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ArQule Announces Top-Line Results of Phase 3 Clinical Study of Tivantinib in Hepatocellular Carcinoma in Japan

BURLINGTON, Mass.--(BUSINESS WIRE)-- ArQule, Inc. (Nasdaq: ARQL) today reported that its partner, Kyowa Hakko Kirin, announced top-line results of the JET-HCC Phase 3 trial of tivantinib in Japan, and that the trial did not meet its primary endpoint of progression free survival (PFS).

JET-HCC is a randomized, double-blind placebo-controlled study that enrolled approximately 190 Japanese patients with c-Met diagnostic-high inoperable hepatocellular carcinoma (HCC) with a history of prior sorafenib therapy, to evaluate the efficacy and safety of tivantinib.

The primary endpoint of the trial is PFS, and the top-line results did not show a significant difference in PFS between the tivantinib group and the placebo group. There were no new safety issues observed in the trial.

The details of the study results will be presented in an upcoming scientific forum.

"I would like to thank our partner, Kyowa Hakko Kirin, and all the participants in their study," said Paolo Pucci, Chief Executive Officer of ArQule. "The results are disappointing as there is a need for a second-line HCC therapy in Japan."

About Hepatocellular Carcinoma (HCC)

Liver cancer is the sixth most common cancer globally with 782,000 new cases in 2012 and is the second most common cause of cancer-related death with 745,000 deaths in 2012.[1] HCC accounts for about 90 percent of primary liver cancers.[2] Cirrhosis, chronic hepatitis B and C and smoking are recognized worldwide as factors increasing the risk of HCC.[2]

About Tivantinib (ARQ 197)

Tivantinib (ARQ 197) is an orally administered, small molecule inhibitor of the c-Met receptor tyrosine kinase ("MET") and its biological pathway. Kyowa Hakko Kirin and ArQule entered into a license agreement for exclusive rights to the development and sale of tivantinib in Japan and certain parts of Asia (China, Korea, and Taiwan) on April 12, 2007.

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: 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; ARQ 092, a selective inhibitor of the AKT serine/threonine kinase, in phase 1 for multiple oncology indications as well as ultra-rare Proteus syndrome, in partnership with the National Institutes of Health (NIH); 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, through toxicology testing and plan to initiate a phase 1 trial by the third quarter of 2017. ArQule's most advanced product is tivantinib (ARQ 197), an oral, selective inhibitor of the c-MET receptor tyrosine kinase, that recently completed two phase 3 trials in second-line MET-overexpressing hepatocellular carcinoma in partnership with Daiichi Sankyo in the West and Kyowa Hakko Kirin in Asia. 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.

About Kyowa Hakko Kirin

Kyowa Hakko Kirin Co., Ltd. is a research-based life sciences company, with special strengths in biotechnologies. In the core therapeutic areas of oncology, nephrology and immunology/allergy, Kyowa Hakko Kirin leverages leading-edge

biotechnologies centered on antibody technologies, to continually discover innovative new drugs and to develop and market those drugs world-wide. In this way, the company is working to realize its vision of becoming a Japan-based global specialty pharmaceutical company that contributes to the health and wellbeing of people around the world. You can learn more about the business at: www.kyowa-kirin.com.

Forward Looking Statements

This press release contains forward-looking statements regarding the Company's clinical trials with tivantinib (ARQ 197). 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 may not demonstrate promising therapeutic effect or 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 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 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., our development partner for tivantinib, 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 companion diagnostic tests to identify MET-overexpressing patients in the METIV-HCC, JET-HCC and other trials. 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 or us 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, Kyowa Hakko Kirin may decide not to continue to develop them. 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.

References:

[1] Ferlay J, et al. Int. J. Cancer. 2015;136:E359-E386.

[2] Llovet JM, et al. J Hepatol. 2012;56(4):908-43.

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