

PRESS RELEASE

Basilea starts phase 1/2 study with derazantinib in urothelial cancer

Basel, Switzerland, August 13, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it has initiated the phase 1/2 FIDES-02 study with the pan-fibroblast growth factor receptor (FGFR) kinase inhibitor derazantinib. The study is evaluating derazantinib alone and in combination with Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab (Tecentriq®) in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR gene aberrations.¹ Urothelial cancer is the sixth most common cancer in the U.S.²

Dr. Marc Engelhardt, Chief Medical Officer, said: "We are very pleased starting this new study with derazantinib in urothelial cancer. Patients with advanced urothelial cancer currently have limited treatment options and there is a high unmet medical need, especially for targeted therapies in this common type of cancer. FGFR gene aberrations occur in about 15 to 20 percent of advanced urothelial cancers and have been established as oncogenic drivers." He added: "This is the first clinical study in which we are exploring derazantinib in combination with atezolizumab. This combination may become a promising new targeted treatment approach for patients with urothelial cancer. Based on *in vitro* data, derazantinib has the potential to enhance the response to atezolizumab's PD-L1 checkpoint inhibition."

The FIDES-02 (**F**ibroblast growth factor **I**nhibition with **D**erazantinib in **S**olid tumors) study is a multi-cohort, open-label phase 1/2 study with the goal to explore Derazantinib as single agent and in combination with atezolizumab in patients with advanced urothelial cancer testing positive for mutations or fusions of FGFR1, FGFR2 or FGFR3 genes. Across a total of four substudies, FIDES-02 potentially can enroll up to approximately 300 patients. The study will be conducted in multiple centers in Asia-Pacific, Europe and North America.

Basilea is the sponsor of the study and Roche will provide clinical supply of atezolizumab for the study.

About derazantinib

Derazantinib (formerly ARQ 087) is an investigational orally administered small molecule panFGFR kinase inhibitor with strong activity against FGFR1, 2, and 3. FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR gene alterations, e.g. gene fusions, amplifications or mutations, have been identified as potentially important therapeutic targets for various cancers, including intrahepatic cholangiocarcinoma (iCCA), urothelial, breast, gastric and lung cancers.³ In these cancers, FGFR gene alterations are found in a range of 5% to 30%.⁴ Derazantinib also inhibits the colony-stimulating-factor-1-receptor kinase (CSF1R).⁵ CSF1R-mediated signaling is important for the maintenance of tumor-promoting macrophages and therefore has been identified as a potential target for anti-cancer drugs.⁴ Pre-clinical data has shown that tumor macrophage depletion through CSF1R blockade renders tumors more responsive to T-cell checkpoint immunotherapy, including approaches targeting PD-L1/PD-1.^{6, 7, 8} Basilea in-licensed derazantinib from ArQule Inc. in April 2018. Derazantinib has demonstrated antitumor activity and a manageable safety profile in previous clinical studies, including a biomarker-driven phase 1/2 study in iCCA patients,⁹ and has received U.S. and EU orphan drug designation for iCCA.

About urothelial cancer

These cancers start in the urothelial cells that line the inside of the bladder. 80,000 new cases of bladder cancer have been estimated in the U.S. for 2017. Up to 20% of patients will have muscle-invasive disease and present with or will later develop metastases.² FGFR gene aberrations occur in about 15-20% of advanced urothelial cancers.^{10, 11} For patients with advanced urothelial cancer, outcomes can be poor due to the often rapid progression of the tumor and the lack of efficacious treatments, especially in relapsed or refractory disease.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company, focused on the development of products that address the medical challenges in the therapeutic areas of oncology and anti-infectives. 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.

Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

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This press release can be downloaded from www.basilea.com.

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