May 3, 2017

Arqule Reports First 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 first quarter of 2017.

For the quarter ended March 31, 2017, the Company reported a net loss of $7,576,000 or $0.11 per share, compared with net loss of $4,981,000 or $0.08 per share, for the quarter ended March 31, 2016.

At March 31, 2017, the Company had a total of approximately $37,540,000 in cash and marketable securities.

Key Highlights

- **ARQ 087, our FGFR inhibitor, will be featured in a poster discussion session highlighting the phase 1/2 trial in second-line intrahepatic cholangiocarcinoma (iCCA) at the American Society of Clinical Oncology (ASCO) on June 3, 2017.** A registrational phase 3 trial in this patient population is planned to commence in the third quarter of 2017.
- **ARQ 092, our lead AKT inhibitor, phase 1/2 company-sponsored trial in Overgrowth Diseases with genetic alterations of the PI3K/AKT1 pathway is open and the first patient has been identified.** The phase 1/2 trial will enroll patients ages six and older with a spectrum of Overgrowth Diseases driven by genetic alterations of the PI3K/AKT1 pathway such as PROS (PIK3CA-Related Overgrowth Spectrum) and Proteus syndrome.
- **ARQ 531, our orally bioavailable, potent and reversible BTK inhibitor, received Investigational New Drug (IND) clearance from the FDA.** A phase 1 trial is planned to commence by the third quarter of 2017 in patients with B-cell malignancies who are refractory to other therapeutic options.
- **ARQ 531 was issued a U.S. Patent by the U.S. Patent and Trademark Office covering composition of matter.** ArQule will be entitled to patent protection through December 2035 in the U.S. for the allowed claims.

"We achieved two important milestones already this year, the initiation of our company-sponsored trial in Overgrowth Diseases with ARQ 092 and the clearance of the IND for ARQ 531 in B-cell malignancies," said Paolo Pucci, Chief Executive Officer of ArQule. "These milestones enable ArQule to execute the next phase of its business plan. In addition, we are looking forward to presenting data from our iCCA trial with ARQ 087 at ASCO in June and initiating the registrational trial in this indication."

"The clearance of the IND for ARQ 531 keeps us on track to begin the phase 1 trial by the third quarter," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "The therapeutic need for a reversible, non-covalent BTK inhibitor that works in wild type and C481S-mutant BTK is significant and we have the potential to be best-in-class in this therapeutic area."

Revenues and Expenses

Revenues for the quarter ended March 31, 2017, were zero compared with revenues of $1,227,000 for the quarter ended March 31, 2016. Research and development revenue in 2016 includes 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 expenses in the first quarter of 2017 were $5,194,000, compared with $4,198,000 for the first quarter 2016.

Research and development expense increased $1.0 million in the first quarter of 2017 compared to the first quarter of 2016 primarily due to higher outsourced pre-clinical, clinical and product development costs.

General and administrative expenses in the first quarter of 2017 were $2,074,000, compared with $2,044,000 for the first quarter of 2016.

Conference Call and Webcast

ArQule will hold its first quarter 2017 financial results call today, May 3, 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 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 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 initiate a phase 1 trial by the third quarter of 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 ARQ 087, ARQ 092 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, ARQ 087, ARQ 092 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 tools in our biomarker-guided clinical trials with ARQ 087, ARQ 092, ARQ 531 and other programs; 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)

	Quarter Ended March 31,	
	2017	2016
Research and development revenue	$ —	$ 1,227
Costs and expenses:		
Research and development	5,194	4,198
General and administrative	2,074	2,044
Total costs and expenses	7,268	6,242
Loss from operations	(7,268)	(5,015)
Interest income	22	34
Interest expense	(330)	—
Net loss	(7,576)	(4,981)
Unrealized gain (loss) on marketable securities	(4)	29
Comprehensive loss	$(7,580)	$(4,952)
Basic and diluted net loss per share	$ (0.11)	$ (0.08)
Weighted average shares used in calculating:		
Basic and diluted loss per share	71,138	65,489

Balance sheet data (in thousands) unaudited:	**March 31, 2017**	**December 31, 2016**
Cash, equivalents and marketable securities- short term	$ 37,540	$ 31,126
Marketable securities- long term	—	—
	$ 37,540	$ 31,126
Total assets	$ 38,490	$ 32,380
Stockholders' equity	$ 16,989	$ 23,680

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