Zacks Small-Cap Research

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Arrowhead Research (ARWR-NASDAQ)

ARWR: Acquisition of Novartis' RNAi assets significantly expands and complements the company's current RNAi estate– maintaining Outperform

Current Recommendation	**Outperform**
Prior Recommendation	N/A
Date of Last Change	06/11/2013
Current Price (03/05/15)	$7.90
Twelve- Month Target Price	**$15.00**

OUTLOOK

ARWR is a clinical stage biotech company focused on RNAi targeted therapy using DPC platform technology. We are optimistic about the Company's lead program ARC-520 for HBV.

ARWR just released initial Phase IIa data, which were as strong as expected, but the program is still viable in our view. The Company plans to initiate Phase IIb of ARC-520 in 1H15. The Company just initiated Phase I trial of its second clinical candidate ARC-AAT for liver disease. The acquisition of Novartis' RNAi assets expands and complements its current RNAi estate.

We maintain our Outperform rating on its shares.

SUMMARY DATA

52-Week High	**$26.57**
52-Week Low	**$5.00**
One-Year Return (%)	**-65.48**
Beta	**2.70**
Average Daily Volume (sh)	**1,661,201**
Shares Outstanding (mil)	**55**
Market Capitalization ($mil)	**$340**
Short Interest Ratio (days)	**4.01**
Institutional Ownership (%)	**N/A**
Insider Ownership (%)	**N/A**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**N/A**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2013 Estimate	**N/A**
P/E using 2014 Estimate	**N/A**
Zacks Rank	**N/A**

Risk Level	**High,**
Type of Stock	**Small-Growth**
Industry	**Biotechnology**
Zacks Rank in Industry	**N/A**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Dec)	Q2 (Mar)	Q3 (Jun)	Q4 (Sep)	Year (Sep)
2014	0.04 A	0.04 A	0.04 A	0.04 A	0.18 A
2015	0.17 A	0.04 E	0.04 E	0.04 E	0.29 E
2016					0.30 E
2017					0.50 E

Earnings per Share
(EPS is operating earnings before non recurring items)

	Q1 (Dec)	Q2 (Mar)	Q3 (Jun)	Q4 (Sep)	Year (Sep)
2014	-$0.19 A	-$0.25 A	-$0.24 A	-$0.43 E	-$1.15 A
2015	-$0.37 A	-$0.40 E	-$0.42 E	-$0.45 E	-$1.64 E
2016					-$1.71 E
2017					-$1.62 E

Zacks Projected EPS Growth Rate - Next 5 Years % **N/A**

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WHAT'S NEW

- **Acquisition of Novartis' RNAi assets significantly expands and complements its current RNAi estate;**
- **Phase I trial of ARC-AAT initiated;**
- **We remain bullish on Arrowhead shares;**

Arrowhead Acquires Novartis' RNAi Research and Development Portfolio

Earlier today (March 5), Arrowhead (ARWR) announced the acquisition of **Novartis' entire RNAi** research and development portfolio and associated assets.

The Deal

The acquisition includes:

- Multiple patent families covering RNAi-trigger design rules and modifications that fall **outside of key patents controlled by competitors,** which will provide freedom to operate for any target and indication;

- Novel intracellular targeting ligands that enhance the activity of RNAi-triggers by targeting the RNA-induced silencing complex (RISC) more effectively and improving stability once RISC is loaded;

- An assignment of Novartis' license from Alnylam Pharmaceuticals granting Arrowhead access to Alnylam intellectual property, excluding delivery, for **30 gene targets** chosen by Novartis;

- A pipeline of three pre-clinical candidates initiated by Novartis and for which Novartis has developed varying amounts of preclinical data.

Per the agreement, Arrowhead made a payment to Novartis of $7 million in cash and will provide an additional payment of $3 million in cash and $25 million in Arrowhead common stock within 30 days. Novartis is also eligible to receive milestones and single digit royalties on sales of future products in connection with the agreement.

Our Takeaways from the Acquisition

We think the acquisition is an important deal for Arrowhead. The RNAi assets acquired from Novartis are some of the best in the RNAi field as Novartis has been working in this area for the past decade.

We believe this acquisition substantially expands and complement Arrowhead's current RNAi assets. With access to non-delivery Alnylam RNAi IP for 30 targets and the three pre-clinical programs developed by Novartis, Arrowhead will have the capability to rapidly expand its pipeline dramatically down the road.

The company now has additional flexibility to optimize each new candidate using the most effective RNAi-trigger design and modifications. We look forward to seeing the new candidates nominated in the coming quarters.

Arrowhead Begins Phase I Trial of ARC-AAT

In late February, Arrowhead initiated dosing in a **Phase I** clinical trial of ARC-AAT for the treatment of liver disease associated with Alpha-1 Antitrypsin Deficiency (AATD) in Australia.

The Phase I trial is a multi-center, randomized, placebo-controlled, double-blind, single dose-escalation study to evaluate the safety, tolerability, pharmacokinetics of ARC-AAT and the effect on circulating alpha-1 antitrypsin (AAT) levels. The study plans to enroll in dose cohorts of six participants each, with participants randomized at a ratio of 2:1 (active:placebo) to receive a single intravenous injection of either ARC-AAT or placebo (normal saline). The study will consist of **two parts**, with Part A planned to be conducted in healthy volunteers and Part B planned to be conducted in patients with PiZZ genotype AATD. In Part A, dose escalation will proceed until the achievement of certain target knockdown parameters. When these AAT knockdown parameters are achieved, dose escalation in healthy volunteers (Part A) is planned to stop and dosing in patients with AATD (Part B) is planned to begin at the highest dose level used in Part A and then dose escalation will proceed. Participants will be evaluated for 28 days following dosing with additional follow-up every 2 weeks until AAT levels return to baseline.

Initial data from this study is expected **in late 2015**.

Arrowhead has signed an agreement with the Alpha-1 Project (TAP), a subsidiary of the **Alpha-1 Foundation**. Under the terms of the agreement, TAP will partially fund the development of ARC-AAT. In addition to the funding, TAP will make its scientific advisors available to Arrowhead, assist with patient recruitment for clinical trials thanks to the Alpha-1 Foundation Patient Research Registry, and engage in other collaborative efforts that support the development of ARC-AAT.

ARC-AAT is Arrowhead's second clinical candidate, which further de-risk its pipeline. In the absence of specific therapy other than liver transplant for AATD associated liver disease, we believe RNAi holds great potential.

With the experience of two candidates ARC-520 and ARC-AAT, the DPC platform, the recently acquired RNAi assets from Novartis, as well as a strong balance sheet, we believe Arrowhead is well positioned to further diversify its pipeline down the road.

We Remain Bullish on Arrowhead's Shares

We maintain our Outperform rating on Arrowhead shares and reiterate our 12-month price target of $15.

The acquisition of Novartis' RNAi development portfolio significantly expands and complements the company's current RNAi assets and will provide the opportunity to rapidly expand its clinical pipeline in the coming years.

Current market price of Arrowhead shares does not reflect the intrinsic value of the company. We think there is still enough room for further price appreciation at current market price.

Based on the current development plan, we expect Arrowhead will be able to initiate **pivotal Phase III** trial of ARC-520 in late 2016, and data should be available in mid-2017. NDA could be filed in late 2017, and the FDA approval is in late 2018.

We estimate Arrowhead will become profitable in fiscal 2019 with an EPS of $1.09 based on revenue of $250 million. We think a P/E ratio of 34x is appropriate for Arrowhead considering its growth in the next few years. Based on this P/E multiple, we think Arrowhead should be valued at $15.00 per share using 25% discount for 4 years. Our price target values Arrowhead at $825 million in market cap, which we think is appropriate and fare compared to its peers.

PROJECTED INCOME STATEMENT

_	2014 (Sept)					2015 (Sept)					2016 (Sept)	2017 (Sept)	2018 (Sept)	2019 (Sept)
$ in millions except per share data	Q1	Q2	Q3	Q4	FY	Q1	Q2	Q3	Q4	FYE	FYE	FYE	FYE	FYE
Revenue	$0.04	$0.04	$0.04	$0.04	$0.18	$0.17	$0.04	$0.04	$0.04	$0.29	$0.30	$0.50	$25.00	$250.00
YOY Growth											-	*66.7%*	*4900.0%*	*900.0%*
Total Revenues	**$0.04**	**$0.04**	**$0.04**	**$0.04**	**$0.18**	**$0.17**	**$0.04**	**$0.04**	**$0.04**	**$0.29**	**$0.30**	**$0.50**	**$25.00**	**$250.00**
YOY Growth	*-73.0%*	*0.0%*	*0.0%*	*1.7%*	*-39.7%*	*297.1%*	*-8.6%*	*-8.6%*	*-10.1%*	*66.1%*	*3.2%*	*66.7%*	*4900.0%*	*900.0%*
Cost of Revenue	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.1	$3.8	$35.0
Gross Income	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.2**	**$0.2**	**$0.0**	**$0.0**	**$0.0**	**$0.3**	**$0.3**	**$0.4**	**$21.3**	**$215.0**
Gross Margin	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*85.0%*	*85.0%*	*86.0%*
R&D	$3.1	$5.2	$6.4	$8.4	$23.1	$17.7	$15.0	$16.0	$17.0	$65.7	$70.0	$75.0	$55.0	$60.0
% R&D	*7286.1%*	*11923.3%*	*14610.7%*	*18868.3%*	*13221.7%*	*10392.0%*	*37500.0%*	*40000.0%*	*42500.0%*	*22612.0%*	*23333.3%*	*15000.0%*	*220.0%*	*24.0%*
SG&A	$3.0	$4.4	$4.0	$7.2	$18.7	$5.2	$5.5	$6.0	$6.5	$23.2	$25.5	$30.0	$37.5	$50.0
% SG&A	*6966.5%*	*10161.3%*	*9227.2%*	*16281.6%*	*10699.1%*	*3067.0%*	*13750.0%*	*15000.0%*	*16250.0%*	*7992.0%*	*8500.0%*	*6000.0%*	*150.0%*	*20.0%*
Other expenses	$0.9	$1.6	$2.3	$2.2	$7.0	$2.3	$1.5	$1.5	$2.0	$7.3	$7.0	$8.5	$10.0	$12.0
% Other	*2150.1%*	*3643.9%*	*5290.8%*	*4962.5%*	*4023.9%*	*1349.9%*	*3750.0%*	*3750.0%*	*5000.0%*	*2512.4%*	*2333.3%*	*1700.0%*	*40.0%*	*4.8%*
Operating Income	**($7.0)**	**($11.2)**	**($12.7)**	**($17.8)**	**($48.7)**	**($25.1)**	**($22.0)**	**($23.5)**	**($25.5)**	**($96.0)**	**($102.2)**	**($113.1)**	**($81.3)**	**$93.0**
Operating Margin	-	-	-	-	-	-	-	-	-	-	-	-	-	*37.2%*
Other Income (Net)	($3.6)	($2.7)	$1.1	$0.7	($4.5)	$2.5	$0.0	$0.0	$0.0	$2.5	($0.5)	($0.5)	($0.5)	($0.5)
Pre-Tax Income	**($10.6)**	**($13.9)**	**($11.6)**	**($17.1)**	**($53.3)**	**($22.6)**	**($22.0)**	**($23.5)**	**($25.5)**	**($93.5)**	**($102.7)**	**($113.6)**	**($81.8)**	**$92.5**
Net Taxes (benefit)	$0.0	$0.0	$0.0	$5.4	$5.4	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Tax Rate	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*-10.2%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*
Reported Net Income	**($10.6)**	**($13.9)**	**($11.6)**	**($22.5)**	**($58.7)**	**($22.6)**	**($22.0)**	**($23.5)**	**($25.5)**	**($93.5)**	**($102.7)**	**($113.6)**	**($81.8)**	**$92.5**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net Margin	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Weighted avg. Shares Out	*37.7*	*44.3*	*51.9*	*53.7*	*46.9*	*54.7*	*55.0*	*56.0*	*56.5*	*55.5*	*60.0*	*70.0*	*80.0*	*85.0*
Reported EPS	**($0.28)**	**($0.31)**	**($0.22)**	**($0.42)**	**($1.25)**	**($0.41)**	**($0.40)**	**($0.42)**	**($0.45)**	**($1.68)**	**($1.71)**	**($1.62)**	**($1.02)**	**$1.09**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-	-	-
One time charge	$3.62	$2.73	($1.07)	($0.73)	$4.55	$2.54	$0.00	$0.00	$0.00	$2.54	$0.00	$0.00	$0.00	$0.00
Non GAAP Net Income	**($7.0)**	**($11.2)**	**($12.7)**	**($23.3)**	**($54.2)**	**($20.0)**	**($22.0)**	**($23.5)**	**($25.5)**	**($90.9)**	**($102.7)**	**($113.6)**	**($81.8)**	**$92.5**
Non GAAP EPS	**($0.19)**	**($0.25)**	**($0.24)**	**($0.43)**	**($1.15)**	**($0.37)**	**($0.40)**	**($0.42)**	**($0.45)**	**($1.64)**	**($1.71)**	**($1.62)**	**($1.02)**	**$1.09**

Source: Company filing and Zacks Investment Research estimates

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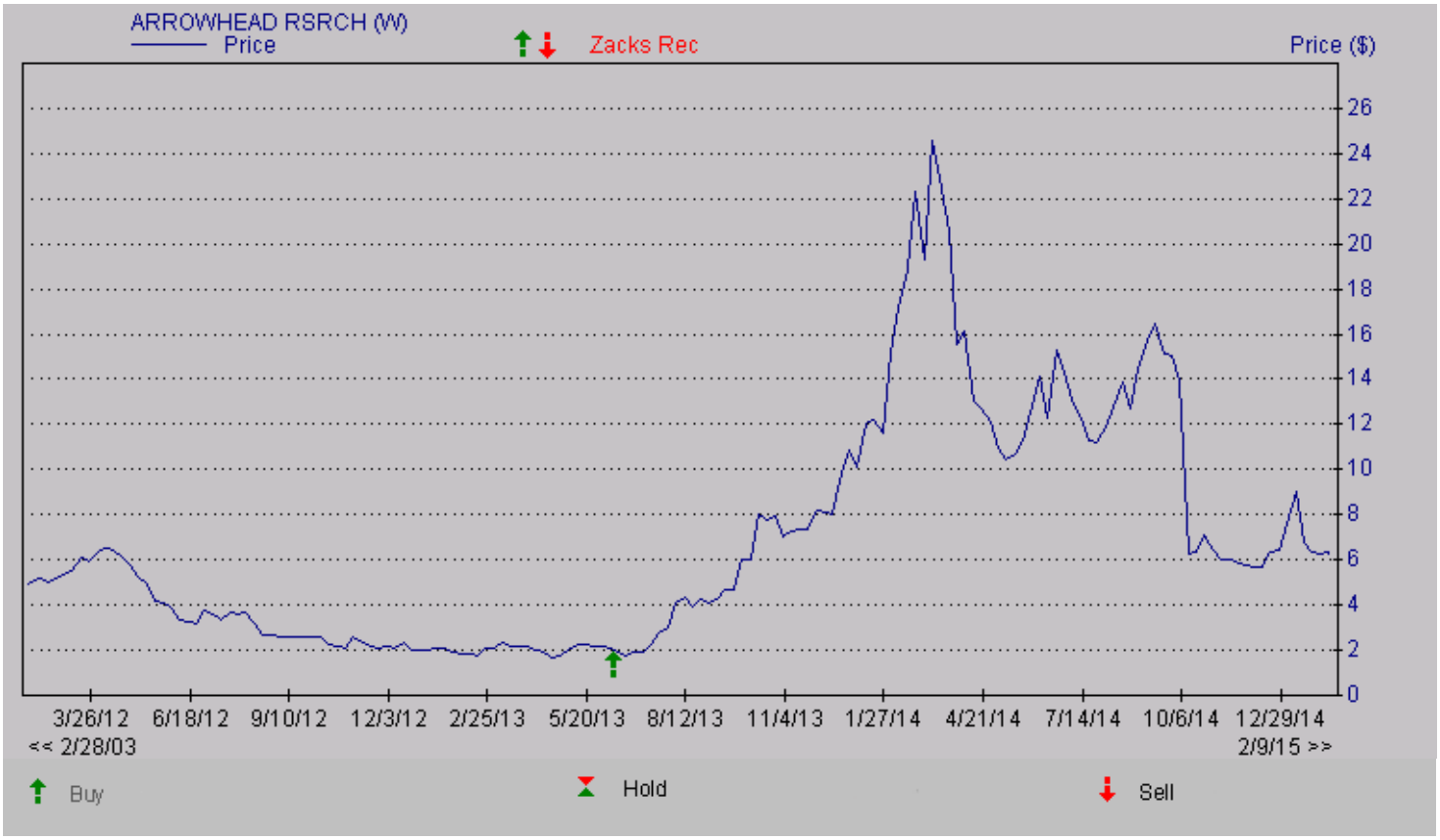

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