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Press release

Basilea announces data presentations at ESMO Virtual Congress 2020

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Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that three abstracts featuring preclinical and clinical data on its fibroblast growth factor receptor (FGFR) kinase inhibitor derazantinib and its tumor checkpoint controller lisavanbulin are going to be presented as e-posters at the European Society for Medical Oncology (ESMO) Virtual Congress 2020, which is taking place from 19-21 September, 2020.

Dr. Marc Engelhardt, Chief Medical Officer, said: “The preclinical derazantinib data may provide an explanation for differences in reported adverse event profiles between various FGFR kinase inhibitors. Furthermore, derazantinib shows promising efficacy in patient-derived tumor models with gene aberrations in FGFR1-3. The data which will be presented at ESMO are part of our broad preclinical and clinical program focused on generating further scientific evidence for the differentiation of derazantinib, in order to explore its full therapeutic potential in FGFR-driven tumors.”

He continued: “We are also pleased that the full data from the phase 1 study with oral lisavanbulin in patients with recurrent glioblastoma will be presented at ESMO. As previously reported, we observed clinical benefit in a subset of patients in the study. In particular, one patient shows an exceptional long-lasting response and the brain tumor tissue of this patient is strongly positive for EB1, a potential response-predictive biomarker. Based on the results from this and other clinical and preclinical studies, we are currently preparing the start of a biomarker-driven phase 2 study in patients with recurrent glioblastoma and subsequently potential additional tumor types.”

The following e-posters are scheduled for presentation at ESMO Virtual Congress 2020:

Presentation #	Title
1960P	Differential induction of gene expression may explain differences in reported adverse event profiles between the FGFR-inhibitors derazantinib and erdafitinib: an analysis in safety relevant normal tissues from urothelial cancer (UC) patient-derived mouse xenograft (PDX) models. Presenting author: Paul McSheehy

541P	Derazantinib (DZB), an oral Fibroblast Growth Factor Receptor inhibitor (FGFRi), shows promising activity in PDX-tumor models with aberrations in FGFR1-3 Presenting author: Paul McSheehy
382P	The potential utility of end-binding protein 1 (EB1) as response-predictive biomarker for lisavanbulin: Final results from a phase 1 study of lisavanbulin (BAL101553) in adult patients with recurrent glioblastoma (GBM) Presenting author: Crescens Tiu

For further information, please visit https://www.esmo.org/meetings/esmo-virtual-congress-2020

About derazantinib

Derazantinib is an investigational orally administered small-molecule FGFR kinase 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 kinase (CSF1R).[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] 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] 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,[8] and has received U.S. and EU orphan drug designation for iCCA. Basilea is currently conducting two 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 (Tecentriq®)[10] in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR genetic aberrations.[11]
Basilea in-licensed derazantinib from ArQule Inc, a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin (BAL101553, the prodrug of BAL27862)[12] is being developed as a potential therapy for diverse cancers.[13, 14, 15] In preclinical studies, lisavanbulin demonstrated in-vitro and in-vivo activity against diverse treatment-resistant cancer

models, including tumors refractory to conventional approved therapeutics and radiotherapy.[16, 17, 18] Lisavanbulin efficiently distributes to the brain, with anticancer activity in glioblastoma models.[19, 20, 21] In preclinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models.[21] The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,[22] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[23]

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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