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Merck’s Bifunctional Immunotherapy M7824 gets FDA orphan drug designation

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FDA grants M7824, an investigational bifunctional immunotherapy, orphan drug designation in biliary tract cancer

Merck, a leading science and technology company, has announced that the US Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to M7824, the first regulatory designation for the bifunctional immunotherapy, for the treatment of biliary tract cancer (BTC).

The FDA orphan drug designation follows the recent presentation of the first clinical data for M7824 in BTC at the European Society of Medical Oncology (ESMO) congress in October. M7824 is an investigational bifunctional immunotherapy designed to combine co-localized blocking of the transforming growth factor-? and anti-PD-L1 immune escape mechanisms.

BTC is a collective term for a group of rare and aggressive gastrointestinal cancers, including intrahepatic cholangiocarcinoma (ICC), extrahepatic cholangiocarcinoma (ECC), and gallbladder carcinoma (GBC). Approximately 16,000 cases of BTC are estimated to occur every year in the US and collectively these cancers present late in the majority of patients.

Treatment options are limited and the median survival rate in the advanced setting is less than one year, objective tumor response with commonly used chemotherapy is typically less than 10% with short duration of response.

"Biliary tract cancer is a rare, notoriously hard-to-treat tumor where existing treatment approaches, such as surgery or chemotherapy, are either not viable or simply don't deliver acceptable patient outcomes," said Luciano Rossetti, Head of Global Research & Development at the Biopharma business of Merck. "As the first regulatory designation for M7824, Merck is excited about the potential of this new class of immunotherapy in a number of challenging cancers and settings."

FDA Orphan Drug Designation (ODD) is granted to medicines intended to treat rare diseases or disorders that affect fewer than 200,000 people in the US, or those that affect more than 200,000 people but are unlikely to recover the costs of developing and marketing the drug. Medicines that meet the FDA's ODD criteria qualify for a number of incentives to help support advancement.

M7824 is an investigational bifunctional immunotherapy that combines a TGF-? trap with the anti-PD-L1 mechanism in one

fusion protein. Designed to combine co-localized blocking of the two immunosuppressive pathways, M7824 is thought to control tumor growth by potentially restoring and enhancing anti-tumor responses. M7824 is an important part of a novel combination approach that seeks to harness the power of the immune system and address the tremendously complex nature of difficult-to-treat tumors.