

Amphista Therapeutics announces first patient dosed in AMX-883 Phase 1 clinical trial for acute myeloid leukemia

- *AMX-883, Amphista's first asset to enter the clinic, is a potent, selective, orally bioavailable DCAF16-mediated BRD9 degrader targeting acute myeloid leukemia (AML)*
- *The first-in-human 'BRAMLIE' (BR(D9) AML Interventional Evaluation) trial will assess the safety, pharmacokinetics and preliminary efficacy of AMX-883 monotherapy in participants with AML and high-risk myelodysplastic syndrome (MDS).*
- *AMX-883 demonstrates karyotype-independent pro-differentiation activity pre-clinically, with trial enrolling patients regardless of mutational status*

Cambridge, UK, 12 October 2026: Amphista Therapeutics Limited ('Amphista' or 'the Company'), a leader in the discovery and development of non-cereblon / VHL targeted protein degradation (TPD) medicines, today announced dosing of the first patient in a Phase 1 trial for its lead candidate, AMX-883. Initiated following authorization from the U.S. Food and Drug Administration in June 2026, the BRAMLIE trial will investigate the safety, pharmacokinetics, and preliminary efficacy of AMX-883 monotherapy in participants with AML and high-risk MDS¹.

BRAMLIE is expected to enroll approximately 54 patients across multiple sites. After establishing the monotherapy profile, a multi-center dose-escalation study would evaluate AMX-883 in combination with current standards of care in participants with relapsed/refractory AML as well as in earlier lines of therapy.

AML is an aggressive blood cancer causing ~130,000 deaths globally each year, with most patients receiving traditional cytotoxic chemotherapy. Although novel targeted agents can be effective, the use of these is restricted to specific mutations – and therefore target only a subset of AML patients - highlighting a need for broad-acting therapeutics.

Acting via a targeted protein degradation (TPD) mechanism, Amphista's Targeted Glue™ degraders are highly selective small molecule therapeutics with properties optimized for human medicines. AMX-883 is a potent, selective, orally bioavailable Targeted Glue™ of BRD9 - a subunit of the chromatin remodeling complex involved in maintaining leukemic stemness and blocking of myeloid cell differentiation. Rapid BRD9 degradation is induced via AMX-883's novel mechanism of action by which it recruits the E3 ligase DCAF16, enabling exceptional selectivity. Preclinically, AMX-883 has demonstrated activity across diverse AML subtypes, independent of mutation status².

Dr Eytan Stein, Director, Program for Drug Development in Leukemia, Division of Hematologic Malignancies at Memorial Sloan Kettering Cancer Center, and Lead Investigator for this trial, said: *"With a five-year survival rate of just 33%, and emerging resistance to standard-of-care treatments like venetoclax, AML is one of the most devastating blood cancers, and there is an urgent need for innovative new therapeutics. AMX-883 is the only BRD9 degrader currently in development, and I look forward to seeing what this clinical stage research will reveal."*

Dr Louise Modis, Chief Executive Officer at Amphista, said: *“The progression of our lead asset into the clinic marks a significant milestone for Amphista in our mission to transform the lives of patients with severe diseases. This study will help us better understand the potential of AMX-883, our lead Targeted Glue™, and how it could establish an important new treatment pathway for patients facing this very difficult disease with high unmet medical need.”*

1. <https://clinicaltrials.gov/study/NCT07723703>
2. <https://www.nature.com/articles/s41467-025-63594-w>

ENDS



Dr Louise Modis, Chief Executive Officer, Amphista



Dr Eytan Stein, Director, Program for Drug Development in Leukemia, Division of Hematologic Malignancies, Memorial Sloan Kettering Cancer Center

For high-resolution images please contact Zyme Communications

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About Amphista Therapeutics:

Amphista Therapeutics is focused on transforming the lives of patients with severe diseases, including acute myeloid leukemia (AML) and non-small cell lung cancer (NSCLC), through the discovery of next-generation targeted protein degradation (TPD) medicines that exploit novel E3 ligases to degrade proteins of interest. With a differentiated mechanism of action and leading drug-like properties, Amphista's portfolio of unique bifunctional Targeted Glue degrader therapeutics has the potential to deliver first- and/or best-in-class therapeutics with performance characteristics beyond the limitations of CRBN- and VHL-based agents. The

Company's lead program for AML, AMX-883, is an orally bioavailable rapid degrader of BRD9 and is now in Phase 1 clinical development..

Amphista was co-founded by Advent Life Sciences and is additionally funded by a premier group of investors, including Forbion, Gilde Healthcare, Novartis Venture Fund, SV's Dementia Discovery Fund, and Eli Lilly. For more information, please visit: www.amphista.com

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