BlossomHill Therapeutics Presents Initial Clinical Data from Dose Escalation in Ongoing Phase 1/1b Trial of Novel Macrocyclic CLK Inhibitor, BH-30236, in Patients with R/R AML or HR-MDS at EHA 2026

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BH-30236 was generally well tolerated and demonstrated preliminary anti-leukemic activity, including in patients previously treated with venetoclax-based regimens, along with favorable pharmacokinetic properties

SAN DIEGO, June 11, 2026 (GLOBE NEWSWIRE) -- BlossomHill Therapeutics, Inc., a privately-held, clinical-stage biopharmaceutical company applying an intentional, chemistry-based approach to design and develop innovative small molecule medicines for the treatment of cancer, today presented initial clinical data from its ongoing first-in-human Phase 1/1b trial of BH-30236 in relapsed or refractory acute myeloid leukemia (R/R AML) and higher-risk myelodysplastic syndrome (HR-MDS) at the European Hematology Association (EHA) 2026 Congress in Stockholm, Sweden. BH-30236 is an intentionally designed novel, orally bioavailable, macrocyclic CDC-like kinase (CLK) inhibitor that modulates aberrant alternative mRNA splicing, a defining feature implicated in cancer progression and therapeutic resistance across both hematologic malignancies and solid tumors.

“The preliminary anti-leukemic activity observed with BH-30236 in R/R AML and HR-MDS, both as a monotherapy and in combination with venetoclax, is very encouraging,” said Geoff Oxnard, M.D., Chief Medical Officer of BlossomHill Therapeutics. “In addition to the reassuring tolerability profile, we have seen provocative responses in patients after progression on prior venetoclax-based therapy, a population with few treatment options. In the ongoing study of BH-30236, we will further characterize the effect of this novel mechanism in myeloid malignancies, both as a monotherapy and in synergy with venetoclax to maximize the potential of this promising program.”

“We intentionally designed BH-30236 to target CLK, which is upstream of multiple resistance pathways, not by addressing one resistance mechanism after it develops, but by suppressing the splicing machinery that enables resistance to begin,” said Jean Cui, Ph.D., Founder and Chief Executive Officer of BlossomHill Therapeutics. “We believe these data further validate our approach of leveraging a deep understanding of disease and protein dynamics and applying our structure-based rational drug design expertise to identify specific structural liabilities that limit existing therapies or approaches and design novel chemical scaffolds to directly address these limitations.”

Presentation Highlights:

- As of the data cutoff date of April 10, 2026, 30 patients received BH-30236 monotherapy at doses ranging from 5 to 120 mg on a continuous daily administration schedule (QD), and 18 patients received BH-30236 at doses ranging from 20 to 90 mg QD in combination with venetoclax
- BH-30236 was generally well tolerated as both a monotherapy and in combination with venetoclax, with most treatment-related adverse events being low-grade and manageable, with one dose limiting toxicity of Grade 3 diarrhea at the 120 mg QD monotherapy dose level
- Dose escalation showed predictable pharmacokinetics without drug accumulation, and no significant drug-drug interactions were observed with venetoclax
- With efficacy follow-up through May 20, 2026, preliminary anti-leukemic activity was observed in patients with R/R AML and HR-MDS when treated with BH-30236 as a monotherapy or in combination with venetoclax, including in patients previously relapsed or refractory to venetoclax-based therapy:
    - In the monotherapy cohort, 28.6% (n=6) of evaluable patients achieved at least a 50% reduction in bone marrow blast counts, including one HR-MDS patient treated at 60 mg with ongoing blast count reduction with treatment ongoing for more than a year
    - In the combination cohort, 87% (n=13) of patients had prior venetoclax exposure and 53% (n=8) of evaluable patients experienced at least a 50% blast reduction, including one patient refractory to all prior therapy including venetoclax, who achieved a minimal residual disease (MRD)-negative complete remission

About R/R AML and HR-MDS
AML is the most common acute leukemia in adults, with approximately 21,000 new diagnoses annually in the United States, with as many as 75% of those being patients resistant to or relapsing on first-line venetoclax therapy. Approximately 14,000 patients are diagnosed with HR-MDS annually in the United States. There is no currently approved targeted therapy for the venetoclax-resistant and refractory segment of AML patients without targetable genetic alterations. Patients with HR-MDS closely mirror AML in clinical course, frontline treatment, and unmet need in the relapsed and refractory setting.

About BH-30236
BH-30236 is an investigational orally bioavailable, macrocyclic inhibitor of the CDC-like kinase (CLK) family. BH-30236 is was intentionally designed to potently inhibit CLK, leading to modulation of aberrant alternative splicing in cancerous tissue, targeting the same aberrant splicing machinery that drives relapsed or refractory (R/R) acute myeloid leukemia (AML) and higher-risk

myelodysplastic syndrome (HR-MDS) disease biology and that cancer cells exploit to develop resistance to venetoclax, FLT3 inhibitors and cytarabine.

BH-30236 is being evaluated in a Phase 1/1b multicenter, open-label, first-in-human dose escalation and expansion trial in adults with R/R AML and HR-MDS. The U.S. Food and Drug Administration (FDA) has granted orphan drug designation to BH-30236 for the treatment of AML.

About BlossomHill Therapeutics
BlossomHill Therapeutics, Inc. is a clinical-stage biopharmaceutical company applying an intentional, chemistry-based approach to design and develop innovative small molecule medicines that address significant unmet medical needs in cancer treatment. Founded and led by industry veteran J. Jean Cui, Ph.D., with her proven track record in oncology drug design and development – including three FDA-approved drugs – BlossomHill applies cutting-edge science to address key oncogenic drivers and improve patient outcomes in difficult-to-treat cancers. The company's lead clinical programs include BH-30643, an investigational, macrocyclic, non-covalent, mutant-selective OMNI-EGFR^{TM} inhibitor for the treatment of EGFR-mutant non-small cell lung cancer (NSCLC) with an initial development focus in the C797S resistance population after 3rd generation EGFR TKI treatment, BH-30236, an investigational macrocyclic CDC-like kinase (CLK) inhibitor initially being developed for the treatment of relapsed or refractory acute myeloid leukemia (R/R AML) or higher-risk myelodysplastic syndrome (HR-MDS) and BH-501284, our pre-clinical, non-covalent, selective, pan-KRAS Switch II inhibitor for potential future development in diverse KRAS-mutant tumors. BlossomHill Therapeutics is headquartered in San Diego, California and has raised over $257 million since its inception from experienced healthcare-focused investors. For more information, visit bhtherapeutics.com and follow us on LinkedIn and X.

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