

PRESS RELEASE

Basilea reports publication of clinical data for anticancer drug candidates BAL101553 in glioblastoma and derazantinib in intrahepatic cholangiocarcinoma at ASCO meeting

Basel, Switzerland, June 3, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today the phase 1 data from an ongoing study with the oral formulation of its novel tumor checkpoint controller, BAL101553, in brain cancer patients with progressive or recurrent glioblastoma, or high-grade glioma. The data were presented at the Annual Meeting of the American Society of Clinical Oncology (ASCO) in Chicago, USA, on June 2nd, 2019.

Results were based on 25 patients dosed in an open-label phase 1 study to explore once-daily oral BAL101553 as a single agent in patients with recurrent glioblastoma, the most common and aggressive primary brain tumor,¹ and patients with high-grade gliomas. BAL101553 was well tolerated and showed clinical activity, with a long-lasting confirmed partial response in one patient whose glioblastoma had been rapidly progressing on two lines of prior therapy. Tumor tissue analyses showed strong expression of the microtubule plus-end binding protein 1 (EB1), which has previously been identified as a potential predictive biomarker of tumor response to BAL101553 in preclinical glioblastoma models.² In addition, five further patients experienced stable disease as a best response.

In preclinical models, BAL101553 has been shown to cross the blood-brain barrier³ with antitumor activity in a panel of brain cancer models.^{3, 4}

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "The safety profile of oral BAL101553 and the signals of clinical activity in glioblastoma patients whose cancer progressed after prior therapy are encouraging. We continue to escalate the dose in the ongoing phase 1 study with the goal to establish a maximum tolerated dose for the oral formulation in glioblastoma, while also exploring whether EB1, or other biomarkers, may have clinical utility for patient stratification."

Basilea anticipates that the study will reach its primary goal, the definition of a maximum tolerated dose, in 2019. Two additional clinical studies with BAL101553 are ongoing: a phase 1 study in combination with standard radiotherapy in newly diagnosed glioblastoma, conducted in collaboration with the Adult Brain Tumor Consortium (ABTC) in the U.S., and a phase 2a expansion study, in which BAL101553 is administered as a weekly 48-hour intravenous (i.v.) infusion in patients with recurrent glioblastoma and in patients with platinum-resistant or refractory ovarian cancer.^{5, 6}

For derazantinib, Basilea's most advanced oncology drug candidate, data from a post-hoc analysis from a previously completed non-comparative phase 2a study⁷ were published in abstract form. The results from this analysis indicated that the antitumor efficacy of derazantinib in patients with intrahepatic cholangiocarcinoma (iCCA), a form of biliary duct cancer, with FGFR2 gene fusions who received derazantinib in a post-second line setting was similar compared to patients who received derazantinib as first or second-line treatment. This suggests that derazantinib could be an effective treatment option at both early and later stages of the disease.

BAL101553 poster at the 2019 ASCO Annual Meeting

- *Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller (TCC), in adult patients with progressive or recurrent glioblastoma (GBM) or high-grade glioma. – Juanita Suzanne Lopez, Rebecca Sophie Kristeleit, Robert Rulach, Noor Md Haris, Mariana Scaranti, Paul James Mulholland, Donna Crawford, Saira Bashir, Caterina Aversa, Alison L. Hannah, Stephanie Anderson, Marc Engelhardt, Thomas Kaindl, Patrice Larger, Phil McKernan, TR Jeffry Evans, Ruth Plummer; Abstract 2025, Poster Board #214. ClinicalTrials.gov Identifier: NCT02490800*

Derazantinib abstract at the 2019 ASCO Annual Meeting

- *Derazantinib (DZB) provides antitumor efficacy regardless of line of therapy in patients (pts) with FGFR2-fusion positive advanced intrahepatic cholangiocarcinoma (iCCA). – Michele Droz Dit Busset, Bassel F. El-Rayes, William Proctor Harris, Nevena Damjanov, Gianluca Masi, Lorena Rimassa, Sherri Bhoori, Monica Niger, Nicola Personeni, Fadi S. Braiteh, Sara Lonardi, Stephan Braun, Marc Engelhardt, Mikael Saulay, Brian E. Schwartz, Julia Kazakin, Walid Labib Shaib, Vincenzo Mazzaferro, Kyriakos P. Papadopoulos; Abstract e15607. ClinicalTrials.gov Identifier: NCT01752920*

For further information, please visit <https://meetings.asco.org/am>.

About BAL101553

Basilea's oncology drug candidate BAL101553 (the prodrug of BAL27862)⁸ is being developed as a potential therapy for diverse cancers. The drug candidate is currently in phase 1/2a clinical evaluation. In Switzerland, a phase 2a expansion study is exploring the drug in recurrent glioblastoma and platinum-resistant ovarian cancer patients using weekly 48-hour infusion. In the UK, a phase 1 dose escalation with daily oral administration is ongoing in patients with progressive or recurrent glioblastoma or high-grade glioma. In the U.S., a phase 1 study is being conducted in collaboration with the Adult Brain Tumor Consortium (ABTC), in which BAL101553 is explored in combination with radiotherapy in patients with newly diagnosed glioblastoma who have a reduced sensitivity to chemotherapy with the standard-of-care drug temozolomide. In preclinical studies, BAL101553 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} BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.^{2, 3, 4} The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,¹² resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.¹³

About derazantinib

Derazantinib (BAL087, formerly ARQ 087) is an investigational orally administered small molecule panFGFR kinase inhibitor with strong activity against FGFR1, 2, and 3. FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR gene alterations, e.g. rearrangements, amplification or mutations, have been identified as potentially important therapeutic targets for various cancers, including intrahepatic cholangiocarcinoma (iCCA), urothelial, breast, gastric and lung cancers.¹⁴ In these cancers, FGFR gene alterations are found in a range of 5% to 30%.¹⁵ Basilea in-licensed derazantinib from ArQule Inc. in April 2018. 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,⁷ and has received U.S. and EU orphan drug designation for iCCA.

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 anti-infectives. 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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