



## ArQule Reports First Quarter 2018 Financial Results

May 7, 2018

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

BURLINGTON, Mass.--(BUSINESS WIRE)--May 7, 2018-- [ArQule](#), Inc. (Nasdaq: ARQL) today announced its financial results for the first quarter of 2018.

For the quarter ended March 31, 2018, the Company reported a net loss of \$6,532,000, or \$0.07 per share, compared with net loss of \$7,576,000, or \$0.11 per share, for the quarter ended March 31, 2017.

At March 31, 2018, the Company had a total of approximately \$42,884,000 in cash and marketable securities.

### Key Highlights

- **ARQ 531, our potent and reversible BTK inhibitor, demonstrated good oral bioavailability and pharmacokinetics in data presented at the American Association for Cancer Research (AACR) on April 15, 2018.** The Phase 1a portion of the Phase 1a/b trial continues to recruit on schedule with no safety concerns, and we plan to present more advanced data at one or more major congresses later in 2018.
- **Miransertib, our lead proprietary AKT inhibitor, was featured in an oral presentation at AACR in which it showed positive signs of activity in hormone- sensitive tumors with AKT1 or PI3K dysregulation.** The Phase 1b trial of miransertib with anastrozole in patients with advanced endometrial cancer produced one complete response and three partial responses out of 8 patients and continues to recruit.
- **Miransertib has been granted Orphan Drug Designation by EMA for the rare disease, Proteus syndrome.** We continue to make progress with our registrational strategy in Proteus syndrome; in addition, we are progressing our rare disease expansion strategy with the Phase 1/2 trial in PROS and Proteus syndrome which is recruiting on schedule.
- **The Company granted Basilea Pharmaceutica Ltd. (“Basilea”) an exclusive license to develop and commercialize derazantinib, our pan-FGFR inhibitor, in all parts of the world except the People’s Republic of China, Hong Kong, Macau and Taiwan, where the Company has licensed rights to Sinovant Sciences, Ltd., a subsidiary of Roivant Sciences Ltd.** Terms of the transaction include a \$10 million upfront payment, an additional \$326 million in regulatory and commercial milestones, and royalties on net sales ranging from single to double digits; Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory.

“We have continued to build on the momentum that we created during 2017 with presentations of important data at the recent AACR meeting and the licensing of derazantinib to our partner, Basilea,” said Paolo Pucci, Chief Executive Officer of ArQule. “This new partnership further supports our mid-term strategy by allowing us to develop derazantinib in ways that we could not have done on our own and by strengthening our balance sheet thus enabling us to focus more on our BTK and ATK programs in potential fast-to-market settings.”

“After a very strong 2017 scientifically, our execution in 2018 continues to be at a high level, highlighted by continuing progress across all pipeline assets,” said Brian Schwartz, M.D., Head of Research and Development and Chief Medical Officer of ArQule. “We are approaching therapeutic levels in the dosing of patients in the Phase 1a/b trial with our BTK inhibitor, ARQ 531, and look forward to presenting a comprehensive data set from that trial later this year. The AKT program is also progressing well in both rare diseases and oncology. We are launching an expansion of the Phase 1b trial with miransertib plus anastrozole in patients with advanced endometrial cancers and are executing our registrational strategy for Proteus and PROS.”

### Revenues and Expenses

Revenues for the quarter ended March 31, 2018, were \$4,138,000 compared with revenues of zero for the quarter ended March 31, 2017. Research and development revenue in the quarter ended March 31, 2018 consisted of \$3,000,000 from the February 2018 Roivant licensing agreement and \$1,138,000 from our October 2017 non-exclusive license agreement for certain library compounds.

Research and development expenses in the first quarter of 2018 were \$5,812,000, compared with \$5,194,000 for the first quarter 2017.

Research and development expenses increased \$0.6 million in the first quarter of 2018 compared to the first quarter of 2017 primarily due to higher outsourced pre-clinical, clinical and product development costs.

General and administrative expenses in the first quarter of 2018 were \$2,351,000, compared with \$2,074,000 for the first quarter of 2017. General and administrative expenses increased \$0.3 million in the first quarter of 2018 compared to the first quarter of 2017 primarily due to higher labor related costs of \$0.2 million and professional fees of \$0.1 million.

### 2018 Updated Financial Guidance

As a result of the April 2018 exclusive license agreement with Basilea, our guidance for 2018 is being updated. For 2018, ArQule now expects revenue

to range between \$14 and \$17 million. Net use of cash is expected to range between \$27 and \$29 million for the year. Net loss is expected to range between \$16 and \$21 million, and net loss per share to range between \$(0.18) and \$(0.24) for the year. ArQule expects to end 2018 with between \$40 and \$42 million in cash and marketable securities.

## Conference Call and Webcast

ArQule will hold its first quarter 2018 financial results call today, May 7, 2018 at 9:00 a.m. ET. The live webcast can be accessed in the "Investors & Media" section of our website, [www.arqule.com](http://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](http://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 pipeline includes: 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; 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  $\beta$ -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. You can follow us on [Twitter](#) and [LinkedIn](#).

## 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 with ARQ 531, miransertib (ARQ 092), derazantinib (ARQ 087) and ARQ 751, 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, 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 partners 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. 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)

Quarter Ended  
March 31,  
2018      2017

Research and development revenue	\$ 4,138	\$ —
Costs and expenses:		
Research and development	5,812	5,194
General and administrative	2,351	2,074
Total costs and expenses	8,163	7,268
Loss from operations	(4,025 )	(7,268 )
Interest income	159	22
Interest expense	(396 )	(330 )
Other expense (1)	(2,270 )	—
Net loss	(6,532 )	(7,576 )
Unrealized loss on marketable securities	(25 )	(4 )
Comprehensive loss	\$ (6,557)	\$ (7,580)
Basic and diluted net loss per share	\$ (0.07 )	\$ (0.11 )
Weighted average shares used in calculating:		
Basic and diluted loss per share	87,112	71,138

(1) Other expense includes a non-cash expense of \$2,270 thousand from the increase in fair value of our preferred stock warrant liability.

<b>Balance sheet data (in thousands) unaudited:</b>	<b>March 31, 2018</b>	<b>December 31, 2017</b>
Cash, equivalents and marketable securities- short term	\$ 42,884	\$ 48,036
Marketable securities- long term	—	—
	\$ 42,884	\$ 48,036
Total assets	\$ 45,103	\$ 48,902
Stockholders' equity	\$ 9,692	\$ 14,181

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Source: ArQule, Inc.

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