Basilea Announces Positive Interim Results from Registrational Phase 2 Study with Oncology Drug Candidate Derazantinib in Intrahepatic Cholangiocarcinoma (iCCA)

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- **21% objective response rate with six confirmed partial responses from 29 evaluable patients**
- **83% disease control rate**
- **Safety profile and tolerability of continuous dosing schedule confirmed**

BURLINGTON, Mass.--(BUSINESS WIRE)--Jan. 9, 2019-- ArQule, Inc.'s (Nasdaq: ARQL) partner, Basilea Pharmaceutica Ltd. (SIX: BSLN), today announced results from the interim analysis of the registrational Phase 2 study with the orally administered pan-fibroblast growth factor receptor (FGFR) kinase inhibitor derazantinib (BAL087). The analysis showed promising activity in patients with FGFR2 gene fusion-expressing intrahepatic cholangiocarcinoma (iCCA) and also confirmed the safety profile and tolerability of the drug candidate observed in previous clinical studies.

The interim analysis in the ongoing registrational Phase 2 study was conducted after 42 patients had been enrolled in the study, with a subset of 29 evaluable patients who had at least one post-baseline imaging assessment. The objective response rate (ORR) in the 29 evaluable patients was 21%. The disease control rate (DCR), reflecting the proportion of patients with a partial response or with stable disease, was 83%. The safety data obtained from all 42 patients enrolled to date was consistent with the results from previous clinical studies with derazantinib.

http://www.basilea.com/Investor-Relations/News-and-Media/

Peter Lawrence, President and Chief Operating Officer of ArQule, said, "We are pleased with derazantinib's continued progress since it was licensed to Basilea Pharmaceutica in April 2018 in the US, EU, Japan and rest of world excluding Greater China. Under the terms of the license agreement, ArQule is eligible to receive up to $326 million in regulatory and commercial milestone payments, and we look forward to further progress and updates from Basilea."

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.

About derazantinib
Derazantinib (BAL087, formerly ARQ 087) is an investigational orally administered small molecule inhibitor of the FGFR family of kinases with strong activity against FGFR 1, 2, and 3. Therefore, it is called a pan-FGFR kinase inhibitor. FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR alterations, e.g., gene fusions, overexpression or mutations, have been identified as potentially important therapeutic targets for various cancers, including iCCA, bladder, breast, gastric and lung cancers.[2] Current scientific literature suggests that FGFR alterations exist in a range of 5% to 30% in these cancers.[3] In iCCA, FGFR2 gene fusions have been reported in 13-22% of the cases[4, 5] and FGFR gene mutations have been reported in up to 5% of the cases.[3] Basilea in-licensed derazantinib from ArQule Inc. in April 2018. The drug candidate has demonstrated favorable clinical data in previous clinical studies, including a biomarker-driven Phase 1/2 study in iCCA patients.[6] Derazantinib has U.S. and EU orphan drug designation for this disease.

About intrahepatic cholangiocarcinoma (iCCA)
Intrahepatic cholangiocarcinoma (iCCA) is a cancer originating from the biliary system. The age-adjusted incidence rate of iCCA in the United States has been increasing over the past decade and is currently estimated to be approximately 1.2 per 100,000.[7] Patients are often diagnosed with advanced or metastatic disease that cannot be surgically removed. Current first-line standard of care is the chemotherapy combination of gemcitabine and platinum-derived agents. The prognosis for patients with advanced disease is poor, with a median survival of less than one year. There is no proven effective treatment for patients who progress on first-line chemotherapy, thus there is a high unmet medical need.[8]

About Basilea
Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company focused on the development of products that address the medical challenge in the therapeutic areas of oncology and anti-infectives. With two commercialized drugs, the company is committed to discovering, developing and commercializing innovative pharmaceutical products to meet the medical needs of patients with serious and life-threatening conditions. Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Additional

information can be found at Basilea's website www.basilea.com.

Forward Looking Statements

This press release contains forward-looking statements regarding clinical trials with derazantinib as well as the potential for future milestone and royalty payments under the Company's exclusive license agreement with Basilea. 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, derazantinib may not demonstrate promising therapeutic effect. In addition, derazantinib may not demonstrate an acceptable 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 during clinical trials or in the course of developing, testing or manufacturing derazantinib that could lead the Company or Basilea to discontinue its 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 Basilea's view of the data or require additional data or information or additional studies. In addition, we or Basilea plan to develop and use a companion diagnostic to identify patients with FGFR2 fusions and possibly other fusions for our future derazantinib clinical trials. We or Basilea intend to outsource the development of such companion diagnostics to one or more third party collaborators. Such collaborators may encounter difficulties in developing and obtaining approval for such companion diagnostics, including issues relating to selectivity/specificity, analytical validation, reproducibility, concordance or clinical validation. Any delay or failure to develop or obtain regulatory approval of such companion diagnostics could delay or prevent approval of derazantinib.Moreover, Basilea has only a limited track record of drug development in oncology. If derazantinib is not successfully developed and as a result of any of the foregoing or other issues, risks or uncertainties, ArQule may not receive any future milestones or royalties under the license agreement with Basilea.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. 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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1 ClinicalTrials.gov identifier: NCT03230318

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Source: ArQule, Inc.

Corporate Contact:
Marc Schegerin, M.D.
Senior Vice President
Head of Strategy, Finance and Communication
ir@arqule.com

Media Contact:
Allison Blum, Ph.D.
LifeSci Public Relations
(646) 627-8383
Allison@lifescipublicrelations.com

www.ArQule.com