

Press release

Basilea initiates targeted biomarker-driven phase 2 study with lisavanbulin in patients with brain cancer

Basel, Switzerland, September 29, 2020

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it has initiated a phase 2 expansion study with its tumor checkpoint controller, lisavanbulin, in patients with recurrent glioblastoma multiforme (GBM), whose tumor has tested positive for the potential response-predictive biomarker EB1 (end-binding protein 1).¹

Glioblastoma is the most common type of primary brain cancer and one of the most lethal types of cancer.² In the open-label study, patients will receive once daily oral lisavanbulin. To identify patients with EB1-positive glioblastoma, a tissue screening program has been implemented using a CE-marked immunohistochemistry clinical trial assay developed for the lisavanbulin program.

Dr. Marc Engelhardt, Chief Medical Officer, said: "The initiation of the phase 2 study is an important step for us to validate our hypothesis that lisavanbulin may be developed for a targeted patient population based on a patient-selection biomarker. We expect interim results in the first half of 2021 and the outcome of this study will define the next development steps for lisavanbulin, including potentially expanding into other tumor types using a biomarker-driven approach."

EB1 was selected by Basilea as a potential response-predictive biomarker for lisavanbulin based on preclinical studies in glioblastoma models and initial clinical signals from earlier clinical studies. One glioblastoma patient in the phase 1 portion of the current study, whose tumor tissue was strongly positive for EB1, was reported as an exceptional long-lasting responder.³ This patient continues on treatment for more than two years now and shows a more than 80% area reduction of the brain tumor.

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin (BAL101553, the prodrug of BAL27862)⁴ is being developed as a potential therapy for diverse cancers.^{1, 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, 12} In preclinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models.¹² The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,¹³ resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.¹⁴



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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