ArQule Announces Oral Presentation for its Pan-AKT Inhibitor, Miransertib, at the 2019 European Society of Human Genetics (ESHG) Conference

June 10, 2019

Presentation highlights preliminary results from phase 1/2 study of miransertib in patients with PIK3CA-related Overgrowth Spectrum (PROS) and Proteus Syndrome (PS)

BURLINGTON, Mass.--(BUSINESS WIRE)--Jun. 10, 2019-- ArQule, Inc. (Nasdaq:ARQL), today announced it will present preliminary results from the company's phase 1/2 study of its pan-AKT inhibitor, miransertib (ARQ 092), in patients with PIK3CA-related Overgrowth Spectrum (PROS) and Proteus syndrome (PS) in an oral presentation at the European Society of Human Genetics Conference held from June 15-18, 2019 in Gothenburg, Sweden.

Presentation Details:

Title:	An open-label, phase 1/2 study of miransertib (ARQ 092), an oral pan-AKT inhibitor, in patients (pts) with PIK3CA-related Overgrowth Spectrum (PROS) and Proteus Syndrome (PS): study design and preliminary results (NCT03094832)
Presentation #:	C17.6
Session:	C18 - Therapies
Date:	Monday, June 17, 2019
Time:	2:15-2:30 p.m. CEST
Location:	F1+F2+F3

About Miransertib
Miransertib (ARQ 092) is an orally available, selective, pan-AKT (protein kinase B) inhibitor that potently inhibits AKT 1, 2 and 3 isoforms. Additionally, it binds both the active and inactive forms of AKT which directly inhibits and prevents membrane localization, respectively. Dysregulation of AKT has been implicated in a variety of rare overgrowth diseases and cancers; however, there are currently no approved inhibitors of AKT. AKT inhibitors, either as single agent or combination therapy, show significant promise in molecularly defined patient populations. We are in process of initiating a registrational trial in both Proteus syndrome and PIK3CA-Related Overgrowth Spectrum (PROS). Miransertib has been granted Rare Pediatric Disease Designation and Fast Track Designation by the U.S. Food and Drug Administration (FDA), as well as Orphan Designation by the FDA and European Medicines Agency in the rare overgrowth disease, Proteus syndrome.

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 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, planned to initiate registrational trial cohorts in Proteus syndrome and PROS in 2019, and in phase 1b in combination with the hormonal therapy, anastrozole, in patients with advanced endometrial cancer; ARQ 751, a next generation highly potent and selective AKT inhibitor, in phase 1 for patients 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.

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