PureTech Reports Positive Topline Data from Phase 1b Trial of LYT-200 in Relapsed/Refractory (R/R) High-Risk (HR) Myelodysplastic Syndrome (MDS) and R/R Acute Myeloid Leukemia (AML)

April 22, 2026

RNS Number : 4332B
PureTech Health PLC
22 April 2026

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Phase 1b dataset with LYT-200 demonstrates complete responses and favorable tolerability across both R/R HR-MDS and R/R AML

PureTech's Founded Entity, Gallop Oncology, to advance LYT-200 first in R/R HR-MDS, with continued development planned in R/R AML

Company plans to engage with the U.S. Food and Drug Administration to discuss the design of a subsequent trial in R/R HR-MDS that has the potential to support registration

PureTech Health plc (Nasdaq: PRTC, LSE: PRTC) ("PureTech" or the "Company"), a hub-and-spoke biotherapeutics company dedicated to giving life to science and transforming innovation into value, today announced positive topline data from the completed Phase 1b clinical trial of LYT-200, a first-in-class, fully human anti-galectin-9 monoclonal antibody, in heavily pretreated patients with relapsed/refractory (R/R) high-risk (HR) myelodysplastic syndrome (MDS) and R/R acute myeloid leukemia (AML). Based on the results, PureTech's Founded Entity, Gallop Oncology, has selected a recommended Phase 2 dose (RP2D) and intends to engage with the U.S. Food and Drug Administration (FDA) to discuss the design of a subsequent trial that could potentially support registration of LYT-200 in R/R HR-MDS.

"The data from the completed Phase 1b trial highlight the potential for LYT-200 to offer a differentiated treatment approach across a range of myeloid hematological malignancies," said Aleksandra Filipovic, M.D., Ph.D., Head of Oncology at PureTech and Chief Medical Officer of Gallop Oncology. "Across patients with R/R HR-MDS and R/R AML, treatment with LYT-200 resulted in deep responses with an exceptionally favorable safety profile. Importantly, the data in R/R HR-MDS were particularly compelling and support prioritizing this indication, especially given the significant unmet need and lack of successful innovation to help these patients. We intend to engage with the FDA to

discuss the design of a subsequent trial in R/R HR-MDS, as our goal is to accelerate delivery of this promising first-in-class therapy to patients while also laying the foundation for broader clinical development, including in AML."

The completed Phase 1b trial (NCT05829226), conducted across nine U.S. sites, evaluated LYT-200 both as a monotherapy and in combination regimens in two heavily pretreated patient populations.
The study included dose escalation of monotherapy LYT-200, followed by dose escalation of LYT-200 in combination with a hypomethylating agent (HMA; azacitidine or decitabine) in patients with R/R HR-MDS and with venetoclax (VEN) and an HMA in R/R AML.

"The safety profile, combinatorial potential, and level of clinical activity observed with LYT-200 in this Phase 1b study across both R/R HR-MDS and R/R AML is very encouraging, particularly given the number of prior lines of treatment and the risk profile in the populations studied," said Amir T. Fathi, M.D., Program Director of the Center for Leukemia at the Mass General Brigham Cancer Institute and Professor of Medicine at Harvard Medical School. "In R/R high-risk MDS, where treatment options are extremely limited and outcomes are poor, the findings are particularly notable. In this context, the potential to achieve clinical responses without added toxicity would represent a meaningful advance in the MDS treatment landscape and warrants continued clinical development."

"The results from this Phase 1b trial provide a strong foundation for the next stage of development of LYT-200," said Eric Elenko, Ph.D., President and Co-founder of PureTech and Acting Chief Executive Officer of Gallop Oncology. "Our decision to prioritize relapsed/refractory high-risk MDS reflects a focused and disciplined approach, grounded in both the data generated to date and the potential to address a tremendous patient need. We intend to engage with the FDA to discuss a subsequent trial design with the potential to support registration, while continuing to evaluate the broader potential of LYT-200."

TOPLINE SAFETY DATA

LYT-200 demonstrated a favorable and consistent safety profile across all cohorts and dose levels studied (N=101), with no dose-limiting toxicities, infusion-related reactions, LYT-200 dose reductions, or LYT-200-related serious adverse events (AEs), discontinuations, or deaths. Importantly, no overlapping or additive toxicities were observed when LYT-200 was combined with an HMA or VEN/HMA.

Six patients at one study site reported experiencing hematology/chemistry-related Grade 3 or 4 AEs attributed as possibly related or related to LYT-200 in the combination arm at the RP2D dose. The reported AEs consisted of decreased levels of platelets, white blood cells, and neutrophils that were below the lower limit of normal physiological levels. The blood count deficits for some of the relevant patients were present at baseline prior to the administration of LYT-200 and are common occurrences in patients due to the underlying advanced MDS/AML, as well as in those receiving VEN/HMA treatment. No other sites reported Grade 3 or greater AEs related to LYT-200 treatment.

TOPLINE EFFICACY DATA

Treatment with LYT-200 in combination with an HMA in R/R HR-MDS patients and VEN/HMA in R/R AML patients demonstrated robust antileukemic activity, including complete responses, bridging to transplant, and durable clinical benefit. The data also provided important insights into the contribution of LYT-200 within combination regimens.

R/R HR-MDS

Across all efficacy-evaluable[1] patients (n=11), the recommended Phase 2 dose (LYT-200 12mg/kg in combination with an HMA) demonstrated:

- 27.3% complete response rate

- 9.1% partial response rate
- 9.1% marrow complete response rate
- 45.5% overall response rate
- 18% conversion to transplant rate

Due to the number of patients alive at the time of study completion (>50%), the upper bound of overall survival could not be calculated; therefore, the median overall survival for this cohort of 6.4 months is not considered fully mature.

Efficacy-evaluable patients had a median of 3 prior lines of therapy (range: 1-5), and all (100%) had previously been treated with an HMA. Additionally, all patients had high-risk cytogenetics, which - coupled with prior exposure to treatment - suggests biologically aggressive, treatment-refractory disease with elevated risk of progression and poor clinical outcomes. Taken together, these attributes underscore the potential mutation-agnostic mechanism of LYT-200 and its potential for broad clinical use.

R/R AML

Across all efficacy-evaluable[1] patients (n=26), LYT-200 12mg/kg in combination with VEN/HMA demonstrated:

- 30.8% composite complete response rate[2]; responders included patients with mutations associated with VEN resistance
- 7.7% partial response rate
- 42.3% overall response rate
- 19.2% conversion to transplant rate

Due to the number of patients alive at the time of study completion (50%), the upper bound of overall survival could not be calculated; therefore, the median overall survival for this cohort of 8.2 months is not considered fully mature.

Efficacy-evaluable patients had a median of 2 prior lines of therapy (range: 1-9), and 84.6% had previously been treated with VEN/HMA.

INITIAL PHARMACODYNAMIC FINDINGS

The systemic effects of LYT-200 were evaluated through pharmacodynamic analyses of peripheral blood mononuclear cells, a population of immune cells in the bloodstream that provides insight into how a treatment affects both the immune system and leukemic blast cells. These analyses suggest that LYT-200 engages complementary and potentially synergistic pathways directed at cancer cell killing and anti-cancer immune responses when combined with VEN and HMA-based therapy, which may contribute to the clinical activity observed in patients with relapsed/refractory disease following HMA and VEN/HMA treatment, in MDS and AML, respectively.

About Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) are a group of serious blood cancers characterized by ineffective blood cell production in the bone marrow, leading to anemia, infections, and bleeding complications. [3], [4] MDS affects approximately 60,000-170,000 people in the United States, with an estimated 30-40% of patients diagnosed with the more aggressive form of the disease known as high-risk (HR) MDS.[3, 5] HR-MDS is associated with poor outcomes, with median survival typically less than two years following diagnosis, and approximately 30% of patients progressing to acute myeloid leukemia (AML).[4-6]

The current standard frontline treatment for HR-MDS are hypomethylating agents (HMAs), such as azacitidine and decitabine; however, most patients do not respond to these therapies or eventually stop benefiting from them.[7] Once the disease becomes relapsed or refractory (R/R), outcomes are especially poor, with survival often limited to only a few months.[7,8]

Treatment options for patients with R/R HR-MDS remain very limited. Only one therapy has been approved specifically for this setting in the past two decades, and it targets only a small subset of patients (~3-5%) with a specific genetic mutation.[7] As a result, there remains a significant need for new treatment approaches for patients with HR-MDS.

About Acute Myeloid Leukemia

Acute myeloid leukemia (AML) is an aggressive blood cancer characterized by the rapid growth of abnormal myeloid blast cells in the bone marrow and blood. It is the most common form of acute leukemia in adults, with a five-year survival rate of less than 30%.[9] Despite available therapies, many patients relapse or fail to respond, and outcomes are especially poor in the relapsed/refractory setting. Around 450,000 people globally are living with AML.[9]

AML is an area of urgent medical need where new therapies with improved safety, efficacy, and durability or responses are critical. Importantly, the incidence of AML is increasing, and the market is expected to grow to $6 billion annually by 2030,[10] underscoring the scale of the opportunity to bring forward therapies that are not only more effective but also applicable across a broader segment of patients.

About LYT-200

LYT-200 is a fully human IgG4 monoclonal antibody in development for the treatment of hematological malignancies. LYT-200 targets galectin-9, which is an important oncogenic driver and potent immunosuppressor in cancer, positioning it as a novel target for cancer therapy.[11] LYT-200 has been granted Fast Track and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) for the treatment of acute myeloid leukemia.

About Gallop Oncology

Gallop Oncology was founded by and is currently wholly-owned by PureTech Health plc (Nasdaq: PRTC, LSE: PRTC). Gallop is a clinical-stage biopharmaceutical company committed to transforming treatment paradigms for hematologic malignancies. Guided by science and driven to deliver meaningful outcomes for patients, Gallop is advancing a novel approach where efficacy, safety, and durability converge. Its lead candidate, LYT-200, is the most advanced candidate targeting galectin-9, an important oncogenic driver and potent immunosuppressor in cancer, offering a differentiated strategy to address some of the most challenging cancers. For more information, please visit www.galloponcology.com.

About PureTech Health

PureTech Health is a hub-and-spoke biotherapeutics company dedicated to giving life to science and transforming innovation into value. We do this through a proven, capital-efficient R&D model focused on opportunities with validated pharmacology and untapped potential to address significant patient needs. This strategy has produced dozens of therapeutic candidates, including three that have received U.S. FDA approval. By identifying, shaping, and de-risking these high-conviction assets, and scaling them through dedicated structures backed by external capital, we accelerate their path to patients while creating sustainable value for shareholders.

For more information, visit www.puretechhealth.com or connect with us on X (formerly Twitter) @puretechh.

Cautionary Note Regarding Forward-Looking Statements

This press release contains statements that are or may be forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation those related to the LYT-200 development program and development plans, its potential benefits to patients, plans for discussions with regulatory authorities, the further development of the program, future presentation of additional data from the program and our future prospects, developments and strategies. The forward-looking statements are based on current expectations and are subject to known and unknown risks, uncertainties and other important factors that could cause actual results, performance and achievements to differ materially from current expectations, including, but not limited to, those risks, uncertainties and other important factors described under the caption "Risk Factors" in our Annual Report on Form 20-F for the year ended December 31, 2024, filed with the SEC and in our other regulatory filings. These forward-looking statements are based on assumptions regarding the present and future business strategies of the Company and the environment in which it will operate in the future. Each forward-looking statement speaks only as at the date of this press release. Except as required by law and regulatory requirements, we disclaim any obligation to update or revise these forward-looking statements, whether as a result of new information, future events or otherwise.

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[1] Efficacy evaluable is defined in the protocol as all patients who received a minimum one full cycle of LYT-200 (four doses) and had a minimum of one post-baseline disease assessment. The intent-to-treat population for the R/R HR-MDS cohort was n=12 and for the R/R AML cohort was n=33.

[2] Complete response + complete response with incomplete hematological recovery

[3] American Cancer Society. (2023). *What Is Myelodysplastic Syndrome?* Retrieved from https://www.cancer.org

[4] National Comprehensive Cancer Network. (2024). *NCCN Clinical Practice Guidelines in Oncology: Myelodysplastic Syndromes (Version 2.2024).* Retrieved from https://www.nccn.org

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[7] Garcia-Manero, G., Fenaux, P., Al-Kali, A., Baer, M. R., Sekeres, M. A., Roboz, G. J., et al. (2016). Rigosertib versus best supportive care for patients with high-risk myelodysplastic syndromes after failure of hypomethylating drugs (ONTIME): A randomised, controlled, phase 3 trial. *Lancet Oncology, 17*(4), 496-508. https://doi.org/10.1016/S1470-2045(16)00009-7

[8] Prébet, T., Gore, S. D., Esterni, B., Gardin, C., Itzykson, R., Thepot, S., Quesnel, B., Dreyfus, F., Beyne-Rauzy, O., Vey, N., Recher, C., Adès, L., Fenaux, P., & Groupe Francophone des Myélodysplasies. (2011). Outcome of patients with higher-risk myelodysplastic syndromes after azacitidine treatment failure. *Journal of Clinical Oncology, 29*(24), 3322-3327. https://doi.org/10.1200/JCO.2011.35.8135

[9] Acute Myeloid Leukemia - Cancer Stat Facts. (n.d.). National Cancer Institute

[10] Grand View Research, Acute Myeloid Leukemia Treatment Market Size, Share & Trends Analysis Report By Disease, By Treatment (Chemotherapy, Targeted Therapy, Immunotherapy), By Route of Administration, By End Use, By Region, And Segment Forecasts, 2025-2030

[11] Karkempetzaki, A. I., Schatton, T., & Barthel, S. R. (2025). Galectin-9-An emerging Glyco-Immune checkpoint target for cancer therapy. *International Journal of Molecular Sciences*, *26*(16), 7998. https://doi.org/10.3390/ijms26167998

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