Y-mAbs Announces Publication of Phase 2 Interim Results in Nature Communications

March 3, 2025

Naxitamab demonstrated clinically meaningful efficacy with manageable safety in patients with relapsed/refractory high-risk neuroblastoma and residual disease in bone/bone marrow

NEW YORK, March 03, 2025 (GLOBE NEWSWIRE) -- Y-mAbs Therapeutics, Inc. (the "Company" or "Y-mAbs") (Nasdaq: YMAB), a commercial-stage biopharmaceutical company focused on the development and commercialization of novel radioimmunotherapy and antibody-based therapeutic products for the treatment of cancer, today announced the publication of interim data from a Phase 2 clinical trial evaluating naxitamab with granulocyte-macrophage colony-stimulating factor (GM-CSF) in patients with relapsed/refractory high-risk neuroblastoma in the journal *Nature Communications*.

The article, titled "*The anti-GD2 monoclonal antibody naxitamab plus GM-CSF for relapsed or refractory high-risk neuroblastoma: a phase 2 clinical trial,*" details the results of a single-arm, global Phase 2 trial (Trial 201, NCT03363373) of patients with relapsed/refractory high-risk neuroblastoma and residual disease in the bone/bone marrow who received naxitamab on days 1, 3, and 5 (3 mg/kg/day) with GM-CSF (days -4 to 5) every 4 weeks, until a complete response (CR) or partial response (PR) was achieved, followed by 5 additional cycles every 4 weeks. Overall, naxitamab demonstrated statistically significant efficacy with a manageable safety profile.

"In this trial, naxitamab showed clinically meaningful outcomes in a well-defined patient population with an urgent need for efficacious treatment options," said Dr. Jaume Mora, Pediatric Cancer Center Barcelona, Hospital Sant Joan de Déu, Barcelona, Spain and lead author. "The study provides robust evidence for targeting residual disease in the bone and bone marrow, a common niche for chemoresistant cells in patients with primary refractory or relapsed disease."

The primary endpoint in the prespecified interim analysis was overall response (per 2017 International Neuroblastoma Response Criteria). Overall response rate (ORR) was 50% (95% CI: 36-64%, N = 52), and CR and PR were observed in 38% and 12%, respectively. Among 26 responders (CR + PR) in the efficacy population, 58% had refractory disease, and 42% had relapsed disease. With the 95% CI lower limit for ORR exceeding 20%, the primary endpoint of overall response was met. Patients with evaluable bone disease had a 58% (29/50) bone compartment response (CR, 40%; PR, 18%). Bone marrow compartment response was 74% (17/23; CR, 74%). One-year overall survival and progression-free survival (secondary endpoints) were 93% (95% CI: 80-98%) and 35% (95% CI: 16-54%), respectively. Notably, of the 13 patients who had been previously treated with anti-GD2 monoclonal antibodies before enrolling in this study, 4 (31%) responded to naxitamab.

In the safety population (N=74), treatment-related adverse events (AEs) were primarily infusion-related (90%), including hypotension (58% Grade 3 and 3 Grade 4 AEs) and bronchospasm (18% Grade 3 and no Grade 4 AEs). Grade 3 pain, a well-characterized class effect of anti-GD2 immunotherapy, was frequently observed and generally resolved within 15 minutes of infusion completion.

"We are pleased to have worked with the investigators and are grateful to our patients and caregivers for participating in this global trial, specifically designed to evaluate responses to a single-agent anti-GD2 monoclonal antibody (with GM-CSF) in patients with residual disease in bone and bone marrow," said Norman LaFrance, MD, Chief Development Officer at Y-mAbs. "Results from the study support naxitamab as an important treatment option for patients with relapsed and refractory high-risk neuroblastoma and their caregivers."

Researchers at Memorial Sloan Kettering Cancer Center ("MSK") developed DANYELZA® (naxitamab-gqgk), which is exclusively licensed by MSK to Y-mAbs. MSK has institutional financial interests in the compound and Y-mAbs.

About Y-mAbs

Y-mAbs is a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, radioimmunotherapy and antibody-based therapeutic cancer products. The Company's technologies include its investigational Self-Assembly DisAssembly ("SADA") Pretargeted Radioimmunotherapy Platform ("PRIT") and bispecific antibodies generated using the Y-BiClone platform. The Company's broad and advanced product pipeline includes the anti-GD2 therapy DANYELZA® (naxitamab-gqgk), the first FDA-approved treatment for patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow after a partial response, minor response, or stable disease to prior therapy.

Forward-Looking Statements

Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Such statements include, but are not limited to, statements about our business model, including financial outlook for 2024 and beyond. Words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "hope," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," "would," "guidance," "goal," "objective," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The Company's business is subject to risks and uncertainties affecting the Company including those described in the "Risk Factors" section included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, and the Company's Quarterly Report on Form 10-Q for the quarterly periods ended March 31, 2024, and September 30, 2024, and future filings and reports by the Company. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company undertakes no obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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