



ArQule to Present Data at the 2018 American Association for Cancer Research (AACR) Annual Meeting

March 15, 2018

ArQule's pipeline will be highlighted in ten presentations at the meeting

BURLINGTON, Mass.--(BUSINESS WIRE)--Mar. 15, 2018-- ArQule, Inc. (Nasdaq: ARQL) today announced that pre-clinical and clinical data on the company's pipeline of drug candidates will be presented at the 2018 AACR Annual Meeting taking place in Chicago from April 14-April 18. Data will be presented in one oral and nine poster sessions on trials conducted by ArQule and its collaborators for BTK inhibitor, ARQ 531, AKT inhibitors, miransertib and ARQ 751, as well as FGFR inhibitor, derazantinib.

The oral presentation will highlight data from a phase 1b trial for miransertib in combination with anastrozole in PIK3CA or AKT1-mutant endometrial and ovarian cancers conducted by Dr. David M. Hyman and colleagues at Memorial Sloan Kettering.

Oral Presentation Details

April 15, 2018

Title: *A phase 1b study of Miransertib (ARQ 092) in combination with anastrozole in patients with PIK3CA or AKT1-mutant ER+ endometrial and ovarian cancer*

Time: 3:00 – 5:00 p.m. CT

Location: Room N427 - McCormick Place North, Level 4

Sponsor: Memorial Sloan Kettering, New York, NY

Poster Presentation Details

April 15, 2018

Title: *ARQ 531, a Novel and Reversible Inhibitor of Bruton's Tyrosine Kinase, Displays Favorable Oral Bioavailability and Exposure in patients with B-cell malignancies*

Time: 1:00 – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 43, Poster Board 18

Sponsor: ArQule, Inc.

Title: *The novel Bruton's tyrosine kinase inhibitor ARQ 531 disrupts survival signaling and triggers apoptosis in AML cells*

Time: 1:00 – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 37, Poster Board 3

Sponsor: University of Genoa, Genova, Italy

Title: *Results of A Phase 1 Dose Escalation Study of ARQ 751 in Adult Subjects with Advanced Solid Tumors with AKT1, 2, 3 Genetic Alterations, Activating PI3K Mutations, PTEN-null, or other known actionable PTEN mutations*

Time: 1:00 – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 42, Poster Board 17

Sponsor: MD Anderson Cancer Center, Houston, TX

April 16, 2018

Title: *ARQ 531, a potent reversible BTK inhibitor exhibits potent antitumor activity in ibrutinib resistant diffuse large B-cell lymphoma*

Time: 8:00 a.m. – 12:00 p.m. CT

Location: Exhibit Hall A, Poster Section 41, Poster Board 8

Sponsor: ArQule, Inc.

Title: *Preclinical Evaluation of the Tyrosine Kinase Inhibitor ARQ531 in AML*

Time: 8:00 a.m. – 12:00 p.m. CT

Location: Exhibit Hall A, Poster Section 38, Poster Board 13

Sponsor: The Ohio State University, Columbus OH

Title: *In vivo combination of Miransertib (ARQ 092) with anti-PD-1 antibody, Trametinib, Lapatinib, Trastuzumab and Paclitaxel*

Time: 1:00 p.m. – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 41, Poster Board 23

Sponsor: ArQule, Inc.

April 17, 2018

Title: *Derazantinib (ARQ 087) Pharmacodynamics: Alterations in FGF19/21/23 and phosphate in Patients with Cholangiocarcinoma*

Time: 8:00 a.m. – 12:00 p.m. CT

Location: Exhibit Hall A, Poster Section 43, Poster Board 22

Sponsor: ArQule, Inc.

Title:*In vitro and in vivo effect of ARQ 531 on Trk family kinases*

Time:1:00 p.m. – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 36, Poster Board 26

Sponsor:ArQule, Inc.

Title:*Combinations of imatinib mesylate with AKT inhibitor (Miransertib, ARQ 751) or FGFR inhibitor (Derazantinib) show synergy in GIST cell lines and pre-clinical models*

Time:1:00 p.m. – 5:00 p.m. CT

Location: Exhibit Hall A, Poster Section 37, Poster Board 7

Sponsor:Fox Chase Cancer Center, Philadelphia, PA

About Miransertib and ARQ 751

Miransertib (ARQ 092) and ARQ 751 are orally bioavailable, selective small molecule inhibitors of the AKT serine/threonine kinase. The AKT pathway when abnormally activated is implicated in multiple oncogenic processes such as cell proliferation and apoptosis. This pathway has emerged as a target of potential therapeutic relevance for compounds that inhibit its activity, which has been linked to a variety of cancers as well as to select non-oncology indications.

Miransertib, the lead compound in ArQule's AKT program, has completed phase 1a clinical testing and has advanced into phase 1b expansion testing in cohorts of patients with endometrial cancer, lymphomas and tumors harboring either AKT or PI3K mutations. A company sponsored phase 1/2 trial is being conducted in the U.S. and E.U. for Overgrowth Diseases, including PROS and Proteus syndrome. Miransertib is also in a phase 1 trial being conducted by the NIH for Proteus syndrome.

About ARQ 531

ARQ 531 is an investigational, orally bioavailable, potent and reversible Bruton's tyrosine kinase (BTK) inhibitor. Biochemical and cellular studies have shown that ARQ 531 inhibits both the wild type and C481S-mutant forms of BTK. The C481S mutation is a known emerging resistance mechanism for first generation irreversible BTK inhibitors. In preclinical studies ARQ 531 has demonstrated high oral bioavailability as well as good ADME, pharmacokinetic and metabolic properties. The Company initiated a phase 1 trial in the third quarter of 2017. BTK is a therapeutic target that has been clinically proven to inhibit B-cell receptor signaling in blood cancers.

About Derazantinib

Derazantinib is a potent, orally administered inhibitor of the fibroblast growth factor receptor (FGFR) family, a key driver of cell proliferation, differentiation, and migration. In a Phase 1/2 study in patients with iCCA harboring FGFR2 gene fusions, treatment with derazantinib resulted in an objective response rate of 21%, nearly 3 times higher than standard-of-care chemotherapy. ArQule is currently conducting a registrational study with derazantinib in patients with FGFR2 fusion-positive second-line iCCA. The open-label single-arm trial is recruiting in both the United States and Europe with objective response rate as the primary endpoint.

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: Derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA; 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), as well as in multiple oncology indications; ARQ 751, a next generation AKT inhibitor, in phase 1 for patients with AKT1 and PI3K mutations; and ARQ 761, a ?-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, in phase 1 for patients with B-cell malignancies refractory to other therapeutic options. 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 regarding the Company's clinical trials with ARQ 531, miransertib (ARQ 092), ARQ 751, and derazantinib (ARQ 087). 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, 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 partner 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. 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. With respect to partnered programs, even if certain compounds show initial promise our collaborators may decide not to continue to develop them. In addition, Sinovant Sciences Ltd. has certain rights to unilaterally terminate its agreement with ArQule. If it were to do so, the Company might not be able to complete development and commercialization of the applicable licensed products on its own in Greater China. 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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