August 3, 2016

ArQule Reports Second 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 second quarter of 2016.

For the quarter ended June 30, 2016, the Company reported a net loss of $5,100,000 or $0.07 per share, compared with a net loss of $4,017,000 or $0.06 per share, for the second quarter of 2015. For the six-month period ended June 30, 2016, the Company reported a net loss of $10,081,000 or $0.15 per share, compared with a net loss of $8,568,000 or $0.14 per share, for the six-month period ended June 30, 2015.

At June 30, 2016, the Company had a total of approximately $43,115,000 in cash, equivalents and marketable securities.

Key Highlights

- **ARQ 087, our proprietary FGFR inhibitor, demonstrated strong anticancer activity in an ongoing phase 1/2 trial in intrahepatic cholangiocarcinoma (iCCA), a rare form of liver cancer.** A 75% disease control rate, including a 25% objective response rate, was observed from the preliminary data presented at the 2016 ESMO GI Congress. Since the Congress presentation, a fourth partial response has been observed.
- **ARQ 531, our proprietary and novel BTK inhibitor, demonstrated in preclinical studies that it is a potent and reversible inhibitor of both wild type and ibrutinib resistant C481S-mutant BTK.** In preclinical testing, ARQ 531 demonstrated biochemical inhibition of both wild type and C481S-mutant BTK at sub-nanomolar levels and potent cellular inhibition in C481S-mutant BTK cells that are resistant to ibrutinib. These data were presented at the 2016 Pan Pacific Lymphoma Conference in July marking the first public showing of data on this potential best-in-class molecule.
- **ARQ 092, our lead AKT inhibitor, continues in the phase 1 trial for Proteus syndrome.** The three patients enrolled in the first cohort are approaching nine months of therapy.
- **Tivantinib - METIV-HCC phase 3 trial in hepatocellular carcinoma is scheduled to conclude by year-end 2016 or early 2017.** Top-line data is expected according to that timeline.

"We are starting to realize the benefits of our precision medicine strategy through the advancement of our proprietary pipeline as evidenced by the strong clinical data recently presented at ESMO GI for ARQ 087 in iCCA, the initiation of a biomarker driven phase 1 trial for our next generation AKT inhibitor, ARQ 751, and the emerging preclinical profile of our novel BTK inhibitor, ARQ 531, recently presented at the Pan Pacific Lymphoma Conference," said Paolo Pucci, Chief Executive Officer of ArQule. "While AKT and FGFR are emerging targets, with ARQ 531 we have the opportunity to work in a well-established target such as BTK and to address a growing therapeutic need of patients who develop resistance to ibrutinib. We look forward to sharing additional data on ARQ 087, ARQ 092 and ARQ 531 later this year."

"With the four partial responses recorded thus far in the iCCA trial, we are nearing a decision for the next stage of clinical development for ARQ 087 and expect to meet with regulatory authorities in the near future," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "The initiation of a pivotal biomarker driven trial in iCCA with FGFR2 genetic alterations would create an opportunity for a fast-to-market strategy in this orphan disease. With clear signs of clinical utility and a manageable safety profile, ARQ 087 has the potential to become a best-in-class compound."

Revenues and Expenses

Revenues for the quarter ended June 30, 2016, were $1,072,000 compared with revenues of $3,004,000 for the quarter ended June 30, 2015. Revenues in the six-months ended June 30, 2016 were $2,299,000 compared with revenues of $5,789,000 in the six-months ended June 30, 2015. Revenue in the three and six-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 June 30, 2016 of $1.0 million from our Daiichi Sankyo METIV-HCC trial and $0.9 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 six months ended June 30, 2016 of $1.6 million from our Daiichi Sankyo METIV-HCC trial and $1.9 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 second quarter of 2016 was $4,337,000, compared with $4,327,000 for the second quarter of 2015. The increase in outsourced clinical and product development costs of $0.4 million in the second quarter of 2016 was offset by lower labor and related costs of $0.3 million and facility costs reductions of $0.1 million.

Research and development expense in the six-months ended June 30, 2016 was $8,535,000 compared with $8,740,000 in the six-months ended June 30, 2015. The $0.2 million decrease in research and development expense in the six-months ended June 30, 2016 was primarily due to lower labor and related costs of $0.5 million, facility costs of $0.5 million, partially offset by increased outsourced clinical and product development costs of $0.8 million.

General and administrative expense was $1,887,000 in the second quarter of 2016 compared with $2,776,000 in the second quarter 2015. General and administrative expense decreased by $0.9 million in the second quarter of 2016 primarily due to lower facility costs of $0.7 million and professional fees of $0.2 million.

General and administrative expense was $3,931,000 in the six-months ended June 30, 2016 compared with $5,963,000 in the six-months ended June 30, 2015. General and administrative expense decreased by $2.0 million in the six-months ended June 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 second quarter 2016 financial results call today, August 3, 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. 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.

ArQule, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss (Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	(In Thousands, Except Per Share Data)			
Research and development revenue	$ 1,072	$ 3,004	$ 2,299	$ 5,789
Costs and expenses:				
Research and development	4,337	4,327	8,535	8,740
General and administrative	1,887	2,776	3,931	5,963
Total costs and expenses	6,224	7,103	12,466	14,703
Loss from operations	(5,152)	(4,099)	(10,167)	(8,914)
Interest income	52	28	86	64
Other income	-	54	-	282
Net loss	(5,100)	(4,017)	(10,081)	(8,568)
Unrealized gain (loss) on marketable securities	-	(8)	29	3
Comprehensive loss	$ (5,100)	$ (4,025)	$(10,052)	$ (8,565)
Basic and diluted net loss per share:				
Net loss per share	$ (0.07)	$ (0.06)	$ (0.15)	$ (0.14)
Weighted average basic and diluted common shares outstanding	71,062	62,754	68,275	62,747

Balance sheet data (in thousands): (Unaudited)	June 30, 2016	December 31, 2015
Cash, equivalents and marketable securities- short term	$ 43,115	$ 38,772

Marketable securities- long term	-	-
	$ 43,115	$ 38,772
Total assets	$ 44,015	$ 40,004
Stockholders' equity	$ 35,479	$ 29,179

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