ArQule Reports First Quarter 2016 Financial Results

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

Burlington, MA, May 4, 2016 – ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the first quarter of 2016.

For the quarter ended March 31, 2016, the Company reported a net loss of $4,981,000 or $0.08 per share, compared with a net loss of $4,551,000 or $0.07 per share, for the quarter ended March 31, 2015.

At March 31, 2016, the Company had a total of approximately $47,642,000 in cash, equivalents and marketable securities.

Key Highlights

- **ARQ 092, our proprietary AKT inhibitor in phase 1 for Proteus syndrome, has demonstrated AKT knockdown in the first three patients dosed:** Our collaborators, the National Human Genome Research Institute (NHGRI) of the National Institutes of Health (NIH), have completed the safety, pharmacokinetics and biomarker evaluation of the first cohort of three patients in the phase 1 trial for ARQ 092 in Proteus syndrome. In all of these patients, ARQ 092 was well tolerated and successfully achieved the pre-specified decrease in AKT signaling. The NIH has opened enrollment to patients ages 12 to 18.
- **ARQ 092 phase 1b trial in oncology has completed enrollment:** The final cohort of patients with AKT1/PI3K mutations has completed enrollment. In total, 10 patients with AKT1 mutations have been enrolled in the phase 1b portion of the trial. We anticipate presenting data from the study by year-end.
- **ARQ 087, our proprietary FGFR inhibitor in phase 2 portion of the trial for intrahepatic cholangiocarcinoma (iCCA), is expected to complete enrollment in the third quarter of 2016:** ArQule recently received a positive opinion from the European Medicines Agency's Committee for Orphan Medicinal Products (COMP) on orphan drug designation for ARQ 087 for biliary tract cancer. The phase 2 portion of the study continues as planned and preliminary data will be available this summer.
- **ARQ 761, our NQO1 inhibitor, in phase 1b/2 for pancreatic cancer completed enrollment of the first cohort:** Our collaborators at the University of Texas Southwestern Medical Center have enrolled the first cohort of patients in combination with gemcitabine and abraxane.

- **ARQ 531, our proprietary BTK inhibitor, proceeds into Good Laboratory Practice (GLP) toxicology studies:** Pre-clinical experiments, including toxicity studies, for ARQ 531 are proceeding as planned.
- **Tivantinib phase 3 trial in second-line hepatocellular carcinoma, METIV-HCC, completed its planned interim assessment and will continue to the final analysis:** The independent data monitoring committee conducted the planned interim assessment and it was determined that the trial will continue to its final analysis. The biomarker-driven phase 3 trial is expected to be completed by year end. The METIV-HCC trial is randomized 2:1 against best supportive care and enrolled approximately 300 MET-high patients with the primary end-point of overall survival.

"We were particularly pleased to report progress with our proprietary pipeline this quarter, and we remain on track for additional data read-outs this year," said Paolo Pucci, Chief Executive Officer of ArQule. "The completion of enrollment for the oncology trial with ARQ 092 and the anticipated completion of the ARQ 087 trial in iCCA next quarter set us up nicely to achieve our 2016 goals. With the METIV-HCC interim analysis behind us, we look forward to concluding the trial by year-end."

"We are pleased to hear from our collaborators at the NIH that the data collected from the first cohort of patients provides compelling *in vivo* evidence of the effect of ARQ 092 in Proteus syndrome," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "The opportunity for ArQule to add rare diseases to its established oncology clinical development program is a significant step forward in our efforts in precision medicine."

Revenues and Expenses

Revenues for the quarter ended March 31, 2016, were $1,227,000 compared with revenues of $2,785,000 for the quarter ended March 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. The revenue decreases in the quarter ended March 31, 2016 of $0.6 million from our Daiichi Sankyo METIV-HCC trial and $1.0 million from our Kyowa Hakko Kirin JET-HCC trial were due to the extension of the development period through December 31, 2016 for both programs.

Research and development expenses in the first quarter of 2016 were $4,198,000, compared with $4,413,000 for the first quarter of 2015. The $0.2 million decrease in research and development expense in the first quarter of 2016 was primarily due to lower facility costs of $0.3 million and lower labor related costs of $0.2 million from reduced headcount. These decreases were partially offset by increased outsourced clinical and product development costs of $0.3 million.

General and administrative costs were $2,044,000 in the first quarter of 2016 compared with $3,187,000 for the first quarter of 2015. General and administrative expense decreased by $1.2 million in the first quarter of 2016 primarily due to lower facility costs of $0.9 million and labor related cost of $0.2 million.

2016 Financial Guidance

For 2016, ArQule expects net use of cash to range between $23 and $25 million. Revenues are expected to range between $4 and $5 million. Net loss is expected to range between $24 and $27 million, and net loss per share to range between $(0.34) and $(0.39) for the year. ArQule expects to end 2016 with between $29 and $31 million in cash and marketable securities. Our guidance has been updated to include the issuance of 8,027,900 shares of common stock related to the stock offering completed during the quarter.

Conference Call and Webcast

ArQule will hold its first quarter 2016 financial results call today, May 4, 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 proprietary clinical-stage pipeline consists of four 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 092, designed to inhibit 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 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; 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. ArQule's current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Company's proprietary library of compounds.

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.

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

	Quarter Ended March 31,	
	2016	**2015**
Research and development revenue	$ 1,227	$ 2,785
Costs and expenses:		
Research and development	4,198	4,413
General and administrative	2,044	3,187
Total costs and expenses	6,242	7,600
Loss from operations	(5,015)	(4,815)
Interest income	34	36
Other income	—	228
Net loss	(4,981)	(4,551)
Unrealized gain on marketable securities	29	11
Comprehensive loss	$ (4,952)	$ (4,540)
Basic and diluted net loss per share	$ (0.08)	$ (0.07)
Weighted average shares used in calculating:		
Basic and diluted loss per share	65,489	62,745

Balance sheet data (in thousands): Unaudited	**March 31, 2016**	**December 31, 2015**
Cash, equivalents and marketable securities	$ 47,642	$ 38,772
Total assets	$ 48,962	$ 40,004
Stockholders' equity	$ 40,120	$ 29,179

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