



## BioLineRx Announces Investigator-Initiated Study for Novel Chronic Myeloid Leukemia Treatment

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**Phase 1/2 study will assess effect of BL-8040 in combination with standard therapy**

JERUSALEM--(BUSINESS WIRE)--Apr. 10, 2014-- BioLineRx (NASDAQ: BLRX)(TASE: BLRX), a clinical-stage biopharmaceutical company dedicated to identifying, in-licensing and developing promising therapeutic candidates, announced today that Prof. Arnon Nagler, Director of the Hematology Division and Bone Marrow Transplantation Center at Sheba Medical Center, Israel, has received final regulatory approval to evaluate BioLineRx's BL-8040 as a treatment for chronic myeloid leukemia (CML) in a Phase 1/2 clinical study. BioLineRx is currently developing BL-8040 in a Phase 2 study for treating acute myeloid leukemia (AML), and in a Phase 1 study for stem cell mobilization, as a pre-treatment for stem cell transplantation. The Company has received orphan drug designation for both these indications.

Dr. Kinneret Savitsky, Chief Executive Officer of BioLineRx, stated, "We are extremely pleased that our promising and versatile therapeutic candidate is ready to commence a Phase 1/2 clinical trial for a third indication, namely CML. Prof. Nagler's decision to pursue BL-8040 for CML in an independent clinical study is a testament to the potential he sees in our molecule for treating hematologic cancers. Each of these indications stands to benefit from the two main characteristics of BL-8040 - its ability to mobilize cells from the bone marrow into the peripheral blood system, and its ability to induce cancer cell death directly."

CML is a cancer of white blood cells that is driven by a constitutively active oncogenic tyrosine kinase. The main treatment today is with tyrosine kinase inhibitors, such as Imatinib (Gleevec), but about 15% of patients do not have an optimal response to the drug, and 40% eventually develop resistance to the drug. In addition, treatment with Gleevec does not always prevent recurrence of the disease, presumably due to the survival of dormant, tumorigenic stem cells in the bone marrow.

The study is designed as a Phase 1/2, randomized, dose-escalation study to assess the combination of BL-8040 with standard-of-care Imatinib for improving the response of CML patients in the first chronic phase of the disease who have achieved a less than optimal response with Imatinib alone. Primary endpoints of the study are the safety and tolerability of BL-8040 in combination with Imatinib, and the secondary endpoints include assessing the efficacy of the combination therapy in achieving improved cytogenetic and molecular response in CML patients. The study will be performed at the Sheba Medical Center, and will include up to 40 patients.

"The bone marrow has a protective effect on CML stem cells, and enables them to evade eradication by existing drugs. Preclinical data have shown that BL-8040 efficiently synergizes with Imatinib *in-vitro*<sup>1</sup> and *in-vivo*, overcoming the protective effect of the bone marrow, and we therefore hope that the combination of these two drugs will override drug resistance and suppress residual disease," stated Prof. Nagler. "It is conceivable that adding BL-8040 to Imatinib therapy in CML patients who have not achieved optimal cytogenetic or molecular responses may improve their response to Imatinib by directly inducing apoptosis of the tumor cells and by mobilizing leukemic stem cells from the bone marrow's protective niches and sensitizing them to Imatinib-induced cell death."

### 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. BL-8040 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 (cell death). Pre-clinical studies show that BL-8040 is efficient, both alone and in combination with anti-cancer drugs such as Rituximab, Imatinib and Bortezomib in lymphoma, CML and multiple myeloma models (respectively).

BL-8040 also mobilizes stem cells from the bone marrow to the peripheral blood, enabling their collection for subsequent autologous or allogeneic transplantation in cancer patients. In a Phase 1/2, open-label, dose escalation, safety and efficacy clinical trial in 18 multiple myeloma patients, BL-8040 demonstrated an excellent safety profile at all doses tested and was highly effective in combination with G-CSF in the mobilization of hematopoietic stem cells and white blood cells from the bone marrow to the peripheral blood. BL-8040 was licensed by BioLineRx from Biokine Therapeutics and was previously developed under the name BKT-140.

### About CML

Chronic myeloid leukemia (CML) is a cancer of white blood cells that is driven by a constitutively active oncogenic tyrosine kinase. Tyrosine kinase inhibitors, such as Imatinib (Gleevec), revolutionized the treatment of CML and remain a major therapeutic strategy for CML patients. However, dormant leukemic stem cells that are resistant to tyrosine kinase inhibitors and may be responsible for CML resistance and recurrence, are protected from therapy-induced cell death by the bone marrow microenvironment and in particular, by bone marrow stromal cells. Therefore, novel drugs are needed that can target leukemic stem cells in the bone marrow.

### 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-1040 for prevention of pathological cardiac remodeling following a myocardial infarction, which has been out-licensed to Bellerophon BCM (f/k/a Ikaria) and is in the midst of a pivotal CE-Mark registration trial; BL-8040 for treating acute myeloid leukemia (AML) and other hematological indications, which is in the midst of a Phase 2 study; BL-7010 for celiac disease, which is in the midst of a Phase 1/2 study; and BL-5010 for non-surgical removal of skin lesions, which is expected to

commence a pivotal CE-mark registration trial in the first half of 2014.

For more information on BioLineRx, please visit [www.biolinerx.com](http://www.biolinerx.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 17, 2014. 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.*

<sup>1</sup> [Combination of Imatinib with CXCR4 antagonist BKT140 overcomes the protective effect of stroma and targets CML in vitro and in vivo.](#)

Beider K, Darash-Yahana M, Blaier O, Koren-Michowitz M, Abraham M, Wald H, Wald O, Galun E, Eizenberg O, Peled A, Nagler A. Mol Cancer Ther. 2014 Feb 6. [Epub ahead of print]

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