



## **BioLineRx Announces Positive Results of Lead-in Period for Phase 3 GENESIS Trial in Stem-Cell Mobilization**

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### **Data from first lead-in patient cohort prompts Data Monitoring Committee to recommend early continuation to randomized placebo-controlled part 2 of trial**

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BioLineRx Ltd. (NASDAQ: BLRX) (TASE: BLRX), a clinical-stage biopharmaceutical company focused on oncology and immunology, announced today positive results from the lead-in period of the GENESIS trial, a double-blind, placebo-controlled Phase 3 trial comparing BL-8040 in combination with granulocyte colony-stimulating factor (G-CSF), to G-CSF alone, for the mobilization of hematopoietic stem cells (HSCs) used for autologous transplantation in multiple myeloma patients.

The open-label, single-arm, lead-in period of the study was designed to include up to 30 patients, with Data Monitoring Committee (DMC) review after completion of approximately 10, 20 and 30 patients, in order to assess safety and efficacy following treatment with BL-8040 plus G-CSF. Results of the first 11 patients show that BL-8040 in combination with standard G-CSF treatment is safe and tolerable. In addition, the data show that 9/11 patients (82%) reached the primary endpoint threshold of  $\geq 6 \times 10^6$  CD34 cells/kg with only one dose of BL-8040 and in up to 2 apheresis sessions.

Furthermore, 7/11 patients (64%) reached the threshold of  $\geq 6 \times 10^6$  CD34 cells/kg in a single apheresis session only. These data demonstrate the potential of BL-8040 treatment to reduce the number of administrations and apheresis sessions, as well as hospitalization costs, related to the preparation of multiple myeloma patients for autologous HSC transplantation.

"Autologous HSC transplantation following high-dose chemotherapy has significantly improved outcomes for multiple myeloma patients," said Dr. John F. DiPersio, Chief, Division of Oncology at the Washington University School of Medicine, and lead investigator of the study. "Current practice involves mobilizing HSCs from the bone marrow to the peripheral blood, after which the cells are collected by apheresis. Results so far show that mobilizing HSCs with a single BL-8040 dose combined with G-CSF is not only safe and tolerable, but also demonstrates robust efficacy regarding the number of collected cells, and may reduce the number of required apheresis sessions to a single session in the majority of patients. This is a very encouraging result that, if corroborated in the placebo-controlled part of the trial, will be of great value to patients as well as to the medical community. I am looking forward to participating in the trial and to potentially improving the quality of treatment available to multiple myeloma patients."

"We are extremely encouraged by these results. Based on the robust data received from the first 11 patients, the DMC issued a positive recommendation to stop the lead-in part of the study and move immediately to the randomized placebo-controlled part of the study," stated Philip Serlin, Chief Executive Officer of BioLineRx. "This is the first Phase 3 trial for our lead BL-8040 program, and as such, it is an important milestone in BL-8040's comprehensive development plan. We look forward to the top-line results from the randomized, double-blind, placebo-controlled part of the study, which are expected in 2020."

#### **About the GENESIS Study**

The GENESIS study is a Phase 3, randomized, double-blind, placebo-controlled, multicenter study, evaluating the safety, tolerability and efficacy of BL-8040 in combination with G-CSF, compared to placebo and G-CSF, for the mobilization of CD34 HSCs for autologous transplantation in multiple myeloma patients. The placebo-controlled part is designed to include 177 patients in more than 25 centers. Treatment will include 5 days of G-CSF, with a single dose of BL-8040 or placebo on day 4 with the option to expand treatment to up to 8 days of G-CSF and up to 2 days of BL-8040. Apheresis for collection of CD34 cells will be performed on day 5. An additional 3 apheresis sessions may be conducted if needed in order to reach the goal of  $\geq 6 \times 10^6$  mobilized CD34 cells/kg.

The primary objective of the study is to demonstrate the superiority of a single dose of BL-8040 in combination with G-CSF, over placebo and G-CSF, in the mobilization of  $\geq 6 \times 10^6$  CD34 cells/kg in up to 2 apheresis sessions, in preparation for autologous stem cell transplantation in multiple myeloma patients. Secondary objectives include time to engraftment of neutrophils and platelets, durability of the engraftment, as well as safety and other efficacy parameters.

#### **About BL-8040**

BL-8040 is a short synthetic peptide for stem cell mobilization and for treatment of hematological malignancies and solid tumors. It functions as a high-affinity best-in-class antagonist for CXCR4, a chemokine receptor that is directly involved in the retention of stem cells in the bone marrow, as well as tumor progression, angiogenesis, metastasis and cell survival. CXCR4 is over-expressed in more than 70% of human cancers and its expression often correlates with disease severity.

HSCs express CXCR4 and are retained in the protective bone marrow niche via binding to CXCL12 (also known as SDF-1). Blocking of the CXCR4-SDF1 interaction by BL-8040 leads to the mobilization of HSCs into the peripheral blood. In a number of clinical and pre-clinical studies, BL-8040 has shown robust mobilization of HSCs.

Furthermore, BL-8040 induce mobilization of leukemic cells and immune-cells from the bone marrow, thereby sensitizing leukemic cells to chemo- and bio-based anti-cancer therapy, as well as a direct anti-cancer effect by inducing cell death (apoptosis). BL-8040 was licensed by BioLineRx from Biokine Therapeutics and was previously developed under the name BKT-140.

#### **About BioLineRx**

BioLineRx is a clinical-stage biopharmaceutical company focused on oncology and immunology. The Company in-licenses novel compounds, develops them through pre-clinical and/or clinical stages, and then partners with pharmaceutical companies for advanced clinical development and/or commercialization.

BioLineRx's leading therapeutic candidates are: BL-8040, a multi-therapy platform, which has successfully completed a Phase 2a study for relapsed/refractory AML, is in the midst of a Phase 2b study as an AML consolidation treatment and has initiated a Phase 3 study in stem cell mobilization for autologous transplantation; and AGI-134, an immunotherapy treatment in development for multiple solid tumors, which has recently initiated a Phase 1/2a study. In addition, BioLineRx has a strategic collaboration with Novartis for the co-development of selected Israeli-sourced novel drug candidates; a collaboration agreement with MSD (known as Merck in the US and Canada), on the basis of which the Company is conducting a Phase 2a study in pancreatic cancer using the combination of BL-8040 and Merck's KEYTRUDA®; and a collaboration agreement with Genentech, a member of the Roche Group, to investigate the combination of BL-8040 and Genentech's atezolizumab in several Phase 1b/2 studies for multiple solid tumor indications and AML.

For additional information on BioLineRx, please visit the Company's website at [www.biolinerx.com](http://www.biolinerx.com), where you can review the Company's SEC filings, press releases, announcements and events. BioLineRx industry updates are also regularly updated on [Facebook](#), [Twitter](#), and [LinkedIn](#).

*Various statements in this release concerning BioLineRx's future expectations constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. These statements include words such as "may," "expects," "anticipates," "believes," and "intends," and describe opinions about future events. These forward-looking statements involve known and unknown risks and uncertainties that may cause the actual results, performance or achievements of BioLineRx to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Some of these risks are: changes in relationships with collaborators; the impact of competitive products and technological changes; risks relating to the development of new products; and the ability to implement technological improvements. These and other factors are more fully discussed in the "Risk Factors" section of BioLineRx's most recent annual report on Form 20-F filed with the Securities and Exchange Commission on March 6, 2018. In addition, any forward-looking statements represent BioLineRx's views only as of the date of this release and should not be relied upon as representing its views as of any subsequent date. BioLineRx does not assume any obligation to update any forward-looking statements unless required by law.*

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