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Press release

Basilea presents preclinical data on anti-angiogenic activity of derazantinib at ENA 2020

Basel, Switzerland, October 26, 2020

Basilea Pharmaceutica Ltd. (SIX: BSLN) today reported that data on the anti-angiogenic activity of the fibroblast growth factor receptor (FGFR) inhibitor derazantinib were presented at the 32nd EORTC-NCI-AACR (ENA) Symposium on Molecular Targets and Cancer Therapeutics, that took place as a virtual event on 24-25 October, 2020. In addition to FGFR1-3 derazantinib also inhibits the vascular endothelial growth factor receptor 2 (VEGFR2). The presented data from several preclinical models demonstrate that derazantinib has an anti-angiogenic effect, which may contribute to its overall anti-tumor activity in FGFR-driven cancers.

The prevention of new blood vessel formation (anti-angiogenesis) is an established approach in cancer therapy as it deprives the growing tumor from oxygen and nutrients. VEGFR2 is a primary target for anti-angiogenic agents in the treatment of cancers.

Dr. Laurenz Kellenberger, Chief Scientific Officer, said: “Our development strategy for derazantinib is focused on strengthening the evidence for its differentiation versus other FGFR inhibitors. The preclinical data on derazantinib’s anti-angiogenic activity presented at the conference show that it may provide additional activity on top of its established primary anti-tumor effects in FGFR-positive solid tumors. Based on its unique kinase inhibition profile, we are exploring derazantinib’s potential for enhanced activity alone and in combination with other anti-cancer agents such as the anti-VEGFR2 antibody ramucirumab, or the PD-L1 immune checkpoint inhibitor atezolizumab within our ongoing clinical program FIDES.“

The following e-poster was presented at the EORTC-NCI-AACR Virtual Symposium 2020:

Presentation #	Authors/title
101	P. McSheehy, J. Boult, S. Robinson, F. Bachmann, M. El-Shemerly, L. Kellenberger, H. Lane Derazantinib, an oral fibroblast growth factor receptor inhibitor, in phase-2 clinical development, shows anti-angiogenic activity in preclinical models

For further information, please visit https://event.eortc.org/ena2020

About derazantinib

Derazantinib is an investigational orally administered small-molecule FGFR inhibitor with strong activity against FGFR1, 2, and 3.[1] FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR genetic aberrations, e.g. gene fusions, mutations or amplifications, have been identified as potentially important therapeutic targets for various cancers, including intrahepatic cholangiocarcinoma (iCCA), urothelial, breast, gastric and lung cancers.[2] In these cancers, FGFR genetic aberrations are found in a range of 5% to 30%.[3] Derazantinib also inhibits the colony-stimulating-factor-1-receptor (CSF1R) kinase.[1, 4] 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.[5] Preclinical data has shown that tumor macrophage depletion through CSF1R blockade renders tumors more responsive to T-cell checkpoint immunotherapy, including approaches targeting PD-1/PD-L1.[6, 7] Derazantinib has demonstrated antitumor activity and a manageable safety profile in a previous biomarker-driven phase 1/2 study in iCCA patients,[8] and has received U.S. and EU orphan drug designation for iCCA. Basilea is currently conducting three clinical studies with derazantinib. The first study, FIDES-01, is a registrational phase 2 study in patients with inoperable or advanced iCCA. It comprises one cohort of patients with FGFR2 gene fusions and another cohort of patients with mutations or amplifications.[9] The second study, FIDES-02, is a phase 1/2 study evaluating derazantinib alone and in combination with Roche's PD-L1-blocking immune-checkpoint inhibitor, atezolizumab, in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR genetic aberrations.[10] The third study, FIDES-03, is a phase 1/2 study evaluating derazantinib alone and in combination with Lilly’s anti-VEGFR2 antibody ramucirumab and paclitaxel or with Roche’s PD-L1 checkpoint inhibitor, atezolizumab, in patients with advanced gastric cancer with FGFR genetic aberrations. Basilea in-licensed derazantinib from ArQule Inc., a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.

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 infectious diseases. 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.

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

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