ArQule and Basilea Enter into Exclusive License Agreement for Derazantinib in the US, EU, Japan and Rest of World Excluding Greater China

April 17, 2018

ArQule eligible to receive up to $336 million including upfront, regulatory and commercial milestone payments

ArQule to host investor conference call including transaction details, AACR highlights and an update on clinical strategy April 18, 2018 at 9:00 A.M. ET

BURLINGTON, Mass.--(BUSINESS WIRE)--Apr. 17, 2018-- ArQule, Inc. (NASDAQ:ARQL) today announced that it has entered into an exclusive license agreement with Basilea Pharmaceutica International Limited(Basilea, SIX: BSLN) to develop and commercialize derazantinib, a pan-FGFR (fibroblast growth factor receptor) inhibitor in the US, EU, Japan and rest of the world excluding the People's Republic of China, Hong Kong, Macau and Taiwan, where Sinovant Sciences Ltd., a Roivant Sciences Ltd. subsidiary, has rights to develop and exclusively commercialize the drug.

Under the terms of the agreement, ArQule will receive an upfront payment of $10 million and is eligible for up to $326 million in regulatory and commercial milestones. ArQule is also entitled to receive staggered single-digit to double-digit royalties on net sales upon commercialization. Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory. Under certain circumstances, ArQule may have the opportunity to promote derazantinib in the US directly.

ArQule is currently conducting a registrational trial for derazantinib in the United States, Canada and Europe as a potential treatment for intrahepatic cholangiocarcinoma (iCCA), a form of biliary tract cancer. As part of the exclusive license agreement, Basilea intends to continue this trial and the further development of derazantinib in iCCA and other tumor types with FGFR dysregulation.

Ronald Scott, Chief Executive Officer of Basilea, said: "We are very excited about this partnership with ArQule. Derazantinib is an ideal match for our existing clinical oncology portfolio. It is a targeted therapy building on a solid biomarker approach in an area where patients currently have limited treatment options. This transaction underscores our continued commitment to expand our R&D portfolio with novel compounds focused on overcoming the clinical problem of resistance in oncology and infectious diseases. Our clinical oncology portfolio now includes three drug candidates in different stages of development. We continue to focus on further broadening our R&D portfolio through internal and external innovation."

"Partnering with Basilea, a company with global drug development experience and expertise, will propel the advancement of derazantinib in ways we could not have achieved independently," said Paolo Pucci, Chief Executive Officer of ArQule. "Basilea will bring a wealth of skills to the expansion of the derazantinib development plan at a time when it will benefit most from these resources, allowing it to reach its full potential in iCCA and beyond."

ArQule will hold a conference call to discuss this agreement tomorrow, April 18, beginning at 9 a.m. EDT. Paolo Pucci, Chief Executive Officer of ArQule, will lead the call. As a result of entering into the exclusive license agreement, ArQule will be updating its financial guidance on the call.

The details of the call are as follows:

Wednesday, April 18, 2018 at 9:00 AM EDT

Audio connection numbers:

US: 1 877-868-1831

Outside US: 1 914-495-8595 PIN: 4089669

A replay of the call will be available two hours after the completion of the call and can be accessed in the "Investors and Media" section of our website, www.arqule.com, under "Events and Presentations." The ArQule investor conference call will be archived and can be accessed in the "Investors and Media" section of ArQule's website, www.arqule.com, under "Events and Presentations."

About Derazantinib

Derazantinib 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. ArQule is currently conducting a registrational study with derazantinib in patients with FGFR2 fusion-positive second-line iCCA. The open-label single-arm trial is recruiting in the United States, Canada and Europe with objective response rate as the primary endpoint. More information on that program is available here.

About Intrahepatic Cholangiocarcinoma

Cholangiocarcinoma (CCA) is the most common biliary malignancy and the second most common hepatic malignancy 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 15% for patients diagnosed with early-stage disease.[2] In China, the incidence of cholangiocarcinoma is more than 7 cases per 100,000 people, and the majority of cases are intrahepatic.[3]

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 proprietary pipeline includes: Derazantinib, a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family, in a registrational trial for iCCA and in phase 1b for multiple oncology indications; 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), as well as in multiple oncology indications; ARQ 751, a next generation AKT inhibitor, in phase 1 for patients with AKT1 and PI3K mutations; 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. In addition, we have advanced ARQ 531, an investigational, 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. ArQule's current discovery efforts are focused on the identification and development of novel kinase inhibitors, leveraging the Company's proprietary library of compounds. You can follow us on Twitter and LinkedIn.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company developing products that address the medical challenge of increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. 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 the Company's clinical trials with derazantinib as well as the potential for future milestone and royalty payments under its 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 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 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.

[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.

[2]American Cancer Society

[3] 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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