# Y-mAbs Receives Breakthrough Therapy Designation for Naxitamab for the treatment of High Risk Neuroblastoma

August 21, 2018

**The FDA has granted Y-mAbs Breakthrough Therapy designation for naxitamab, in combination with GM-CSF, for the treatment of high risk neuroblastoma**

**NEW YORK, August 21, 2018** – Y-mAbs Therapeutics, Inc. (YmAbs), an immunotherapy company discovering and developing innovative treatments for patients with cancer, today announced that the Company has received a Breakthrough Therapy designation for naxitamab, in combination with GM-CSF, for the treatment of high risk neuroblastoma refractory to initial therapy or with incomplete response to salvage therapy in patients older than 12 months of age with persistent, refractory disease limited to bone marrow with or without evidence of concurrent bone involvement.

YmAbs Founder, President and Head of Business Development and Strategy, Thomas Gad said, “We are very pleased that the FDA has granted the Breakthrough Therapy designation to naxitamab and we look forward to continuing to work with the FDA to make this therapy potentially available to children facing an unmet medical need. We believe that Naxitamab provides a new opportunity for pediatric patients otherwise faced with little or no options. This is an important milestone achievement for YmAbs, and we continue to work with the regulatory authorities to advance naxitamab to patients suffering from high risk neuroblastoma as quickly as possible.”

Dr. Claus Møller, Chief Executive Officer further notes, “This is the first time naxitamab has earned the distinction of a Breakthrough Therapy Designation. We are pleased that the FDA continues to recognize the potential of naxitamab to help patients with high risk neuroblastoma.”

**About Breakthrough Therapy Designation:**

The Breakthrough Therapy Designation was enacted as part of the 2012 FDA Safety and Innovation Act (FDASIA) and is intended to expedite development of drugs to treat serious and life-threatening medical conditions when preliminary clinical evidence demonstrates that the drug may have substantial improvement on at least one clinically significant endpoint over available therapies. Breakthrough Therapy Designation includes all the features of the Fast Track Designation, as well as more intensive guidance from the FDA on a drug's clinical development program.

**About YmAbs:**

YmAbs is a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer. We have a broad and advanced product pipeline, including two pivotal-stage product candidates - naxitamab and omburtamab—which target tumors that express GD2 and B7-H3, respectively. We are developing naxitamab for the treatment of pediatric patients with relapsed or refractory, or R/R, high-risk neuroblastoma, or NB, and radiolabeled omburtamab for the treatment of pediatric patients with central nervous system, or CNS, leptomeningeal metastases, or LM, from NB. NB is a rare and almost exclusively pediatric cancer that develops in the sympathetic nervous system and CNS/LM is a rare and usually fatal complication of NB in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord in the CNS. In addition we are developing 131I-omburtamab, which is omburtamab radiolabeled with Iodine-131, for the treatment of DSRCT and 124I-omburtamab, which is omburtamab radiolabeled with Iodine-124, for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. We have two additional product candidates in pre-clinical development, omburtamab-DTPA (diethylenetriamine pentaacetate), a Lutetium-177 conjugated antibody, and huB7-H3, a humanized version of omburtamab, each targeting indications with large adult patient populations where we believe there is a significant unmet medical need. We are also advancing a pipeline of novel bispecific antibodies (BsAbs) through late pre-clinical development, including our huGD2-BsAb product candidate for the treatment of refractory GD2-positive adult and pediatric solid tumors and our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs. We intend to advance and expand our product pipeline into certain adult cancer indications either independently or in collaboration with potential partners.

To learn more, visit www.ymabs.com.

**SOURCE:**

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