



BioLineRx Reports Results of Phase 2 Study for BL-8040 Monotherapy in Stem Cell Mobilization for Allogeneic Bone Marrow Transplantation

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**- BL-8040 was safe and well tolerated, and all transplanted recipients were successfully engrafted -
- Full top-line results will be presented at the 23rd EHA Congress -**

TEL AVIV, Israel, May 17, 2018 /PRNewswire/ -- BioLineRx Ltd. (NASDAQ: BLRX) (TASE: BLRX), a clinical-stage biopharmaceutical company focused on oncology and immunology, announced today positive results from a Phase 2 clinical trial assessing BL-8040 as a single agent for hematopoietic stem cell mobilization in an allogeneic transplantation setting. The full top-line results of the study will be presented at the [23rd Congress of European Hematology Association \(EHA\)](#), to take place June 14-17, 2018 in Stockholm, Sweden.

Mobilization of hematopoietic stem and progenitor cells (HSPCs) for the purpose of donor (allogeneic) transplantation after high-dose chemotherapy is currently performed using a 4-5 day treatment cycle with G-CSF and a 1-2 day apheresis procedure. Single-agent treatment with BL-8040, a novel, high-affinity CXCR4 antagonist with rapid HSPC mobilizing kinetics, showed similar efficacy in only one administration. In addition, BL-8040 showed non-inferiority in recipient engraftment, with all transplanted recipients successfully engrafting with BL-8040-mobilized grafts.

This proof-of-concept Phase 2 study, consisting of 24 donor/recipient pairs, assesses the ability of BL-8040 monotherapy to mobilize HSPCs for transplantation in a single administration. In the first part of the trial, HLA-identical donors were treated with a single dose of 1 mg/kg of BL-8040 for evaluating safety and tolerability. The second part of the study, which is still ongoing, includes both HLA-identical and haploidentical pairs, and donors were treated with 1.25 mg/kg of BL-8040. HSPCs were collected by leukapheresis after a single BL-8040 injection. The primary endpoint was collection of $\geq 2 \times 10^6$ CD34 cells/kg of recipient weight in up to 2 leukapheresis sessions.

Of the 21 evaluable donors that have been enrolled in the study to date, 11 out of 13 donors (85%) treated at the 1 mg/kg dose and 8/8 donors (100%) treated at the 1.25 mg/kg dose of BL-8040 reached the primary goal of $\geq 2 \times 10^6$ CD34 cells/kg of recipient weight in up to 2 leukapheresis sessions. BL-8040 was safe and well tolerated, with adverse events consisting of injection site reactions and transient systemic reactions, all of which were resolved. No related serious adverse events were observed. All 19 transplanted recipients were successfully engrafted with BL-8040-mobilized grafts, 13 of which have reached the secondary endpoint of 100 days post-transplant. Preliminary graft-versus-host disease (GVHD) data are in line with current standard-of-care incidence rates. The full effect of BL-8040 on acute and chronic GVHD, as well as on relapse rates, await longer follow-up periods and will be reported at a later stage once available.

"Hematopoietic stem cell transplant is vital for the treatment of various hematological malignancies," stated Dr. Geoffrey Uy, from the Section of Blood and Marrow Transplantation and Leukemia in the Division of Oncology at the Washington University School of Medicine, and the principal investigator of the study. "Currently, hematopoietic stem cells are mobilized from the bone marrow to the peripheral blood using repeated administrations of G-CSF, followed by leukapheresis, with the whole process lasting almost a week. It is therefore encouraging to see these top-line results, supporting the application of BL-8040 as a single agent for rapid mobilization of hematopoietic stem cells after only one injection."

"We are very pleased with the results of this proof-of-concept Phase 2 clinical trial, showing that a single administration of BL-8040, followed by apheresis, results in rapid and effective HSPC mobilization and leads to prompt hematopoietic recovery after allogeneic transplantation," stated Philip A. Serlin, Chief Executive Officer of BioLineRx. "These robust results in an allogeneic setting continue to strongly support BL-8040's mechanism of action, and demonstrate the ability of BL-8040 as a fast and effective mobilizing agent, thereby giving us further confidence in our ongoing Phase 3 study in stem cell mobilization for autologous transplants. We look forward to the results of the lead-in period from the Phase 3 trial, which are expected in the next few months. In parallel, we are further evaluating the allogeneic transplant landscape in order to decide on the best development pathway forward for this complementary indication."

About BL-8040

BL-8040 is a short peptide for the treatment of acute myeloid leukemia, solid tumors, and stem cell mobilization. It functions as a high-affinity antagonist for CXCR4, a chemokine receptor that is directly involved in 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. In a number of clinical and pre-clinical studies, BL-8040 has shown robust mobilization of cancer cells and immune-cells from the bone marrow, thereby sensitizing cancer cells to chemo- and bio-based anti-cancer therapy, as well as a direct anti-cancer effect by inducing cell death (apoptosis) and mobilizing immune-cells. In addition, BL-8040 has also demonstrated robust stem-cell mobilization, including the mobilization of colony-forming cells, T, B and NK cells. BL-8040 was licensed by BioLineRx from Biokine Therapeutics and was previously developed under the name BKT-140.

About Stem Cell Mobilization

High-dose chemotherapy followed by stem cell transplantation has become an established treatment modality for a variety of hematologic malignancies, including multiple myeloma, as well as various forms of lymphoma and leukemia. Stem cells are mobilized from the bone marrow using granulocyte colony-stimulating factor (G-CSF), harvested from the peripheral blood by apheresis, and infused to the patient after chemotherapy. This type of treatment often replaces the use of traditional bone marrow transplantation, because the stem cells are easier to collect and the treatment allows for a quicker recovery time and fewer complications.

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 cancer 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 is expected to initiate a first-in-man study in mid-2018. 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 carrying out 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, 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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