



BioLineRx Announces Initiation of Phase 1/2 Trial for Novel Treatment in Two Bone Marrow Failure Conditions

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Efficacy and safety study, in collaboration with MD Anderson Cancer Center, will assess BL-8040 as combination treatment with immunosuppressants

Interim results of study, in patients with hypoplastic myelodysplastic syndrome (hMDS) and aplastic anemia (AA), expected by end of 2016

TEL AVIV, Israel--(BUSINESS WIRE)--Nov. 23, 2015-- [BioLineRx Ltd.](#) (NASDAQ/TASE: BLRX), a clinical-stage biopharmaceutical company dedicated to identifying, in-licensing and developing promising therapeutic candidates, announced today that it has commenced a Phase 1/2 trial for BL-8040, in combination with standard of care immunosuppressive therapy, as a novel treatment for two bone marrow failure conditions: hypoplastic myelodysplastic syndrome (hMDS) and aplastic anemia (AA).

The open-label trial, conducted in collaboration with MD Anderson Cancer Center in Houston, will examine BL-8040's ability to improve bone marrow cellularity and peripheral blood counts in up to 25 patients suffering from these bone marrow failure conditions. The study's primary endpoint is to evaluate the safety and tolerability of treatment with BL-8040 on top of the standard immunosuppressive regimen of Anti-Thymocyte Globulin (hATG), Cyclosporine and Methylprednisolone (steroids) in hMDS and AA patients. Secondary endpoints include assessment of the clinical efficacy (response rate), time and duration of response to the treatment, and overall survival following treatment. Safety and efficacy will be assessed at defined time points throughout the study. Duration of response and overall survival will also be assessed as part of the study's long term follow up protocol.

Study patients will initially receive BL-8040 as a monotherapy for ten days. From Day 11 through Day 14, patients will receive hATG, Methylprednisolone and Cyclosporine. Subsequently, during the first month of treatment, from Day 15 until Day 30, patients will continue treatment only with Methylprednisolone and Cyclosporine. Beginning in the second month, patients will continue daily treatments with Cyclosporine through the end of the sixth month, which is the end of the study treatment. In addition, beginning in the second month, BL-8040 will be administered daily as part of the maintenance period for the first 5 days of each month, also until the end of the six-month study period.

Both hMDS and AA are characterized by a T cell-driven autoimmune attack on the bone marrow that results in depletion of hematopoietic precursors, leading to anemia and low white blood cell counts. In this regard, high CXCR4 expression on pathogenic T cells has been suggested to facilitate infiltration to the bone marrow. BL-8040, a CXCR4 antagonist, is expected to inhibit migration of pathogenic T cells to the bone marrow, thereby mitigating the severe depletion of hematopoietic stem and progenitor cells.

In addition, BL-8040 may directly affect the number of hematopoietic precursors. Preclinical studies in mice showed that multiple doses of BL-8040 led to a marked increase in the number of hematopoietic progenitor cells and hematopoietic stem cells in both the bone marrow and peripheral blood. BL-8040 also promoted production of megakaryocytes in the bone marrow, leading to a prolonged increased platelet production. These direct effects of BL-8040, along with the exclusion of the pathogenic T cells from the bone marrow, may improve bone marrow cellularity and peripheral blood counts.

Dr. Kinneret Savitsky, CEO of BioLineRx, stated, "We are very pleased to commence an additional clinical trial for BL-8040, our unique oncology platform, in these two orphan designations that represent significant unmet medical needs. This study expands our existing successful collaboration with the MD Anderson Cancer Center, where we are already running a Phase 2 trial in relapsed/refractory AML. The hMDS/AA trial will assess BL-8040 in combination with standard of care immunosuppressive therapy, with interim results expected by the end of 2016. We are very hopeful that BL-8040, as part of a novel treatment regimen, will significantly improve bone marrow cellularity and peripheral blood counts in patients suffering from these difficult bone marrow failure conditions."

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 is currently in the midst of a Phase 2 study for relapsed/refractory acute myeloid leukemia (AML) and has recently initiated a Phase 2b study as an AML consolidation treatment. In addition, 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 hMDS and AA

Hypoplastic myelodysplastic syndrome (hMDS) and aplastic anemia (AA) are hematological conditions caused by progressive bone marrow failure, and characterized by ineffective production of all blood cells, leading to severe anemia and cytopenias (low blood counts). hMDS and AA result from disorders of the hematopoietic stem cells in the bone marrow. Hematopoiesis is disrupted and the number and quality of blood-forming cells decline irreversibly, further impairing blood production. Treatment may include immunosuppressive therapy, chemotherapy or hematopoietic stem cell transplant.

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 AML, has recently initiated a Phase 2b study as an AML consolidation treatment, has recently initiated a Phase 1/2 study in hMDS and AA, 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.bioglinerx.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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