ArQule Reports First Quarter 2019 Financial Results

May 1, 2019

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

BURLINGTON, Mass.--(BUSINESS WIRE)--May 1, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the first quarter, 2019.

For the quarter ended March 31, 2019, the Company reported a net loss of $10,267,000, or $0.09 per share, compared with net loss of $6,532,000, or $0.07 per share, for the quarter ended March 31, 2018.

As of March 31, 2019, the Company had a total of approximately $92,223,000 in cash, cash equivalents, and marketable securities.

Key Highlights from Q1, 2019

- **ARQ 531, our potent and reversible dual inhibitor of both wild-type and C481S-mutant BTK.** Reported in March that the first evaluable CLL patient with a C481S mutation enrolled in cohort 7 (65 mg QD) of our phase 1 trial achieved a partial response that has since been confirmed with a subsequent scan. This is in addition to a previously reported follicular lymphoma patient who had also achieved a partial response and continues on therapy. Cohort 7 has been cleared for safety and we have begun enrolling patients in cohort 8 (75 mg QD). No additional DLTs have been observed at any dose. We plan to present detailed, updated data from this ongoing trial at the European Hematological Association meeting in June
- **Miransertib, our potent and selective first-generation AKT inhibitor.** Concluded interactions with the FDA and defined the registrational trial designs for both Proteus syndrome and PIK3CA-Related Overgrowth Spectrum (PROS). We have finalized the protocol and received the first conditional IRB approvals
- **ARQ 751, our highly potent and selective next-generation AKT inhibitor.** Signal generation work in genetically-defined solid tumors continues, and we plan to present the final data set at a major conference by year end
- **Derazantinib, our FGFR inhibitor, partnered with Basilea and Sinovant, in a registrational trial for intrahepatic cholangiocarcinoma.** Substantially completed the timely recruitment and transfer of clinical and other responsibilities to Sinovant and Basilea

Paolo Pucci, Chief Executive Officer of ArQule, commented, “We have made tremendous progress across our pipeline in Q1, and we are particularly pleased with the safety and dose dependent clinical activity profile that is emerging with ARQ 531.”

“We are busy collecting data for our ARQ 531 presentation at EHA, and we are confident that we will demonstrate meaningful incremental clinical activity in addition to the two PRs already announced”, commented Dr. Brian Schwartz, Chief Medical Officer of ArQule. “We are also pleased by the rapid review of the initial IRBs for our registrational trial with miransertib in Proteus syndrome and PROS.”

Revenues and Expenses

Revenues for the first quarter, 2019, were $1,345,000 compared with revenues of $4,138,000 for the first quarter, 2018.

Research and development expenses in the first quarter, 2019 were $7,448,000 compared with $5,812,000 for the first quarter, 2018.

General and administrative expenses in the first quarter, 2019 were $4,300,000 compared with $2,351,000 for the first quarter, 2018.

2019 Financial Guidance

Our 2019 financial guidance has not changed. For 2019, ArQule expects revenue to range between $3 and $5 million. Net loss is expected to range between $40 and $43 million, and net loss per share to range between $(0.37) and $(0.39) for the year. ArQule expects to end 2019 with between $60 and $63 million in cash and marketable securities.

Conference Call and Webcast

ArQule will hold its first quarter financial results call today, May 1, 2019 at 9:00 a.m. ET. The live webcast can be accessed in the “Investors and Media” section of our website, www.arqule.com, under “Events and 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 “Investors and Media” section of our website, www.arqule.com, under “Events and 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 pipeline includes: ARQ 531, an orally bioavailable, potent and reversible dual 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 potent and selective inhibitor of the AKT serine/threonine kinase, planned to initiate registrational trial cohorts in Proteus syndrome and PROS in 2019, and in phase 1b in combination with the hormonal therapy, anastrozole, in patients with advanced endometrial cancer; ARQ 751, a next generation highly potent and selective AKT inhibitor, in phase 1 for patients with AKT1 and PI3K mutations; and derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA in collaboration with Basilea and Sinovant. 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 headings "Key Highlights from Q1, 2019," and quotes of management in connection with the Company's clinical trials and planned clinical trials, as well as under "2019 Financial Guidance" with respect to projected financial results. 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.For example, while initial results from the development of ARQ 531, miransertib, ARQ 751 and derazantinib have been promising, such results are not necessarily indicative of results that will be obtained from ongoing or subsequent trials and the results achieved in ongoing or later stage trials may not be sufficient to meet applicable regulatory standards or to justify further development.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. 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 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. 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. In addition, the Company uses or expects to use companion diagnostics in biomarker-guided clinical trials with its product candidates. The Company or its 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. 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, the Company may not have the financial or human resources to successfully pursue drug discovery in the future. With respect to partnered programs, even if certain compounds show initial promise our collaborators may decide not to continue to develop them. Our collaborators in the development of derazantinib have certain rights to unilaterally terminate their agreement with ArQule.If either were to do so, the Company might not be able to complete development and commercialization of derazantinib on its own in the affected territory. 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 disclaims any obligation to update the information contained in this press release as new information becomes available.

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

	Quarter Ended March 31,	
	2019	**2018**
Research and development revenue	$ 1,345	$ 4,138
Costs and expenses:		
Research and development	7,448	5,812
General and administrative	4,300	2,351
Total costs and expenses	11,748	8,163
Loss from operations	(10,403)	(4,025)
Interest income	566	159
Interest expense	(430)	(396)
Other expense (1)	—	(2,270)
Net loss	(10,267)	(6,532)
Unrealized gain (loss) on marketable securities	117	(25)
Comprehensive loss	$ (10,150)	$ (6,557)

Basic and diluted net loss per share	$ (0.09)	$ (0.07)
Weighted average shares used in calculating:		
Basic and diluted loss per share	109,020	87,112

(1) Non-cash expense associated with the change in fair value of our preferred stock warrant liability which was converted to common stock and common stock warrants in May 2018. Accordingly, at March 31, 2019, the warrant liability was zero.

Balance sheet data (in thousands):	**March 31, 2019**	**December 31, 2018**
Cash, equivalents and marketable securities- short term	$ 92,223	$ 99,558
Marketable securities- long term	—	—
	$ 92,223	$ 99,558
Total assets	$ 98,473	$ 106,676
Stockholders' equity	$ 71,086	$ 78,968

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