BACKGROUND, RATIONALE AND STUDY DESIGN

**BGBC003 Study Rationale**

- AXL, a receptor tyrosine kinase, is expressed in approx. 50% of AML cases and is a negative prognostic factor.
- Bemcentinib is a first-in-class highly selective, potent, and orally bioavailable inhibitor of AXL.
- Bemcentinib potentiates the efficacy of chemotherapy, prevents development of acquired drug resistance, and enhances the efficacy of immune checkpoint inhibitors in several in vivo tumor models.
- Bemcentinib inhibits survival of AML cells in vitro and enhances sensitivity to cytoreduction in preclinical AML models and AML cell lines.
- Bemcentinib is being developed as a monotherapy and in combination with chemotherapy in AXL/MSD and a broader development program at BerGenBio is ongoing, including other indications.

**Proposed Mechanism**

- **Key inclusion criteria**
  - Diagnoses of AML (except FAB M2)
  - Ineligible for intensive chemotherapy (due to advanced age or comorbidities)
  - Relapsed or refractory after at least 1 prior line of therapy
  - Suitable to receive treatment with Bemcentinib+LDAC

**Endpoints**

- **Primary:** Safety and tolerability
- **Secondary:**
  - CR (overall response rate)
  - PR (complete response rate)
  - OS (overall survival)
  - PK profile

**Demographics and disease characteristics**

- **All patients in B2 and B5:**
  - Median age 73 years, 76% male, 81% white

- **Bemcentinib + LDAC combination—cohorts B2 and B5**
  - Median age 72 years, 80% male, 76% white

**Single cell multimomics of longitudinal patient bone marrow samples**

- 10x gene expression has been performed on 30 single-gene bone marrow samples of 11 patients within the combination arm B2 & B5.
- Transcriptomes were acquired using a panel of 25 ToilSeq antibodies.
- Gene and protein expression counts were obtained from sequencing data using Cell Ranger and cell doublets were inferred using Sceneria.
- UMAP visualizations and clustering results were obtained by state-of-the-art workflows for bulk-model single-cell data using functions from Sceneria and screen.
- Cell type annotations were based on the identified clusters and were inferred from the expression of known marker features on both the RNA and the protein level.
- Response was defined as CR, CRi, and PR.
- Sub-clustering of the T-NK cell compartment showed differences with respect to response.

**Sub-clustering of the T-NK cell compartment**

- **Safety profile of combination treatment**
  - Consistent with that of the individual drugs

**ACKNOWLEDGMENTS**

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**REFERENCES**


2. BerGenBio ASA. Ongoing BGBC003. https://www.bergenbio.com/pr/pr210419-bgc003


**CONTACT INFORMATION**

BerGenBio Ltd.
11 Robert Robinson Ave.
Oxford
OX4 4QA, UK

BerGenBio ASA
1 Robert Robinson Ave.
Bergen
5018 Bergen, Norway

Soraya Leguizamon
sonia.loges@medma.uni

+49 17682243855

David Gribble
BerGenBio

+47 99939526

www.bergenbio.com

@BerGenBio

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THE COMBINATION OF AXL INHIBITOR BEMCENTINIB AND LOW-DOSE CYTARABINE IS WELL TOLERATED AND EFFICACIOUS IN ELDERLY RELAPSED AML PATIENTS: UPDATE FROM THE ONGOING BGBC003 PHASE II TRIAL (NCT02488408)


Soraya Leguizamon
sonia.loges@medma.uni

+49 17682243855

www.bergenbio.com

@BerGenBio