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Press release

Basilea reports new prevalence data for EB1, a potential response-predictive biomarker for lisavanbulin in glioblastoma and other tumor types, at ASCO Annual Meeting

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Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that data on the prevalence of end-binding protein 1 (EB1) in glioblastoma and other tumor types is being presented at the American Society of Clinical Oncology (ASCO) Annual Meeting that takes place online from June 4 to 8, 2021.

EB1 plays a pivotal role in the regulation of microtubule dynamics during cell division and has been shown to interact with microtubule-targeting agents, such as lisavanbulin, inhibiting tumor growth.[1] EB1 has been identified as a response-predictive biomarker for Basilea's tumor checkpoint controller lisavanbulin in pre-clinical glioblastoma models.[2] In the previously reported phase 1 portion of the ongoing phase 1/2 clinical study, long-lasting clinical benefit was observed in two patients with recurrent glioblastoma whose tumor tissues show EB1-positive staining. Both patients are ongoing in the study for more than 2 years.[3]

The prevalence assessment of EB1-positivity or strong EB1-staining using immunohistochemistry methods presented at ASCO was based on 565 patient tissue samples from 14 different tumor types, including more than 100 glioblastoma samples. Approximately 5% of glioblastoma tissue samples were found to be EB1-positive. 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.[4]

Dr. Marc Engelhardt, Chief Medical Officer, commented: "We are very pleased that two patients with EB1-positive recurrent glioblastoma have obtained long-lasting clinical benefit in our phase 1 study with lisavanbulin. We are looking forward to the interim results in our phase 2 study, which is enrolling patients with recurrent glioblastoma that is EB1-positive, towards the end of 2021. The new prevalence data presented at ASCO are consistent with our initial frequency estimates of EB1-positive glioblastoma. A clinical proof-of-concept in glioblastoma based on positive interim results would support exploring the selection of patients based on

EB1-positivity in other tumor types as well, such as melanoma, breast, colorectal and lung cancers or rare cancer types such as medulloblastomas or neuroblastomas."

The rationale and study design of the ongoing phase 2 study with lisavanbulin in patients with EB1-positive recurrent glioblastoma is being presented in a second poster at the ASCO Annual meeting.[5]

The following posters are presented at the 2021 ASCO Annual Meeting:

Abstract #	Title
3118	**Expression of end-binding protein 1 (EB1), a potential response-predictive biomarker for lisavanbulin, in glioblastoma and various other solid tumor types** Authors: Magdalena Skowronska, Crescens Tiu, Alexandar Tzankov, Fatima König, Joanne Lewis, Igor Vivanco, Malte Kleinschmidt, Kirk Beebe, Stephanie Anderson, Felix Bachmann, Marc Engelhardt, Heidi A. Lane, Thomas Kaindl, Alexandru C Stan, Elizabeth Ruth Plummer, T. R. Jeffry Evans, Inti Zlobec, Juanita Suzanne Lopez
TPS2068	**The potential utility of end-binding protein 1 (EB1) as response-predictive biomarker for lisavanbulin: A phase 2 study of lisavanbulin (BAL101553) in adult patients with recurrent glioblastoma** Authors: Crescens Tiu, Sarah Derby, Noor Md. Haris, Liam Welsh, Anna Stansfeld, Thomas Hundsberger, Patrick Roth, Fatima König, Joel Robert Eisner, Malte Kleinschmidt, Stephanie Anderson, Felix Bachmann, Heidi A. Lane, Marc Engelhardt, Thomas Kaindl, Karine Litherland, Alexandru C. Stan, T. R. Jeffry Evans, Elizabeth Ruth Plummer, Juanita Suzanne Lopez

For further information, please visit https://conferences.asco.org/am/abstracts-posters

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin (BAL101553, the prodrug of BAL27862)[6] is being developed as a potential therapy for diverse cancers.[1, 7, 8] 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.[9, 10, 11]
Lisavanbulin efficiently distributes to the brain, with anticancer activity in glioblastoma models.[12, 13] In preclinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models.[2] 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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3. ClinicalTrials.gov identifier: NCT02490800. Phase 1 results: C. Tiu, A. Tzankov, R. Plummer et al. The potential utility of end-binding protein 1 (EB1) as response-predictive biomarker for lisavanbulin: Final results from a phase I study of lisavanbulin (BAL101553) in adult patients with recurrent glioblastoma (GBM). Annals of Oncology 2020 (31) supplement 4, S396-S408
4. M. Skowronska, C. Tiu, A. Tzankov, et al. Expression of end-binding protein 1 (EB1), a potential response-predictive biomarker for lisavanbulin, in glioblastoma and various other solid tumor types. J Clin Oncol 39, 2021 (suppl 15; abstr 3118)

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