August 4, 2017

ArQule Reports Second Quarter 2017 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 second quarter of 2017.

For the quarter ended June 30, 2017, the Company reported a net loss of $7,201,000 or $0.10 per share, compared with a net loss of $5,100,000 or $0.07 per share, for the second quarter of 2016. For the six-month period ended June 30, 2017, the Company reported a net loss of $14,777,000 or $0.21 per share, compared with a net loss of $10,081,000 or $0.15 per share, for the six-month period ended June 30, 2016.

At June 30, 2017, the Company had a total of approximately $31,007,000 in cash, equivalents and marketable securities.

Key Highlights

- **Derazantinib (ARQ 087), a pan-FGFR inhibitor, has begun recruiting in a registrational phase 3 trial for FGFR2 fusion positive second-line intrahepatic cholangiocarcinoma (iCCA).** Enrollment is planned to commence in the current quarter. In May, compelling data from the phase 1/2 trial in second-line iCCA was presented at the American Society of Clinical Oncology (ASCO) meeting highlighting a disease control rate of 83% and an objective response rate of 21%.
- **ARQ 531, an orally bioavailable, potent and reversible BTK inhibitor, has been dosed in a phase 1a/b trial.** The trial is enrolling patients with B-cell malignancies, including B-cell lymphomas, chronic lymphocytic leukemia, and Waldenstrom's macroglobulinemia, who are refractory to other therapeutic options, including ibrutinib. Up to 120 patients can be enrolled in the trial. The company also presented preclinical data for ARQ 531 in diffuse large B-cell lymphoma at the Annual Congress of the European Hematology Association which further strengthens the preclinical package for this molecule.
- **ARQ 092, lead AKT inhibitor, has been dosed in a phase 1/2 company-sponsored trial in Overgrowth Diseases with genetic alterations of the PI3K/AKT1 pathway, including PROS (PIK3CA-related Overgrowth Spectrum) and Proteus syndrome.** The trial is designed to enroll six patients in a dose escalation cohort as part of the phase 1 portion of the trial. An additional 10 patients will be enrolled in an expansion cohort as part of the phase 2 portion of the trial. The objective of this study is to determine a clinically meaningful endpoint to pursue in a registrational trial.

"We have made significant progress over the past few months by initiating phase 1 trials for ARQ 092 and ARQ 531 with the aim of achieving clinical proof of principle, and we are now poised to initiate a registrational trial with derazantinib," said Paolo Pucci, Chief Executive Officer of ArQule. "We achieved all of our targeted pipeline milestones for the first half of 2017, most notably moving ARQ 531, our BTK inhibitor, into the clinic. We believe ARQ 531 was the first reversible BTK inhibitor to be dosed in patients with B-cell malignancies. With four programs in the clinic, including derazantinib, ARQ 092, ARQ 751, and ARQ 531, we are poised to continue to achieve our goals for 2017."

"Our pipeline achieved two important milestones with the dosing of the first patient in two biomarker driven clinical trials targeting patients in areas of high unmet need," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "In the first of these clinical trials, ARQ 531 aims to demonstrate its potential to address a large patient population with B-cell malignancies who become refractory to current therapies. This is a significant emerging clinical need, particularly in C481S-mutant patients. In the second trial, ARQ 092 is now being dosed in Overgrowth Diseases driven by the PI3K/AKT1 mutation targeting a completely unmet clinical need in a patient population comprised of multiple orphan diseases. Both programs have the potential to be transformational and represent well ArQule's mission to bring life-changing therapies to address unmet medical needs."

Revenues and Expenses

Revenues for the quarter ended June 30, 2017, were zero compared with revenues of $1,072,000 for the quarter ended June 30, 2016. Revenues in the six-months ended June 30, 2017 were zero compared with revenues of $2,299,000 in the six-months ended June 30, 2016. Revenue in the three and six-month periods of 2016 is comprised of revenue from the

Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement. No further revenue is anticipated from these agreements.

Research and development expense in the second quarter of 2017 was $4,983,000, compared with $4,337,000 for the second quarter of 2016. Research and development expense increased $0.6 million in the second quarter of 2017 primarily due to higher outsourced preclinical, clinical and product development costs.

Research and development expense in the six-months ended June 30, 2017 was $10,177,000 compared with $8,535,000 in the six-months ended June 30, 2016. The $1.6 million increase in research and development expense in the six-months ended June 30, 2017 was primarily due to higher outsourced preclinical, clinical and product development costs.

General and administrative expense was $1,866,000 in the second quarter of 2017 compared with $1,887,000 in the second quarter 2016.

General and administrative expense was $3,940,000 in the six-months ended June 30, 2017 compared with $3,931,000 in the six-months ended June 30, 2016.

Conference Call and Webcast

ArQule will hold its second quarter 2017 financial results call today, August 4, 2017 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. 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 a phase 1/2 company sponsored study for Overgrowth Diseases, in a phase 1 study for ultra-rare Proteus syndrome conducted by the National Institutes of Health (NIH), as well as in multiple oncology indications; 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, in phase 1 for patients with B-cell malignancies refractory to other therapeutic options. 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 regarding clinical trials with derazantinib (ARQ 087), ARQ 092, ARQ 751 and ARQ 531 as well as projected financial results and 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, derazantinib, ARQ 092, 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 and, if applicable, 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, as applicable, its partners' views of the data or require additional data or information or additional studies. In addition, the planned timing of completion of the Phase 1 clinical trial for ARQ 092 in Proteus syndrome is subject to the ability of the National Institutes of Health, our collaborator responsible for such trial, to enroll patients, and overcome technical hurdles and other issues related to the conduct of the trial. There is a risk that these issues may not be successfully resolved. In addition, we are utilizing or expect to utilize diagnostic tests in our

biomarker-guided clinical trials with derazantinib, 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, concordance or clinical validation. Any delay or failure by our collaborators or us 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. Furthermore, ArQule may not have the financial or human resources to successfully pursue drug discovery in the future. Our use of cash is subject to a number of risks and uncertainties including how successful we and, as applicable, our collaborators are in executing our clinical strategy and the results obtained therefrom. 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 June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Research and development revenue	$ —	$ 1,072	$ —	$ 2,299
Costs and expenses:				
Research and development	4,983	4,337	10,177	8,535
General and administrative	1,866	1,887	3,940	3,931
Total costs and expenses	6,849	6,224	14,117	12,466
Loss from operations	(6,849)	(5,152)	(14,117)	(10,167)
Interest income	37	52	59	86
Interest expense	(389)	—	(719)	—
Net loss	(7,201)	(5,100)	(14,777)	(10,081)
Unrealized gain (loss) on marketable securities	(5)	—	(9)	29
Comprehensive loss	$ (7,206)	$ (5,100)	$ (14,786)	$(10,052)
Basic and diluted net loss per share:				
Net loss per share	$ (0.10)	$ (0.07)	$ (0.21)	$ (0.15)
Weighted average basic and diluted common shares outstanding	71,149	71,062	71,143	68,275

Balance sheet data (in thousands) (Unaudited):	**June 30, 2017**	**December 31, 2016**
Cash, equivalents and marketable securities- short term	$ 31,007	$ 31,126
Marketable securities-long term	-	-
	$ 31,007	$ 31,126
Total assets	$ 31,774	$ 32,380
Stockholders' equity	$ 10,086	$ 23,680

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