



November 9, 2017

ArQule Reports Third Quarter 2017 Financial Results

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

BURLINGTON, Mass.--(BUSINESS WIRE)-- [ArQule](#), Inc. (Nasdaq: ARQL) today announced its financial results for the third quarter of 2017.

For the quarter ended September 30, 2017, the Company reported a net loss of \$6,666,000 or \$0.09 per share, compared with a net loss of \$5,817,000 or \$0.08 per share, for the third quarter of 2016. For the nine-month period ended September 30, 2017, the Company reported a net loss of \$21,443,000 or \$0.30 per share, compared with a net loss of \$15,898,000 or \$0.23 per share, for the nine-month period ended September 30, 2016.

At September 30, 2017, the Company had a total of approximately \$27,603,000 in cash, equivalents and marketable securities. In October and November 2017, the Company raised approximately \$25 million in net proceeds from two equity offerings.

Key Highlights

- | **Miransertib (ARQ 092), lead AKT inhibitor, has met its primary endpoint of determining a biologically active dose in a phase 1 trial for Proteus syndrome lead by the National Human Genome Research Institute (NHGRI) of the National Institutes of Health (NIH).** The NIH presented preliminary data from the ongoing phase 1 clinical trial for Proteus syndrome at the Proteus Syndrome Foundation Family Conference in September 2017. In five of the six patients, a reduction of a least 50% of phospho-AKT levels was reported. This met the primary objective of the study. Most significantly the NIH observed disease modification in the cerebiform connective tissue nevus (CCTN) lesions which are considered one of the hallmarks of the disease.
- | **Miransertib was granted by the U.S. Food and Drug Administration (FDA) Rare Pediatric Disease Designation.** Under the FDA's rare pediatric disease priority review voucher program, the sponsor may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug application.
- | **Miransertib continues to dose in the phase 1/2 trial for Overgrowth Diseases driven by PI3K and AKT mutations and opened its first U.S. clinical trial site.** This trial is part of the company's continued progress towards expanding its rare disease strategy in Overgrowth Diseases.
- | **ARQ 531, an orally bioavailable, potent and reversible BTK inhibitor, continues dosing as planned in a phase 1a/b trial.** The trial is enrolling patients with B-cell malignancies, including B-cell lymphomas, chronic lymphocytic leukemia, and Waldenstrom's macroglobulinemia, who are refractory to other therapeutic options, including ibrutinib. Up to 120 patients can be enrolled in the trial.
- | **Derazantinib (ARQ 087), a pan-FGFR inhibitor, in a registrational trial in FGFR2 fusion positive second-line intrahepatic cholangiocarcinoma (iCCA) is recruiting with six active sites in the U.S.** The trial is planned to enroll up to 100 iCCA patients and provides an opportunity for a conditional approval as part of a fast-to-market strategy and includes an interim analysis that will be performed after the first 40 patients have been enrolled and evaluated for response.
- | **Company raised approximately \$29 million in capital through a private placement of \$15.7 million of common stock, a private placement of \$9.5 million of convertible preferred stock and an additional \$4 million through unrelated business development activities and other sources.** Net proceeds will be used to advance ArQule's proprietary pipeline and for general business purposes, including working capital.

"The ArQule clinical pipeline is the strongest it has ever been, and the most recent positive developments are the compelling clinical data in Proteus syndrome and the granting of Rare Pediatric Disease Designation for miransertib in this indication," said Paolo Pucci, Chief Executive Officer of ArQule. "We are now well capitalized to see our pipeline assets through major inflection points."

"Based on the preliminary results from the phase 1 NIH-sponsored Proteus syndrome trial, miransertib is the first drug to demonstrate activity and achieve clinical proof of concept in this indication," said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. "We are thankful to the NIH for their work in identifying the mutation driving the disease, conducting the first clinical trial and ultimately identifying potential clinical endpoints. We plan

to engage regulatory authorities to define a clinical path to registration in this indication. In parallel and consistent with the regulatory interactions we will continue to enroll in our ongoing phase 1/2 trial in Overgrowth Diseases driven by mutations in the PI3K or AKT pathway, including Proteus syndrome, and provide the drug under compassionate policy."

Revenues and Expenses

Revenues for the quarter ended September 30, 2017, were zero compared with revenues of \$1,223,000 for the quarter ended September 30, 2016. Revenues in the nine-months ended September 30, 2017 were zero compared with revenues of \$3,522,000 in the nine-months ended September 30, 2016. Revenue in the three and nine-month periods of 2016 is comprised of revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement. No further revenue is anticipated from these agreements.

Research and development expense in the third quarter of 2017 was \$4,570,000, compared with \$5,265,000 for the third quarter of 2016. Research and development expense decreased \$0.7 million in the third quarter of 2017 primarily due to lower outsourced preclinical, clinical and product development costs.

Research and development expense in the nine-months ended September 30, 2017 was \$14,747,000 compared with \$13,800,000 in the nine-months ended September 30, 2016. The \$0.9 million increase in research and development expense in the nine-months ended September 30, 2017 was primarily due to higher outsourced clinical and product development costs.

General and administrative expense was \$1,762,000 in the third quarter of 2017 compared with \$1,824,000 in the third quarter 2016.

General and administrative expense was \$5,702,000 in the nine-months ended September 30, 2017 compared with \$5,755,000 in the nine-months ended September 30, 2016.

2017 Financial Guidance

As a result of ArQule's recent stock offerings and business development activities the company is updating 2017 guidance. For 2017, ArQule expects net use of cash to range between \$25 and \$27 million. Net loss is expected to range between \$28 and \$30 million, net loss per share is expected to range between \$(0.38) and \$(0.40) for the year. ArQule expects to end 2017 with between \$47 and \$49 million in cash and marketable securities.

Conference Call and Webcast

[ArQule](#) will hold its third quarter 2017 financial results call today, November 9, 2017 at 9:00 a.m. ET. The live webcast can be accessed in the "Investors & 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 "Investor and Media" section of our website, www.arqule.com, under "[Events & 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 proprietary pipeline includes: Derazantinib (ARQ 087), a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in phase 2 for iCCA and in phase 1b for multiple oncology indications; 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), as well as in multiple oncology indications; ARQ 751, a next generation AKT inhibitor, in phase 1 for patients with AKT1 and PI3K mutations; 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. In addition, we have advanced ARQ 531, an investigational, 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. ArQule's current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Company's proprietary library of compounds. You can follow us on [Twitter](#) and [LinkedIn](#).

Forward-Looking Statements

This press release contains forward-looking statements, including, without limitation, those related to clinical trials and the progress of the Company's clinical pipeline as well as the achievement of certain clinical and regulatory milestones with derazantinib (ARQ 087), miransertib (ARQ 092) and ARQ 531, use of offering proceeds and financial guidance with respect to cash at year end and the Company's 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. Positive information about pre-clinical and early stage clinical trial results does not ensure that later stage milestones will be met or that later stage or larger scale clinical trials will be successful. Moreover, derazantinib, miransertib, and ARQ 531 or other programs 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 partners' view of data or require additional data or information or additional studies. In addition, the planned timing of completion of clinical trials for miransertib in Proteus syndrome is dependent in part on the National Institutes of Health, our collaborator responsible for the phase 1 trial in Proteus syndrome, 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 are utilizing a companion diagnostic to identify patients in our registration trial with derazantinib in intrahepatic cholangiocarcinoma with FGFR2 fusions, and we are utilizing or expect to utilize diagnostic tools in our other biomarker-guided clinical trials with derazantinib, miransertib, ARQ 751 and ARQ 531; 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. 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's expectations regarding its use of cash are subject to numerous risks and uncertainties, including, without limitation, those set forth above. The Company may not have the financial or human resources to successfully pursue all of its drug discovery programs 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		NINE MONTHS ENDED	
	September 30,	September 30,	September 30,	September 30,
	2017	2016	2017	2016
	(IN THOUSANDS, EXCEPT PER SHARE DATA)			
Research and development revenue	\$ —	\$ 1,223	\$ —	\$ 3,522
Costs and expenses:				
Research and development	4,570	5,265	14,747	13,800
General and administrative	1,762	1,824	5,702	5,755
Total costs and expenses	<u>6,332</u>	<u>7,089</u>	<u>20,449</u>	<u>19,555</u>
Loss from operations	(6,332)	(5,866)	(20,449)	(16,033)
Interest income	66	49	125	135
Interest expense	(400)	—	(1,119)	—
Net loss	<u>(6,666)</u>	<u>(5,817)</u>	<u>(21,443)</u>	<u>(15,898)</u>
Unrealized gain (loss) on marketable securities	<u>6</u>	<u>(10)</u>	<u>(3)</u>	<u>19</u>

Comprehensive loss	\$ (6,660)	\$ (5,827)	\$ (21,446)	\$ (15,879)
Basic and diluted net loss per share:				
Net loss per share	\$ (0.09)	\$ (0.08)	\$ (0.30)	\$ (0.23)
Weighted average basic and diluted common shares outstanding	71,541	71,083	71,282	69,247

Balance sheet data (in thousands) (Unaudited):	September 30, December 31,	
	2017	2016
Cash, equivalents and marketable securities- short term	\$ 27,603	\$ 31,126
Marketable securities-long term	-	-
	<u>\$ 27,603</u>	<u>\$ 31,126</u>
Total assets	\$ 28,240	\$ 32,380
Stockholders' equity	6,058	\$ 23,680

View source version on [businesswire.com: http://www.businesswire.com/news/home/20171109005148/en/](http://www.businesswire.com/news/home/20171109005148/en/)

ArQule, Inc.
Dawn Schottlandt, 781-994-0300
Vice President, Investor Relations/ Corp. Communications
www.ArQule.com

Source: ArQule, Inc.

News Provided by Acquire Media