

Press release

Basilea announces U.S. FDA Orphan Drug Designation granted to lisavanbulin for the treatment of malignant glioma

Basel, Switzerland, July 29, 2021

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to Basilea's tumor checkpoint controller, lisavanbulin, for the treatment of malignant glioma (brain cancer). This includes glioblastoma, the most common type of primary brain cancer and one of the most lethal types of cancer.¹ Orphan Drug Designation qualifies the sponsor of the drug for various incentives, including longer regulatory market exclusivity.

Dr. Marc Engelhardt, Chief Medical Officer, commented: "The Orphan Drug Designation of lisavanbulin by the U.S. FDA is an important milestone for the development of lisavanbulin. Glioblastoma is associated with a poor prognosis and there are only very limited therapeutic options available. Lisavanbulin, as a targeted treatment, could be a useful new approach to expand the treatment options for patients with this devastating disease."

Basilea is currently conducting a phase 1/2 study in patients with recurrent glioblastoma, using end-binding protein 1 (EB1) for patient selection.² In the previously reported phase 1 part of the study, long-lasting clinical benefit was observed in two patients with recurrent glioblastoma whose tumor tissues showed EB1-positive staining.³ Interim results from the phase 2 part of the study are expected in the second half of 2021.

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin (BAL101553, the prodrug of BAL27862)⁴ is currently being developed as a potential therapy for glioblastoma.^{2, 5, 6} 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.^{7, 8, 9}

Lisavanbulin efficiently distributes to the brain, with anticancer activity in glioblastoma models.^{10, 11} In preclinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models and strong EB1-positivity was shown in about 5% of tissue samples from glioblastoma patients.^{12, 13} The strongest expression of EB1 in non-glioblastoma tumors was detected in tissue samples from medulloblastomas and neuroblastomas, which are cancers that occur predominantly in the pediatric population. EB1-positive staining was also found in tissue samples from metastatic melanoma (skin

cancer). Other tumors expressing slightly lower levels of EB1 staining include non-small cell lung cancer, colorectal cancer and triple-negative breast cancer.¹³ The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,¹⁴ resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.¹⁵

About Basilea

Basilea is a commercial-stage biopharmaceutical company founded in 2000 and headquartered in Switzerland. We are committed to discovering, developing and commercializing innovative drugs to meet the medical needs of patients with cancer and infectious diseases. We have successfully launched two hospital brands, Cresemba for the treatment of invasive fungal infections and Zevtera for the treatment of severe bacterial infections. We are conducting clinical studies with two targeted drug candidates for the treatment of a range of cancers and have a number of preclinical assets in both cancer and infectious diseases in our portfolio. Basilea is listed on the SIX Swiss Exchange (SIX: BSLN). Please visit basilea.com.

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

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