March 7, 2017

ArQule Reports Fourth Quarter and Full Year 2016 Financial Results

Conference call scheduled today at 9:00 a.m. ET

BURLINGTON, Mass.--(BUSINESS WIRE)-- ArQule, Inc. (NASDAQ: ARQL) today announced its financial results for the fourth quarter and full year of 2016.

For the quarter ended December 31, 2016, the Company reported a net loss of $6,820,000 or $0.10 per share, compared with net loss of $2,852,000 or $0.05 per share, for the quarter ended December 31, 2015. The Company reported a net loss of $22,718,000 or $0.33 per share, for the year ended December 31, 2016, compared with a net loss of $13,774,000 or $0.22 per share, for the year ended December 31, 2015.

At December 31, 2016, the Company had a total of approximately $31,126,000 in cash and marketable securities.

Key Highlights

- **ARQ 087, our proprietary FGFR inhibitor, is planned to advance to a pivotal phase 3 biomarker driven trial in second-line intrahepatic cholangiocarcinoma (iCCA) patients with FGFR2 fusions.** A phase 1/2 trial including 29 second-line iCCA patients harboring FGFR2 fusions has completed enrollment and generated compelling clinical evidence to support a pivotal trial. Preliminary data from this phase 1/2 iCCA trial demonstrate a 24% response rate. Of 25 evaluable patients, six have achieved partial responses and a median time on therapy of approximately 26 weeks has been observed. To date, the disease control rate for the overall trial is 76%.
- **ARQ 092, our lead proprietary AKT inhibitor, is planned to advance to a company-sponsored phase 1/2 trial in PROS (PIK3CA Rare Overgrowth Spectrum), including Proteus syndrome.** A phase 1/2 trial evaluating patients with PROS, including more advanced and younger patients, has received regulatory approval in the U.S. and EU.
- **ARQ 531, our proprietary reversible BTK inhibitor, is on track to begin its first clinical trial.** Preclinical toxicology testing has completed and the company plans to file an Investigational New Drug (IND) application with FDA in Q1'17, and to subsequently begin its first clinical trial.
- **Tivantinib phase 3 METIV-HCC trial did not meet its primary endpoint of increased overall survival in hepatocellular carcinoma (HCC).** METIV-HCC is a biomarker-selected, double-blind, placebo-controlled, randomized phase 3 study evaluating tivantinib (2:1) versus best supportive care in patients with MET-overexpressing, inoperable HCC intolerant to or previously-treated with systemic therapy. Full results from the trial will be presented at an upcoming scientific forum. The phase 3 JET-HCC trial conducted by Kyowa Hakko Kirin is expected to provide final results in late Q1 or early Q2'17.
- **Company announced it raised approximately $15 million from a debt offering.** Net proceeds from the offering will be used to advance ArQule's proprietary pipeline and for general business purposes, including working capital.

2017 Goals

ARQ 087 - FGFR Inhibitor

- Initiate phase 3 trial for second-line iCCA patients with FGFR2 fusions in Q3'17
- Apply for Breakthrough Therapy Designation in the U.S.
- Present data on phase 1/2 trial in iCCA at a scientific congress

ARQ 092 - AKT Inhibitor

- Initiate company-sponsored phase 1/2 trial in PROS, including Proteus syndrome, in Q2'17
- Continue to pursue Pediatric Rare Disease Voucher Designation from the FDA
- Continue to support the NIH phase 1 trial in Proteus syndrome

ARQ 531 - BTK Inhibitor

- File an IND application in Q1'17
- Initiate phase 1 trial by Q3'17

ARQ 751 - Next-Generation AKT Inhibitor

- Evaluate initial data from phase 1 trial and define next steps

"We are focused on advancing our proprietary pipeline to meaningful inflection points," said Paolo Pucci, Chief Executive Officer of ArQule. "We are looking forward to beginning our first phase 3 trial with a proprietary asset, ARQ 087, in iCCA, and initiating company-sponsored clinical trials in PROS with ARQ 092 and in oncology with ARQ 531 this year."

"We are pleased with the results, including response rate and durability, that ARQ 087 has demonstrated in the phase 1/2 iCCA trial," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "After meetings with regulatory agencies, we are planning to progress to a pivotal phase 3 response rate driven trial of approximately 100 iCCA patients with FGFR2 fusions at sites across the U.S. and E.U. iCCA is an orphan disease with few treatment options."

Revenues and Expenses

Revenues for the quarter ended December 31, 2016, were $1,187,000, compared with revenues of $2,797,000 for the quarter ended December 31, 2015. Revenues for the year ended December 31, 2016 were $4,709,000 compared with revenues of $11,239,000 for the year ended December 31, 2015. Research and development revenue in 2016 and 2015 includes revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement.

Research and development expenses in the fourth quarter of 2016 were $6,242,000, compared with $3,641,000 for the fourth quarter 2015. Fiscal 2016 research and development expenses were $20,042,000, compared with $15,561,000 for fiscal 2015.

Research and development expense increased $2.6 million in the fourth quarter of 2016 compared to the fourth quarter of 2015 primarily due to higher outsourced clinical and product development costs. Research and development expense increased $4.5 million in 2016 primarily due to higher outsourced clinical and product development costs of $5.3 million, partially offset by lower labor costs of $0.5 million and facility costs of $0.4 million.

General and administrative expenses in the fourth quarter of 2016 were $1,808,000, compared with $2,028,000 for the fourth quarter of 2015. General and administrative expenses for fiscal 2016 were $7,563,000, compared to $9,830,000 for fiscal 2015.

General and administrative expense decreased by $0.2 million in the fourth quarter of 2016 compared to the fourth quarter of 2015 due to lower labor costs. General and administrative expense in 2016 decreased by $2.3 million primarily due to lower facility costs of $1.6 million and labor related costs of $0.6 million.

2017 Financial Guidance

For 2017, ArQule expects net use of cash to range between $25 and $27 million. Net loss is expected to range between $30 and $32 million, and net loss per share to range between $(0.42) and $(0.45) for the year. ArQule expects to end 2017 with between $18 and $20 million in cash and marketable securities.

Conference Call and Webcast

ArQule will hold its fourth quarter and full year financial results call today, March 7, 2017 at 9:00 a.m. ET. The live webcast can be accessed in the "Investors and Media" section of our website, www.arqule.com, under "Events and Presentations." You may also listen to the call by dialing (877) 868-1831 within the U.S. or (914) 495-8595 outside the U.S. A replay will be available two hours after the completion of the call and can be accessed in the "Investors and Media" section of our website, www.arqule.com, under "Events and Presentations."

About ArQule

ArQule is a biopharmaceutical company engaged in the research and development of targeted therapeutics to treat cancers and rare diseases. ArQule's mission is to discover, develop and commercialize novel small molecule drugs in areas of high unmet need that will dramatically extend and improve the lives of our patients. Our clinical-stage pipeline consists of five drug candidates, all of which are in targeted, biomarker-defined patient populations, making ArQule a leader among companies our size in precision medicine. ArQule's proprietary pipeline includes: ARQ 087, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in phase 2 for iCCA and in phase 1b for multiple oncology indications; ARQ 092, a selective inhibitor of the AKT serine/threonine kinase, in phase 1 for multiple oncology indications as well as ultra-rare Proteus syndrome, in partnership with the National Institutes of Health (NIH); ARQ 751, a next generation AKT inhibitor, in phase 1 for patients with AKT1 and PI3K mutations; and ARQ 761, a β-lapachone analog being evaluated as a promoter of NQO1-mediated programmed cancer cell necrosis, in phase 1/2 in multiple oncology indications in partnership with the University of Texas Southwestern Medical Center. In addition, we have advanced ARQ 531, an investigational, orally bioavailable, potent and reversible inhibitor of both wild type and C481S-mutant BTK, through toxicology testing and plan to file an Investigational New Drug Application in the first quarter of 2017. ArQule's most advanced product, in phase 3 clinical development, is tivantinib (ARQ 197), an oral, selective inhibitor of the c-MET receptor tyrosine kinase, for second-line treatment of MET-overexpressing hepatocellular carcinoma in partnership with Daiichi Sankyo in the West and Kyowa Hakko Kirin in Asia. ArQule's current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Company's proprietary library of compounds. You can follow us on Twitter and LinkedIn.

Forward Looking Statements

This press release contains forward-looking statements, including without limitation under the heading "Goals," in connection with the Company's clinical trials and planned clinical trials with tivantinib (ARQ 197), ARQ 092, ARQ 087, ARQ 751, and ARQ 531 as well as under "2017 Financial Guidance" with respect to projected financial results and its ability to fund operations with current cash and marketable securities. These statements are based on the Company's current beliefs and expectations, and are subject to risks and uncertainties that could cause actual results to differ materially. Positive information about pre-clinical and early stage clinical trial results does not ensure that later stage or larger scale clinical trials will be successful. For example, the pivotal phase 3 METIV-HCC with tivantinib did not meet its primary endpoint of overall survival. Moreover, ARQ 087, ARQ 092, ARQ 761, ARQ 751, and ARQ 531 may not demonstrate promising therapeutic effect; in addition, they may not demonstrate appropriate safety profiles in current or later stage or larger scale clinical trials as a result of known or as yet unanticipated side effects. The results achieved in later stage trials may not be sufficient to meet applicable regulatory standards or to justify further development. Problems or delays may arise prior to the initiation of planned clinical trials, during clinical trials or in the course of developing, testing or manufacturing these compounds that could lead the Company or its partners and collaborators to fail to initiate or to discontinue development. Even if later stage clinical trials are successful, unexpected concerns may arise from subsequent analysis of data or from additional data. Obstacles may arise or issues may be identified in connection with review of clinical data with regulatory authorities. Regulatory authorities may disagree with the Company's or its partners' view of the data or require additional data or information or additional studies. In addition, the planned timing of completion of clinical trials for tivantinib is subject to the ability of the Company as well as Kyowa Hakko Kirin, a licensee of tivantinib, and for ARQ 092 the National Institutes of Health, our collaborator responsible for the phase 1 trial in Proteus syndrome, to enroll patients, enter into agreements with clinical trial sites and investigators, and overcome technical hurdles and other issues related to the conduct of the trials for which each of them is responsible. There is a risk that these issues may not be successfully resolved. In addition, we and our partner are utilizing a companion diagnostic to identify MET-high patients in the JET-HCC trial, and we are utilizing or expect to utilize diagnostic tools in our biomarker-guided clinical trials with ARQ 087, ARQ 092, ARQ 751 and ARQ 531; we or our collaborators may encounter difficulties in developing and obtaining approval for companion diagnostics, including issues relating to access to certain technologies, selectivity/specificity, analytical validation, reproducibility, or clinical validation. Any delay or failure by our collaborators or ourselves to develop or obtain regulatory approval of companion diagnostics could delay or prevent approval of our product candidates. Drug development involves a high degree of risk. Only a small number of research and development programs result in the commercialization of a product. Positive pre-clinical data may not be supported in later stages of development. Furthermore, ArQule may not have the financial or human resources to successfully pursue drug discovery in the future. Moreover, with respect to partnered programs, even if certain compounds show initial promise, Kyowa Hakko Kirin or the NIH may decide not to continue to develop them. In addition, Daiichi Sankyo and Kyowa Hakko Kirin have certain rights to unilaterally terminate their agreements with ArQule. If either company were to do so, the Company might not be able to complete development and commercialization of the applicable licensed products on its own. For more detailed information on the risks and uncertainties associated with the Company's drug development and other activities, see the Company's periodic reports filed with the Securities and Exchange Commission. The Company does not undertake any obligation to publicly update any forward-looking statements.

ArQule, Inc.
Condensed Statement of Operations and Comprehensive Loss
(In Thousands, Except Per Share Amounts)
(Unaudited)

	Quarter Ended December 31,		Year Ended December 31,	
	2016	2015	2016	2015
Research and development revenue	$ 1,187	$ 2,797	$ 4,709	$ 11,239
Costs and expenses:				
Research and development	6,242	3,641	20,042	15,561
General and administrative	1,808	2,028	7,563	9,830
Total costs and expenses	8,050	5,669	27,605	25,391
Loss from operations	(6,863)	(2,872)	(22,896)	(14,152)
Interest income	43	20	178	101
Other income	-	-	-	277
Net loss	(6,820)	(2,852)	(22,718)	(13,774)
Unrealized gain (loss) on marketable securities	(20)	2	(1)	13
Comprehensive loss	$(6,840)	$(2,850)	$(22,719)	$(13,761)
Basic and diluted net loss per share:				
Net loss per share	$ (0.10)	$ (0.05)	$ (0.33)	$ (0.22)
Weighted average basic and diluted common shares outstanding	71,106	62,861	69,714	62,808

Balance sheet data (in thousands): (Unaudited)	December 31, 2016	December 31, 2015
Cash, equivalents and marketable securities- short term	$ 31,126	$ 38,772
Marketable securities- long term	-	-
	$ 31,126	$ 38,772
Total assets	$ 32,380	$ 40,004
Stockholders' equity	$ 23,680	$ 29,179

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