



ArQule to Present Preclinical Data on Miransertib for the Treatment of Vascular Malformations at the 2019 American Society of Human Genetics (ASHG) Annual Meeting

October 8, 2019

BURLINGTON, Mass.--(BUSINESS WIRE)--Oct. 8, 2019-- ArQule, Inc. (Nasdaq: ARQL) today announced that it will be presenting research on the company's pan-AKT inhibitor, miransertib (ARQ 092), in a poster presentation at the 2019 ASHG Annual Meeting being held from October 15 to October 19, 2019 in Houston, Texas.

The presentation will detail results from a preclinical study of miransertib demonstrating its potential for treating *PIK3CA*-driven vascular malformations.

Details on the presentation are as follows:

Presentation Title: PgmNr 2702/F: Miransertib (ARQ 092) prevents the formation of PIK3CAH1047R-driven vascular malformations in mice

Abstract Number: 1920179

Presenter: Piotr Kobialka, MS, Institut d'Investigacio Biomedica de Bellvitge

Date: October 18, 2019

Poster Viewing Time: 2:00 p.m.- 3:00 p.m. CDT

Location: Exhibit Hall-Level 1/ George R. Brown Convention Center

Additional details can be found on the [meeting website](#). A copy of the presentation materials can be accessed by visiting the [Publications & Presentations](#) section of the ArQule website after the presentation concludes.

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 and 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 a single agent or in combination therapy, show significant promise in molecularly defined patient populations. Miransertib has been granted Rare Pediatric Disease Designation for Proteus syndrome by the U.S. Food and Drug Administration (FDA) as well as Orphan Drug Designation by both the FDA and European Medicines Agency. Fast Track Designation has been granted by the FDA for PROS.

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.

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