May 7, 2014

ArQule Reports First Quarter 2014 Financial Results

Conference call scheduled today at 9:00 a.m. eastern time

WOBURN, Mass.--(BUSINESS WIRE)-- ArQule, Inc. (NASDAQ: ARQL) today announced its financial results for the first quarter of 2014.

For the quarter ended March 31, 2014, the Company reported a net loss of $7,141,000 or $0.11 per share, compared to a net loss of $5,775,000, or $0.09 per share, for the first quarter of 2013.

At March 31, 2014, the Company had a total of $85,758,000 in cash, equivalents and marketable securities.

Operational Update

- Enrollment is proceeding in the Phase 3 METIV-HCC trial of tivantinib (ARQ 197) in hepatocellular carcinoma (HCC) conducted by ArQule and its partner, Daiichi Sankyo Co., in the U.S. and Europe;
- Kyowa Hakko Kirin, the Company's partner in Asian territories, initiated the Phase 3 JET-HCC trial of tivantinib in HCC in Japan in February, 2014;
- Patient enrollment is continuing in a number of NIH-sponsored trials with tivantinib, including Phase 2 randomized trials in prostate cancer, head and neck cancer, and kidney cancer;
- The dose escalation portions of Phase 1 trials with the Company's proprietary compounds, ARQ 092 and ARQ 087, are nearing completion.

"The two Phase 3 trials with tivantinib in HCC are proceeding," said Paolo Pucci, chief executive officer of ArQule. "These trials, which include METIV-HCC in the West and JET-HCC in Japan, are unique in that they are the only biomarker-directed trials ongoing in second-line HCC. We expect to provide updates on the projected time frame to completion of patient enrollment as more patients complete screening to ensure that they have MET-diagnostic high disease upon entry into the trial.

"The most advanced clinical trials with tivantinib that are being conducted under the NIH-CRADA program are randomized Phase 2 trials taking place in prostate cancer, head and neck cancer and kidney cancer," said Mr. Pucci. "We plan to announce data from these independent trials as they are made available by sponsoring investigators.

"Our earlier clinical-stage programs are focused on ARQ 092, an Akt inhibitor, and ARQ 087, an FGFR inhibitor," said Mr. Pucci. "Phase 1 trials with both of these products are nearing the achievement of maximum tolerated dose, and we plan to announce data from these trials later this year."

Revenues and Expenses

The Company reported research and development revenue of $2,676,000 for the quarter ended March 31, 2014, compared with $5,661,000 for the quarter ended March 31, 2013. Revenue in the three months ended March 31, 2014 is comprised of revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement for tivantinib.

The $3.0 million revenue decrease in the quarter ended March 31, 2014 is primarily due to revenue decreases of $0.6 million from our Daiichi Sankyo tivantinib program, $0.6 million from our Daiichi Sankyo ARQ 092 agreement that ended in June 2013, and $1.8 million of other revenue related to a one-time research project in the quarter ended March 31, 2013.

Total costs and expenses for the quarter ended March 31, 2014 were $9,981,000 compared to $11,581,000 for the first quarter of 2013. Research and development costs for the quarter ended March 31, 2014 were $6,731,000 compared to $8,181,000 for the first quarter of 2013. These decreases were primarily due to lower labor related costs of $1.2 million and reduced lab expenses of $0.4 million. These cost decreases were partially offset by $0.3 million higher outsourced clinical and product development costs related to our pipeline programs, including ARQ 092 and ARQ 087. General and administrative costs for the quarter ended March 31, 2014 were $3,250,000, compared to $3,400,000 for the first quarter of 2013. This decrease was principally due to lower non-cash stock compensation expenses.

Conference Call and Webcast

Conference call details

Date:	Wednesday, May 7, 2014
Time:	9:00 a.m. Eastern Time
Conference Call Numbers	
Domestic:	877-868-1831
International:	914-495-8595
Web cast:	http://www.arqule.com

A replay of the conference call will be available beginning two hours after the completion of the call until the end of May 9, 2014 and can be accessed by dialing toll-free 855-859-2056 or 800-585-8367, and outside the U.S. 404-537-3406. The confirmation code for replayed calls is 30148648.

About ArQule

ArQule is a biotechnology company engaged in the research and development of next-generation, small-molecule cancer therapeutics. The Company's targeted, broad-spectrum products and research programs are focused on key biological processes that are central to human cancers. ArQule's lead product, in Phase 2 and Phase 3 clinical development, is tivantinib (ARQ 197), an oral, selective inhibitor of the c-MET receptor tyrosine kinase. The Company's pipeline includes: ARQ 092, designed to inhibit the Akt serine/threonine kinase, and ARQ 087, designed to inhibit fibroblast growth factor receptor (FGFR). ArQule's current discovery efforts, which are based on the ArQule Kinase Inhibitor Platform (AKIP™), are focused on the identification of novel kinase inhibitors that are potent, selective and do not compete with ATP (adenosine triphosphate) for binding to the kinase.

This press release contains forward-looking statements regarding the Company's clinical trials with tivantinib (ARQ 197) and other candidate compounds in earlier stages of development as well as 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. 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, tivantinib, ARQ 092 and ARQ 087 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 during clinical trials or in the course of developing, testing or manufacturing these compounds that could lead the Company or its partners 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 view of the data or require additional data or information or additional studies. In addition, the planned timing of initiation and completion of clinical trials for tivantinib is subject to the ability of the Company as well as Daiichi Sankyo, Inc. and Kyowa Hakko Kirin, a licensee of tivantinib, 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 companion diagnostic to identify MET-high patients in the METIV-HCC and JET-HCC trials, and we may encounter difficulties in developing and obtaining approval for companion diagnostics, including issues relating to selectivity/specificity, analytical validation, reproducibility, or clinical validation. Any delay or failure by our collaborators to develop or obtain regulatory approval of the 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. Positive pre-clinical data may not be supported in later stages of development. Furthermore, ArQule may not have the financial or human resources to successfully pursue drug discovery in the future. Moreover, with respect to partnered programs, even if certain compounds show initial promise, Daiichi Sankyo or Kyowa Hakko Kirin may decide not to license or continue to develop them, as the case may be. In addition, Daiichi Sankyo and Kyowa Hakko Kirin have certain rights to unilaterally terminate their agreements with ArQule. If either company were to do so, the Company might not be able to complete development and commercialization of the applicable licensed products on its own. 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 March 31,	
	2014	2013
Research and development revenue (1)	$ 2,676	$ 5,661
Costs and expenses:		
Research and development	6,731	8,181
General and administrative	3,250	3,400
Total costs and expenses	9,981	11,581
Loss from operations	(7,305)	(5,920)
Interest income	95	151
Interest expense	(7)	(4)
Other income (expense)	76	(2)
Net loss	(7,141)	(5,775)
Unrealized gain (loss) on marketable securities	(17)	9
Comprehensive loss	$ (7,158)	$ (5,766)
Basic and diluted net loss per share	$ (0.11)	$ (0.09)
Weighted average shares used in calculating:		
Basic and diluted loss per share	62,583	62,384

(1) Research and development revenue is shown net of collaboration contra-revenue of $82 thousand and $216 thousand for the quarters ended March 31, 2014 and 2013, respectively.

Balance sheet data (in thousands):	March 31, 2014	December 31, 2013
Cash, equivalents and marketable securities- short term	$ 71,728	$ 74,695
Marketable securities- long term	14,030	20,391
	$ 85,758	$ 95,086
Total assets	$ 88,574	$ 98,179
Notes payable	$ 1,700	$ 1,700
Stockholders' equity	$ 54,554	$ 60,626

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Source: ArQule, Inc.