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This Special Edition of *Bioshares* provides analysis of Antisense Therapuetics, which has released the final results of its Phase IIa study of ATL1102 in Duchenne's Muscular Dystrophy, and Pharmaxis, which announced that Boehringer Ingelheim will discontinue the development of BI 1467335 (formerly PXS 4728A) in the indication of NASH, but is continuing with its development in diabetic retinopathy.

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Blake Industry & Market Analysis Pty Ltd
ACN 085 334 292
PO Box 193
Richmond Vic 3121
AFS Licence No. 258032
Enquiries for *Bioshares*
Ph: (03) 9326 5382
Fax: (03) 9329 3350
Email: info[at}bioshares.com.au

David Blake - Editor/Analyst
Ph: (03) 9326 5382
Email: david[at]bioshares.com.au

Mark Pachacz - Editor/Analyst
Ph: 0403 850 425
Email: mark[at]bioshares.com.au

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Bioshares

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Delivering independent investment research to investors on Australian biotech, pharma and healthcare companies

Extract from Bioshares –

Antisense Therapeutics to Move into Phase IIb with Positive Phase II Data

Antisense Therapeutics (ANP: $0.086) has released data from the full nine patients in its Phase IIa Duchenne Muscular Dystrophy (DMD) study with ATL1102 with clear positive indicators which support moving the program into a Phase IIb study.

That Phase IIb study will be conducted in Europe, and pending the quality of the results, may become a pivotal study which would allow for registration of the therapy. A Phase IIb study would likely involve 80 children with DMD who are non-ambulant (in wheelchairs) and should start next year. Patients are likely to be treated for 12 months with ATL1102 and we expect it to take two years to generate results.

ATL1102 is an antisense drug compound. It inhibits CD49d present on T cells and it has been found that children with DMD with higher levels of CD49d progress more rapidly with the disease. Inhibiting CD49d is thought to lower the level of inflammation that contributes to the deterioration in muscle growth and function that characterises this disease. Antisense Therapeutics has previously shown in a Phase II study that CD49d levels can be reduced in patients with multiple sclerosis and control the disease at a much higher dose of 400mg a week, however there are some side effect concerns at higher doses.

In this children's study, a dose of 25mg per week was evaluated with no safety issues. The Phase IIb study is likely to explore higher doses of the therapy.

Phase IIa Results

Antisense's Phase IIa trial results were compared to what can be expected from children at a similar age and also non-ambulant boys treated with corticosteroids as detailed in a paper by Ricotti et al earlier this year. Antisense CEO Mark Diamond said there are at least three other papers showing the expected decline in muscle function with boys on corticosteroid treatment.

The Phase IIa results showed that as measured by a standard MyoPinch test, there was no deterioration on average in the children treated with ATL1102, compared to a 0.38 point drop or 15% loss in pinch strength in boys on corticosteroids for six months.

With grip strength, the boys treated with ATL1102 showed a 0.2 point increase in strength, compared to a 0.5 point drop in those followed in the Ricotti paper or a 10.5% drop.

On the PUL2.0 measure, which assesses upper limb strength, seven of the nine boys either maintained their strength or registered an improvement. The mean gain was 0.9. This compares to another study conducted by Pane *et al* and published last year, in non-ambulant boys at a similar age treated only with corticosteroids, that showed a decline in function over six months of 1.09 points. Once boys with DMD become non-ambulant, a linear deterioration in physical function tends to occur.

Continued over

– ANP cont'd

There were particularly encouraging results from the level of CD49d+ T cells. As measured by the CD3+ CD49d+ T cells, there was a median 9.78% drop at 24 weeks. Importantly, just four weeks after treatment had stopped, this level increased to a net increase of 9.93% from baseline, which confirms the mechanism of action of this drug candidate.

Professor Thomas Voit, an author on the Ricotti paper, commented on these results that "Disease stabilisation or indeed improvement in function scores in non-ambulant DMD boys is almost unheard of and a very encouraging result."

Summary

Antisense Therapeutics should continue to attract further interest from investors in the year ahead, given that the company will be moving into a Phase IIb study next year in DMD, a study which may be sufficient for achieving approval in Europe.

The stock will be responsive to the company's confirming the design of the Phase IIb study, receiving regulatory and ethics clearance for the trial, completing manufacture of drug material for the study, and results of discussions with regulatory agencies.

The company retained cash of $2 million at September 30, 2019, and is expected to receive a tax refund of $0.55 million this quarter. An underwriting agreement has recently been signed that should see $5.5 million raised through the exercise of options.

Antisense Therapeutics is capitalised at $42 million, including the exercise of all outstanding listed options.

Milestones for 2020 include:

- Manufacturing of material for proposed Phase IIb trial (up to 4,000 doses)
- Clinical trial design
- Clinical trial approval and ethics clearance in Europe
- Results of discussions with FDA
- Commencement of Phase IIb study

Bioshares recommendation: **Speculative Buy Class B**

Bioshares

How Bioshares Rates Stocks

For the purpose of valuation, Bioshares divides biotech stocks into two categories. The first group are stocks with existing positive cash flows or close to producing positive cash flows. The second group are stocks without near term positive cash flows, history of losses, or at early stages of commercialisation. In this second group, which are essentially speculative propositions, Bioshares grades them according to relative risk within that group, to better reflect the very large spread of risk within those stocks. For both groups, the rating "Take Some Profits" means that investors may re-weight their holding by selling between 25%-75% of a stock.

Group A

Stocks with existing positive cash flows or close to producing positive cash flows.

Buy	CMP is 20% < Fair Value
Accumulate	CMP is 10% < Fair Value
Hold	Value = CMP
Lighten	CMP is 10% > Fair Value
Sell	CMP is 20% > Fair Value

(CMP–Current Market Price)

Group B

Stocks without near term positive cash flows, history of losses, or at early stages commercialisation.

Speculative Buy – Class A
These stocks will have more than one technology, product or investment in development, with perhaps those same technologies offering multiple opportunities. These features, coupled to the presence of alliances, partnerships and scientific advisory boards, indicate the stock is relative less risky than other biotech stocks.

Speculative Buy – Class B
These stocks may have more than one product or opportunity, and may even be close to market. However, they are likely to be lacking in several key areas. For example, their cash position is weak, or management or board may need strengthening.

Speculative Buy – Class C
These stocks generally have one product in development and lack many external validation features.

Speculative Hold – Class A or B or C

Sell

Corporate Subscribers: Cogstate, Bionomics, LBT Innovations, Opthea, ResApp Health, Pharmaxis, Dimerix, Adalta, Actinogen Medical, Patrys, Cyclopharm, Emvision, Antisense Therapeutics, Heramed, Imugene, Exopharm, Immutep, Neuroscientific Biopharmaceuticals