November 7, 2016

ArQule Reports Third Quarter 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 third quarter of 2016.

For the quarter ended September 30, 2016, the Company reported a net loss of $5,817,000 or $0.08 per share, compared to a net loss of $2,354,000 or $0.04 per share, for the third quarter of 2015. For the nine-month period ended September 30, 2016, the Company reported a net loss of $15,898,000 or $0.23 per share, compared to a net loss of $10,922,000 or $0.17 per share for the nine-month period ended September 30, 2015.

At September 30, 2016, the Company had a total of $37,659,000 in cash, equivalents and marketable securities.

Key Highlights

- **ARQ 087, our proprietary FGFR inhibitor, is approaching completion of enrollment in the phase 2 portion of the phase 1/2 trial in intrahepatic cholangiocarcinoma (iCCA).** Discussions with regulatory agencies in the U.S. and Europe are nearing completion for the design of a potential pivotal trial in iCCA. We plan to finalize the trial design by year end pending final trial results.
- **ARQ 531, our proprietary reversible BTK inhibitor, continues to demonstrate best-in-class potential with preclinical data to be presented at the 2016 American Society of Hematology (ASH) Annual Meeting.** The data, to be presented by The Ohio State University, demonstrate that ARQ 531 effectively inhibits C481S mutant BTK in patient derived cells and in a TCL1 mouse model shows efficacy superior to that of ibrutinib in Chronic Lymphocytic Leukemia (CLL). The company plans to complete preclinical studies and file an Investigational New Drug (IND) application in early 2017 to begin clinical trials with an initial focus on the fast-to-market, ibrutinib resistant C481S mutant BTK CLL population.
- **ARQ 092, our lead proprietary AKT inhibitor, continues to demonstrate the potential utility of targeting AKT in rare non-oncological indications and will be the focus of an oral presentation at the ASH Annual Meeting.** The data, to be presented by The University of Illinois College of Medicine, demonstrate that in neutrophils and platelets from Sickle Cell Disease (SCD) patients *in vitro* and cell-cell interactions in a mouse model of SCD, ARQ 092 attenuates neutrophil-platelet interactions. The study provides evidence that ARQ 092 could be a novel therapy in treating and preventing acute vaso-occlusive complications in SCD. The data warrants further studies of ARQ 092 in SCD.
- **ARQ 092 phase 1 trial for Proteus syndrome continues to enroll and our collaborator, the National Institutes of Health (NIH), is in the final stages of implementing an updated enrollment protocol that will facilitate logistics for patients and their families.** To date, the drug has been well tolerated, and we are looking forward to assessing full data from the initial two cohorts in the early part of next year.
- **ARQ 092 clinical research to be expanded into PROS (PIK3CA-Related Overgrowth Spectrum) family of rare diseases**. The company received approval of its IND application from the Food and Drug Administration (FDA) in the PROS family of rare diseases, including Proteus syndrome, for a potential clinical trial.
- **Tivantinib METIV-HCC phase 3 trial for hepatocellular carcinoma (HCC) is scheduled to conclude in early 2017.** Top-line data is expected in the first quarter of 2017.

"It is exciting to see our proprietary pipeline being pursued independently and through collaborations with the top researchers in the fields of oncology, hematology and selected rare diseases," said Paolo Pucci, Chief Executive Officer of ArQule. "We are encouraged that several projects have attracted the interest of leading scientific institutions including ARQ 531, our BTK inhibitor, which is in preclinical testing with our collaborators at The Ohio State University. ARQ 092 continues to progress in Proteus syndrome through a program that includes a phase 1 trial conducted by the NIH. In addition, we now have preclinical data for ARQ 092 in Sickle Cell Disease through the work of The University of Illinois College of Medicine. Lastly, we are pleased to have moved closer to a potential pivotal trial in an attractive fast to market opportunity for ARQ 087 in iCCA."

"Our lead proprietary drug candidate, ARQ 087, is nearing completion of the phase 2 iCCA trial," said Dr. Brian Schwartz,

M.D., Head of Research and Development and Chief Medical Officer at ArQule. "Encouraging discussions and positive feedback from the regulatory agencies, combined with the totality of the efficacy data we have observed in the clinical trials, has moved us closer to defining a pivotal trial design for ARQ 087 in this indication."

Revenues and Expenses

Revenues for the quarter ended September 30, 2016, were $1,223,000 compared with revenues of $2,653,000 for the quarter ended September 30, 2015. Revenues in the nine-months ended September 30, 2016 were $3,522,000 compared with revenues of $8,442,000 in the nine-months ended September 30, 2015. Revenue in the three and nine-month periods of 2016 and 2015 is comprised of revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement.

The revenue decreases in the quarter ended September 30, 2016 of $0.5 million from our Daiichi Sankyo METIV-HCC trial and $1.0 million from our Kyowa Hakko Kirin JET-HCC trial were principally due to the March 2016 extension of the development period through December 31, 2016 for both programs. The revenue decreases in the nine months ended September 30, 2016 of $2.0 million from our Daiichi Sankyo METIV-HCC trial and $2.9 million from our Kyowa Hakko Kirin JET-HCC trial were also principally due to the extension of the development period through December 31, 2016.

Research and development expense in the third quarter of 2016 was $5,265,000 compared with $3,180,000 for the third quarter of 2015. The $2.1 million increase in research and development expense in the third quarter of 2016 was principally due to increased outsourced clinical and product development costs of $1.9 million and professional fees of $0.2 million.

Research and development expense in the nine-months ended September 30, 2016 was $13,800,000 compared with $11,920,000 in the nine-months ended September 30, 2015. The $1.9 million increase in research and development expense in the nine-months ended September 30, 2016 was primarily due to increased outsourced clinical and product development costs of $2.6 million and professional fees of $0.2 million, partially offset by decreased labor and related costs of $0.4 million and facility costs of $0.5 million.

General and administrative expense was $1,824,000 in the third quarter of 2016 compared with $1,839,000 in the third quarter 2015.

General and administrative expense was $5,755,000 in the nine-months ended September 30, 2016 compared with $7,802,000 in the nine-months ended September 30, 2015. General and administrative expense decreased by $2.0 million in the nine-months ended September 30, 2016 primarily due to lower facility costs of $1.6 million, labor related costs of $0.2 million and professional fees of $0.2 million.

Conference Call and Webcast

ArQule will hold its third quarter 2016 financial results call today, November 7, 2016 at 9:00 a.m. ET. The live webcast can be accessed in the "Investors & Media" section of our website, www.arqule.com, under "Events & 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 "Investor and Media" section of our website, www.arqule.com, under "Events & Presentations".

About ArQule

ArQule is a biopharmaceutical company engaged in the research and development of targeted therapeutics to treat cancers and rare diseases. Our 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 lead 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 hepatocellular carcinoma in partnership with Daiichi Sankyo in the West and Kyowa Hakko Kirin in Asia. 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, into toxicology testing and plan to file an Investigational New Drug Application in early 2017. 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.

This press release contains forward-looking statements regarding the Company's clinical trials and planned clinical trials with tivantinib (ARQ 197), ARQ 092, ARQ 087, ARQ 761, ARQ 751, and ARQ 531 as well as 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, tivantinib, ARQ 092, ARQ 087, 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 view of the data or require additional data or information or additional studies. In addition, the planned timing of initiation and completion of clinical trials for tivantinib is subject to the ability of the Company as well as Daiichi Sankyo, Inc., our development partner for tivantinib, and Kyowa Hakko Kirin, a licensee of tivantinib, and the National Institutes of Health, our collaborator responsible for the phase 1 trial with ARQ 092 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 partners are utilizing a companion diagnostic to identify MET-high patients in the METIV-HCC and JET-HCC trials, and we are utilizing or expect to utilize diagnostic tools in our biomarker-guided clinical trials with ARQ 087, ARQ 092 and ARQ 751; we or our collaborators may encounter difficulties in developing and obtaining approval for companion diagnostics, including issues relating to 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, Daiichi Sankyo, 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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	**2015**	**2016**	**2015**
Research and development revenue	$ 1,223	$ 2,653	$ 3,522	$ 8,442
Costs and expenses:				
Research and development	5,265	3,180	13,800	11,920
General and administrative	1,824	1,839	5,755	7,802
Total costs and expenses	7,089	5,019	19,555	19,722
Loss from operations	(5,866)	(2,366)	(16,033)	(11,280)
Interest income	49	17	135	81
Other income (expense)	-	(5)	-	277
Net loss	(5,817)	(2,354)	(15,898)	(10,922)

Unrealized gain (loss) on marketable securities	(10)	8	19	11
Comprehensive loss	$ (5,827)	$ (2,346)	$ (15,879)	$ (10,911)
Basic and diluted net loss per share:				
Net loss per share	$ (0.08)	$ (0.04)	$ (0.23)	$ (0.17)
Weighted average basic and diluted common shares outstanding	71,083	62,827	69,247	62,753

Balance sheet data, unaudited (in thousands):	**September 30, 2016**	**December 31, 2015**
Cash, equivalents and marketable securities- short term	$ 37,659	$ 38,772
Marketable securities- long term	—	—
	$ 37,659	$ 38,772
Total assets	$ 38,530	$ 40,004
Stockholders' equity	$ 30,081	$ 29,179

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