



ArQule Announces Results of Phase 2 Trial with ARQ 197 in Advanced Non-Small Cell Lung Cancer

ARQ 197 in combination with erlotinib prolongs median progression free survival Conference call scheduled today at 8:30 a.m. eastern time

WOBURN, Mass., Mar 31, 2010 (BUSINESS WIRE) -- ArQule, Inc. (Nasdaq: ARQL) today announced that ARQ 197, when used in combination with erlotinib, demonstrated a 66% improvement in median Progression-Free Survival (PFS) in patients with advanced, refractory non-small cell lung cancer (NSCLC). In the intent to treat (ITT) population (n = 167), median PFS was 16.1 weeks in the ARQ 197 plus erlotinib arm, compared with 9.7 weeks in the erlotinib plus placebo arm.

The difference in PFS between the two arms did not achieve statistical significance (hazard ratio = 0.809) by applying a log-rank test. When adjusting for imbalances in the distribution of key prognostic factors, the difference in PFS was statistically significant (hazard ratio = 0.675) by applying a Cox regression analysis specified for secondary efficacy analyses.

Improvement in median PFS was more pronounced in the pre-defined sub-group of patients with non-squamous histology (n = 117); median PFS was 18.9 weeks in the treatment arm versus 9.7 weeks in the control arm, which represents a 94% improvement. Based on an exploratory Cox regression analysis, the endpoint of PFS was met in the sub-group and achieved statistical significance (hazard ratio = 0.613).

There were no clinically relevant differences in adverse event rates between the treatment and control arms. The majority of adverse events were mild in intensity and included rash, diarrhea and fatigue.

"We believe the treatment benefit observed in this trial would represent a meaningful clinical improvement over standard therapy if replicated in Phase 3 trials," said Dr. Brian Schwartz, chief medical officer of ArQule. "We are especially encouraged by the potential benefit for the large sub-group of non-squamous cell patients. We will thoroughly analyze our extensive database from this trial, including additional patient sub-group characteristics, to optimize ongoing and future trials of ARQ 197."

Complete data from this trial, which will include biomarker analyses, will be presented at a future medical meeting during 2010.

One hundred sixty-seven patients were evaluated in the Phase 2 trial. Participating patients were EGFR (epidermal growth factor receptor) inhibitor naïve and were randomized one-to-one to receive either the combination of ARQ 197 plus erlotinib or placebo plus erlotinib in second and third line settings. ARQ 197 is an orally available, small molecule inhibitor of the c-Met receptor tyrosine kinase. Erlotinib, marketed as Tarceva(TM), is an inhibitor of the EGFR tyrosine kinase.

ARQ 197 is also currently being evaluated in clinical trials as a single agent and in combination with other anti-cancer therapies in a number of indications, including c-Met-associated soft-tissue sarcomas, hepatocellular carcinoma, pancreatic adenocarcinoma, germ cell tumors and colorectal cancer.

Patients, physicians and other healthcare professionals seeking additional information regarding trials involving ARQ 197 may call 1-800-373-7827.

The American Cancer Society's estimates of the impact of lung cancer in the U.S. during 2009 include approximately 219,000 new cases (both non-small cell and small cell) and 159,000 deaths resulting from the disease, accounting for 28 percent of all cancer deaths. Lung cancer is the leading cause of cancer death among both men and women.

Conference Call and Webcast

ArQule will hold a conference call at 8:30 a.m. eastern time today, March 31, 2010 to discuss the results of the trial described above.

Date: Wednesday, March 31, 2010

Time: 8:30 a.m., Eastern Time

Conference Call Numbers

Domestic: (877) 868-1831

International: (914) 495-8595
Webcast: www.arqule.com

A replay of the conference call will be available beginning at Noon on March 31, 2010 for seven days and can be accessed by dialing toll-free (800) 642-1687 and outside the U.S. (706) 645-9291. The confirmation code for replayed calls is 66413046.

About c-Met and ARQ 197

When abnormally activated, the c-Met receptor tyrosine kinase plays multiple roles in aspects of human cancer, including cancer cell growth, survival, angiogenesis, invasion and metastasis. Pre-clinical data have demonstrated that ARQ 197 inhibits c-Met activation in a range of human tumor cell lines and shows anti-tumor activity against several human tumor xenografts. In clinical trials to date, treatment with ARQ 197 has been well tolerated and has resulted in tumor responses and prolonged stable disease across broad ranges of tumors and doses.

About ArQule, Inc. and Daiichi Sankyo, Co., Ltd.

On December 19, 2008, ArQule and Daiichi Sankyo, Co., Ltd. signed a license, co-development and co-commercialization agreement to co-develop ARQ 197 in the U.S., Europe, South America and the rest of the world, excluding Japan, China (including Hong Kong), South Korea and Taiwan, where Kyowa Hakko Kirin Co., Ltd. has exclusive rights for development and commercialization.

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 clinical development, is ARQ 197, an inhibitor of the c-Met receptor tyrosine kinase. The Company is also conducting Phase 1 clinical testing with ARQ 621, designed to inhibit the Eg5 kinesin motor protein. The Company's pre-clinical pipeline includes a compound designed to inhibit the B-RAF kinase. ArQule's current discovery efforts, which are based on the ArQule Kinase Inhibitor Platform (AKIP(TM)), 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. The most advanced AKIP(TM) program is focused on the discovery of inhibitors of fibroblast growth factor receptor (FGFR).

This press release contains forward-looking statements regarding the progress of the Company's clinical trials, including its Phase 2 trial with ARQ 197 in non-small cell lung cancer (NSCLC) and trials which may be conducted by Daiichi Sankyo and/or Kyowa Hakko Kirin under their agreements with the Company. 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 early stage clinical trial results is not necessarily indicative of clinical efficacy and does not ensure that later stage or larger scale clinical trials will be successful. For example, ARQ 197 may not demonstrate promising therapeutic effect; in addition, this compound may not demonstrate an appropriate safety profile in further pre-clinical testing and in current, 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. 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 partner to discontinue development. Even if later stage clinical trials are successful, the risk exists that unexpected concerns may arise from analysis of data or from additional data or that obstacles may arise or issues be identified in connection with review of clinical data with regulatory authorities or that regulatory authorities may disagree with the Company's view of the data or require additional data, information or studies. In addition, the planned timing of initiation and completion of clinical trials for ARQ 197 are subject to the ability of the Company or Daiichi Sankyo, its partner, and Kyowa Hakko Kirin, a licensee of ARQ 197, to enroll patients, enter into agreements with clinical trial sites and investigators, and other technical hurdles and issues that may not be resolved. Moreover, Daiichi Sankyo has certain rights to unilaterally terminate the ARQ 197 license, co-development and co-commercialization agreement. 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 pursue drug discovery successfully in the future. 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.

SOURCE: ArQule, Inc.

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