Delcath Receives Orphan Drug Designation From FDA For Melphalan To Treat Cholangiocarcinoma

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NEW YORK, July 20, 2015 /PRNewswire/ -- Delcath Systems, Inc. (NASDAQ: DCTH), a specialty pharmaceutical and medical device company focused on oncology with an emphasis on the treatment of primary and metastatic liver cancers, announced today that the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development (OOPD) has granted Orphan Drug Designation for melphalan for the treatment of cholangiocarcinoma. The OOPD is tasked with evaluating the scientific and clinical data submissions from sponsors to identify and designate products as promising for rare diseases and to further advance scientific development of such promising medical products.

Orphan drug designation provides certain exclusivity benefits, tax credits for certain research and a waiver of the New Drug Application user fee. Cholangiocarcinoma is recognized by the FDA as an orphan disease, usually defined as a condition that affects fewer than 200,000 people nationwide.

Intrahepatic cholangiocarcinoma (ICC), a sub-category of cholangiocarcinoma, is a tumor in the bile duct that arises within the liver. It is the second most common primary liver tumor and represents approximately 15% of new HCC cases diagnosed annually. Surgical resection, the standard of care, is not possible for an estimated 80% to 90% of patients diagnosed with ICC.

The Company recently announced the expansion of its global Phase 2 clinical study in primary liver cancer (HCC) to include an ICC cohort, which is investigating the safety and efficacy of Melphalan/HDS treatment in patients with unresectable ICC confined to the liver. The study is being conducted at the same hospitals in Europe participating in the Company's Phase 2 HCC trial, and is expected to enroll 11 patients. The ICC cohort will evaluate tumor response (objective response rate) as measured by modified Response Evaluation Criteria in Solid Tumor (mRECIST), and will assess progression-free survival and safety. Additional analyses will be conducted to characterize the systemic exposure of melphalan administered by Melphalan/HDS, as well as to assess patient-reported clinical outcomes, or quality-of-life.

"We are pleased with the receipt of orphan drug designation for melphalan in the treatment of patients with cholangiocarcinoma as it is a key milestone that supports our broader regulatory and development strategy for our Melphalan/Hepatic Delivery System (Melphalan/HDS) as a therapy for primary and metastatic liver cancers," said Jennifer Simpson, Ph.D., M.S.N., C.R.N.P., President and Chief Executive Officer of Delcath. "ICC is a disease of significant unmet medical need and our Melphalan/HDS treatment may offer clinical benefit for ICC patients who face limited treatment options."

About Delcath Systems
Delcath Systems, Inc. is a specialty pharmaceutical and medical device company focused on oncology with an emphasis on the treatment of primary and metastatic liver cancers. Our proprietary Melphalan Hydrochloride for Injection for use with the Delcath Hepatic Delivery System (Melphalan/HDS) is designed to administer high-dose chemotherapy to the liver while controlling systemic exposure. In April 2012 we obtained authorization to affix a CE Mark to our second-generation system, which is currently marketed in Europe as a device under the trade name Delcath Hepatic CHEMOSAT® Delivery System for Melphalan (CHEMOSAT). In the U.S. the Melphalan/HDS system is considered a combination drug and device product, and is regulated as a drug by the U.S. Food and Drug Administration (FDA). The Melphalan/HDS system has not been approved for sale in the U.S. We have commenced a global Phase 2 clinical trial in Europe and the U.S. to investigate the Melphalan/HDS system for the treatment of primary liver cancer (HCC) and intrahepatic cholangiocarcinoma (ICC), and expect to initiate a global Phase 3 trial in ocular melanoma (OM) that has metastasized to the liver.

Private Securities Litigation Reform Act of 1995 provides a safe harbor for forward-looking statements made by the Company or on its behalf. This news release contains forward-looking statements, which are subject to certain risks and uncertainties that can cause actual results to differ materially from those described. Factors that may cause such differences include, but are not limited to, uncertainties relating to: future patient outcomes and clinical trial results consistent with the data contained in the SSO abstract, acceptance and publication of the Phase 3 trial manuscript and the impact of publication to support the Company's efforts, the timing and results of the Company's clinical trials including without limitation the HCC, ICC and OM clinical trial programs timely enrollment and treatment of patients in the global Phase 2 HCC and ICC clinical trial, FDA approval of the global Phase 3 OM clinical trial protocol, IRB or ethics committee clearance of the Phase 2 HCC/ICC and/or Phase 3 OM protocols from participating sites and the timing of site activation and subject enrollment in each trial, the impact of the presentations at ESSO and future clinical results consistent with the data presented, approval of Individual Funding Requests for reimbursement of the CHEMOSAT procedure, the impact, if any of Value 4 status on potential CHEMOSAT product use and sales in Germany, clinical adoption, use and resulting sales, if any, for the CHEMOSAT system to deliver and filter melphalan in Europe including the key markets of Germany and the UK, the Company's ability to successfully commercialize the Melphalan HDS/CHEMOSAT system and the potential of the Melphalan HDS/CHEMOSAT system as a treatment for patients with primary and metastatic disease in the liver, our ability to obtain reimbursement for the CHEMOSAT system in various markets, the Company's ability to satisfy the requirements of the FDA's Complete Response Letter and provide the same in a timely manner, approval of the current or future Melphalan HDS/CHEMOSAT system for delivery and filtration of melphalan or other chemotherapeutic agents for various indications in the U.S. and/or in foreign markets, actions by the FDA or other foreign regulatory agencies, the Company's ability to successfully enter into strategic partnership and distribution arrangements in foreign markets and the timing and revenue, if any, of the same, uncertainties relating to the timing and results of research and development projects, our ability to maintain NASDAQ listing, and uncertainties regarding the Company's ability to obtain financial and other resources for any research, development, clinical trials and commercialization activities. These factors, and others, are discussed from time to time in our filings with the Securities and Exchange Commission. You should not place undue reliance on these forward-looking statements, which speak only as of the date they are made. We undertake no obligation to publicly update or revise these forward-looking statements to reflect events or circumstances after the date they are made.

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