BioLineRx Announces Oral Presentation on Data from Phase 1 Clinical Trial Evaluating Motixafortide for CD34+ Hematopoietic Stem Cell Mobilization for Gene Therapies in Sickle Cell Disease at ASH 2024

November 5, 2024 2:00 PM IST

- Findings suggest motixafortide alone, and in combination with natalizumab, could support the collection of the large number of stem cells required by gene therapies for sickle cell disease within a single apheresis cycle -

- Data from proof-of-concept study shows that motixafortide was safe and well tolerated -

- Oral presentation at ASH 2024 on Saturday, December 7, 2024 in San Diego, California -

TEL AVIV, Israel and WALTHAM, Mass., Nov. 5, 2024 /PRNewswire/ -- BioLineRx Ltd. (NASDAQ: BLRX) (TASE: BLRX), a commercial stage biopharmaceutical company pursuing life-changing therapies in oncology and rare diseases, today announced that an abstract including the initial results from a Phase 1 clinical trial evaluating motixafortide as monotherapy and in combination with natalizumab for CD34+ hematopoietic stem cell (HSC) mobilization for gene therapies in sickle cell disease (SCD) was accepted for oral presentation at the 66th American Society of Hematology (ASH) Annual Meeting & Exposition taking place December 7-10, 2024 in San Diego, California. The proof-of-concept study, conducted in collaboration with Washington University School of Medicine in St. Louis, is exploring alternative HSC mobilization strategies that could significantly improve the treatment journey of patients with sickle cell disease seeking gene therapy.

"Currently available gene therapies for sickle cell disease rely on the collection of significant quantities of CD34+ hematopoietic stem cells, posing challenges for many patients," said Zachary Crees, MD, principal investigator for the trial, Division of Oncology, Washington University School of Medicine. "The findings in this trial suggest that patients with sickle cell disease given motixafortide alone, or in combination with natalizumab, could mobilize and potentially collect the number of stem cells required for approved gene therapies in a single apheresis cycle. These are encouraging findings that we look forward to presenting in greater detail at ASH 2024."

"We are encouraged by the initial findings in this Phase 1 study showing that motixafortide is safe and well-tolerated and may hold potential to improve the overall treatment process and access to gene therapy for more people with SCD," said Philip Serlin, Chief Executive Officer of BioLineRx. "We look forward to continued collaboration with Washington University on this important research and our ongoing work to develop motixafortide for the potential benefit of patients with sickle cell disease."

The Phase 1 safety and feasibility study is evaluating motixafortide (CXCR4 inhibitor) as monotherapy and in combination with natalizumab (VLA-4 inhibitor) as novel regimens to mobilize CD34+ hematopoietic stem cells for gene therapies in SCD. As reported in the abstract, five patients completed mobilization and apheresis with motixafortide alone, and four of five with motixafortide in combination with natalizumab.

Motixafortide alone, and in combination with natalizumab, were safe and well-tolerated in the trial. Common adverse events (AEs) were transient and included Grade 1-2 injection site (pruritis, tingling/pain) and systemic reactions (pruritis, hives). No Grade 4 AEs or vaso-occlusive events occurred.

Motixafortide alone, and in combination with natalizumab, resulted in robust CD34+ HSC mobilization to peripheral blood (PB). Motixafortide alone mobilized a median of 198 CD34+ cells/µl (range 77-690) to PB with median 3.49x10 CD34+ cells/kg as part of a single blood volume collection, projecting the collection of 13.9x10^6 HSCs in a normal, single-day four blood volume apheresis collection session. Motixafortide in combination with natalizumab mobilized a median of 231 CD34+ cells/µl (range 117-408), with median 4.64x10 CD34+ cells/kg collected as part of a single blood volume collection, projecting the collection of 18.6x10^6 CD34+ HSCs in a single day four blood volume apheresis collection session.

The two approved gene therapies for sickle cell disease in the U.S. require 16.5 million, and 22 million, total CD34+ HSCs, respectively.[i,ii] Unfortunately, granulocyte colony-stimulating factor (G-CSF), the most commonly used drug to support the collection of stem cells, is contraindicated in patients with SCD. The use of the mobilization agent plerixafor is the current standard of care for collecting HSCs for SCD gene therapies; however, plerixafor alone requires multiple mobilization attempts and often yields suboptimal HSC numbers. For some, gene therapy may be prohibitive due to the failure to obtain adequate numbers of HSCs.

In the trial, patients who underwent prior mobilization with plerixafor, experienced 2.8- fold greater HSC mobilization with motixafortide alone, and 3.2-fold greater HSC mobilization with motixafortide in combination with natalizumab compared to plerixafor.

Oral Presentation at ASH 2024
San Diego Convention Center, San Diego, California
Oral Presentation Details

Session Name: 711. Cell Collection and Manufacturing of HSPCs, CAR-T Cells, and Other Cellular Therapy Products: Innovations in Mobilization, Collection, and Manufacturing for Cellular Therapies

Title: Motixafortide (CXCR4 Inhibition) Alone and in Combination with Natalizumab (VLA-4 Inhibition) As a Novel Regimen to Mobilize Hematopoietic

Stem Cells for Gene Therapies in Sickle Cell Disease: A First-in-Human, Proof-of-Principle Safety and Feasibility Study

Presenter: Zachary D. Crees, MD, Division of Oncology, Washington University School of Medicine, Saint Louis, MO

Abstract ID#: 193210

Date: Saturday, December 7, 2024

Time: 12:00 PM

Location: San Diego Convention Center, Room 25

About the Clinical Trial of Motixafortide in Sickle Cell Disease (SCD)
The trial (ClinicalTrials.gov Identifier: NCT05618301) is a safety and feasibility study to evaluate motixafortide (CXCR4 inhibitor) as monotherapy and in combination with natalizumab (VLA-4 inhibitor) as novel regimens to mobilize CD34+ hematopoietic stem cells for gene therapies in SCD. The study enrolled five adults with a diagnosis of SCD who are receiving automated red blood cell exchanges via apheresis. The trial's primary objective is to assess the safety and tolerability of motixafortide alone and the combination of motixafortide + natalizumab in SCD patients, defined by dose-limiting toxicities. Secondary objectives include determining the number of CD34+ hematopoietic stem and progenitor cells (HSPCs) mobilized via apheresis; and determining the kinetics of CD34+ HSPC mobilization to peripheral blood in response to motixafortide alone and motixafortide + natalizumab in SCD patients.

About Sickle Cell Disease
Sickle cell disease (SCD) is one of the most common genetic diseases globally, affecting millions of people throughout the world and disproportionately impacting persons of color. Sickle cell disease arises from mutations in the hemoglobin gene, ultimately leading to the production of abnormally shaped (sickle) red blood cells that tend to stick within blood vessels causing their occlusion. The clinical manifestations of SCD include anemia and blood vessel occlusion which can lead to both acute and chronic pain, as well as tissue ischemia across multiple organ systems (e.g., stroke, heart attack, respiratory failure), ultimately compromising end organ function. The cumulative impact of these complications significantly impacts morbidity and mortality for patients with SCD.

About BioLineRx
BioLineRx Ltd. (NASDAQ: BLRX) (TASE: BLRX) is a commercial stage biopharmaceutical company pursuing life-changing therapies in oncology and rare diseases. The company's first approved product is APHEXDA® (motixafortide) with an indication in the U.S. for stem cell mobilization for autologous transplantation in multiple myeloma. BioLineRx is advancing a pipeline of investigational medicines for patients with sickle cell disease, pancreatic cancer, and other solid tumors. Headquartered in Israel, and with operations in the U.S., the company is driving innovative therapeutics with end-to-end expertise in development and commercialization, ensuring life-changing discoveries move beyond the bench to the bedside.

Learn more about who we are, what we do, and how we do it at www.biolinerx.com, or on Twitter and LinkedIn.

Forward Looking Statement
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 "anticipates," "believes," "could," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," and "would," and describe opinions about future events. These include statements regarding management's expectations, beliefs and intentions regarding, among other things, the potential benefits of APHEXDA, the execution of the launch of APHEXDA and the plans and objectives of management for future operations and expectations and commercial potential of motixafortide, as well as its potential investigational uses. These forward-looking statements involve known and unknown risks, uncertainties and other factors 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. Factors that could cause BioLineRx's actual results to differ materially from those expressed or implied in such forward-looking statements include, but are not limited to: the initiation, timing, progress and results of BioLineRx's preclinical studies, clinical trials, and other therapeutic candidate development efforts; BioLineRx's ability to advance its therapeutic candidates into clinical trials or to successfully complete its preclinical studies or clinical trials; whether BioLineRx's collaboration partners will be able to execute on collaboration goals in a timely manner; whether the clinical trial results for APHEXDA will be predictive of real-world results; BioLineRx's receipt of regulatory approvals for its therapeutic candidates, and the timing of other regulatory filings and approvals; the clinical development, commercialization and market acceptance of BioLineRx's therapeutic candidates, including the degree and pace of market uptake of APHEXDA for the mobilization of hematopoietic stem cells for autologous transplantation in multiple myeloma patients; whether access to APHEXDA is achieved in a commercially viable manner and whether APHEXDA receives adequate reimbursement from third-party payors; BioLineRx's ability to establish, operationalize and maintain corporate collaborations; BioLineRx's ability to integrate new therapeutic candidates and new personnel; the interpretation of the properties and characteristics of BioLineRx's therapeutic candidates and of the results obtained with its therapeutic candidates in preclinical studies or clinical trials; the implementation of BioLineRx's business model and strategic plans for its business and therapeutic candidates; the scope of protection BioLineRx is able to establish and maintain for intellectual property rights covering its therapeutic candidates and its ability to operate its business without infringing the intellectual property rights of others; estimates of BioLineRx's expenses, future revenues, capital requirements and its needs for and ability to access sufficient additional financing, including any unexpected costs or delays in the commercial launch of APHEXDA; risks related to changes in healthcare laws, rules and regulations in the United States or elsewhere; competitive companies, technologies and BioLineRx's industry; statements as to the impact of the political and security situation in Israel on BioLineRx's business; and the impact of the COVID-19 pandemic, the Russian invasion of Ukraine, the declared war by Israel against Hamas and the military campaigns against Hamas and other terrorist organizations, which may exacerbate the magnitude of the factors discussed above. 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 26, 2024. 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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i LYFGENIA Prescribing Information; December 2023.

ii CASGEVY Prescribing Information; December 2023.

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