Omega Therapeutics Receives Orphan Drug Designation for OTX-2002 for the Treatment of Hepatocellular Carcinoma

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Phase 1/2 MYCHELANGELO™ I Clinical Trial Underway to Evaluate Safety, Tolerability and Preliminary Antitumor Activity of OTX-2002

CAMBRIDGE, Mass., Nov. 2, 2022 /PRNewswire/ -- Omega Therapeutics, Inc. ("Omega"), a clinical-stage biotechnology company pioneering the first systematic approach to use mRNA therapeutics as a new class of programmable epigenetic medicines, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for OTX-2002, the company's first-in-class epigenomic controller engineered to downregulate c-Myc (MYC), for the treatment of hepatocellular carcinoma (HCC).

The company recently announced that the first patient had been dosed in its Phase 1/2 MYCHELANGELO™ I clinical trial investigating the safety, tolerability, pharmacokinetics, pharmacodynamics, and preliminary antitumor activity of OTX-2002 as a monotherapy and in combination with standard of care therapies in patients with relapsed or refractory HCC and other solid tumor types known for association with the MYC oncogene.

"HCC is a devastating illness that often develops resistance to current standard of care therapeutics. The FDA's decision to grant orphan drug designation for OTX-2002 underscores the need for novel therapies to address HCC and the potential of epigenomic programming to transform the treatment landscape," said Mahesh Karande, President and Chief Executive Officer of Omega Therapeutics. "We look forward to continuing to work with clinical investigators, patients and the FDA as we advance our MYCHELANGELO clinical program and evaluate the potential of OTX-2002 to bring a new treatment option to the liver cancer patient community."

About Orphan Drug Designation

The FDA's Orphan Drug Designation program provides orphan status to drugs defined as those intended for the treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemptions and seven-year marketing exclusivity upon FDA approval.

About Hepatocellular Carcinoma

Hepatocellular carcinoma (HCC) is a leading cause of cancer deaths worldwide and represents an unmet clinical need with few therapeutic options. Tyrosine kinase inhibitors (TKIs) have been used as a systemic therapy for HCC, but patients frequently develop resistance with oncogenic MYC identified as a correlating prognostic factor. The MYC oncogene is associated with aggressive disease in up to 70% of patients with HCC.

About OTX-2002

OTX-2002 is a first-in-class Omega Epigenomic Controller ™ in development for the treatment of hepatocellular carcinoma (HCC). OTX-2002 is an mRNA therapeutic delivered via lipid nanoparticles (LNPs) and is designed to downregulate MYC expression pre-transcriptionally through epigenetic modulation while potentially overcoming MYC autoregulation. MYC is a master transcription factor that regulates cell proliferation, differentiation and apoptosis and plays a significant role in more than 50% of all human cancers. OTX-2002 is currently being evaluated in the Phase 1/2 MYCHELANGELO ™ I trial in patients with relapsed or refractory HCC and other solid tumor types known for association with the MYC oncogene; visit clinicaltrials.gov (NCT05497453) for more details.

About Omega Therapeutics

Omega Therapeutics, founded by Flagship Pioneering, is a clinical-stage biotechnology company pioneering the first systematic approach to use mRNA therapeutics as a new class of programmable epigenetic medicines. The company's OMEGA Epigenomic Programming™ platform harnesses the power of epigenetics, the mechanism that controls gene expression and every aspect of an organism's life from cell genesis, growth, and differentiation to cell death. Using a suite of technologies, paired with Omega's process of systematic, rational, and integrative drug design, the OMEGA platform enables control of fundamental epigenetic processes to correct the root cause of disease by returning aberrant gene expression to a normal range without altering native nucleic acid sequences. Omega's modular and programmable mRNA medicines, Omega Epigenomic Controllers™, target specific epigenomic loci within insulated genomic domains, EpiZips™, from amongst thousands of unique, mapped, and validated genome-wide DNA-sequences, with high specificity to durably tune single or multiple genes to treat and cure diseases through Precision Genomic Control™. Omega is currently advancing a broad pipeline of development candidates spanning a range of disease areas, including oncology, regenerative medicine, multigenic diseases including immunology, and select monogenic diseases, including alopecia.

For more information, visit omegatherapeutics.com, or follow us on Twitter and LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without

limitation statements regarding the timing and design of our Phase 1/2 MYCHELANGELO ™ I clinical trial; the potential of the OMEGA platform to engineer programmable epigenetic mRNA therapeutics that successfully regulate gene expression by targeting insulated genomic domains; expectations surrounding the potential of our product candidates, including our lead OEC candidate OTX-2002; and expectations regarding our pipeline, including trial design, initiation of preclinical studies and advancement of multiple preclinical development programs in oncology, immunology, regenerative medicine, and select monogenic diseases. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: the novel technology on which our product candidates are based makes it difficult to predict the time and cost of preclinical and clinical development and subsequently obtaining regulatory approval, if at all; the substantial development and regulatory risks associated with epigenomic controller machines due to the novel and unprecedented nature of this new category of medicines; our limited operating history; the incurrence of significant losses and the fact that we expect to continue to incur significant additional losses for the foreseeable future; our need for substantial additional financing; our investments in research and development efforts that further enhance the OMEGA platform, and their impact on our results; uncertainty regarding preclinical development, especially for a new class of medicines such as epigenomic controllers; potential delays in and unforeseen costs arising from our clinical trials; the fact that our product candidates may be associated with serious adverse events, undesirable side effects or have other properties that could halt their regulatory development, prevent their regulatory approval, limit their commercial potential, or result in significant negative consequences; the impact of increased demand for the manufacture of mRNA and LNP based vaccines to treat COVID-19 on our development plans; difficulties manufacturing the novel technology on which our OEC candidates are based; our ability to adapt to rapid and significant technological change; our reliance on third parties for the manufacture of materials; our ability to successfully acquire and establish our own manufacturing facilities and infrastructure; our reliance on a limited number of suppliers for lipid excipients used in our product candidates; our ability to advance our product candidates to clinical development; and our ability to obtain, maintain, enforce and adequately protect our intellectual property rights. These and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, and our other filings with the SEC, could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change.

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