FDA Approves Y-mAbs' DANYELZA® (naxitamab-gqgk) for the Treatment of Neuroblastoma

November 25, 2020

NEW YORK, Nov. 25, 2020 (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, antibody-based therapeutic products for the treatment of cancer, today announced that the U.S. Food and Drug Administration ("FDA") has approved DANYELZA (naxitamab-gqgk) 40mg/10ml. DANYELZA 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 is approved under accelerated approval regulation 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 is a humanized, monoclonal antibody that targets the ganglioside GD2, which is highly expressed in various neuroectoderm-derived tumors and sarcomas. DANYELZA is administered to patients three times in a week in an outpatient setting and the treatment is repeated every four weeks. The product has received Priority Review, Orphan Drug, Breakthrough Therapy, and Rare Pediatric Disease designations from the FDA.

"Today is an important day for children living with refractory/relapsed high-risk neuroblastoma," said Thomas Gad, founder, Chairman and President. "It's very exciting to see this treatment go from being an experimental therapy used at my daughter's bedside to now being FDA approved. On behalf of Y-mAbs, I want to thank all the patients and physicians who took part in our clinical trials and our scientific partner, Memorial Sloan Kettering, for helping us achieve this goal."

"We believe that DANYELZA in combination with GM-CSF is a much-needed treatment for patients with relapsed/refractory high-risk neuroblastoma in the bone or bone marrow who have historically not had approved treatments available. This approval of Y-mAbs' first BLA represents a key step in working towards our mission of becoming a world leader in developing better and safer antibody-based oncology products addressing unmet pediatric and adult medical needs," said Claus Moller, Chief Executive Officer.

The FDA approval of DANYELZA is supported by clinical evidence from two pivotal studies in patients with high-risk neuroblastoma with refractory or relapsed disease. DANYELZA appears to be well tolerated with few discontinuations of treatment in the clinical trials and adverse events were clinically manageable. See below for information related to adverse reactions.

The FDA granted approval under the accelerated approval regulation. The postmarketing clinical trial required by the FDA to verify and to further characterize the clinical benefit is the ongoing Study 201, which will enroll a minimum of 80 patients and report overall response rate ("ORR"), duration of response ("DOR"), progression free survival ("PFS") and overall survival ("OS"). The ORR is the primary endpoint for the study, DOR is the secondary endpoint, PFS and OS are secondary endpoints in long-term follow up.

DANYELZA is expected to be available in the United States in the coming weeks. To learn more about DANYELZA, visit DANYELZA.com. To help patients get started on DANYELZA, Y-mAbs Connect™ has been created to answer questions about access, health insurance coverage, financial support programs and other resources available for qualifying patients. To learn more about Y-mAbs Connect, visit ymabsconnect.com.

Researchers at Memorial Sloan Kettering Cancer Center ("MSK") developed DANYELZA, which is exclusively licensed by MSK to Y-mAbs. As a result of this licensing arrangement, MSK has institutional financial interests related to the compound and Y-mAbs.

About High-Risk Neuroblastoma

Neuroblastoma is a solid tumor of childhood that arises in the nervous system, outside of the brain. The clinical behavior of neuroblastoma is highly variable, with some tumors being easily treatable, but the majority being very aggressive. All patients are staged based on the International Neuroblastoma Staging System Committee ("INSS") system, ranging from stage 1 through stage 4S. All patients with stage 4 disease diagnosed after one year of age are classified in the high-risk category, where the neuroblastoma tumor cells have already metastasized to other sites in the body, such as the bone or bone marrow. Essentially all patients who have tumors with many copies, or amplification, of the MYCN oncogene also have high-risk disease, even if they do not have evidence of the tumor having spread.

Important Safety Information and Indication for DANYELZA® (naxitamab-gqgk)

Indication

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 is 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.

Important Safety Information

Please click here to see the full Prescribing Information for DANYELZA.

Contraindications

DANYELZA is contraindicated in patients with a history of severe hypersensitivity reaction to naxitamab-gqgk. Reactions have included anaphylaxis.

Warnings and Precautions

DANYELZA has been approved with a box warning.

In clinical studies, DANYELZA has been shown to cause serious infusion reactions including anaphylaxis, cardiac arrest, bronchospasm, stridor, and hypotension. Infusion reactions generally occurred within 24 hours of completing a DANYELZA infusion, most often within 30 minutes of initiation. Infusion reactions are most frequent during first infusion in each cycle. Premedicate with an antihistamine, acetaminophen, an H2 antagonist and corticosteroid as recommended in the label. Monitor patients closely for signs and symptoms of infusion reactions during and for at least 2 hours

following completion of each DANYELZA infusion in a setting where cardiopulmonary resuscitation medication and equipment are available. Reduce the rate, interrupt infusion, or permanently discontinue DANYELZA based on severity and institute appropriate medical management as needed.

Based on its mechanism of action, DANYELZA can cause severe pain. Premedicate with gabapentin and e.g. oral oxycodone. Treat break-through pain with intravenous hydromorphone or equivalent.

One case of transverse myelitis (Grade 3) has been reported. Permanently discontinue DANYELZA therapy in case of transverse myelitis.

DANYELZA may cause severe hypertension. The onset of hypertension may be delayed. Monitor blood pressure during and after infusion. Interrupt DANYELZA infusion and resume at a reduced rate, or permanently discontinue DANYELZA based on the severity.

Two cases of posterior reversible encephalopathy syndrome ("PRES") have been reported. Monitor blood pressure during and following DANYELZA infusion and assess for neurologic symptoms. Permanently discontinue DANYELZA in case of symptomatic PRES.

Adverse Reactions
The most common adverse events were mainly mild and moderate and included infusion-related reaction, pain, tachycardia, vomiting, cough, nausea, diarrhea, decreased appetite, hypertension, fatigue, erythema multiforme, peripheral neuropathy, urticaria, pyrexia, headache, edema, anxiety, localized edema and irritability.

This is not the complete list of Warnings, Precautions and Adverse Reactions. For further information see label.

To report suspected adverse reactions, contact Y-mAbs Therapeutics, Inc., at 1-833-339-6227 (1-833-33YMABS), or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

About Y-mAbs

Y-mAbs is a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer. The Company has a broad and advanced product pipeline, including DANYELZA and omburtamab, which target tumors that express B7-H3.

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 The Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements about our business model and development and commercialization plans; the benefits, safety and efficacy of DANYELZA, current and future clinical and pre-clinical studies and our research and development programs; expectations related to the timing of the initiation and completion of regulatory submissions; regulatory, marketing and reimbursement approvals; rate and degree of market acceptance and clinical utility as well as pricing and reimbursement levels; retaining and hiring key employees; our commercialization, marketing and manufacturing capabilities and strategy; our intellectual property position and strategy; additional product candidates and technologies; collaborations or strategic partnerships and the potential benefits thereof; expectations related to the use of our cash and cash equivalents, and the need for, timing and amount of any future financing transaction; our financial performance, including our estimates regarding revenues, expenses, capital expenditure requirements; developments relating to our competitors and our industry; and other statements that are not historical facts. Words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will", "would" 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 our financial condition and need for additional capital; risks associated with our development work; cost and success of our product development activities and clinical trials; the risks of delay in the timing of our regulatory submissions or failure to receive approval of our drug candidates; the risks related to commercializing any approved pharmaceutical product including the rate and degree of market acceptance of our product candidates; development of our sales and marketing capabilities and risks associated with failure to obtain sufficient reimbursement for our products; the risks related to our dependence on third parties including for conduct of clinical testing and product manufacture; our inability to enter into partnerships; the risks related to government regulation; risks related to market approval, risks associated with protection of our intellectual property rights; risks related to employee matters and managing growth; risks related to our common stock and other risks and uncertainties affecting the Company including those described in the "Risk Factors" section included in our Annual Report on Form 10-K and in our other SEC filings. 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.

Contact:

Y-mAbs Therapeutics, Inc.
230 Park Avenue, Suite 3350
New York, NY 10169
USA

+1 646 885 8505

E-mail: info@ymabs.com

Source: Y-mAbs Therapeutics, Inc