ArQule Announces First Patient Dosed in Registrational MOSIAC Trial of Miransertib for the Treatment of Proteus Syndrome and PIK3CA-related Overgrowth Spectrum

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MOSAIC trial represents the first registrational trial for Proteus Syndrome and PIK3CA-related overgrowth spectrum

BURLINGTON, Mass.--(BUSINESS WIRE)--Oct. 2, 2019--

ArQule, Inc. (Nasdaq: ARQL), today announced that the first patient was dosed in the registrational MOSAIC (Miransertib in Overgrowth Syndromes in Adults and Children) trial of its oral, selective pan-AKT inhibitor, miransertib, for the treatment of Proteus syndrome (PS) and PIK3CA-related Overgrowth Spectrum disorders (PROS). The MOSAIC trial represents the first registrational trial of its kind for patients suffering from these rare genetic conditions characterized by mutations in the PI3K/AKT pathway that emerge in childhood and early adolescence.

"After years of extensive preclinical and clinical efforts among ArQule and our collaborators, the MOSAIC trial represents an important milestone for patients with Proteus syndrome and PROS, their families and caregivers," said Brian Schwartz, Chief Medical Officer of ArQule. "These patients currently have extremely limited therapeutic options and no approved or effective drug for their treatment. We are pleased that the FDA has granted miransertib Fast Track Designation for PROS and Rare Pediatric Disease Designation for Proteus syndrome."

"PS and PROS are chronic, progressive and debilitating conditions affecting both children and adults. At Texas Children's Hospital (TCH) Vascular Anomalies Center we are very excited and hopeful that miransertib may help our patients," said Dr. Ionela Iacobas, medical director of the Vascular Anomalies Center at Texas Children's Hospital and assistant professor of pediatrics – hematology and oncology at Baylor College of Medicine. "With the current research advances in the vascular anomalies field, we have been able to provide early diagnosis for syndromes caused by genetic abnormalities. Still, until now there was no open clinical trial specifically targeted for PS and PROS. We have made a commitment to our patients to offer comprehensive multidisciplinary medical and surgical care and to bring them both state-of-the-art standard-of-care as well as the newest promising experimental therapies. Opening the MOSAIC study at TCH Vascular Anomalies Center is part of that commitment."

The MOSAIC trial is an international, multi-center, open-label study that will evaluate the objective response to miransertib in patients with PS and PROS. The study will enroll approximately 30-35 patients with documented somatic mutations in the AKT1 or PIK3CA genes in the registrational cohorts for PS and PROS. Thirty to 35 additional patients will be enrolled in 2 other cohorts for patients under compassionate use or those ineligible to enter the registrational cohorts.

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 Proteus syndrome

Proteus syndrome is an ultra-rare condition characterized by the aberrant overgrowth of multiple tissues of the body. Patients with Proteus syndrome experience changes in the shapes of certain body structures over time, including abnormal, often asymmetric, massive growth (overgrowth) of the skeleton, skin, adipose tissue and central nervous system out of proportion to the rest of the body. Although patients may have minimal or no manifestations at birth, the disease develops and becomes apparent in early childhood (6-18 months) and rapidly progresses with intense growth in the first 10 years of life. The worldwide incidence is believed to be approximately one in a million. There are currently no approved medicinal treatments for Proteus syndrome, leaving patients with minimal treatment options to manage the disease and a mortality of 25% by age 22.

About PROS

PROS is a term used to refer to a spectrum of rare diseases identified by somatic mutations in the PIK3CA gene, that result in excess growth in certain areas of the body. While the individual diseases that fall within the overgrowth spectrum have similar symptoms, each disease is defined by unique clinical characteristics. The implementation of genetic sequencing has led to the identification of the underlying genetic mutations that drive these overgrowth disorders, allowing for the development of medicines that target the specific causes of disease.

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 statements regarding the MOSAIC registrational study in Proteus syndrome and PROS. 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, miransertib 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. 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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