ArQule Reports Third Quarter 2019 Financial Results

October 30, 2019

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Oct. 30, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the third quarter of 2019.

For the quarter ended September 30, 2019, the Company reported a net loss of $10.7 million, or $0.09 per basic share, compared with net loss of $5.6 million, or $0.05 per basic share, for the quarter ended September 30, 2018.

As of September 30, 2019, the Company had a total of approximately $174.1 million in cash, cash equivalents, and marketable securities.

Key Highlights from Q3, 2019

- **ARQ 531, our potent and reversible dual inhibitor of both wild-type and C481-mutant BTK:**
  - Completed recruitment of the phase 1 dose escalation trial and determined a recommended phase 2 dose (RP2D) of 65 mg once a day
  - Submitted American Society of Hematology (ASH) abstract in July, which is scheduled to be made public at 9:00 a.m. ET, November 6, 2019
  - Began dosing phase 2 expansion cohorts in multiple B cell malignancies at the RP2D
  - Requested and received a meeting date with the FDA to discuss registrational trial design
- **Miransertib, our potent and selective first-generation AKT inhibitor:**
  - Initiated multiple additional sites in our previously described registrational trial, MOSAIC (Miransertib in Overgrowth Syndromes in Adults and Children), for both Proteus syndrome (PS) and PIK3CA-related Overgrowth Spectrum (PROS)
  - Recently announced the first patient dosed under the MOSAIC protocol at Texas Children's Hospital (TCH) Vascular Anomalies Center
- **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:**
  - Continued to interact with our partners, Basilea and Sinovant

Paolo Pucci, Chief Executive Officer of ArQule, commented, "We are very excited to present the final phase 1 data set for ARQ 531 at ASH in December. These data will include meaningful updates on clinical activity and durability that provide a more complete picture of the potential of ARQ 531 and will reinforce its position as the leading reversible BTK inhibitor."

"The tremendous progress ARQ 531 has made in such a short period has allowed us to provide first ever proof of concept with a reversible BTK inhibitor in the emerging unmet medical need of C481-mutant CLL patients. We are now actively recruiting patients in the multi-arm phase 2 trial," commented Dr. Brian Schwartz, Chief Medical Officer of ArQule. "It is also very gratifying to have begun patient enrollment in our registrational MOSAIC trial in Proteus syndrome and PROS."

Revenues and Expenses

Revenues for the third quarter, 2019, were $0.2 million compared with revenues of $5.0 million for the third quarter, 2018.

Research and development expenses in the third quarter, 2019 were $8.3 million compared with $7.3 million for the third quarter, 2018.

General and administrative expenses in the third quarter, 2019 were $3.2 million compared with $3.4 million for the third quarter, 2018.

2019 Financial Guidance

For 2019, ArQule expects revenue to range between $2 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. ArQule 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, October 30, 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 & 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. and entering the conference ID: 4289763. A replay will be available two hours after the completion of the call and can be accessed in the “Investors & 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 1/2 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 registrational trial with cohorts in Proteus syndrome and PROS; ARQ 751, a next generation highly potent and selective AKT inhibitor, in phase 1 for patients with solid tumors 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 Q3, 2019,” and quotes of management in connection with the Company’s clinical trials and planned clinical trials with ARQ 531 and miransertib, 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 Statements of Operations and Comprehensive Loss
(In Thousands, Except Per Share Amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Research and development revenue	$ 208	$ 4,979	$ 1,834	$ 22,823

Costs and expenses:				
Research and development	8,313	7,261	22,091	19,860
General and administrative	3,170	3,429	10,638	8,014
Total costs and expenses	11,483	10,690	32,729	27,874
Loss from operations	(11,275)	(5,711)	(30,895)	(5,051)
Interest income	961	514	2,085	843
Interest expense	(425)	(422)	(1,288)	(1,235)
Other expense (1)	—	—	—	(1,552)
Net Loss	(10,739)	(5,619)	(30,098)	(6,995)
Unrealized gain (loss) on marketable securities	47	(10)	222	(16)
Comprehensive loss	$ (10,692)	$ (5,629)	$ (29,876)	$ (7,011)
Basic and diluted net loss per share:				
Net loss per share	$ (0.09)	$ (0.05)	$ (0.27)	$ (0.07)
Weighted average basic and diluted common shares outstanding	120,374	107,445	113,126	95,678

1. Includes 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 September 30, 2019 and at September 30, 2018 there was no remaining balance in the warrant liability.

Balance sheet data (in thousands) (Unaudited):	**September 30, 2019**	**December 31, 2018**
Cash, equivalents and marketable securities- short term	$ 125,213	$ 99,558

Marketable securities-long term	48,900	—
	$ 174,113	$ 99,558
Total assets	$ 180,342	$ 106,676
Stockholders' equity	$ 153,126	$ 78,968

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