



ArQule to Present Clinical Data for its BTK Inhibitor, ARQ 531, at the 2018 American Society of Hematology (ASH) Annual Meeting

November 19, 2018

Presentation highlights clinical data in patients with relapsed or refractory hematologic malignancies

BURLINGTON, Mass.--(BUSINESS WIRE)--Nov. 19, 2018-- ArQule, Inc. (Nasdaq:ARQL) today announced that it will present clinical data on the company's BTK inhibitor, ARQ 531, in a poster presentation at the 60th American Society of Hematology (ASH) Annual Meeting and Exposition to be held from December 1 to 4, 2018 in San Diego.

Updated safety, PK, biomarker and anti-tumor activity data from the company's Phase 1 dose escalation study in patients with relapsed or refractory hematologic malignancies (ARQ 531-101) will be presented.

Presentation Details

Title: [A Phase 1 Dose Escalation Study of ARQ 531 in Selected Patients with Relapsed or Refractory Hematologic Malignancies](#)

Abstract #: 3136

Session: CLL: Therapy, excluding Transplantation: Poster II

Date: Sunday, December 2, 2018

Time: 6:00-8:00 p.m. PT

Location: San Diego Convention Center, Hall GH

About BTK and ARQ 531

Bruton's tyrosine kinase, BTK, is a therapeutic target that has been clinically proven to inhibit B-cell receptor signaling in blood cancers. ARQ 531 is an orally bioavailable, potent and reversible 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 resistance mechanism for first generation irreversible BTK inhibitors. In preclinical studies, ARQ 531 has demonstrated good oral bioavailability as well as favorable pharmacokinetic, pharmacodynamic and metabolic properties.

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 pipeline includes: ARQ 531, an 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; 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), and in Phase 1b in combination with the hormonal therapy, anastrozole, in patients with advanced endometrial cancer; ARQ 751, a next generation AKT inhibitor, in Phase 1 for patients with AKT1 and PI3K mutations; Derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA; 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. ArQule's current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Company's proprietary library of compounds.

Forward-Looking Statements

This press release contains forward-looking statements, including without limitation those regarding the current clinical trial with ARQ 531. 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 from those set forth in this press release. Positive information about early stage clinical trial results does not ensure that later stage or larger scale clinical trials will be successful. For example, ARQ 531 may not demonstrate promising therapeutic effect; in addition, it may not demonstrate an appropriate safety profile 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 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 expect to utilize diagnostic tools in ongoing and future biomarker-guided clinical trials with ARQ 531. 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 us to develop or obtain regulatory approval of companion diagnostics could delay or prevent approval of our product candidates. 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. For more detailed information on the risks and uncertainties associated with the Company's drug development, financial condition 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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