

January 27, 2015

Arrowhead Research (Nasdaq/ARWR)
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Neutral
Waiting on the FDA
Arrowhead Research is a clinical stage targeted therapeutics company
Investment Highlights

1) Arrowhead has met a bump in the road with its lead clinical candidate, ARC-520, following a recent call from the US FDA placing a partial clinical hold on its Phase 2b study in chronic hepatitis B (HBV) infected patients. Under the recent hold, announced in mid-January, Arrowhead must start its new multiple-dose study at 1 mg/kg of ARC-520, rather than the proposed parallel study design of 2 and 4 mg/kg dosages, until the Company can provide the agency more information on the ongoing Phase 2a trial, which included dosage levels of 1, 2, 3 and later 4 mg/kg. The FDA intends to provide Arrowhead with a more complete letter regarding the agencies' concerns and information needs by mid-February. Late last year, Arrowhead submitted its IND application to the FDA for two proposed Phase 2b studies with ARC-520, including the Heparc-2002 study for patients who test negative for hepatitis B e-antigen (HBeAg) at screening and the Heparc-2003 study for patients who test positive for HBeAg. The primary objectives of the studies will be to evaluate the depth of HBsAg decline in response to multiple doses of ARC-520 in patients with HBV, with secondary objectives assessing safety and tolerability, as well as pharmacokinetics when co-administered with entecavir and tenofovir. Meanwhile, Arrowhead is completing its Phase 2a clinical trials with ARC-520, including the key final dosage level of 4 mg/kg, and expects to complete the trial by mid-year 2015.

2) Last year Arrowhead also diversified its R&D portfolio with the mid-year nomination of its ARC-AAT clinical candidate for patients with Alpha-1 antitrypsin deficiency (AATD), a large-scale orphan liver disease with approximately 100,000 sufferers in the US. Arrowhead has filed for regulatory approval in Australia to begin a Phase 1 trial of up to 48 patients, with a single ascending dose administered to both healthy volunteers and AATD patients. The primary objectives of the trial will be to measure the safety and tolerability of escalating doses of ACR-AAT and also to evaluate the pharmacokinetics of the various dosage levels. Secondary objectives will include the evaluation of the

Current Price \$6.77
Price Target N/A

Estimates (FYE Sept)	F2012A	F2013A	F2014A
Revenues(\$000s)	\$147	\$290	\$175
EPS	(\$1.90)	(\$1.30)	(\$1.25)

Stock Data

52-Week Range	\$4.95-\$27.63
Shares Outstanding (mil.)	54.7
Market Capitalization (mil.)	\$370.3
Enterprise Value (mil.) - Pro Forma	\$193.1
Debt to Capital (9/14)	0.0%
Book Value/Share (9/14)	\$3.03
Price/Book	2.2
Average Trading Volume (10-Day)	1,655,000
Insider Ownership	6.1%
Institutional Ownership	76.4%
Short interest (Millions)	12.9
Dividend / Yield	\$0.00/0.0%


Price target and ratings changes over the past 3 yrs:

Initiated - February 26, 2013 - Buy - Price Target \$4.00

Updated - August 8, 2013 - Buy - Price Target increased to \$6.50

Downgraded to Neutral - January 30, 2014

depth and duration of the decline in serum total alpha-1 antitrypsin levels as well as the time needed for serum alpha- antitrypsin levels to return to baseline. The early plan for Arrowhead's ARC-AAT clinical program follows closely with that of its ARC-520, also conducted in Australia, where time between initial filing of the Phase 1 plan, completion of enrollment, and publication of final results was only six months, approximately, between May and December of 2013. Arrowhead also continues to explore advancement of additional clinical candidates, focusing on siRNA candidates in the liver disease as well as extra-hepatic medical areas, as well as maintaining its R&D facility in Madison, Wisconsin and pursuing strategic partnerships and research collaborations.

3) Arrowhead recently reported financial results for its fiscal 2014 year ending September, including revenues of \$175,000 and a net loss of \$58.6 million, or (\$1.25) per share, as compared with revenues of \$290,000 and a net loss of \$31.1 million or (\$1.30) per share in fiscal 2013. The higher net loss in 2014 was due primarily to increased R&D activities, which was evident in higher R&D expenses of \$23.1 million versus \$8.7 million, as well as increased salaries and payroll-related costs of \$12.8 million as compared with \$6.7 million. Arrowhead also incurred approximately \$11.6 million in non-cash charges and one-time items, and operating cash burn in 2014 for the Company was about \$47 million. At the end of the fiscal year, Arrowhead held over \$177.2 million in cash and investments, boosted significantly during 2014 through two securities offerings. For current fiscal year 2015E, we are estimating that stepped-up R&D activities, including a new Phase 2b clinical trial for ARC-520 and a Phase 1 trial for ARC-AAT, which may begin in the near-term, will increase operating expenses, and thus expanding net losses for Arrowhead to \$65.7 million or (\$1.20) per share, however, well within the Company's current level of cash resources.

Conclusion

Arrowhead shares have dropped in recent months in response to preliminary results from its ARC-520 Phase 2a trial and delay in the start of its Phase 2b trial, down to the point where ARWR shares are currently trading at valuations of only 2.3X book value and 2.1X recent cash per share, and just slightly above our most recent price target from 2013. Still, until more clarity can be seen related to the direction of its clinical programs, especially ARC-520, we are maintaining our Neutral rating on ARWR shares at the present time.

Catalysts/Investor Timeline

- 1) Publications in peer-reviewed scientific journals on DPCs and Peptide targeting – Ongoing 2014-15
- 2) Presentation of complete ARC-520 data on chronic HBV chimpanzee at AASLD – early November 2013
- 3) Completion of Phase 1 clinical dosing study on healthy volunteers (Australia) with ARC-520 – Calendar Q4/2013
- 4) Complete patient dosing in Phase 2a ARC-520 trial – mid-2015
- 5) Initiation of Phase 2b trial for ARC-520 in Chronic HBV patients – 2015
- 6) Initiation of Phase 1 trial for ARC-AAT in AATD patients in Australia – H1/2015
- 7) Initiation of proof-of-concept clinical trials for new drug candidates – 2015-16

Risk Factors

In addition to normal economic and market risk factors that impact most equities and the common risks shared by Arrowhead with other companies in the industry, we believe an investment in Arrowhead Research involves the following risks:

- **FDA and regulatory risks** – Arrowhead is subject to regulatory review for its ongoing research and development activities, principally the US Food and Drug Administration but also potentially with other international regulatory agencies as well.
- **Reliance on joint venture partners** — At present, Arrowhead has a number of partnership agreements signed in both the development and drug discovery areas. Partnerships and joint ventures bring certain risks that are not present in internal operations, such as potential delays, company disagreements, or unforeseen financial difficulties at the partnering entity, and there can be no assurance that these partnerships will prove to ultimately successful.
- **Need to defend patents and other intellectual property** – At present, Arrowhead holds a large number of patents in the US and in foreign jurisdictions with additional patents pending in the US and internationally related to drug delivery platform technologies and processes. The Company may need to defend its patents in the US and overseas in the future, particularly if one or more products receive regulatory approval and are successfully marketed.
- **Need to raise additional capital** - Currently, Arrowhead has enough cash on hand to fund ongoing research and marketing development programs well beyond calendar 2016, approximately. However, the Company does not have a history of profitable operations and unforeseen events including potential delays in regulatory approvals could require Arrowhead to raise additional capital through the sale of equity within a shorter time-frame, therefore potentially diluting current shareholders.
- **Limited stock liquidity** – Trading volume in Arrowhead stock is comparatively light. As such, news regarding Arrowhead, its target market, partners and/or competitors could lead to significant volatility in the stock price.
- **Competitive Markets** – The Company will potentially compete in its target pharmaceutical markets with a number of other large multi-national manufacturers, generic drug companies, and specialty pharma firms, some of which represent much larger companies. There can be no assurance that the Company will be able to successfully launch new products into these competitive markets in the future.
- **Recent acquisition activity** – Over the past several years, Arrowhead has completed several major acquisitions. These acquisitions will require the Company to integrate existing operations and staff over time, and there can be no assurance that Arrowhead management will be able to avoid unforeseen problems with past (or future) acquisitions. In addition, several of these acquisitions were completed at least in part through the issuance of common stock in Arrowhead, which can be dilutive and harmful to trading activity in certain cases should these shares come out into the market in a non-orderly fashion.

Arrowhead Research Corporation
Consolidated Statements of Income
(in \$000s, except EPS)

Robert M. Wasserman

Fiscal Year Ending September	<u>2011</u>	<u>2012</u>	<u>2013</u>	<u>2014</u>	<u>2015E</u>	<u>2016E</u>
Revenues						
Discovery and service contracts	\$296.1	\$146.9	\$290.3	\$175.0	\$175.0	\$1,000.0
Product sales royalties and regulatory milestones	<u>0.0</u>	<u>0.0</u>	<u>0.0</u>	<u>0.0</u>	<u>0.0</u>	<u>20,000.0</u>
Total revenues	<u>296.1</u>	<u>146.9</u>	<u>290.3</u>	<u>175.0</u>	<u>175.0</u>	<u>21,000.0</u>
Expenses						
Salaries and payroll-related costs	1,408.4	6,414.9	6,667.7	12,829.4	15,000.0	18,000.0
General and administrative and other	3,795.4	6,439.3	3,488.9	5,894.0	6,500.0	7,500.0
Research and development	3,277.8	5,391.5	8,705.6	23,138.1	25,000.0	29,000.0
Other, non-cash and one-time charges	<u>1,644.9</u>	<u>2,990.4</u>	<u>6,017.4</u>	<u>11,589.9</u>	<u>12,000.0</u>	<u>14,000.0</u>
Total operating expenses	<u>10,126.4</u>	<u>21,236.1</u>	<u>24,879.5</u>	<u>53,451.3</u>	<u>58,500.0</u>	<u>68,500.0</u>
Loss from operations	(9,830.3)	(21,089.2)	(24,589.3)	(53,276.3)	(58,325.0)	(47,500.0)
Interest expense, net	86.5	36.0	(97.9)	645.5	500.0	1,000.0
Other expense, net	<u>6,251.3</u>	<u>(1,057.5)</u>	<u>(7,016.2)</u>	<u>(6,089.3)</u>	<u>(8,000.0)</u>	<u>(8,500.0)</u>
Net income (loss)	(3,492.4)	(22,110.7)	(31,703.4)	(58,720.1)	(65,825.0)	(55,000.0)
Non-controlling interests and taxes	<u>363.5</u>	<u>984.8</u>	<u>560.1</u>	<u>89.9</u>	<u>100.0</u>	<u>100.0</u>
Net income (loss) attributable to common stock	<u>(3,128.9)</u>	<u>(21,125.9)</u>	<u>(31,143.3)</u>	<u>(58,630.2)</u>	<u>(65,725.0)</u>	<u>(54,900.0)</u>
Basic and diluted income (loss) per share	<u>(\$0.44)</u>	<u>(\$1.90)</u>	<u>(\$1.30)</u>	<u>(\$1.25)</u>	<u>(\$1.20)</u>	<u>(\$1.00)</u>
Basic and diluted shares outstanding	7,181.1	11,129.8	24,002.2	46,933.0	54,700.0	54,700.0
Key ratios:						
Cash Flow/share	(\$1.13)	(\$1.62)	(\$0.78)	(\$0.87)	(\$0.84)	(\$0.59)

Balance Sheets

(\$000s)

<i>Assets:</i>	<u>9/30/13</u>	<u>9/30/14</u>
Cash and marketable securities	\$28,144.7	\$154,163.6
Receivables	83.7	
Prepaid expenses and other	615.4	637.1
Note receivable, net	<u>0.0</u>	<u>0.0</u>
Total current	28,843.7	154,800.8
Property & equip., net	3,513.2	3,872.8
Intangible assets and other long-term	<u>4,972.7</u>	<u>24,143.2</u>
TOTAL ASSETS	37,329.6	182,816.8
<i>Liabilities:</i>		
Accounts payable	1,199.6	2,579.5
Accrued expenses	1,544.7	4,668.0
Deferred revenue	103.1	103.1
Notes payable	971.6	50.0
Other current	<u>4,906.1</u>	<u>4,446.4</u>
Total current	8,725.0	11,847.0
Notes payable, long-term	50.0	
Other long-term	2,819.8	4,984.5
Stockholders' equity	<u>25,734.8</u>	<u>165,985.3</u>
TOTAL LIAB & EQ	37,329.6	182,816.8

Source: Dawson James Securities, Inc. estimates; Company documents

Important Disclosures:

Price Chart:



Price target and ratings changes over the past 3 years:

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 Downgraded to Neutral – January 30, 2014

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	# of Companies	% of Total	# of Companies	% of Totals
Market Outperform (Buy)	15	71%	9	60%
Market Perform (Neutral)	6	29%	4	67%
Market Underperform (Sell)	0	0%	0	0%
Total	21	100%	13	62%

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