

## RESEARCH REPORT

## Antisense Therapeutics (ANP)

Potential Competitor on the Canvas

Share Price  
& Estimated  
Future Price

12-Month Target\* \$0.43

Price \$0.20

Implied Return 115%

\*Implied Return

**Sarepta Therapeutics' (NASDAQ: SRPT) SRP-9001, a putative gene therapy (GT) for all Duchenne muscular dystrophy (DMD) patients has failed to demonstrate an improvement clinical function in a double-blind, randomised, placebo-controlled phase II trial in 41 patients. This event significantly improves the commercial prospects Antisense Therapeutics' DMD therapy, ATL1102.**

**The Event & Interpretation:** Approved drugs for the treatment of DMD are based on a technology called exon-skipping, with the precise problem with the dystrophin gene a patient carries determining their eligibility for an approved drug, if one exists for that problem. This means many drugs will be required to cover the entire DMD population. SRP-9001 aims to treat essentially all DMD patients by simply giving them a functional, although scaled-down, gene. The protein it produces is called micro-dystrophin and is also scaled down, but functional.

While a very big reach, it was hoped SRP-9001 could cure DMD patients. The more likely hoped for outcome was a universal treatment that would benefit all DMD patients. **The former will not be happening and the latter is looking very unlikely.**

The top line results released by Sarepta showed that despite a high mean micro-dystrophin concentration of 28.1% produced by SRP-9001 in the test arm, benefit at the clinical/functional was not shown. The reason(s) for this result are unclear.

GTs have been an alluring, but mistakenly simple, idea for decades. A strong belief formed in the 2000's that we had the technologies to develop them. Nonetheless, the majority of currently approved GTs require the cells they need to affect to be removed from the patient, treated, then, put back in the patient for them to work. That is systemic delivery of GTs has proven difficult. In the case of SRP-9001, the skeletal muscle it needs to affect cannot be uniformly accessed for a variety of reasons, such as the differences in the size, shape and blood supply to the various different muscles.

Consequently, while the market got quite bullish on SRP-9001's prospects as the trial results approached, we have been much more subdued. The market was so bullish on SRP-9001, that Sarepta share price fell 53% on release of the results, equating to a loss of more than USD7b in value, as judged by market capitalisation.

The big surprise in the trial data was the big increase in micro-dystrophin levels in treated patients without clinical benefit. An argument for existing DMD drugs is that the low levels of dystrophin found in healthy patients mean therapeutics need only produce a small amount of dystrophin to have a big clinical impact in DMD.

There are a myriad of possible explanations for SRP-9001's poor trial performance. Sarepta has pulled out the often-stated excuse of trial imbalances, which can be code for, we have no idea, but do not blame our drug. We tend to believe that there is simply something unknown about the pathogenesis of DMD that has led to this result. **SRP-9001 is not out cold, but it is on the canvas and the referee is counting.**

**SRP-9001's results are good for ATL1102 on many levels.** Firstly, they indicate that a possible DMD GT cure is decades away. Secondly, a universal DMD GT capable of a real, if muted, clinical impact is many years away, if not longer. Finally, and maybe most importantly, the discrepancy between micro-dystrophin levels and clinical outcome may mean inflammation is a much bigger driver in the pathogenesis of DMD than originally thought. That would make ATL1102 the prominent DMD drug.

This event has caused us to **significantly increase the slope of ATL1102's sales curve** in our model, while also **increasing the volume at which market growth rates kick in.**

**Conclusion:** It is never nice to look at the bright side of a drug failure, but the event discussed herein has significant implications for the value of Antisense Therapeutics. Accordingly, **we increase our price target to 43 cents per share.**

**Analyst: Marc Sinatra, BSc(Hons), MBA.**

## Company Information

ASX Ticker	ANP
Shares on Issue	574.0 million
Fully Diluted Shares on Issue	619.0 million
Market Capitalisation	\$115.8 million
ASX Vol. (Shares/Day)*	9.5 million

\* Shares per Day for the Last 20 Trading Days.

## Cash Sufficiency

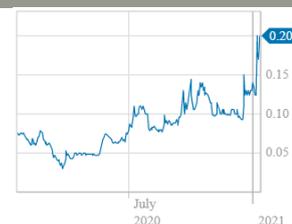
	\$ Million
A) Last Appendix 4C	End Sept 2020
B) Cash and Equivalents at 4C	3.1
C) Burn <sup>1</sup>	-0.9
D) Estimated Current Q Burn <sup>2</sup>	-1.1
E) Estimated Cash Raised Post 4C <sup>3</sup>	9.1
F) Quarters (Q) Cash Remaining <sup>4</sup>	11.0
<b>G) Estimated Current Cash<sup>5</sup></b>	<b>10.2</b>
H) Significant Estimated New Commitment(s) <sup>6</sup>	5.5
Description of Commitment(s): Phase IIb study of ATL1102 in non-ambulant DMD boys.	

<sup>1</sup> Burn = Net Cash from/used In Operating Activities;<sup>2</sup> Equals C \* (# Days Since previous Q end / # Days in Qs post 4C);<sup>3</sup> Equals Capital Raising(s) - Estimated Costs + R&D Rebate + Other Income;<sup>4</sup> Equals (B + D + E) / C ;<sup>5</sup> Equals B + D + E<sup>6</sup> Equals estimated maximum significant commitments that the company has or is likely to become contractually or ethically committed to.

## Key Personnel

Mr Robert Moses	Chairman
Mr Mark Diamond	MD and CEO
Mr William Goolsbee	NED
Dr Graeme Mitchell	NED
Dr Gary Pace	NED
Dr George Tachas	Director, Drug Discovery and Patents

## Share Price Chart



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