



## BioLineRx Announces Positive Clinical Results from First Part of Phase 2 Trial in r/r AML

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**- Data strongly suggest BL-8040 has potent anti-leukemic activity in combination with Ara-C in AML -**

**- Results to be presented at 2015 ASH Annual Meeting -**

TEL AVIV, Israel--(BUSINESS WIRE)--Nov. 5, 2015-- BioLineRx Ltd. (NASDAQ, TASE: BLRX), a clinical-stage biopharmaceutical company dedicated to identifying, in-licensing and developing promising therapeutic candidates, announced today positive results from the dose escalation part of BL-8040's Phase 2 clinical trial in relapsed or refractory acute myeloid leukemia (r/r AML). The results will be presented at the [57th American Society of Hematology \(ASH\) Annual Meeting](#), to be held December 5-8, 2015 in Orlando, Florida.

Results showed that BL-8040, as a single agent and in combination with Cytarabine (Ara-C), was safe and well tolerated at all doses tested up to and including the highest dose level of 1.5 mg/kg, with no major adverse events. The composite complete remission rate, including both complete remission (CR) and complete remission with incomplete blood count recovery (CRi), was 38% in subjects receiving only one cycle of BL-8040 treatment at doses of 1 mg/kg and higher (n=16). Patients included in this part of the study were patients that had undergone a significant number of prior treatment cycles or that were refractory to induction treatment.

Treatment with BL-8040 had a triple effect on the leukemic cells. First, following only two days of monotherapy, BL-8040 triggered an average 40-fold mobilization of immature AML progenitor cells from the bone marrow to the peripheral blood, thereby sensitizing these cells to the Ara-C chemotherapy and improving its efficacy. Second, BL-8040 showed a direct and significant apoptotic effect on the immature leukemia progenitor cells in the bone marrow following the two days of monotherapy. Last, BL-8040 induced leukemia progenitor cells towards differentiation, as evidenced by a 58% median decrease in the number of bone marrow leukemia progenitor cells, along with a three-fold increase in differentiated granulocytes, in the bone marrow biopsy conducted on Day 3 of the treatment cycle prior to the Ara-C treatment, as compared to the biopsy performed at baseline.

Dr. Jorge Cortes, Chief of the AML and CML Sections at the MD Anderson Cancer Center in Houston, stated, "The clinical results from the dose escalation stage of the Phase 2 study for BL-8040 in r/r AML are very encouraging and fully support continued development of the compound in the AML space."

Dr. Kinneret Savitsky, CEO of BioLineRx, commented, "We are very pleased and encouraged by the positive results from the first part of this Phase 2 trial with BL-8040 for the treatment of r/r AML, especially in light of the severity of the patient population recruited and the short treatment regimen of one cycle. The results continue to demonstrate that BL-8040 not only significantly induces mobilization of leukemic cells from the protective microenvironment of the bone marrow into the peripheral blood, but also directly leads to apoptosis of leukemic progenitor cells and triggers terminal differentiation of the cells into granulocytes. Combined with the reported 38% remission rate from subjects receiving BL-8040 doses of at least 1 mg/kg, the results strongly suggest that BL-8040 has potent anti-leukemic activity and, in combination with Ara-C, may improve the response typically achieved in this advanced AML population." Dr. Savitsky continued, "In light of the encouraging results, we look forward to discussions with the regulatory authorities regarding the future development plan for AML. We currently anticipate reporting topline results from the full study by early next year."

"In order to further expand and enhance the potential of this unique oncology platform, we are continuing to perform and plan multiple additional clinical studies for BL-8040. These include a Phase 2b study which we recently initiated for BL-8040 as an AML consolidation treatment; a Phase 2 study in the planning stages as a novel stem cell mobilization treatment, based on input that we received from the FDA last month; and two additional studies in certain bone marrow failure indications and for the treatment of AML patients with the FLT3-ITD mutation," concluded Dr. Savitsky.

### **About the r/r AML Phase 2 study**

The Phase 2 trial is a multicenter, open-label study under an IND, conducted at ten clinical sites in the U.S. and Israel, and is designed to assess the safety, efficacy pharmacodynamics and pharmacokinetic parameters of BL-8040 in combination with Cytarabine (Ara-C) for the treatment of adult relapsed or refractory AML patients. Twenty-two patients with r/r AML were enrolled in the dose escalation stage of the study (16 of which received a dose of 1 mg/kg and higher), which includes a dose escalation stage followed by an expansion stage. In the dose escalation stage, each patient received a once daily dose of BL-8040 monotherapy (from 0.5 to 1.5 mg/kg) on days 1-2 followed by the same dose of BL-8040 plus Ara-C on days 3-7. Extensive pharmacodynamic parameters, such as mobilization of leukemic cells and induction of apoptosis, were assessed after monotherapy with BL-8040 using peripheral blood sampling and bone marrow aspirates at baseline and on Day 3 prior to Ara-C administration. Clinical response to treatment was evaluated by bone marrow biopsy on Day 30.

### **About BL-8040**

BL-8040 is a clinical-stage drug candidate for the treatment of acute myeloid leukemia, as well as other hematological indications. It is a short peptide that functions as a high-affinity antagonist for CXCR4, a chemokine receptor that is directly involved in tumor progression, angiogenesis (growth of new blood vessels in the tumor), metastasis (spread of the disease to other organs or organ parts) and cell survival. CXCR4 is over-expressed in more than 70% of human cancers and its expression often correlates with disease severity. In a Phase 1/2, open-label, dose escalation, safety and efficacy clinical trial in 18 multiple myeloma patients, BL-8040, when combined with G-CSF, demonstrated an excellent safety profile at all doses tested and was highly effective in the mobilization of hematopoietic stem cells and white blood cells from the bone marrow to the peripheral blood. Additionally, in a Phase 1 stem-cell mobilization study in healthy volunteers, BL-8040 as a single agent was safe and well tolerated at all doses tested and resulted in efficient stem-cell mobilization and collection in all study participants. Importantly, the results of this study support the use of BL-8040 as one-day, single-dose collection regimen, which is a significant improvement upon the current standard of care.

BL-8040 also mobilizes cancer cells from the bone marrow and may therefore sensitize these cells to chemo- and bio-based anti-cancer therapy. Importantly, BL-8040 has also demonstrated a direct anti-cancer effect by inducing apoptosis. Pre-clinical studies show that BL-8040 inhibits the growth of various tumor types including multiple myeloma, non-Hodgkin's lymphoma, leukemia, non-small cell lung carcinoma, neuroblastoma and melanoma. BL-8040 also significantly and preferentially stimulated apoptotic cell death of malignant cells (multiple myeloma, non-Hodgkin's lymphoma and leukemia). Significant synergistic and/or additive tumor cell killing activity has been observed in-vitro and in-vivo when tumor cells were treated with BL-8040 together with Rituximab, Bortezomib, Imatinib, Cytarabine and the FLT-3 inhibitor AC-220 (in NHL, MM, CML, AML, and

AML-FLT3-ITD models, respectively). In addition, the current Phase 2 clinical trial in AML patients has demonstrated robust mobilization and apoptosis of cancer cells. BL-8040 was licensed by BioLineRx from Biokine Therapeutics and was previously developed under the name BKT-140.

### **About Acute Myeloid Leukemia (AML)**

Acute myeloid leukemia (AML) is a cancer of the blood and bone marrow and is the most common type of acute leukemia in adults. According to the American Cancer Society, approximately 19,000 new cases of AML were diagnosed in the United States in 2014, and the median age of AML patients was 67 years old. The first treatment line for patients with AML includes a combination of chemotherapy drugs and is called induction treatment. The median survival for AML patients receiving induction chemotherapy is less than two years, with shorter survival for patients over the age of 60 or for those with certain gene or chromosome aberrations. Due to relapsed or refractory disease (where the disease is not responsive to standard treatments), the overall five-year survival rate for AML is between 10 and 40 percent.

### **About BioLineRx**

BioLineRx is a publicly-traded, clinical-stage biopharmaceutical company dedicated to identifying, in-licensing and developing promising therapeutic candidates. The Company in-licenses novel compounds primarily from academic institutions and biotech companies based in Israel, develops them through pre-clinical and/or clinical stages, and then partners with pharmaceutical companies for advanced clinical development and/or commercialization.

BioLineRx's current portfolio consists of a variety of clinical and pre-clinical projects, including: BL-8040, a cancer therapy platform, which is in the midst of a Phase 2 study for relapsed/refractory acute myeloid leukemia (AML), has recently initiated a Phase 2b study as an AML consolidation treatment, and has successfully completed a Phase 1 study in stem cell mobilization; and BL-7010 for celiac disease, which has successfully completed a Phase 1/2 study.

In December 2014, BioLineRx entered into a strategic collaboration with Novartis for the co-development of selected Israeli-sourced novel drug candidates. The companies intend to co-develop a number of pre-clinical and early clinical therapeutic projects through clinical proof-of-concept for potential future licensing by Novartis.

For more information on BioLineRx, please visit [www.bioplinerx.com](http://www.bioplinerx.com) or download the investor relations mobile device app, which allows users access to the Company's SEC documents, press releases, and events. BioLineRx's IR app is available on the iTunes App Store as well as the Google Play Store.

*Various statements in this release concerning BioLineRx's future expectations, including specifically those related to the development and commercialization of BL-8040, 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 23, 2015. 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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