ArQule Announces Release of American Society of Hematology Abstract Detailing Results of the Ongoing Phase 1 Study of Reversible BTK Inhibitor, ARQ 531, in Patients with Relapsed/Refractory Hematologic Malignancies

November 6, 2019

- *ARQ 531 demonstrates substantial anti-tumor activity in refractory CLL patients and manageable safety profile*
- *A total of 10 patients experienced partial responses (PRs) as of July 19th, the cutoff date for data included in the abstract, primarily at the higher doses: 7 with CLL/SLL, 1 with FL, 1 with Richter's Transformation and 1 with DLBCL*

BURLINGTON, Mass.--(BUSINESS WIRE)--Nov. 6, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced the publication of the abstract highlighting data, as of July 19, 2019, from the phase 1 trial of ARQ 531, the company's potent and reversible dual inhibitor of both wild type and C481-mutant Bruton's tyrosine kinase (BTK), in patients with relapsed or refractory B-cell malignancies on the American Society of Hematology (ASH) website (link here). A poster containing the final data set from the phase 1 portion of this study will be presented at the ASH annual meeting in Orlando, FL on December 9, 2019 and will detail additional data with respect to ARQ 531's safety profile, clinical activity and durability across multiple refractory B-Cell malignancies, including C481-mutant chronic lymphocytic leukemia (CLL).

Dr. Brian Schwartz, Chief Medical Officer of ArQule, commented, "ARQ 531 continues to demonstrate profound effects at well-tolerated doses in a highly refractory patient population. Data on clinical activity, in CLL in particular, has improved further since our last presentation at EHA in June, and I'm looking forward to presenting important durability data for these patients at ASH. In addition, the unique kinase inhibition profile and favorable molecular properties of ARQ 531 are proving to be valuable in other, hard-to-treat B-cell malignancies, such as Richter's Transformation."

The reported data are from the ongoing phase 1, open label, single arm dose escalation 3+3 study and include data from the first eight cohorts (n=40) at dose levels of 5, 10, 15, 20, 30, 45, 65 and 75 mg once a day in patients with relapsed or refractory (R/R) CLL, small lymphocytic leukemia (SLL), Richter's Transformation and other B-cell Non-Hodgkin lymphomas.

Key findings of the abstract include:

- ARQ 531 continues to be well-tolerated through 65 mg QD and has a manageable safety profile in multiple B-cell malignancies
- Pharmacokinetic (PK) data show that patients receiving 65 mg QD of ARQ 531 exhibited steady-state mean C_{min} of above 1 µM, with complete pBTK inhibition
- Robust, dose-dependent, anti-tumor activity was observed, including 10 PRs, especially at the higher doses
    - Of the 6 evaluable patients recruited in cohort 7 with R/R CLL/SLL and dosed initially at 65 mg QD, 5 experienced a PR as of July 19, 2019
    - Two additional R/R CLL patients experienced a PR: 1 patient dose escalated from 45 to 65 mg QD and another de-escalated from 75 to 65 mg QD
    - Three additional PRs were observed outside of CLL including 1 patient with Follicular Lymphoma, 1 with Richter's Transformation and 1 with Diffuse Large B-Cell Lymphoma

Presentation Details
Title: Final Results of Phase 1, Dose Escalation Study Evaluating ARQ 531 in Patients with Relapsed or Refractory B-Cell Lymphoid Malignancies
Abstract #: 4298
Session Name: CLL: Therapy, excluding Transplantation: Poster III
Date: Monday, December 9, 2019
Presentation Time: 6:00 PM - 8:00 PM ET
Location: Orange County Convention Center, Hall B

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 dual inhibitor of both wild type and C481S-mutant BTK. The C481S-mutation is a known resistance mechanism for first generation irreversible BTK inhibitors. ARQ 531 has demonstrated a manageable safety profile, predictable PK, profound pharmacodynamic effects and emerging signs of dose-proportional clinical activity in phase 1 clinical testing.

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 four 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 dual inhibitor of both wild type and C481S-mutant BTK, in phase 1/2 for patients with B-cell malignancies refractory to other therapeutic options; miransertib (ARQ 092), a potent and selective inhibitor of the AKT serine/threonine kinase, in a registrational trial with cohorts in

Proteus syndrome and PROS; ARQ 751, a next generation highly potent and selective AKT inhibitor, in phase 1 for patients with solid tumors with AKT1 and PI3K mutations; and derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA in collaboration with Basilea and Sinovant. 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 ongoing clinical trial with ARQ 531 and the additional data to be presented at the ASH annual meeting. 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 adequate 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 current or 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 or intellectual property, 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 diagnostic could delay or prevent approval of ARQ 531. Only a small number of research and development programs result in the commercialization of a product. Furthermore, the Company 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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