ArQule Reports Second Quarter 2018 Financial Results

August 1, 2018

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Aug. 1, 2018-- ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the second quarter of 2018.

For the quarter ended June 30, 2018, the Company reported net income of $5,156,000 or $0.05 per diluted share, compared with a net loss of $7,201,000 or $0.10 per basic share, for the second quarter of 2017. For the six-month period ended June 30, 2018, the Company reported a net loss of $1,376,000 or $0.02 per basic share, compared with a net loss of $14,777,000 or $0.21 per basic share, for the six-month period ended June 30, 2017.

As of June 30, 2018, the Company had a total of approximately $46,075,000 in cash, equivalents and marketable securities.

Key Highlights

- In July 2018, the Company raised approximately $70 million of gross proceeds in a public offering of common stock. Net proceeds will be used to fund its core clinical programs and for general corporate purposes
- ARQ 531, our potent and reversible BTK inhibitor, demonstrated good oral bioavailability, pharmacokinetics and early signs of activity in data presented at the European Hematological Association (EHA) in June 2018. The Phase 1 portion of the Phase 1a/b trial continues to recruit on schedule, and we plan to present additional data at a major congress later in 2018
- A comprehensive preclinical publication on ARQ 531 has been accepted in a major scientific journal
- We and our academic collaborators at Memorial Sloan Kettering have initiated an expansion cohort for miransertib in combination with anastrazole in patients with endometrial cancer, and are targeting to enroll up to 40 patients in this cohort
- We and our scientific collaborators have compiled a foundational clinical data set from miransertib in rare diseases that we plan to present at the American Society of Human Genetics (ASHG) Annual Meeting in October

“We were gratified by the high level of interest and quality of investors that participated in our recent public offering,” said Paolo Pucci, Chief Executive Officer of ArQule. "Our core clinical programs continue to progress, and the capital we received in the upsized offering significantly enhances our ability to expand our plans and to sustain them over a longer period of time.”

Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer of ArQule said, “We are pleased with the continued progress of our clinical programs in oncology and rare diseases. Data recently presented at EHA on ARQ 531, our reversible BTK inhibitor, have further validated the potential of this promising drug candidate, and we are now planning for the next data releases in the second part of the year.”

Revenues and Expenses

Revenues for the quarter ended June 30, 2018, were $13,706,000 compared with revenues of zero for the quarter ended June 30, 2017. Research and development revenue in the quarter ended June 30, 2018 consisted of $13,706,000 from our April 2018 Basilea licensing agreement.

Revenues for the six months ended June 30, 2018, were $17,844,000 compared with revenues of zero for the six months ended June 30, 2017. Research and development revenue in the six months ended June 30, 2018 consisted of $13,706,000 from our April 2018 Basilea licensing agreement, $3,000,000 from our February 2018 Roivant licensing agreement and $1,138,000 from our October 2017 non-exclusive license agreement for certain library compounds.

Research and development expense in the second quarter of 2018 was $6,787,000 compared with $4,983,000 for the second quarter of 2017. Research and development expense increased $1.8 million in the second quarter of 2018 primarily due to higher outsourced preclinical, clinical and product development costs.

Research and development expense in the six-months ended June 30, 2018 was $12,599,000 compared with $10,177,000 in the six months ended June 30, 2017. The $2.4 million increase in research and development expense in the six months ended June 30, 2018 was primarily due to higher outsourced preclinical, clinical and product development costs.

General and administrative expense was $2,234,000 in the second quarter of 2018 compared with $1,866,000 in the second quarter 2017. The $0.4 million increase in general and administrative expense in the three months ended June 30, 2018 was primarily due to increased professional fees.

General and administrative expense was $4,585,000 in the six months ended June 30, 2018 compared with $3,940,000 in the six months ended June 30, 2017. The $0.6 million increase in general and administrative expense in the six months ended June 30, 2018 was primarily due to increased professional fees and labor related costs.

2018 Updated Financial Guidance

As a result of the July 2018 stock offering and the prior conversion of our preferred stock into 8,370,000 shares of common stock, we have updated our

2018 guidance. For 2018, ArQule now expects revenue to range between $21 and $23 million. Net use of cash is expected to range between $28 and $30 million for the year. Net loss is expected to range between $10 and $14 million, and net loss per share to range between $(0.10) and $(0.14) for the year. ArQule expects to end 2018 with between $100 and $102 million in cash and marketable securities.

Conference Call and Webcast

ArQule will hold its second quarter 2018 financial results call today, August 1, 2018 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 pipeline includes: ARQ 531, an 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; Miransertib (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), and in Phase 1b in combination with the hormonal therapy, anastrozole, in patients with advanced endometrial cancer; ARQ 751, a next generation AKT inhibitor, in Phase 1 for patients with AKT1 and PI3K mutations; Derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA; 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.

Forward-Looking Statements

This press release contains forward-looking statements, including without limitation under the heading, "Key Highlights," in the quotes of management in connection with the Company's clinical trials and planned clinical trials, as well as under the heading, "2018 Updated Financial Guidance," with respect to projected financial results and our 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 from those set forth in this press release. 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 531, miransertib, ARQ 751 and derazantinib 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 or its collaborators' view of data or require additional data or information or additional studies. In addition, the planned timing of completion of clinical trials is subject to the ability of the Company and, in certain cases, its collaborators 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 break apart FISH diagnostic to identify patients in the trial with derazantinib in iCCA, and are utilizing or expect to utilize diagnostic tools in other biomarker-guided clinical trials with derazantinib, miransertib, ARQ 531 and ARQ 751. 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, 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. Moreover, each of Basilea and Sinovant, our collaborators for derazantinib, has only a limited or no track record of drug development in oncology.In addition, both Basilea and Sinovant have certain rights to unilaterally terminate their respective agreements with ArQule.If either party were to do so, the Company might not be able to complete development and commercialization of derazantinib. Even if derazantinib were to show promise, our collaborators may decide not to continue to develop it. If derazantinib is not successfully developed as a result of any of the foregoing or other issues, risks or uncertainties, ArQule may not receive any future milestones or royalties under its agreements with Basilea and Sinovant.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. For more detailed information on the risks and uncertainties associated with the Company's drug development, financial condition 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,	
	2018	**2017**	**2018**	**2017**

Research and development revenue	$ 13,706	$ —	$ 17,844	$ —
Costs and expenses:				
Research and development	6,787	4,983	12,599	10,177
General and administrative	2,234	1,866	4,585	3,940
Total costs and expenses	9,021	6,849	17,184	14,117
Income (loss) from operations	4,685	(6,849)	660	(14,117)
Interest income	170	37	329	59
Interest expense	(417)	(389)	(813)	(719)
Other income (expense) (1)	718	—	(1,552)	—
Net income (loss)	5,156	(7,201)	(1,376)	(14,777)
Unrealized gain (loss) on marketable securities	19	(5)	(6)	(9)
Comprehensive income (loss)	$ 5,175	$ (7,206)	$ (1,382)	$ (14,786)
Basic and diluted net income (loss) per share:				
Basic net income (loss) per share	$ 0.06	$ (0.10)	$ (0.02)	$ (0.21)
Diluted net income (loss) per share	$ 0.05	$ (0.10)	$ (0.02)	$ (0.21)
Weighted average shares used in calculating:				
Basic net income (loss) per share	92,241	71,149	89,691	71,143
Diluted net income (loss) per share	100,532	71,149	89,691	71,143

(1) Includes non-cash income (expense) associated with the change in fair value of our preferred stock warrant liability. At June 30, 2018 there was no remaining balance in the warrant liability.

Balance sheet data (in thousands) (Unaudited):	**June 30, 2018**	**December 31, 2017**
Cash, equivalents and marketable securities- short term	$ 46,075	$ 48,036
Marketable securities-long term	-	-
	$ 46,075	$ 48,036
Total assets	$ 50,181	$ 48,902
Stockholders' equity	$ 27,244	$ 14,181

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Source: ArQule, Inc.

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