# BerGenBio's Bemcentinib Meets Efficacy Endpoint in BERGAMO Phase II Trial in Patients with High Risk Myelodysplastic Syndromes or Acute Myeloid Leukemia

- *Primary endpoint of overall response rate was met in the fully recruited single arm phase II study*

**Bergen, Norway,18 August 2020 -** BerGenBio ASA (OSE: BGBIO), a clinical-stage biopharmaceutical company developing novel, selective AXL kinase inhibitors for unmet medical need, announces that the primary endpoint of overall response rate has been met in the BERGAMO Phase II Trial investigating BerGenBio's selective AXL inhibitor bemcentinib in patients with High Risk Myelodysplastic Syndromes (HR-MDS) or Acute Myeloid Leukemia (AML), for whom treatment with hypomethylating agents (HMAs) is ineffective.

The multicenter phase 2 BERGAMO trial (NCT03824080) evaluated the safety and efficacy of bemcentinib monotherapy in patients with HR-MDS or AML who were refractory to or in relapse after at least six or four cycles of the HMAs azacitidine (AZA) or decitabine (DAC), respectively. HMAs represent the current standard of care in both indications for patients not eligible for intensive chemotherapy or allogeneic stem cell transplantation. The study enrolled 45 eligible patients from 10 sites across Germany, France and the Netherlands.

The primary endpoint was met and included overall response rate (CR, CRi, PR or SD) assessed after 4 treatment cycles. All patients who achieved CR, CRi, PR or SD after 4 cycles of bemcentinib were considered as responders and allowed to continue treatment for a total of up to 9 cycles. Non-responding patients stopped treatment after 4 cycles. Secondary endpoints of the trial include a translational project evaluating the role of biomarkers and response.

The BERGAMO trial is an investigator sponsored trial co-ordinated by The European Myelodysplastic Syndromes Cooperative Group (EMSCO). The chief investigator is Prof. Uwe Platzbecker, MD, from Leipzig University Hospital, Germany, and the study included 10 clinical research sites in Germany, France and Netherlands.

Further details of results of the BERGAMO trial will be presented at appropriate scientific medical conferences later in 2020.

**Richard Godfrey, Chief Executive Officer of BerGenBio, said:** "*These headline results are in-line with previously reported efficacy data on bemcentinib in relapsed MDS and AML patients. Despite recent improvements of first line treatment options there remains a substantial need for novel therapeutics which target difficult-to-treat patient groups including patients with HR-MDS and AML who have failed front-line therapy with hypomethylating agents. This is a group with a very poor prognosis so we are very encouraged by these data and believe they provide further validation for our clinical development strategy in these indications.''*

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**About MDS and AML**

Hypomethylating agents (HMAs) are the standard of care for patients with higher-risk myelodysplastic syndrome (HR-MDS) or acute myeloid leukemia (AML), not eligible for intensive chemotherapy or allogeneic stem cell transplantation. However, the majority of

patients do not respond to these agents or relapse, still having a dismal outcome with very limited treatment options available.
Bemcentinib (BEM) is a selective small molecule inhibitor of AXL, a surface membrane protein kinase receptor mediating resistance to chemotherapeutic agents and decreased antitumor immune response. AXL is overexpressed on leukemic cells, especially in the stem cell compartment, and represents a potential novel target in patients with MDS and AML.

**About AXL**

AXL kinase is a cell membrane receptor and an essential mediator of the biological mechanisms underlying life-threatening diseases. In cancer, AXL suppresses the body's immune response to tumours and drives cancer treatment failure across many indications. AXL inhibitors, therefore, have potential high value at the centre of cancer combination therapy, addressing significant unmet medical needs and multiple high-value market opportunities. Research has also shown that AXL mediates other aggressive diseases.

**About Bemcentinib**

Bemcentinib (formerly known as BGB324), is a potentially first-in-class selective AXL inhibitor in a broad phase II clinical development programme. Ongoing clinical trials are investigating bemcentinib in multiple solid and haematological tumours, in combination with current and emerging therapies(including immunotherapies, targeted therapies and chemotherapy), and as a single agent. Bemcentinib targets and binds to the intracellular catalytic kinase domain of AXL receptor tyrosine kinase and inhibits its activity. Increase in AXL function has been linked to key mechanisms of drug resistance and immune escape by tumour cells, leading to aggressive metastatic cancers.

**About BerGenBio ASA**

BerGenBio is a clinical-stage biopharmaceutical company focused on developing transformative drugs targeting AXL as a potential cornerstone of therapy for aggressive diseases, including immune-evasive, drug resistant cancers. The company's proprietary lead candidate, bemcentinib, is a potentially first-in-class selective AXL inhibitor in a broad Phase II oncology clinical development programme focused on combination and single agent therapy in lung cancer and leukaemia. A first-in-class functional blocking anti-AXL antibody is undergoing Phase I clinical testing. In parallel, BerGenBio is developing a companion diagnostic test to identify those patient populations most likely to benefit from bemcentinib: this is expected to facilitate more efficient registration trials supporting a precision medicine-based commercialisation strategy.

BerGenBio is based in Bergen, Norway with a subsidiary in Oxford, UK. The company is listed on the Oslo Stock Exchange (ticker: BGBIO). For more information, visit www.bergenbio.com

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**Forward looking statements**

This announcement may contain forward-looking statements, which as such are not historical facts, but are based upon various assumptions, many of which are based, in turn, upon further assumptions. These assumptions are inherently subject to significant known and unknown risks, uncertainties and other important factors. Such risks, uncertainties, contingencies and other important factors could cause actual events to differ materially from the expectations expressed or implied in this announcement by such forward-looking statements.

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