Topline Results from MYLOX-1 Trial Demonstrate Reduction in Fibrosis of the Bone Marrow in Patients with Myelofibrosis

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Six of ten evaluable myelofibrosis patients who received GB2064 monotherapy for at least six months experienced a ≥ 1-grade reduction in collagen fibrosis of the bone marrow, validating LOXL2 as a clinical fibrosis target

BOSTON, Dec. 21, 2023 (GLOBE NEWSWIRE) -- Galecto, Inc. (NASDAQ: GLTO), a clinical stage biotechnology company focused on the development of novel treatments for fibrosis and cancer, today announced positive topline results from a Phase 2a trial of GB2064 for the treatment of myelofibrosis (the "MYLOX-1 trial", NCT04679870).

The MYLOX-1 trial dosed a total of 18 myelofibrosis patients, of which 11 (61%) patients had previously received janus kinase inhibitor (JAKi) therapy with ruxolitinib, with eight of those patients being refractory and three being intolerant to JAKi therapy. Six out of ten evaluable myelofibrosis patients who received GB2064 monotherapy for at least six months experienced a ≥ 1-grade reduction in collagen fibrosis of the bone marrow, an improvement suggesting that GB2064 could impact the progression of the disease and be disease-modifying.

Fibrosis is a key disease mechanism of myelofibrosis that destroys bone marrow function. Reducing fibrosis is required to slow the progression of the disease. Bone marrow biopsies taken during the study showed that GB2064 penetrated the bone marrow and could exert its anti-fibrotic effect directly in the disease compartment. Furthermore, GB2064 demonstrated target engagement systemically by binding to LOXL2 in plasma.

All six patients who experienced a > 1-grade reduction in bone marrow fibrosis score also showed stable hematological parameters, including hemoglobin, white blood cell count and platelets. At six months of treatment, one patient obtained a ≥35% reduction in spleen volume, two patients reduced their Total Symptom Score (TSS) by more than 50% and one patient had an anemia response. Four of these patients have entered the extension phase of the study due to the clinical benefit derived from GB2064 as evaluated by the treating physician, with one patient receiving treatment for more than 30 months.

Professor Claire Harrison, Guy's and St Thomas' NHS Foundation Trust, and Chair of the Safety Review Committee for the MYLOX-1 trial, commented, "It is exciting and encouraging to see that the data from the MYLOX-1 trial affirms the safety and effectiveness of LOXL-2 inhibition in the challenging landscape of myelofibrosis. I am especially intrigued by the unique observed improvements in bone marrow collagen fibrosis, showcasing the targeted impact on a crucial aspect of this relentless disease."

GB2064 showed a generally acceptable tolerability profile in the MYLOX-1 trial. Eighteen patients were dosed with GB2064 monotherapy in the MYLOX-1 trial. Eight patients completed treatment in the core phase of the MYLOX-1 trial and ten patients discontinued treatment due to an adverse event or disease progression. The most commonly observed treatment-related adverse events were gastrointestinal in nature and were manageable in most patients with standard therapy. The only treatment-related serious adverse event was a case of fall, which was assessed as possibly related to GB2064 by the investigator.

Dr. Hans Schambye, President and Chief Executive Officer of Galecto, commented, "We believe that the topline results from the MYLOX-1 trial reaffirm the anti-fibrotic activity observed in the intermediate assessment of the trial announced in September 2022. We are very excited with the proof of principle achieved with GB2064, showcasing its strong anti-fibrotic impact in a very challenging patient population. The encouraging topline results from the MYLOX-1 trial reinforce our confidence in GB2064's potential as a transformative therapy for various cancers and a range of fibrotic diseases, but we will not make any decisions relating to funding additional trials with GB2064 until we complete our previously announced strategic alternative process."

About the MYLOX-1 Trial
The MYLOX-1 trial was a Phase 2, open-label, single-arm study in myelofibrosis patients who were ineligible, refractory, or intolerant to JAKi therapy. These patients have a progressive disease with poor quality of life, high mortality rates and very limited treatment options. Patients received GB2064 orally at a dose of 1000mg twice daily for nine months and undergo bone marrow biopsies at the beginning of the trial and again at months 3, 6 and 9. The primary endpoint of the MYLOX-1 trial was to assess the safety and tolerability of GB2064.

Apart from evaluating the safety and tolerability of GB2064, key secondary objectives of the MYLOX-1 trial were to evaluate hematological parameters as well as the direct anti-fibrotic activity of GB2064 by blocking lysyl oxidase-like 2 (LOXL2) in an indication that allows for repeated tissue biopsies.

About Myelofibrosis
Myelofibrosis is a hematological cancer that causes fibrosis of the bone marrow and disrupts the body's normal production of blood cells, which can lead to multiple negative impacts and a significantly reduced quality of life and mortality. The bone marrow is destroyed by fibrosis, forcing out the production of blood components and aggravating symptoms, including anemia, thrombocytopenia, leukocytosis, and spleen enlargement. JAKi therapy is the current standard of care for patients with myelofibrosis; however, these therapies do not address the core of the underlying disease biology and have not shown a consistent effect on fibrosis, biomarkers of disease modification, or overall survival.

About LOXL2 and GB2064
GB2064, a potentially first-in-class, oral, lysyl oxidase-like 2 (LOXL2) inhibitor candidate, is in development for the treatment of fibrotic diseases and cancer. LOXL2 is an enzyme that plays a key role in myelofibrosis and contributes to the fibrotic progression of the disease. LOXL2 catalyzes the cross-linking of collagen, forming the backbone of fibrosis. The molecular target for GB2064 is LOXL2, an enzyme that plays a central role in the crosslinking of collagen in tissue fibrosis and is involved in multiple types of fibrotic diseases, including myelofibrosis. In contrast to previous attempts to inhibit LOXL2 with a monoclonal antibody, GB2064 is designed to completely inhibit the LOXL2 enzymatic activity.

About Galecto
Galecto is a clinical stage company incorporated in the U.S. that is developing small molecule-based inhibitors of galectin-3 and LOXL2. Galecto has

multiple Phase 2 clinical opportunities in fibrosis and cancer, including (i) an orally active LOXL2 inhibitor (GB2064) for the treatment of myelofibrosis; (ii) an orally active galectin-3 inhibitor (GB1211) for the treatment of liver cirrhosis; and (iii) an orally active galectin-3 inhibitor (GB1211) in combination with a checkpoint inhibitor for various oncology indications.

Galecto intends to use its website as a means of disclosing material non-public information. For regular updates about Galecto, visit www.galecto.com.

Forward-Looking Statements
This press release contains forward-looking statements that involve a number of risks and uncertainties. Such forward-looking statements include statements about the safety and efficacy of GB2064 and Galecto's plans, strategies and prospects for clinical development of GB2064. Such forward-looking statements include statements about Galecto's focus, plans for clinical development, product candidates and pipeline. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. For such statements, Galecto claims the protection of the Private Securities Litigation Reform Act of 1995. Actual events or results may differ materially from Galecto's expectations. Factors that could cause actual results to differ materially from the forward-looking statements include risks and uncertainties related to whether preliminary data that is reported herein changes following a more comprehensive review of the data related to the clinical trial and as more patient data become available or as additional analyses are conducted, the ongoing development of Galecto's product candidates and evaluation of their therapeutic potential, including emerging data on the safety profile of such candidates and their potential for disease-modifying activity, having adequate funds and their use, and those disclosed in Galecto's filings with the Securities and Exchange Commission (SEC), including, but not limited to, Galecto's Annual Report on Form 10-K, as filed with the SEC on March 9, 2023, and Galecto's Quarterly Reports on Form 10-Q. These forward-looking statements represent Galecto's judgment as of the time of this release. Galecto disclaims any intent or obligation to update these forward-looking statements, other than as may be required under applicable law.

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