Sinovant Sciences Announces Approval of Derazantinibâ€™s Clinical Trial Application by the China National Medical Products Administration

Chinese registrational clinical trial for derazantinib to begin in 2H 2019

ArQule, Inc.â€™s (Nasdaq: ARQL) partner, Sinovant Sciences, announced that its Clinical Trial Application (CTA) for derazantinib has been accepted by the Center for Drug Evaluation at the China National Medical Products Administration (NMPA), enabling the initiation of a registrational clinical trial in patients with second-line intrahepatic cholangiocarcinoma (iCCA) in the second half of 2019.

â€œThis is an important step forward for derazantinib in China, where the unmet need in iCCA is particularly acute,â€• said Peter Lawrence, President and COO of ArQule. â€œWe are very pleased with the operational and regulatory progress our partner has made in this region and look forward to further clinical updates.â€•

To read the full Sinovant Sciences press release announcing the approval, click here.

In 2018, ArQule and Sinovant Sciences, entered into a licensing agreement for derazantinib in China. Under the terms of the agreement, ArQule granted Sinovant Sciences, a subsidiary of Roivant Sciences, an exclusive license to develop and commercialize derazantinib in the People's Republic of China, Hong Kong, Macau and Taiwan (greater China). In addition to the $3 million upfront payment and $2.5 million developmental milestone it has already received, ArQule is eligible for regulatory and commercial milestones and royalties on future sales of derazantinib in Greater China.

About ArQuleArQule is a biopharmaceutical company engaged in the research and development of targeted therapeutics to treat cancers and rare diseases. ArQuleâ€™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. ArQuleâ€™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. ArQuleâ€™s current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Companyâ€™s proprietary library of compounds.

About DerazantinibDerazantinib is a potent, orally administered inhibitor of the fibroblast growth factor receptor (FGFR) family, a key driver of cell proliferation, differentiation, and migration. In a phase 1/2 study in patients with iCCA harboring FGFR2 gene fusions, treatment with derazantinib resulted in an objective response rate of 21%, nearly 3 times higher than standard-of-care chemotherapy.

About Intrahepatic CholangiocarcinomaCholangiocarcinoma (CCA) is the most common biliary malignancy and the second most common malignancy in the liver after hepatocellular carcinoma (HCC).1 Depending on the anatomic location, CCA is classified as intrahepatic (iCCA), perihilar (pCCA), and extrahepatic (eCCA). iCCA originates from the intrahepatic biliary ductal system and forms an intrahepatic mass. iCCA is an aggressive cancer, with a median 5-year survival rate of only 15% for patients diagnosed with early-stage disease.2 Reports show that in China's most populous cities the incidence of cholangiocarcinoma is more than 7 cases per 100,000 people, and a majority of cases are intrahepatic.3

About SinovantSinovant is a Chinese biopharmaceutical company dedicated to conducting globally innovative biomedical R&D in China to meet the needs of patients in Greater China and around the world. Sinovant's mission is to develop and commercialize new medicines that address the most pressing public health challenges in China while simultaneously advancing Chinese biopharmaceutical research abroad. For further information, please visit www.sinovant.com.

Forward Looking StatementsThis press release contains forward-looking statements regarding clinical trials with derazantinib under the Companyâ€™s license agreement with Sinovant Sciences. These statements are based on the Companyâ€™s current beliefs and expectations and are subject to risks and uncertainties that could cause actual results to differ materially. Positive information from early stage clinical trials does not ensure that later stage or larger scale clinical trials will be successful. The results achieved in later stage trials may not be sufficient to meet applicable regulatory standards or to justify further development. 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. Problems or delays may arise during clinical trials or in the course of developing, testing or manufacturing derazantinib that could lead the Company or Sinovant Sciences to delay or discontinue its development. Even if later stage clinical trials are successful, unexpected concerns may arise from subsequent analysis of data or from additional data. Regulatory authorities may disagree with the Companyâ€™s or Sinovant Sciencesâ€™ view of the data or require additional data or information or additional studies. If derazantinib is not successfully developed and as a result of any of the foregoing or other issues, risks or uncertainties,Â the CompanyÂ may not receive future milestones or royalties under the license agreement. For more detailed information on the risks and uncertainties associated with the Companyâ€™s drug development and other activities, see the Companyâ€™s periodic reports filed with theÂ Securities and Exchange Commission. The Company does not undertake any obligation to publicly update any forward-looking statements.

1 Welzel TM, et al. Impact of classification of hilar cholangiocarcinomas (Klatskin tumors) on the incidence of intra- and extrahepatic cholangiocarcinoma in the United States. Journal of the National Cancer Institute 2006; 98(12), 873-875.2American Cancer Society3 Banales JM, et al. Cholangiocarcinoma: current knowledge and future perspectives consensus statement from the European Network for the Study of Cholangiocarcinoma (ENS-CCA). Nature Reviews: Gastroenterology & Hepatology 2016; 13, 261-280.

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