Omburtamab as intraperitoneal radioimmunotherapy in desmoplastic small round cell tumor

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NEW YORK, April 16, 2018 – Y-mAbs Therapeutics, Inc. (YmAbs), an immunotherapy company discovering and developing innovative treatments for patients with cancer, today announced Phase I clinical trial data for omburtamab in Desmoplastic Small Round Cell Tumor (DSRCT), which is a rare sarcoma of adolescents and young adults. Dr. Shakell Modak from Memorial Sloan Kettering Cancer Center (MSK) presented data at the American Association for Cancer Research Annual Meeting on April 15, 2018 in Chicago, Illinois.

The phase I study of radioiodinated omburtamab was conducted at MSK to evaluate toxicity, pharmacokinetics, biodistribution and efficacy. Cohorts of 3-6 patients were treated with escalated doses of intraperitoneal 131I- omburtamab. A prior dose of 2mCi 124I-omburtamab intraperitoneal was used to acquire PET images and biodistribution data. A total of 41 DSRCT patients were treated at doses of 30-90mCi/m2. Maximum tolerated dose was not reached and there were no dose-limiting toxicities. Major adverse events were grade 4 neutropenia (n=3). Median progression-free survival in patients undergoing complete cytoreductive surgery (n=20) followed by RIT and whole-abdominal radiotherapy was 18.5±2.4 months from administration of RIT. Of these, 18 and 11 patients are overall and progression-free survivors, respectively, at a median follow up of 14 months post-RIT. In contrast a patient with gross residual disease pre- RIT (n=1) had median progression free survival of 5.3±3.8 months (p<0.05 for PFS and OS compared to patients undergoing complete surgery). 131I-omburtamab intraperitoneal RIT appears to have activity against micro-metastatic DSRCT.

"We are very pleased to see omburtamab, currently also being studied in CNS/Leptomeningeal Metastasis from Neuroblastoma, DIPG and Medulloblastoma, generate encouraging data for patients with DSRCT, an ultra-rare disease," said YmAbs' Founder, Chairman and President, Thomas Gad. "The Phase II trial in this indication will commence shortly."

Dr. Claus Møller, Chief Executive Officer added, "Progression free survival of almost 18 months in this population of patients is very encouraging, and we are excited to move forward in the clinic with this 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 RIR, 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 CNSLM 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 131 I-omburtamab, which is omburtamab radiolabeled with Iodine-131, for the treatment of DSRCT and 124 I-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. 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.

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