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Zacks Small-Cap Research

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Arrowhead Pharm (ARWR-NASDAQ)

ARWR: Q1 Results. Clinical Trial Activity Accelerating

Relative valuation metrics indicates a fair value at $30/share.

Current Price (02/12/19)	$16.89
Valuation	**$30.00**

OUTLOOK

Arrowhead's clinical programs are showing no signs of slowing down with 2019 already slated to be a very busy year. In fact, management's roster of goals for fiscal 2019 implies that activity should accelerate further and include data from of several of their ongoing and new clinical trials and initiation of a Phase II program for ARO-AAT (in alpha-1 antitrypsin deficiency). Data is expected during the year from ARO-HBV (chronic hepatitis B) Phase I/II as well as two Phase I studies in hypertriglyceridemia; ARO-ANG3 (targeting ANGPTL3) and ARO-APOC3 (targeting apoC-III). ARO-ANG3 commenced dosing in early January (see below) while the Phase I ARO-APOC3 study, which has ethics approval, is awaiting regulatory go-ahead. We could also potentially get results from the Phase I study of AMG 890 (cardiovascular disease), for which ARWR is partnered with Amgen.

In addition, Arrowhead hopes to make additional progress with their preclinical candidates including anticipated filing of clinical trial applications (CTA) for their two non-liver programs; ARO-ENaC (cystic fibrosis) and ARO-HIF2 (clear cell renal cell carcinoma).

SUMMARY DATA

52-Week High	**$22.39**
52-Week Low	**$4.77**
One-Year Return (%)	**218.64**
Beta	**2.23**
Average Daily Volume (sh)	**1,702,284**
Shares Outstanding (mil)	**94**
Market Capitalization ($mil)	**$1,586**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**60**
Insider Ownership (%)	**5**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**285.1**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2019 Estimate	**13.3**
P/E using 2020 Estimate	**29.7**
Zacks Rank	**N/A**

Risk Level	**High,**
Type of Stock	**Mid-Growth**
Industry	**Med-Drugs**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Dec)	Q2 (Mar)	Q3 (Jun)	Q4 (Sep)	Year (Sep)
2017	4.37 A	8.99 A	9.34 A	8.71 A	31.4 A
2018	3.50 A	0.65 A	0.73 A	11.26 A	16.14 A
2019	34.66 A	43.33 E	58.70 E	43.07 E	179.6 E
2020					121.9 E

Price/Sales Ratio (Industry = 2.5x)

	Q1 (Dec)	Q2 (Mar)	Q3 (Jun)	Q4 (Sep)	Year (Sep)
2017	-$0.21 A	-$0.08 A	-$0.08 A	-$0.14 A	-$0.50 A
2018	-$0.18 A	-$0.18 A	-$0.18 A	-$0.12 A	-$0.65 A
2019	$0.13 A	$0.17 E	$0.29 E	$0.22 E	$0.81 E
2020					$0.14 E

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

WHAT'S NEW

2019 Goals Include: Data from HBV, ANG3 and APOC3 (and possibly AMG-890), Begin AAT Ph2 Program

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In addition, Arrowhead hopes to make additional progress with their preclinical candidates including anticipated filing of clinical trial applications (CTA) for their two non-liver programs; ARO-ENaC (cystic fibrosis) and ARO-HIF2 (clear cell renal cell carcinoma).

As it relates to a Phase II program for ARO-AAT…

Management indicated on the Q1 call that a Phase II study for ARO-AAT could potentially not only move along rapidly, but also possibly even act as a pivotal study for the alpha-1 antitrypsin deficiency candidate. Long-term toxicology studies are now complete and discussions, which initiated in October, are ongoing with FDA around design of a Phase II study. Given that AAT-related liver disease is a novel target, ARWR and FDA are essentially designing from scratch – while that may imply a possibly more involved undertaking, eventual 'success' (i.e. FDA's blessing) could represent a more atypically significant step forward and a potential value inflection event. We will be eager to hear future updates on ARWR's discussions with FDA regarding the study design, including proposed endpoints.

As it relates to ARO-ANG3 Phase I Study…

In early January Arrowhead announced commencement of dosing of the initial subjects enrolled in their Phase I study (ARO-ANG1001) evaluating the safety, tolerability, PK and PD effects of ARO-ANG3 in healthy individuals and those with dyslipidemia. As a reminder, ARO-ANG3 is ARWR's subcutaneously-administered RNAi-based candidate developed to target angiopoietin like protein 3 (ANGPTL3), which has shown to be involved in the regulation of lipoprotein levels, including triglycerides, LDL cholesterol, HDL and very low-density lipoprotein cholesterol.

This Phase I study, expected to enroll up to 70 subjects, has single and multiple dose phases. The single-dose portion will include 4 cohorts, each consisting of 10 healthy adults (6 treatment, 4 placebo), with each participant receiving a single dose (ARO-ANG3 or placebo) at 35mg, 100mg, 200mg or 300mg. The multi-dose portion encompasses up to 4 patient cohorts including those with non-alcoholic fatty liver disease (NAFLD), those on statin treatment with high LDL cholesterol and triglycerides, those with familial hypercholesterolemia, and patients with severe hypertriglyceridemia.

Preclinical (mouse and monkey models) results have been promising, showing substantial and durable reductions in serum ANGPTL3 and liver mRNA, as well as reductions in triglycerides and LDL levels. Over 90% knock down was observed in mouse models. In addition to representing a potential eventual treatment for dyslipidemia, ARO-ANG3 could have utility in certain metabolic diseases as well, including NAFLD and nonalcoholic steatohepatitis (NASH). All of these represent significantly sized markets and with unmet therapeutic needs.

As it relates to ARO-APOC3 Phase I study…

Also in early January, ARWR announced that they submitted an application to New Zealand regulatory authorities requesting approval to begin a phase I study of ARO-APOC3, ARWR's subcutaneously-administered RNAi-based candidate targeting Apolipoprotein C-III (apoC-III) and being developed for the treatment of hypertriglyceridemia. Management noted on the fiscal Q1 2019 call that they have received

ethics approval, are awaiting regulatory feedback and are preparing to commence the study once approvals are granted.

The study, expected to enroll up to 63 subjects, also has single and multiple dose phases and will be used to help inform subsequent development. It is expected to enroll both healthy subjects and various populations of patients with elevated triglycerides. Up to 90% knock down was observed in rodent models.

Q1 2019 Results

Arrowhead reported financial results for their fiscal 2019 first quarter ending December 31, 2018. **Total revenue** was $34.7M (versus our $55.8M estimate), representing recognition of a portion of the $197.8M transaction price of the Janssen / JJDC collaboration agreement, which closed in late October 2018. Management expects the majority of the $197.8M to be recognized as revenue in the current fiscal year, with the remainder expected to be booked in 2020.

As a reminder, ARWR received $250M upfront and is eligible for another $3.5B in potential additional milestones and for royalties on eventual commercial sales. The collaboration is expected to speed development of ARWR's hepatitis B therapeutic candidate, ARO-HBV (which subsequently changed names to JNJ 3989). The upfront payment consists of $175M cash from Janssen plus a $75M equity investment (@ $23.00/share) from Johnson & Johnson Innovation – JJDC, Inc.

In return, Janssen receives worldwide exclusive license to ARO-HBV and an option to collaborate with ARWR on up to three additional RNAi therapeutics for new targets (to be selected by Janssen). Janssen will be solely responsible for development and commercialization beyond ARWR's ongoing Phase 1/II study. The other optional targets will leverage ARWR's TRIM platform technology but will not include any of the company's current pipeline. For these optioned targets, Janssen will fund initial discovery and preclinical work by ARWR and will take over each program following an IND filing by ARWR.

Specifically, as it relates to potential milestone payments, ARWR is eligible to receive $1.6B for the HBV license, which is inclusive of $50M in a milestone for a Phase 2 study. Up to another $1.9B could be received for the agreement related to other three targets. Commercial sales royalties would be tiered and at a rate of up to the mid-teens percentage.

Q1 operating expenses were $23.7M (versus our $22.7M estimate), which includes $17.6M ($17.2M E) of R&D expense and $6.1M ($5.5M E) in salary/G&A expense. We continue to expect OpEx, and in particular R&D expense, to increase as ARWR's various studies progress to later stages and as new clinical candidates enter the pipeline.

EPS was $0.13, compared to our and average consensus estimates of $0.38 and $0.54, respectively.

Cash

ARWR exited fiscal Q1 with $303.3M in cash and investments, inline with management's prior guidance of ending the quarter with approximately $305M. The increase from fiscal Q4 2018 ($30.1M) is mostly a result of proceeds from the Janssen collaboration. ARWR generated $168.3M (or $15.8M ex-changes in working capital) in cash from operations.

Clinical Programs Refresher

Clinical Data of ARO-HBV (JNJ 3989) Presented

In November 2018 preliminary data from ARWR's Phase I/II study of ARO-HBV for the treatment of HBV was presented as a late-breaking poster at the Liver Meeting of the Annual Meeting of the American Association for the Study of Liver Disease (AASLD).

Background of the ARO-HBV Program

In Mach 2018, Arrowhead initiated a **Phase I/II** first-in-human study of **ARO-HBV** for the treatment of chronic hepatitis B virus **(HBV)** infection in New Zealand. Dosing began on May 14, 2018.

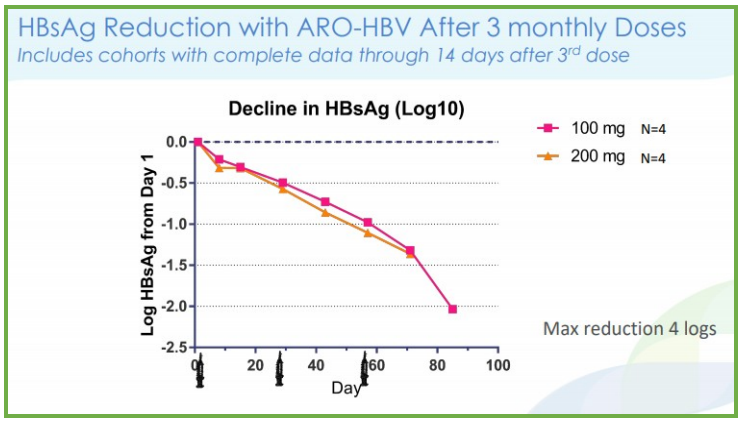
The study, **AROHBV1001** (NCT03365947), is a Phase I/II study to evaluate the safety, tolerability, and pharmacokinetic effects of single-ascending doses (**SAD**) of ARO-HBV in healthy adult volunteers, and to evaluate the safety, tolerability, and pharmacodynamic effects of multiple-ascending doses (**MAD**) of ARO-HBV in patients with chronic HBV.

The SAD portion is designed to include up to 5 cohorts of 6 subjects per cohort. Each SAD subject will receive a single-dose administration of either placebo or ARO-HBV at up to 5 dose levels (35, 100, 200, 300, 400 mg). The MAD portion is designed to include up to 8 cohorts of 4 HBV patients per cohort. Each MAD patient will receive 3 doses of ARO-HBV at up to 4 dose levels (100, 200, 300, 400 mg).

In May 2018, the company completed enrollment and dosing of all 5 planned cohorts of healthy adult volunteers in the SAD portion of the Phase I/II study of ARO-HBV. The first 2 MAD cohorts at doses of 100 mg and 200 mg have been fully enrolled.

The Clinical Data Presented at the Summit

On Sep. 6, 2018, Arrowhead presented initial clinical data for **ARO-HBV** at the 18th World Gastroenterologists Summit in Auckland, New Zealand. Arrowhead presented initial data of **eight patients** from the first two MAD dose cohorts of the AROHBV1001 clinical study: 100mg and 200mg.



Following is the summary of the key clinical data presented at the Summit:

- Maximum reduction of HBsAg was 4.0 log10 (99.99%) after three monthly doses of ARO-HBV
- Mean reduction of HBsAg was 2.0 log10 (99%) on day 85 in cohort 2b (100 mg) and 1.4 log10 (96%) on day 71 in cohort 3b (200 mg)
- Minimum HBsAg reduction in all patients from cohorts 2b and 3b was 1.2 log10 (93%)
- Activity was demonstrated in all patient types (HBeAg pos/neg, NUC naïve/treated)

ARO-HBV was generally well-tolerated with generally mild and self-limiting injection site adverse events being the most common reported event in chronic HBV patients, occurring in around 10% of injections. The other most commonly reported events included symptoms consistent with upper respiratory tract infection and headache.

These results represent the first clinical data presented on ARO-HBV, which was very encouraging in our view. The data indicated that ARO-HBV is highly active in the treatment of HBV with good safety profile.

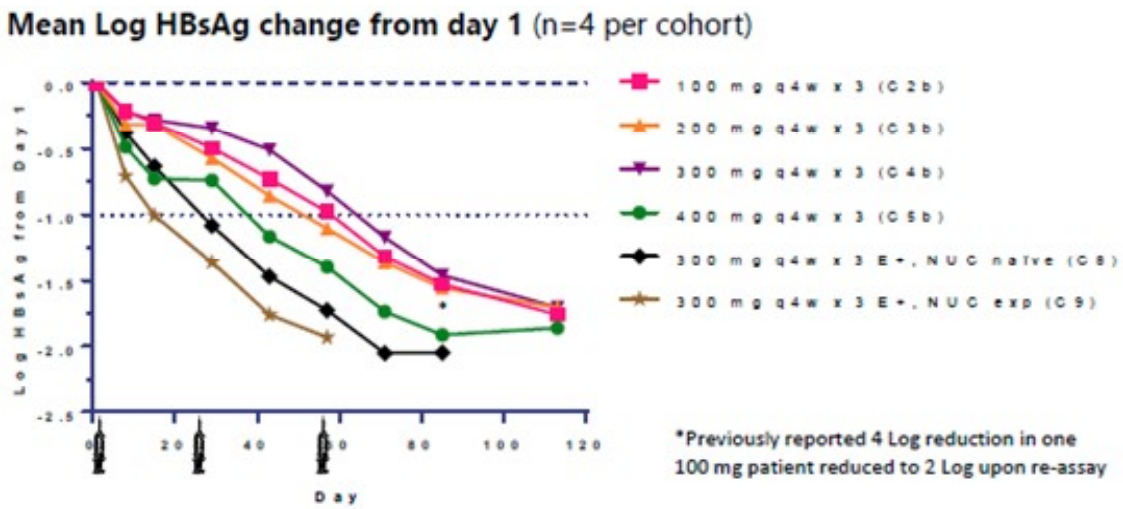
Clinical Data Presented at the AASLD in November 2018

In November 2018 preliminary data from ARWR's Phase I/II study of ARO-HBV for the treatment of HBV was presented as a late-breaking poster at the Liver Meeting of AASLD. This is the first data presentation of ARO-HBV since consummating the collaboration with Janssen

Following is the summary of the key clinical data presented at AASLD:

- Well-tolerated: while mild injection-site reactions were reported in ~12% of injections, ARO-HBV appears to be well-tolerated at single and multiple doses up to 400mg
- Strong HBsAg responses in both HBeAg positive and HBeAg negative patients:
  - all patients showed strong response with mean NADIR of -1.9 Log10 (-98.7%) and ranging from -1.3 (-95.0%) to -3.8 Log10 (-99.98%)
  - HBeAg-positive: Mean HBsAg NADIR in HBeAg positive (n=11) -2.1 Log10
  - HBeAg-negative: Mean HBsAg NADIR in HBeAg negative (n=13) -1.8 Log10
- Reductions similar for NUC naïve and NUC experienced patients
  - Mean HBsAg reduction on day 57 for cohort 8 (n=4) -1.7 Log10
  - Mean HBsAg reduction on day 57 for cohort 9 (n=4) -1.9 Log10
- Improved response from 1st-gen: responses observed were superior to those of the first-generation (ARC-520), which targeted only HBV transcripts
- Response silences HBV: investigators believe that the observed responses are consistent with the ability of ARO-HBV to silence HBV mRNA from cccDNA and host integrated viral DNA
- Other viral parameters showed response: including HBV DNA, HBV RNA, HBeAg and HBcrAg
- While no strong dose response…was observed between 100mg and 400mg, additional patients are enrolling to hopefully better understand possible dose response

AASLD Poster: Strong Response Observed in All Patients



SOURCE: Gane, E. et al. ARWR. AASLD Poster Presentation. Nov 2018

Arrowhead also expects to present additional data from this study and noted on their fiscal Q1 2019 call that they have already accepted presentations at the Asian Pacific Association for the Study of the Liver (APASL) meeting (February 20th – 24th) as well as the EASL International Liver Congress (April). ARWR also expects to report additional data throughout the current year and submit additional abstracts for publishing.

With Janssen now onboard as a collaboration partner, there is no concern about funding the ARO-HBV development program. And, ARWR expects to recognize revenue in the amount of $198M (~$35M of which has been recognized to-date) as they complete the oversight of the Phase 1/2 study of ARO-HBV. This reflects the upfront payment from Janssen, the premium paid by JJDC on ARWR common stock and material on-hand and to be manufactured for the study.

Update on Amgen Collaboration Programs

On August 1, 2018, Arrowhead announced that it has earned a $10 million milestone payment from Amgen following the administration of the first dose of AMG 890, formerly referred to as ARO-LPA, in a clinical study.

Amgen is evaluating **AMG 890** in a **Phase I** clinical study designed to assess its safety in volunteers with elevated levels of lipoprotein (a) (Lp(a)). AMG 890 is an RNAi therapeutic designed to lower Lp(a) for the treatment of cardiovascular disease. Initial data from the study could be available later this year or early 2020.

In September 2016, Arrowhead announced two license and collaboration agreements with Amgen to develop and commercialize **two cardiovascular programs** based on Arrowhead's RNAi platform. These programs will utilize Arrowhead's proprietary **subcutaneous RNAi delivery technology**.

Pursuant to one agreement, Amgen receives a worldwide, exclusive license to Arrowhead's novel, RNAi **ARC-LPA program**, which is designed to reduce production of apolipoprotein A, a key component of lipoprotein(a), which has been genetically linked with increased risk of cardiovascular diseases, independent of cholesterol and LDL levels. ARC-LPA is Arrowhead's first drug candidate to use a subcutaneously administered delivery construct. Elevated lipoprotein(a), or Lp(a), is widely viewed as a key risk factor for cardiovascular diseases, including coronary artery disease, atherosclerosis, thrombosis and stroke.

Under the second agreement, Amgen receives an option to a worldwide, exclusive license for a RNAi therapy for an **undisclosed genetically validated cardiovascular target**. In both agreements, Amgen will be wholly responsible for clinical development and commercialization.

In connection with the two collaborations, Arrowhead received $35 million in upfront payments; $21.5 million in the form of an equity investment by Amgen in Arrowhead common stock (about 3 million shares); and up to $617 million in option payments, and development, regulatory and sales milestone payments. Arrowhead is further eligible to receive single digit royalties for sales of products against the undisclosed target and up to low double-digit royalties for sales of products under the ARC-LPA agreement.

Update on ARO-AAT Phase I Program for Alpha-1 Liver Disease

On Aug. 31, 2018, Arrowhead announced that it completed dosing and in November updated data from this study was the subject of a late-breaking poster presentation at AASLD in November (see below). This follows a presentation in late-June of **preclinical and initial clinical data** on **ARO-AAT** for the treatment of alpha-1 antitrypsin **(AAT)** deficiency at the Alpha-1 National Education Conference in San Francisco.

Background of the Phase I Study

In February 2018, Arrowhead received approval from the New Zealand Medicines and Medical Devices Safety Authority (MEDSAFE) and from the local Ethics Committee to proceed with a first-in-human **Phase I** study of **ARO-AAT** for the treatment of **alpha-1 antitrypsin deficiency (AATD)**. The study began **dosing** patients in March 2018.

The study, which is designated as **AROAAT1001** (NCT03362242), is a Phase I single- and multiple-ascending dose study to evaluate the safety, tolerability, pharmacokinetics, and effect of ARO-AAT on serum alpha-1 antitrypsin levels in healthy adult volunteers. The study has two parts: double blind and unblinded.

DOUBLE BLIND PART

- 4 treatment arms
  - 35, 100, 200 and 300 mg
  - 100, 200, 300 mg receive ***3 monthly doses***
  - 4 active, 4 placebo
- Assessments of safety, tolerability, plasma levels of ARO-AAT, plasma AAT changes

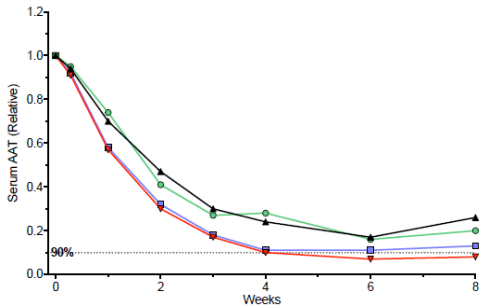
UNBLINDED PART

- No placebo
- 3 groups
  - ***Single doses*** of 100, 200 and 300 mg of ARO-AAT
  - 4 per cohort
- Assessments of safety, tolerability, depth and duration of AAT reductions after a single dose

Key Initial Clinical Data Presented at the Alpha-1 National Education Conference

In the AROAAT1001 **Phase I** clinical study, a single, open-label dose of 100 mg of ARO-AAT in four subjects achieved 93% maximum serum AAT knockdown and 87% mean maximum serum AAT knockdown. At 8 weeks post-dose, mean serum AAT knockdown remained at 83%.

Open Label AAT Plasma Data at 100 mg:
Single Dose, Healthy Volunteers



93%: Maximum Serum AAT Reduction achieved 6-weeks following a single dose
87%: Mean maximum serum AAT reduction achieved 6-weeks following a single dose

The single 100 mg dose of ARO-AAT equates to an average dose of 1.4 mg/kg (range 1.0-1.6 mg/kg) in the subjects studied, who had an average weight of 72.9 kg (range 61.8-98.9 kg).

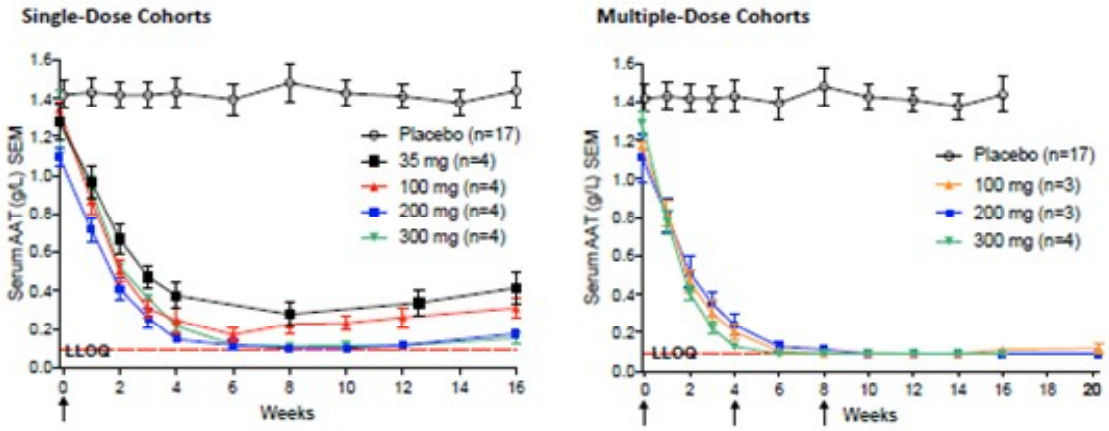
ARO-AAT appeared to be generally well-tolerated and as of the data cutoff of June 11, 2018, the following safety measures were observed in 40 subjects (24 received ARO-AAT and 16 received placebo):

- No serious or severe adverse events (AEs)
- Most AEs reported were mild (one moderate gastroenteritis)
- Two cases of injection site erythema at 100 mg after 1st dose, both were classified as mild and resolved within 48 hours
- No clinically meaningful adverse changes in BUN, creatinine, ALT, AST or total bilirubin
- No dose-related pattern of adverse laboratory changes seen

Poster Presentation at AASLD: No Serious Safety Concerns, Substantial NADIR Serum Reductions…
On Aug. 31, 2018, Arrowhead announced that it completed dosing and in November updated data from this study was the subject of a late-breaking poster presentation at AASLD in November (see below). Results showed that ARO-AAT was well tolerated at doses as high as 300mg administered 3x/day for 28 days and no deaths or serious adverse events were reported. Moreover, maximum serum nadir reductions were substantial even among the single-dose cohorts.

Single dose reductions were 79%, 87%, >91% and >91% at 35, 100, 200 and 300mg doses, respectively. Meanwhile, multi-dose nadir reductions were all greater than 91% with most patients below the level of quantification. Maximum serum reduction was 94% (in the 300mg multi-dose cohort). Data to-date has shown that among the 100mg cohort, a 90% reduction was sustained for at least 8 weeks (100mg cohort is the only one so far with this length of post-dosing data).

While no serious adverse events were reported, the most common (non-serious) adverse events were headache (22%) and rhinorrhea (13%), or runny nose. Given the substantial nadir reduction and duration of effect, investigators believe that quarterly or less frequent dosing may be feasible – which presumably would further benefit the safety profile.



Serum AAT Relative Percentage Reduction Summary

	NADIR Single Dose				NADIR Multiple-Dose		
	35 mg N=4	100 mg N=4	200 mg N=4	300 mg N=4	100 mg N=3	200 mg N=3	300 mg N=4
Average Max	79.0	87.7	>91.5	>91.1	>92.2	>91.2	>93.0
SD	8.5	4.5	0.6	4.0	0.2	2.7	0.7
Max	91.1	93.3	>92.0	>94.9	>92.7	>93.3	>93.7
BLQ, n=	0	0	3	3	2	3	4

SOURCE: ARWR, Schwabe C. et al. AASLD Poster Presentation Nov 2018

ARO-AAT Phase II Studies…
Management indicated on the fiscal Q1 2019 (Feb 7th) call that a Phase II study for ARO-AAT could potentially not only move along rapidly, but also possibly even act as a pivotal study for the alpha-1 antitrypsin deficiency candidate. Long-term toxicology studies are now complete and discussions, which initiated in October, are ongoing with FDA around design of a Phase II study. Given that AAT-related liver disease is a novel target, ARWR and FDA are essentially designing from scratch – while that may imply a possibly more involved undertaking, eventual 'success' (i.e. FDA's blessing) could represent a more atypically significant step forward and a potential value inflection event. We will be eager to hear future updates on ARWR's discussions with FDA regarding the study design, including proposed endpoints.

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Additional Pipeline Update
Arrowhead has also achieved continued progress with the company's **extra-hepatic platform** and pipeline, including:

- ARO-Lung1, Arrowhead's first candidate against an undisclosed gene target in the lung, which achieved nearly 90% target knockdown following inhaled administration in rodents
- ARO-HIF2, the Company's candidate targeting renal cell carcinoma, which achieved 85% target gene knockdown in a rodent tumor model. Arrowhead hopes to file a CTA for ARO-HIF2 this year

- ARO-ENaC, Arrowhead's candidate targeting the epithelial sodium channel (ENaC) alpha subunit for treatment of cystic fibrosis. The company hopes to file a CTA for ARO-ENaC this year

Update on ARO-HIF2 for Kidney Cancer

In September 2015, Arrowhead nominated **ARO-HIF2** as its first therapeutic candidate delivered using a new Dynamic Polyconjugate™ (DPC™) designed to target tissues outside of the liver. Arrowhead believes that ARO-HIF2, which uses RNA interference to silence transcription factor hypoxia-inducible factor 2α (HIF-2α), is a promising new candidate for the treatment of clear cell renal cell carcinoma **(ccRCC).**

ARO-HIF2 is designed to inhibit the production of HIF-2α, which has been linked to tumor progression and metastasis in ccRCC. Using ARC-HIF2 in a preclinical ccRCC tumor model, mice treated with weekly injections led to greater than 80% knockdown of HIF-2α mRNA in tumors. Furthermore, tumors from treated mice exhibited statistically significant reductions in size and weight, extensive tumor cell death, reduction in the tumor-expressed VEGF-A biomarker, and destruction of the blood vessels feeding the tumors.

The company presented positive **preclinical data** at the European Cancer Congress 2015 (ECC2015) in Vienna on September 27, 2015 in a poster titled "HIF-2α targeting with a novel RNAi delivery platform as therapy for renal cell carcinoma," (abstract #353). The company further presented positive preclinical data on ARO-HIF2 at AACR2016.

The poster presentation described data from various stages of development of ARO-HIF2, including RNAi trigger selection, HIF2-α target validation, delivery and targeting ligand validation, and multiple RCC tumor models. These data show that important advancements are being made in this program and for Arrowhead's Dynamic Polyconjugate™ (DPCTM) delivery platform generally, including the following key findings:

- Proof-of-concept ligand dependent, functional delivery was demonstrated using the DPC targeted delivery platform
- Silencing HIF2-α expression by RNA interference resulted in reduction of HIF-2α regulated genes
- In two different RCC tumor bearing mouse models, ARO-HIF2 inhibited tumor growth and promoted tumor cell death and structural degeneration

The company is in the process of manufacturing scale up to allow for initiation of IND-enabling studies. Arrowhead hopes to file a CTA for ARO-HIF2 this year.

Valuation

We Remain Optimistic about the Prospect of Arrowhead

We continue to be optimistic about the prospect of Arrowhead and maintain our fair valuation of $30 per share. We believe management decision to focus on the subQ and extra-hepatic programs is prudent. The Janssen collaboration brings significant operating capital, nearly completely de-risks ARO-HBV (at least from a financial standpoint) and provides possible additional shots on goal with potential future additions to the pipeline.

Each of ARWR's pipeline candidates has been shown to be highly active against its respective target. For example, ARC-AAT achieved 90% knockdown of serum AAT, which is believed to be near full suppression of liver production of the protein, in a **Phase I** clinical study. The AAT and HBV programs have moved along rapidly with both showing highly encouraging results to-date. Safety and tolerability appear to be acceptable while suggestive efficacy of both are, in our opinion, extremely encouraging. Phase 2 studies, if successful, could represent value inflection events.

ARWR's other pipeline programs, including ARO-APOC3 and ARO-ANG3, represent additional incremental - although at this stage, more option-like value, in our opinion. Further progress on the

earlier-stage pipeline should provide additional insight into risk-adjustments and ultimate potential tangible commercial value of those candidates.

Our price target $30 per share values the company at about $2.8 billion in market capitalization, which we think is appropriate at this time.

PROJECTED INCOME STATEMENT

	2018 (Sept)					2019 (Sept)					2020 (Sept)
$ in millions except per share data	Q1	Q2	Q3	Q4	FYE	Q1	Q2	Q3	Q4	FYE	FYE
Revenue	$3.51	$0.65	$0.73	$11.26	$16.14	$34.66	$43.13	$58.70	$43.07	$179.56	$121.90
YOY Growth	*-19.6%*	*-92.8%*	*-92.2%*	*29.2%*	*-48.6%*	*887.5%*	*6534.1%*	*7970.1%*	*282.7%*	*1012.3%*	*251.7%*
Total Revenues	**$3.51**	**$0.65**	**$0.73**	**$11.26**	**$16.14**	**$34.66**	**$43.13**	**$58.70**	**$43.07**	**$179.56**	**$121.90**
YOY Growth	*-19.6%*	*-92.8%*	*-92.2%*	*29.2%*	*-48.6%*	*887.5%*	*6534.1%*	*7970.1%*	*282.7%*	*1012.3%*	*251.7%*
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
Gross Income	**$3.5**	**$0.7**	**$0.7**	**$11.3**	**$16.1**	**$34.7**	**$43.1**	**$58.7**	**$43.1**	**$179.6**	**$121.9**
Gross Margin	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*	*100.0%*
R&D	$12.9	$12.0	$12.1	$16.0	$53.0	$17.6	$22.2	$24.1	$14.6	$78.5	$82.6
% R&D	*368.1%*	*1846.2%*	*1657.0%*	*142.1%*	*328.1%*	*50.7%*	*51.5%*	*41.1%*	*33.9%*	*43.7%*	*67.8%*
Salary and G&A	$4.4	$3.7	$4.6	$6.4	$19.1	$6.1	$5.8	$6.6	$7.0	$25.5	$26.9
% SG&A	*125.5%*	*566.3%*	*631.6%*	*57.1%*	*118.4%*	*17.7%*	*13.4%*	*11.2%*	*16.3%*	*14.2%*	*22.1%*
Other expenses	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
% Other	*0.0%*	*0.0%*	*0.0%*	*0.1%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*
Operating Income	**($13.8)**	**($15.0)**	**($15.9)**	**($11.2)**	**($55.9)**	**$10.9**	**$15.1**	**$28.0**	**$21.5**	**$75.5**	**$12.4**
Operating Margin	-	-	-	-	-	*31.6%*	*35.1%*	*47.7%*	*49.8%*	*42.1%*	*10.2%*
Other Income (Net)	$0.6	$0.1	$0.3	$0.4	$1.5	$1.1	$1.1	$0.9	$0.7	$3.7	$2.2
Pre-Tax Income	**($13.2)**	**($14.9)**	**($15.6)**	**($10.8)**	**($54.5)**	**$12.0**	**$16.2**	**$28.9**	**$22.1**	**$79.3**	**$14.6**
Net Taxes (benefit)	$0.0	$0.0	$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%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*
Reported Net Income	**($13.2)**	**($14.9)**	**($15.6)**	**($10.8)**	**($54.5)**	**$12.0**	**$16.2**	**$28.9**	**$22.1**	**$79.3**	**$14.6**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-
Net Margin	-	-	-	-	-	*34.7%*	*37.6%*	*49.2%*	*51.4%*	*44.2%*	*12.0%*
Weighted avg. Shares Out	*74.8*	*84.1*	*87.6*	*88.1*	*83.6*	*95.6*	*97.0*	*99.2*	*102.1*	*98.5*	*104.5*
Reported EPS	**($0.18)**	**($0.18)**	**($0.18)**	**($0.12)**	**($0.65)**	**$0.13**	**$0.17**	**$0.29**	**$0.22**	**$0.81**	**$0.14**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-

Zacks Small-Cap Research

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