BlossomHill Therapeutics to Present Initial Clinical Dose Escalation Data from the Phase 1/1b Trial of BH-30236 in Patients with R/R AML or HR-MDS at EHA2026

May 12, 2026

Treatment with BH-30236, a novel CLK inhibitor, was generally well tolerated as monotherapy and in combination with venetoclax

Preliminary anti-leukemic activity observed, including patients that were previously treated with venetoclax based regimens

U.S. FDA orphan drug designation granted to BH-30236 for the treatment of AML

SAN DIEGO, May 12, 2026 (GLOBE NEWSWIRE) -- BlossomHill Therapeutics, Inc., a privately-held, clinical-stage biopharmaceutical company applying an intentional, chemistry-based approach to develop innovative small molecule medicines for the treatment of cancer, today announced that initial dose escalation data from its Phase 1/1b trial of BH-30236, a macrocyclic CDC-link kinase (CLK) inhibitor, in relapsed or refractory acute myeloid leukemia (R/R AML) and higher-risk myelodysplastic syndrome (HR-MDS) will be presented during a poster session on June 12, 2026, at the European Hematology Association (EHA) 2026 Congress in Stockholm, Sweden. Additionally, BH-30236 has been granted orphan drug designation by the U.S. Food and Drug Administration for the treatment of AML.

“We are highly encouraged by the initial safety data and early signs of anti-leukemic activity observed with BH-30236, both as a monotherapy and in combination with venetoclax,” said Jean Cui, Ph.D., Founder and Chief Executive Officer of BlossomHill Therapeutics. “By modulating aberrant alternative mRNA splicing, our potential first-in-class CLK inhibitor, BH-30236, represents a novel approach for patients with relapsed or refractory AML and higher-risk MDS, who face a significant unmet medical need. We look forward to presenting additional data from more patients with longer follow-up at the meeting and to advancing BH-30236 in both monotherapy and combination settings.”

BH-30236 was designed to target the CLK kinase family, leading to the modulation of aberrant alternative mRNA splicing, a defining feature implicated in cancer progression and therapeutic resistance across both hematologic malignancies and solid tumors. Our Phase 1/1b trial evaluating BH-30236 was initiated following encouraging preclinical data, including evidence of synergistic activity between BH-30236 and venetoclax, a BCL-2 inhibitor and an established standard of care therapy for patients with AML. The trial is currently enrolling patients in dose escalation, evaluating BH-30236 as both a monotherapy and in combination with venetoclax.

As of the January 23, 2026 cutoff date for the EHA abstract submission, 28 patients received BH-30236 monotherapy at 5-120 mg on a continuous daily administration schedule (QD) and 11 patients received BH-30236 at 20-60mg QD in combination with venetoclax. Initial findings demonstrated:

- 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, as well as one grade 3 DLT (diarrhea)
- Dose escalation showed predictable pharmacokinetics without drug accumulation or reduction, and no significant drug-drug interactions were observed with venetoclax
- Early signs of clinical activity were observed:
    - In the monotherapy cohort, 29% (n=5) 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 duration of treatment 7.6 months.
    - In the combination cohort, 55% (n=5) 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.

“In March of this year, the U.S. Food and Drug Administration granted Orphan Drug Designation to BH-30236 for the treatment of acute myeloid leukemia, underscoring the need for new therapies in this rare malignancy,” said Dr. Geoff Oxnard, Chief Medical Officer of BlossomHill Therapeutics. “This designation is an important milestone that reflects the potential of BH-30236 to address a significant unmet need and provides benefits that may support our ongoing clinical development, as well as potential market exclusivity upon approval.”

Poster Session Details

- **Title:** A First-in-Human Study of the Oral CLK Inhibitor BH-30236 in Adults with Relapsed/Refractory Acute Myeloid Leukemia or Higher-Risk Myelodysplastic Syndrome: Monotherapy and Venetoclax Combination
- **Presenting Author:** Eytan M. Stein, MD, Chief of the Leukemia Service, Associate Attending Physician, Clinical Investigator and Director of the Program for Drug Development in Leukemia on the Leukemia Service at Memorial Sloan

Kettering Cancer Center
- **Date & Time:** Friday, June 12 from 12:45 to 1:45 ET / 18:45 - 19:45 CEST
- **Abstract Code:** PF494

About BH-30236
BH-30236 is an investigational orally bioavailable, macrocyclic inhibitor of the CDC-like kinase (CLK) family. BH-30236 is designed to modulate aberrant alternative splicing in cancerous tissue, targeting the same aberrant splicing machinery that drives 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 currently in clinical development for the treatment of relapsed or refractory AML (R/R AML) and HR-MDS.

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 has granted orphan drug designation to BH-30236 for the treatment of AML.

About BlossomHill Therapeutics
BlossomHill Therapeutics, Inc. is a privately held, clinical-stage biopharmaceutical company applying an intentional, chemistry-based approach to develop innovative small molecule medicines which 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, a potential first-in-class, macrocyclic, non-covalent, mutant-selective OMNI-EGFR^{TM} inhibitor for the treatment of EGFR-mutated non-small cell lung cancer (NSCLC), and BH-30236, a macrocyclic CLK inhibitor for the treatment of relapsed or refractory acute myeloid leukemia (R/R AML) or higher-risk myelodysplastic syndrome (HR-MDS), representing a potential first-in-class opportunity. BlossomHill Therapeutics is headquartered in San Diego, California and has raised over $257 million since its inception from leading life sciences investors. For more information, visit bhtherapeutics.com and follow us on LinkedIn and X.

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