Y-mAbs Presents Neuroblastoma Research on Naxitamab and SADA PRIT Technology Platform at AACR Special Conference on Advances in Pediatric Cancer Research

September 6, 2024

NEW YORK, Sept. 06, 2024 (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 new clinical and preclinical data from studies evaluating naxitamab and GD2-SADA, respectively, in neuroblastoma. The results are summarized in poster presentations scheduled to be presented September 6 – 7, 2024, at the American Academy of Cancer Research ("AACR") Special Conference in the Advances in Pediatric Cancer Research in Toronto, Canada.

Naxitamab maintains disease control in patients with refractory/relapsed high-risk neuroblastoma: A poster titled "Disease control in patients treated with naxitamab for refractory/relapsed high-risk neuroblastoma" (poster #B055) will be presented on September 7, 2024, during poster session B. This study analyzed the disease control rate with a 12-week minimum of stable disease from the start of naxitamab treatment, based on data from a prespecified interim analysis of Trial 201 (NCT03363373).

Patients with refractory/relapsed high-risk neuroblastoma and residual disease in the bone and/or bone marrow who were treated with naxitamab in combination with granulocyte-macrophage colony-stimulating factor ("GM-CSF") achieved a disease control rate of 63%, with the results suggesting consistent disease control irrespective of baseline Curie score.

"Naxitamab's ability to maintain disease control provides an important measure for those who are most at risk for disease progression," said Vignesh Rajah, MBBS, DCH, MRCP (UK), Chief Medical Officer. "The results reflect our deep commitment to advancing the science and therapeutic management of neuroblastoma."

High-affinity binding of GD2-SADA to Tb-DOTA: A poster titled "GD2-SADA, a bispecific fusion protein that forms self-assembling and disassembling ("SADA"), GD2-avid tetramers with high affinity for chelated radiolanthanides" (poster # A075) will be presented today, September 6, 2024, during poster session A.

The study demonstrated tight binding interactions between GD2-SADA and DOTA-chelated terbium, a metal in the same lanthanide family as lutetium with multiple medical isotopes of potential benefit in diagnosis and therapy. Building on previous *in vitro* findings, the study also characterized the self-assembly and disassembly of GD2-SADA, a dynamic equilibrium that permits high avidity binding of non-radiolabeled GD2-SADA tetramers to GD2-expressing tumors and the renal clearance of disassembled monomers during the first step of pre-targeted radiotherapy ("PRIT"). In a preclinical model of neuroblastoma, also presented in the poster, the chelated radioisotope is administered during the second step of PRIT and binds to GD2-SADA on tumor cells, delivering cytotoxic radiation with minimal off-target exposure.

The results have informed ongoing PK/PD modeling and the initial dosing in Trial 1001 (NCT05130255), a first-in-human Phase 1 trial of GD2-SADA PRIT with ^{177}Lu-DOTA in adolescent and adult patients with GD2-positive solid tumors, and with Trial 1002 planned for pediatric patients with high-risk neuroblastoma.

"The data highlight the importance of continued innovation in the treatment of high-risk neuroblastoma, an aggressive and relentless tumor, and the most common extracranial solid tumor in children," said Dr Rajah. "We remain committed to unlocking the full potential of naxitamab and our novel SADA PRIT technology platform in our mission of improving the lives of patients."

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. Researchers at MSK, including Dr. Nai-Kong Cheung, developed the SADA technology for radioimmunotherapy, which is exclusively licensed by MSK to Y-mAbs. Dr. Cheung has intellectual property rights and interests in technology, and as a result of this licensing arrangement, MSK has institutional financial interests in the technology.

About DANYELZA® (naxitamab-gqgk)
DANYELZA® (naxitamab-gqgk) is indicated, in combination with granulocyte-macrophage colony-stimulating factor ("GM-CSF"), for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. This indication was approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefits in a confirmatory trial. DANYELZA® includes a Boxed Warning for serious infusion-related reactions, such as cardiac arrest and anaphylaxis, and neurotoxicity, such as severe neuropathic pain and transverse myelitis. See full Prescribing Information for complete Boxed Warning and other important safety information.

About GD2-SADA PRIT
GD2-SADA is a bispecific fusion protein that tightly binds to the glycolipid GD2 and Lutetium 177 (Lu 177)-DOTA, a chelated or "caged" radionuclide. In the first step of pre-targeted radiotherapy, non-radiolabeled GD2-SADA tetramers are infused and bind to GD2-expressing solid tumors, while unbound GD2-SADA protein disassembles into low molecular weight monomers that are removed by the kidney. The second infusion delivers the "radioactive payload," which binds directly to GD2-SADA on tumor cells for localized irradiation. GD2- SADA PRIT with Lutetium 177-DOTA is currently being investigated in adults and adolescents in Trial 1001 (NCT05130255).

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, including estimated operating expenses, cash burn and DANYELZA product revenue and sufficiency of cash resources and related assumptions; implied and express statements regarding the future of the Company's business, expectations related to the use of cash and cash equivalents, and the need for, timing and amount of any future financing transaction; expectations with respect to the Company's future financial performance; and other statements that are not historical facts. 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. Our product candidates and related technologies are novel approaches to cancer treatment that present significant challenges. Actual results may differ materially from those indicated by such forward-looking statements as a result of various factors, including but not limited to: risks associated with the Company's financial condition and need for additional capital; the risks that actual results of the Company's restructuring plan and revised business plan will not be as expected; risks associated with the Company's development work; cost and success of the Company's product development activities and clinical trials; the risks of delay in the timing of the Company's regulatory submissions or failure to receive approval of its drug candidates; the risks related to commercializing any approved pharmaceutical product including the rate and degree of market acceptance of product candidates; All statements are subject to the risks 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, the Company's Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2024, the Company's Quarterly Report on Form 10-Q for the quarterly period ended June 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.

Investor Contact:
Courtney Dugan
VP, Head of Investor Relations
cdu@ymabs.com

Source: Y-mAbs Therapeutics, Inc.