



Press Release

Daiichi Sankyo and ArQule Announce the Completion of the METIV-HCC Phase 3 Study of Tivantinib in Second-Line Treatment of MET-Overexpressing Hepatocellular Carcinoma

• ArQule to host investor conference call on February 17, 2017 at 8:30 A.M. ET

Burlington, MA, Tokyo, Japan, Munich, Germany and Parsippany, NJ – February 17, 2017 – ArQule, Inc. (Nasdaq: ARQL) and Daiichi Sankyo today announced that the METIV-HCC phase 3 study of tivantinib in hepatocellular carcinoma (HCC) did not meet its primary endpoint of improving overall survival.

METIV-HCC is a biomarker-selected, double-blind, placebo-controlled, randomized phase 3 study evaluating tivantinib (2:1) versus best supportive care in patients with MET-overexpressing, inoperable HCC intolerant to or previously-treated with systemic therapy. A total of 340 patients with MET-overexpressing HCC analyzed by a validated immunohistochemical assay were randomized in the intent-to-treat population for efficacy analysis. The primary endpoint of the study is overall survival. Secondary endpoints include progression-free survival and safety. Full results from the trial will be presented at an upcoming scientific forum.

"HCC is a disease with high unmet need, especially in the second-line setting, so these results are disappointing for the patients as well as the investigators and the companies," said Paolo Pucci, Chief Executive Officer of ArQule.

"Despite the negative outcome of this study, we remain committed to applying rigorous science to unmet needs for patients with cancer," said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. "We would like to take this opportunity to thank all of the investigators, and especially the patients, for their participation in this study."

The ArQule investor conference call can be accessed in the "Investors and Media" section of ArQule's 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. and using the passcode 74015633. 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 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. HCC accounts for about 90 percent of primary liver cancers. Cirrhosis, chronic hepatitis B and C and smoking are recognized worldwide as factors increasing the risk of HCC.

About Tivantinib (ARQ 197)

ArQule and Daiichi Sankyo have a licensing, co-development and co-commercialization agreement for tivantinib in the U.S., Europe, South America and the rest of the world, excluding Japan, China (including Hong Kong), South Korea and Taiwan.

About ArQule

ArQule is a biopharmaceutical company engaged in the research and development of targeted therapeutics to treat cancers and rare diseases. Our 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 lead product, in phase 3 clinical development, is tivantinib (ARQ 197), an oral, selective inhibitor of the c-MET receptor tyrosine kinase, for second-line treatment of patients with MET-overexpressing hepatocellular carcinoma in partnership with Daiichi Sankyo in the West and Kyowa Hakko Kirin in Asia. 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, into toxicology testing and plan to file an Investigational New Drug Application in early 2017. 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 Daiichi Sankyo Cancer Enterprise

The vision of Daiichi Sankyo Cancer Enterprise is to leverage our world-class, innovative science and push beyond traditional thinking in order to create meaningful treatments for patients with cancer. We are dedicated to transforming science into value for patients, and this sense of obligation informs everything

we do. Anchored by our Antibody Drug Conjugate (ADC) and Acute Myeloid Leukemia (AML) franchises, our cancer pipeline includes more than 20 small molecules, monoclonal antibodies and ADCs stemming from our powerful research engines: our two laboratories for biologic/immuno-oncology and small molecules in Japan, and Plexxikon Inc., our small molecule structure-guided R&D center in Berkeley, CA. Compounds in development include: quizartinib, an oral FLT3 inhibitor, for FLT3-ITD+ AML; DS-8201, a HER2-targeting ADC, for HER2-expressing breast or gastric cancer or other HER2-expressing solid tumors; pexidartinib, an oral CSF-1R inhibitor, for tenosynovial giant cell tumor (TGCT), which is also being explored in a range of solid tumors in combination with the anti-PD1 immunotherapy, pembrolizumab; and tivantinib, an oral MET inhibitor, for second-line treatment of patients with MET-overexpressing hepatocellular carcinoma in partnership with ArQule, Inc.

About Daiichi Sankyo

Daiichi Sankyo Group is dedicated to the creation and supply of innovative pharmaceutical products to address diversified, unmet medical needs of patients in both mature and emerging markets. With over 100 years of scientific expertise and a presence in more than 20 countries, Daiichi Sankyo and its 16,000 employees around the world draw upon a rich legacy of innovation and a robust pipeline of promising new medicines to help people. In addition to a strong portfolio of medicines for hypertension and thrombotic disorders, under the Group's 2025 Vision to become a "Global Pharma Innovator with a Competitive Advantage in Oncology," Daiichi Sankyo research and development is primarily focused on bringing forth novel therapies in oncology, including immuno-oncology, with additional focus on new horizon areas, such as pain management, neurodegenerative diseases, heart and kidney diseases, and other rare diseases. For more information, please visit: www.daiichisankyo.com. Daiichi Sankyo, Inc., headquartered in Parsippany, New Jersey, is a member of the Daiichi Sankyo Group. For more information on Daiichi Sankyo, Inc., please visit: www.dsi.com.

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, 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.

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References

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