



ArQule Reports Third Quarter 2018 Financial Results

October 31, 2018

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Oct. 31, 2018-- [ArQule](#), Inc. (Nasdaq: ARQL) today announced its financial results for the third quarter of 2018.

For the quarter ended September 30, 2018, the Company reported a net loss of \$5,619,000 or \$0.05 per share, compared with a net loss of \$6,666,000 or \$0.09 per share, for the third quarter of 2017. For the nine-month period ended September 30, 2018, the Company reported a net loss of \$6,995,000 or \$0.07 per share, compared with a net loss of \$21,443,000 or \$0.30 per share, for the nine-month period ended September 30, 2017.

At September 30, 2018, the Company had a total of approximately \$105,088,000 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
- In August 2018, extensive preclinical data on ARQ 531, our reversible BTK inhibitor, was published in the scientific journal, *Cancer Discovery*, highlighting the profile of this potential first and best-in-class molecule
- In September 2018, miransertib (ARQ 092) was granted Fast Track Designation for the treatment of PROS (PIK3CA-Related Overgrowth Spectrum), opening the way for enhanced interactions with regulators
- In October 2018, three clinical presentations for miransertib were given at the American Society of Human Genetics, confirming its potential for treating patients with Proteus syndrome and PROS

"We continue to execute on our strategy to develop rapidly ARQ 531 in hematological malignancies, miransertib in PROS and Proteus syndrome, as well as miransertib and ARQ 751 in hormone sensitive solid tumors," said Paolo Pucci, Chief Executive Officer of ArQule. "Each of our drug candidates holds tremendous promise and each candidate is being increasingly validated by the data that we are placing in the public domain."

Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer of ArQule said, "We are pleased with the *Cancer Discovery* publication for ARQ 531 and how the pre-clinical data highlighted in that paper is beginning to be validated in the on-going Phase 1 trial." "I am also proud of the work that we continue to perform with miransertib in PROS and Proteus syndrome and am encouraged by the accumulating clinical data that supports the potential utility of miransertib to fulfill the serious unmet medical need in these diseases, particularly in children."

Revenues and Expenses

Revenues for the quarter ended September 30, 2018, were \$4,979,000 compared with revenues of zero for the quarter ended September 30, 2017. Research and development revenue in the quarter ended September 30, 2018 consisted of \$2,852,000 from our February 2018 Sinovant licensing agreement, \$1,996,000 from our April 2018 Basilea licensing agreement and \$131,000 from a non-exclusive license agreement for certain of our library compounds.

Revenues for the nine months ended September 30, 2018, were \$22,823,000 compared with revenues of zero for the nine months ended September 30, 2017. Research and development revenue in the nine months ended September 30, 2018 consisted of \$5,852,000 from our February 2018 Sinovant licensing agreement \$15,702,000 million from our April 2018 Basilea licensing agreement and \$1,269,000 from a non-exclusive license agreement for certain of our library compounds.

Research and development expense in the third quarter of 2018 was \$7,261,000 compared with \$4,570,000 for the third quarter of 2017. The \$2.7 million increase in research and development expense in the third quarter of 2018 was primarily due to higher outsourced preclinical, clinical and product development costs.

Research and development expense in the nine months ended September 30, 2018 was \$19,860,000 compared with \$14,747,000 in the nine months ended September 30, 2017. The \$5.1 million increase in research and development expense in the nine months ended September 30, 2018 was primarily due to higher outsourced preclinical, clinical and product development costs.

General and administrative expense was \$3,429,000 in the third quarter of 2018 compared with \$1,762,000 in the third quarter 2017. The \$1.7 million increase in general and administrative expense in the third quarter of 2018 was primarily due to higher consulting and professional fees of \$1.4 million and labor and related costs of \$0.2 million.

General and administrative expense was \$8,014,000 in the nine months ended September 30, 2018 compared with \$5,702,000 in the nine months ended September 30, 2017. The \$2.3 million increase in general and administrative expense in the nine months ended September 30, 2018 was principally due to higher consulting and professional fees of \$1.8 million and labor and related costs of \$0.5 million.

2018 Updated Financial Guidance

As a result of our advancing development programs and collaborations and the likely timing of cash receipts and disbursements, we have updated our

2018 guidance. Net use of cash is expected to be approximately \$34 million for the year, which is slightly higher than our previous guidance due primarily to an acceleration of the clinical development costs associated with proprietary and partnered clinical programs. Net loss is expected to range between \$14 and \$17 million for the year, and net loss per share to range between \$(0.14) and \$(0.17). We are also adjusting our revenue guidance upward to between \$24 and \$25 million primarily in connection with services provided to our derazantinib partners.

ArQule expects to end 2018 with approximately \$100 million in cash and marketable securities, which we believe will be sufficient to finance our operations into 2021.

Conference Call and Webcast

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. 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 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 test to identify patients in the registrational trial with derazantinib in iCCA, and are utilizing or expect to utilize diagnostic tests 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 September 30,	Nine months Ended September 30,
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	2018	2017	2018	2017
Research and development revenue	\$ 4,979	\$ —	\$ 22,823	\$ —
Costs and expenses:				
Research and development	7,261	4,570	19,860	14,747
General and administrative	3,429	1,762	8,014	5,702
Total costs and expenses	10,690	6,332	27,874	20,449
Income (loss) from operations	(5,711)	(6,332)	(5,051)	(20,449)
Interest income	514	66	843	125
Interest expense	(422)	(400)	(1,235)	(1,119)
Other expense (1)	—	—	(1,552)	—
Net loss	(5,619)	(6,666)	(6,995)	(21,443)
Unrealized gain (loss) on marketable securities	(10)	6	(16)	(3)
Comprehensive loss	\$ (5,629)	\$ (6,660)	\$ (7,011)	\$ (21,446)
Basic and diluted net loss per share:				
Net loss per share	\$ (0.05)	\$ (0.09)	\$ (0.07)	\$ (0.30)
Weighted average basic and diluted common shares outstanding	107,445	71,541	95,678	71,282

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

Balance sheet data (in thousands) (Unaudited):	September 30, 2018	December 31, 2017
Cash, equivalents and marketable securities- short term	\$ 100,181	\$ 48,036
Marketable securities-long term	4,907	-
	\$ 105,088	\$ 48,036
Total assets	\$ 112,549	\$ 48,902
Stockholders' equity	\$ 87,097	\$ 14,181

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