ArQule Reports Second Quarter 2019 Financial Results

August 7, 2019

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Aug. 7, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the second quarter of 2019.

For the quarter ended June 30, 2019, the Company reported a net loss of $9.1 million, or $0.08 per basic share, compared with net income of $5.2 million, or $0.05 per diluted share, for the quarter ended June 30, 2018.

As of June 30, 2019, the Company had a total of approximately $182.8 million in cash, cash equivalents, and marketable securities.

Key Highlights from Q2, 2019

- In June 2019, the Company raised approximately $104 million of gross proceeds in a public offering of common stock. Net proceeds will be used to fund clinical programs and for general corporate purposes
- **ARQ 531, our potent and reversible dual inhibitor of both wild-type and C481S-mutant BTK.**
  - Presented first-of-its kind clinical proof-of-concept data at the 2019 European Hematology Association (EHA) Annual meeting from the ongoing phase 1 trial, including 4 Partial Responses (PRs) out of 6 evaluable CLL patients in the 65 mg QD dose cohort, a PR in a Richter's transformation patient at the same dose and a PR in a follicular lymphoma patient on therapy for approximately two years. Subsequent to the EHA presentation an additional PR was observed in a DLBCL patient and was disclosed in the context of the recent public offering
  - Selected 65 mg QD as the recommended starting phase 2 dose (RP2D) after assessing incremental clinical activity and tolerability data from cohorts 7 (65 mg QD) and 8 (75 mg QD); no additional dose limiting toxicities have been observed and a maximum tolerated dose has not been reached
  - Commenced preparations for the next phase of development, including planned regulatory interactions and design of expansion cohorts at the RP2D in multiple B-Cell malignancies, including C481S mutant CLL
- **Miransertib, our potent and selective first-generation AKT inhibitor.**
  - Presented updated Proteus syndrome and PIK3CA-related Overgrowth Spectrum (PROS) data at the 2019 European Society of Human Genetics conference in Gothenburg, Sweden
  - Finalized the registrational protocol, received initial IRB approvals, nearing dosing of first patient
- **ARQ 751, our highly potent and selective next-generation AKT inhibitor.**
  - Continued the signal generation work in genetically-defined solid tumors
- **Derazantinib, our FGFR inhibitor, partnered with Basilea and Sinovant, in a registrational trial for intrahepatic cholangiocarcinoma.**
  - Clinical activities ongoing with our partners, Basilea and Sinovant

Paolo Pucci, Chief Executive Officer of ArQule, commented, "Our second quarter was punctuated by the proof-of-concept data presented at EHA for ARQ 531, and we are gratified that our recent public offering will allow us to pursue the next phase of clinical development with ARQ 531."

"Having observed substantial clinical activity with ARQ 531 at a well-tolerated dose in the target populations, we have been able to select a phase 2 dose and are aggressively taking the drug into the expansion phase," commented Dr. Brian Schwartz, Chief Medical Officer of ArQule. "We are also excited to have opened additional sites in our registrational MOSAIC study in Proteus syndrome and PROS."

Revenues and Expenses

Revenues for the second quarter, 2019, were $0.3 million compared with revenues of $13.7 million for the second quarter, 2018.

Research and development expenses in the second quarter, 2019 were $6.3 million compared with $6.8 million for the second quarter, 2018.

General and administrative expenses in the second quarter, 2019 were $3.2 million compared with $2.2 million for the second quarter, 2018.

2019 Financial Guidance

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 $(0.35) and $(0.37) for the year. As a result of our common stock offering in June, we are updating our cash guidance. ArQule now expects to end 2019 with approximately $160 million in cash and marketable securities which will support the current business plan into 2022.

Conference Call and Webcast

ArQule will hold its second quarter financial results call today, August 7, 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, in a planned registrational trial with cohorts in Proteus syndrome and PROS to initiate in 2019; 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 Q2, 2019,” and quotes of management in connection with the Company’s clinical trials and planned clinical trials with ARQ 531, miransertib, ARQ 751 and derazantinib, 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 Income (Loss)
(In Thousands, Except Per Share Amounts)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Research and development revenue	$ 281	$ 13,706	$ 1,626	$ 17,844
Costs and expenses:				

Research and development	6,330	6,787	13,778	12,599
General and administrative	3,168	2,234	7,468	4,585
Total costs and expenses	9,498	9,021	21,246	17,184
Income (loss) from operations	(9,217)	4,685	(19,620)	660
Interest income	558	170	1,124	329
Interest expense	(433)	(417)	(863)	(813)
Other income (expense) (1)	—	718	—	(1,552)
Net income (loss)	(9,092)	5,156	(19,359)	(1,376)
Unrealized gain (loss) on marketable securities	58	19	175	(6)
Comprehensive income (loss)	$ (9,034)	$ 5,175	$ (19,184)	$ (1,382)
Basic and diluted net income (loss) per share:				
Basic net income (loss) per share	$ (0.08)	$ 0.06	$ (0.18)	$ (0.02)
Diluted net income (loss) per share	$ (0.08)	$ 0.05	$ (0.18)	$ (0.02)
Weighted average shares used in calculating:				
Basic net income (loss) per share	109,860	92,241	109,442	89,691
Diluted net income (loss) per share	109,860	100,532	109,442	89,691

1. Includes non-cash income (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 June 30, 2019 and at June 30, 2018 there was no remaining balance in the warrant liability.

Balance sheet data (in thousands) (Unaudited):	**June 30, 2019**	**December 31, 2018**

Cash, equivalents and marketable securities- short term	$ 179,121	$ 99,558
Marketable securities-long term	3,638	—
	$ 182,759	$ 99,558
Total assets	$ 187,193	$ 106,676
Stockholders' equity	$ 161,616	$ 78,968

View source version on businesswire.com: https://www.businesswire.com/news/home/20190807005066/en/

Source: ArQule, Inc.

Corporate Contact:
Kathleen Farren
Investor Relations
and Executive Assistant to the CFO
ir@arqule.com

Media Contact:
Cait Williamson, Ph.D.
LifeSci Public Relations
(646) 751-4366
cait@lifescipublicrelations.com

www.ArQule.com