ArQule Reports Fourth Quarter and Full Year 2018 Financial Results

March 7, 2019

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Mar. 7, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced its financial results for the fourth quarter and full year of 2018.

For the quarter ended December 31, 2018, the Company reported a net loss of $8,487,000 or $0.08 per share, compared with net loss of $7,760,000 or $0.09 per share, for the quarter ended December 31, 2017. The Company reported a net loss of $15,482,000 or $0.16 per share, for the year ended December 31, 2018, compared with a net loss of $29,203,000 or $0.39 per share, for the year ended December 31, 2017.

As of December 31, 2018, the Company had a total of approximately $99,558,000 in cash, equivalents, and marketable securities.

Key Highlights from 2018

- **ARQ 531, our potent and reversible dual inhibitor of both wild-type and C481S-mutant BTK.** Successfully progressedrecruitment in our ongoing phase 1 dose escalation trial in 2018, enrolling over 20 patients at 7 dose cohorts ranging from 5mg to 65mg QD. Data from this trial was presented at 3 major conferences (AACR, EHA, ASH) in 2018 and demonstrated a good safety profile, profound target engagement and encouraging signs of dose-dependent clinical activity in both lymphomas and C481S-mutant CLL
- **Miransertib, our potent and selective first-generation AKT inhibitor.** Presented first-of-its kind clinical data in Proteus syndrome and PROS at the American Society of Human Genetics (ASHG), received FDA Fast Track Designation in PROS, and worked with regulators to define registrational trial designs for both indications
- **ARQ 751, our highly potent and selective next-generation AKT inhibitor.** Progressed the phase 1 basket trial in R/R or metastatic cancer patients harboring an AKT, PI3K or PTEN mutation, identified a recommended phase 2 dose of 75mg QD and presented data at the EORTC/AACR/NCI congress in November
- **Derazantinib, our FGFR inhibitor, partnered with Basilea and Sinovant, in a registrational trial for intrahepatic cholangiocarcinoma.** Continued the timely recruitment and transfer of clinical and other responsibilities to Sinovant and Basilea following the outlicensing to both companies in February and April of last year, respectively
- **Capital Structure.** Strengthened our capital structure through business development activities, which included funding from our collaborators and a successful offering of our common stock which raised gross proceeds of about $70 million
- **Stock Index Inclusions.** Added to the family of Russell 2000 Index companies in June and the NASDAQ Biotechnology Index in December

Key Catalysts & Goals for 2019

ARQ 531: Dual BTK Inhibitor in B-Cell Malignancies

- Complete the dose escalation portion of phase 1 trial
- Determine a recommended phase 2 dose and initiate expansion cohort(s)
- Present results of dose escalation phase 1 trial at major industry conference(s)

Miransertib: AKT Inhibitor in Proteus syndrome and PROS

- Finalize regulatory interactions with the FDA
- Initiate registrational trial cohorts in both Proteus syndrome and PROS

ARQ 751 (and Miransertib): AKT Inhibitors in Oncology

- Complete recruitment in ongoing clinical trials
- Present data sets at major medical conferences this year

Derazantinib: FGFR Inhibitor in iCCA

- Complete the orderly and timely transfer of all clinical, manufacturing and regulatory responsibilities to our partners, Basilea and Sinovant

“2018 was a watershed year for ArQule,” remarked Paolo Pucci, Chief Executive Officer. “The clinical progress we made across the entire portfolio

positions us well for an even more transformational 2019. Specifically in rare disease, we now have the ability to expand the scope of the miransertib registrational trial beyond Proteus syndrome to the PROS family of overgrowth spectrum disorders which represents a particularly exciting opportunity because of their significantly larger prevalence."

Dr. Brian Schwartz, Chief Medical Officer, added "We made tremendous progress in 2018, enrolling over 20 patients across 7 cohorts in our dose escalation study with ARQ 531 which is now positioned as the first and best in class drug candidate to address the emerging medical need in BTK mutated malignancies. We are looking forward to advance ARQ 531, miransertib and ARQ 751 respectively into the next phase of development. I want to thank our collaborators such as the Ohio State University, the National Institute of Health and Bambino Gesu', and others for their continued support in advancing these programs."

Revenues and Expenses

Revenues for the quarter ended December 31, 2018, were $2,941,000 compared with revenues of zero for the quarter ended December 31, 2017. Revenues for the year ended December 31, 2018 were $25,764,000 compared with revenues of zero for the year ended December 31, 2017. Research and development revenue increased in 2018 due to revenue of $5.9 million from our February 2018 Sinovant licensing agreement, $18.5 million from our April 2018 Basilea licensing agreement and $1.3 million from a non-exclusive license agreement for certain of our library compounds.

Research and development expenses in the fourth quarter of 2018 were $8,850,000 compared with $4,721,000 for the fourth quarter 2017. Fiscal 2018 research and development expenses were $28,710,000 compared with $19,468,000 for fiscal 2017. The $4.1 million increase in research and development expense in the fourth quarter of 2018 compared with the fourth quarter of 2017 was primarily due to higher outsourced preclinical, clinical and product development costs of $3.5 million and $0.6 million from labor and related costs.

The $9.2 million increase in research and development expense in 2018 was primarily due to higher outsourced preclinical, clinical and product development costs of $8.5 million and $0.7 million from labor and related costs.

General and administrative expenses in the fourth quarter of 2018 were $2,739,000, compared with $1,849,000 for the fourth quarter of 2017. General and administrative expenses for fiscal 2018 were $10,753,000, compared to $7,551,000 for fiscal 2017. The $0.9 million increase in general and administrative expense in the fourth quarter of 2018 compared with the fourth quarter of 2017 was principally due to higher consulting and professional fees of $0.4 million and labor and related costs of $0.5 million. The $3.2 million increase in general and administrative expense in 2018 was principally due to higher consulting and professional fees of $2.1 million and labor and related costs of $1.1 million.

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 $(0.37) 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 fourth quarter and full year financial results call today, March 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, 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 2018," "Key Catalysts & Goals for 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 Loss
(In Thousands, Except Per Share Amounts)
(Unaudited)

	Quarter Ended December 31,		Year Ended December 31,	
	2018	**2017**	**2018**	**2017**
Research and development revenue	$ 2,941	$ -	$ 25,764	$ -
Costs and expenses:				
Research and development	8,850	4,721	28,710	19,468
General and administrative	2,739	1,849	10,753	7,551
Total costs and expenses	11,589	6,570	39,463	27,019
Loss from operations	(8,648)	(6,570)	(13,699)	(27,019)
Interest income	592	113	1,435	238
Interest expense	(431)	(401)	(1,666)	(1,520)
Other expense (1)	-	(902)	(1,552)	(902)
Net loss	(8,487)	(7,760)	(15,482)	(29,203)
Unrealized loss on marketable securities	(63)	(15)	(79)	(18)
Comprehensive loss	$ (8,550)	$ (7,775)	$ (15,561)	$ (29,221)
Basic and diluted net loss per share:				
Net loss per share	$ (0.08)	$ (0.09)	$ (0.16)	$ (0.39)
Weighted average basic and diluted common shares outstanding	108,994	85,292	99,035	74,813

(1) Non-cash expense associated with the change in fair value of our preferred stock warrant liability. At December 31, 2018 there was no remaining balance in the warrant liability.

Balance sheet data (in thousands): (Unaudited)	**December 31, 2018**	**December 31, 2017**
Cash, equivalents and marketable securities- short term	$ 99,558	$ 48,036
Marketable securities- long term	-	-
	$ 99,558	$ 48,036
Total assets	$ 106,676	$ 48,902
Stockholders' equity	$ 78,968	$ 14,181

View source version on businesswire.com: https://www.businesswire.com/news/home/20190307005110/en/

Source: ArQule, Inc.

Corporate Contact:
Marc Schegerin, M.D.
Senior Vice President
Head of Strategy, Finance and Communication
ir@arqule.com

Media Contact:
Allison Blum, Ph.D.
LifeSci Public Relations (646) 627-8383
Allison@lifescipublicrelations.com
www.ArQule.com