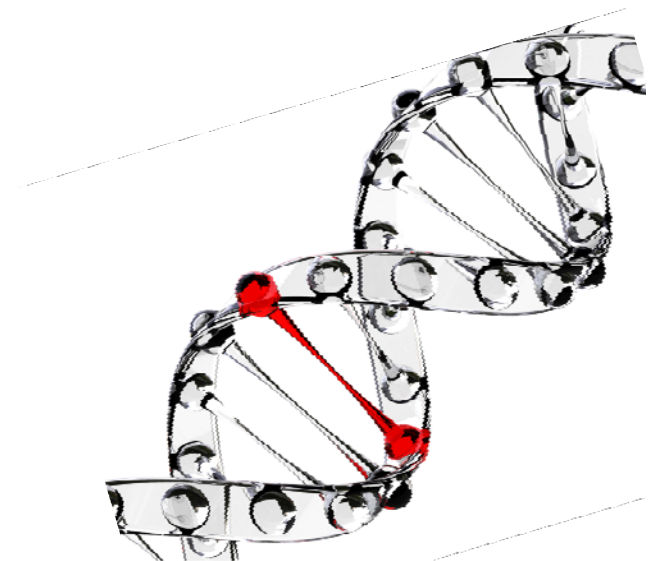


# ATL1102

## 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





# Phase II trial results published in *Neurology*

Published Ahead of Print on September 19, 2014 as 10.1212/WNL.0000000000000926

## CD49d antisense drug ATL1102 reduces disease activity in patients with relapsing-remitting MS

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### ABSTRACT

**Objective:** This study evaluated the efficacy and safety of ATL1102, an antisense oligonucleotide that selectively targets the RNA for human CD49d, the  $\alpha$  subunit of very late antigen 4, in patients with relapsing-remitting multiple sclerosis (RRMS).

**Methods:** In a multicenter, double-blind, placebo-controlled randomized phase II trial, 77 patients with RRMS were treated with 200 mg of ATL1102 subcutaneously injected 3 times in the first week and twice weekly for 7 weeks or placebo and monitored for a further 8 weeks. MRI scans were taken at baseline and weeks 4, 8, 12, and 16. The primary endpoint was the cumulative number of new active lesions (either new gadolinium-enhancing T1 lesions or nonenhancing new or enlarging T2 lesions) at weeks 4, 8, and 12.

**Results:** A total of 72 patients completed the study and 74 intention-to-treat patients were assessed. ATL1102 significantly reduced the cumulative number of new active lesions by 54.4% compared to placebo (mean 3.0 [SD 6.12] vs 6.2 [9.89],  $p = 0.01$ ). The cumulative number of new gadolinium-enhancing T1 lesions was reduced by 67.9% compared to placebo ( $p = 0.002$ ). Treatment-emergent adverse events included mild to moderate injection site erythema and decrease in platelet counts that returned to within the normal range after dosing.

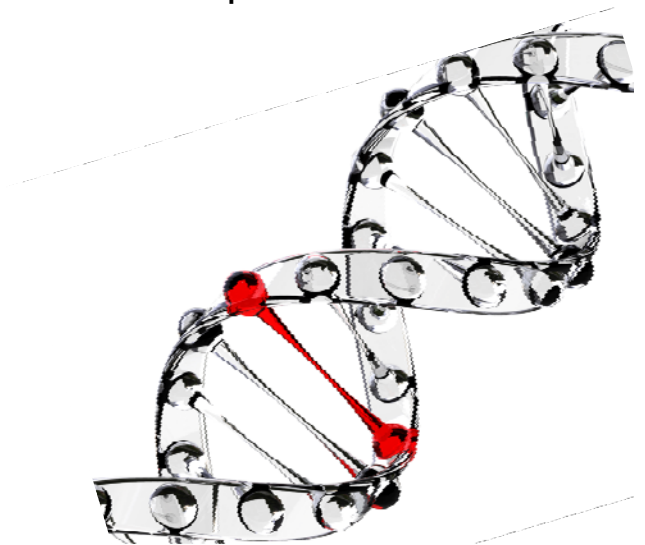
**Conclusions:** In patients with RRMS, ATL1102 significantly reduced disease activity after 8 weeks of treatment and was generally well-tolerated. This trial provides evidence for the first time that antisense oligonucleotides may be used as a therapeutic approach in neuroimmunologic disorders.

**Classification:** This study provides Class I evidence that for patients with RRMS, the antisense oligonucleotide ATL1102 reduces the number of new active head MRI lesions. *Neurology*® 2014;83:1-9



# 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 the process to attract a pharmaceutical company partner to undertake the Phase IIb trial
- Early Access Program now established initially for use of ATL1102 in Europe





# Antisense Therapeutics

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