



ArQule Reports Fourth Quarter and Full Year 2017 Financial Results

March 5, 2018

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

BURLINGTON, Mass.--(BUSINESS WIRE)--Mar. 5, 2018-- ArQule, Inc. (NASDAQ: ARQL) today announced its financial results for the fourth quarter and full year of 2017.

For the quarter ended December 31, 2017, the Company reported a net loss of \$7,760,000 or \$0.09 per share, compared with net loss of \$6,820,000 or \$0.10 per share, for the quarter ended December 31, 2016. The Company reported a net loss of \$29,203,000 or \$0.39 per share, for the year ended December 31, 2017, compared with a net loss of \$22,718,000 or \$0.33 per share, for the year ended December 31, 2016.

At December 31, 2017, the Company had a total of approximately \$48,036,000 in cash and marketable securities.

Key Highlights

- **ARQ 531, orally bioavailable, potent and reversible BTK inhibitor, is recruiting on schedule in the phase 1a trial.** Initial phase 1a data will be presented at American Association for Cancer Research (AACR) which will be held April 14 through April 18, 2018.
- **Miransertib, lead proprietary AKT inhibitor, is recruiting at three sites for Overgrowth Diseases.** The phase 1/2 trial for Overgrowth Diseases evaluating patients with PROS and Proteus syndrome, age two and older in the U.S. and EU, continues to dose.
- **Data supporting miransertib in combination with the aromatase inhibitor, anastrozole, for the treatment of advanced endometrial cancer will be presented at AACR.** Phase 1b trial conducted at Memorial Sloan Kettering presents potential clinical path forward in this hard to treat disease setting.
- **Derazantinib, a pan-FGFR inhibitor, continues to enroll on schedule in a registrational trial for FGFR2 fusion driven intrahepatic cholangiocarcinoma (iCCA).** The recruitment of patients needed to perform the interim analysis is expected to be completed by year-end.
- **ArQule has granted a Roivant Sciences Ltd. subsidiary an exclusive license to develop and commercialize derazantinib in the People's Republic of China, Hong Kong, Macau, and Taiwan.** Deal terms include an upfront payment to ArQule of \$3 million and an additional \$2.5 million development milestone within the first year. ArQule is also eligible for regulatory and commercial milestones and royalties on future sales of derazantinib in Greater China.

2018 Goals

ARQ 531 – BTK Inhibitor

- Complete phase 1a trial in refractory B-cell malignancies
- Publish foundational pre-clinical paper including chemical and crystal structure
- Present initial phase 1a data at AACR and additional data later this year

Miransertib – AKT Inhibitor Rare Diseases

- Initiate registrational program in Proteus syndrome
- Publish NIH phase 1 trial findings and compassionate use case reports

Miransertib and ARQ 751 – AKT Inhibitors Oncology

- Present data from phase 1b in oncology for miransertib
- Present data from phase 1a in oncology for ARQ 751

Derazantinib – FGFR Inhibitor

- Complete dosing of patients needed to conduct interim analysis in registrational trial in 2nd line iCCA
- Explore opportunities in additional tumor types

"During 2017 we have worked diligently to advance all the assets in our proprietary pipeline, and we are now in the position to make 10 presentations at AACR in April demonstrating our commitment to precision medicine," said Paolo Pucci, Chief Executive Officer of ArQule. "2018 has the prospect of being a pivotal year for ArQule as we see a complete data set from the phase 1a trial of our BTK inhibitor, ARQ 531, launch a registrational program in

Proteus syndrome, and explore emerging late-stage opportunities in oncology with our AKT program.”

“For ArQule the scientific highlights of 2017 were achieving, in collaboration with the NIH, clinical proof of concept with miransertib in Proteus syndrome, initiating a registrational trial with derazantinib and initiating a phase 1 trial for ARQ 531,” said Dr. Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer at ArQule. “Consistent with our strategy we are now preparing to launch a registrational program for Proteus syndrome, as well as continuing with our ongoing trials for our BTK inhibitor, ARQ 531, and derazantinib. In addition, the data to be presented at AACR for miransertib in combination with anastrozole will highlight a new opportunity.”

Revenues and Expenses

Revenues for the quarter ended December 31, 2017, were zero, compared with revenues of \$1,187,000 for the quarter ended December 31, 2016. Revenues for the year ended December 31, 2017 were zero compared with revenues of \$4,709,000 for the year ended December 31, 2016. Research and development revenue in 2016 includes revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement.

Research and development expenses in the fourth quarter of 2017 were \$4,721,000, compared with \$6,242,000 for the fourth quarter 2016. Fiscal 2017 research and development expenses were \$19,468,000 compared with \$20,042,000 for fiscal 2016.

Research and development expense decreased \$1.5 million in the fourth quarter of 2017 compared to the fourth quarter of 2016 primarily due to lower outsourced clinical and product development costs of \$1.0 million, professional fees of \$0.3 million and labor related costs of \$0.2 million. Research and development expense decreased \$0.6 million in 2017 primarily due to lower labor costs.

General and administrative expenses in the fourth quarter of 2017 were \$1,849,000, compared with \$1,808,000 for the fourth quarter of 2016. General and administrative expenses for fiscal 2017 were \$7,551,000, compared to \$7,563,000 for fiscal 2016.

2018 Financial Guidance

For 2018, ArQule expects revenue to range between \$3 and \$4 million. Net use of cash is expected to range between \$26 and \$28 million for the year. Net loss is expected to range between \$27 and \$30 million, and net loss per share to range between \$(0.30) and \$(0.34) for the year. ArQule expects to end 2018 with between \$23 and \$25 million in cash and marketable securities.

Conference Call and Webcast

ArQule will hold its fourth quarter and full year financial results call today, March 5, 2018 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](http://www.arqule.com) 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](http://www.arqule.com) a leader among companies our size in precision medicine. ArQule’s proprietary pipeline includes: Derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial 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](https://twitter.com/ArQule) and [LinkedIn](https://www.linkedin.com/company/arqule).

Forward-Looking Statements

This press release contains forward-looking statements, including without limitation under the headings “Key Highlights,” “2018 Goals,” and quotes of management in connection with the Company’s clinical trials and planned clinical trials with ARQ 531, miransertib (ARQ 092), derazantinib (ARQ 087) and ARQ 751 as well as under “2017 Financial Guidance” with respect to projected financial results and its 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, the registrational trial of derazantinib in iCCA may not meet its primary endpoint of overall response rate. Moreover, ARQ 531, miransertib, and ARQ 751 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 partner are utilizing a break apart FISH diagnostic to identify patients in the trial with derazantinib in iCCA, and are utilizing or expect to utilize diagnostic tools 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. 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. With respect to partnered programs, even if certain compounds show initial promise our collaborators may decide not to continue to develop them. In addition, Sinovant Sciences Ltd. has certain rights to unilaterally terminate its agreement with ArQule. If it were to do so, the Company might not be able to complete development and commercialization of the applicable licensed products on its own in Greater China. 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 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)

	Quarter Ended		Year Ended	
	December 31,		December 31,	
	2017	2016	2017	2016
Research and development revenue	\$ -	\$ 1,187	\$ -	\$ 4,709
Costs and expenses:				
Research and development	4,721	6,242	19,468	20,042
General and administrative	1,849	1,808	7,551	7,563
Total costs and expenses	6,570	8,050	27,019	27,605
Loss from operations	(6,570)	(6,863)	(27,019)	(22,896)
Interest income	113	43	238	178
Interest expense	(401)	-	(1,520)	-
Other income (expense) (1)	(902)	-	(902)	-
Net loss	(7,760)	(6,820)	(29,203)	(22,718)
Unrealized gain (loss) on marketable securities	(15)	(20)	(18)	(1)
Comprehensive loss	\$(7,775)	\$(6,840)	\$(29,221)	\$(22,719)
Basic and diluted net loss per share:				
Net loss per share	\$(0.09)	\$(0.10)	\$(0.39)	\$(0.33)
Weighted average basic and diluted common shares outstanding	85,292	71,106	74,813	69,714

(1) Other income (expense) in 2017 includes a non-cash expense of \$902 thousand from the increase in fair value of our preferred stock warrant liability.

	December 31, December 31,	
Balance sheet data (in thousands): (Unaudited)	2017	2016
Cash, equivalents and marketable securities- short term	\$ 48,036	\$ 31,126
Marketable securities- long term	-	-
	\$ 48,036	\$ 31,126
Total assets	\$ 48,902	\$ 32,380
Stockholders' equity	\$ 14,181	\$ 23,680

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Source: ArQule, Inc.

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