



NEWS RELEASE

FDA Grants Breakthrough Therapy Designation to Omeros' MASP-2 Inhibitor OMS721 for the Treatment of High-Risk Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy

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– Discussions Ongoing with FDA and European Regulators for Expedited Approval –

SEATTLE--(BUSINESS WIRE)--Apr. 26, 2018-- Omeros Corporation (NASDAQ: OMER) today announced that the U.S. Food and Drug Administration (FDA) has granted breakthrough therapy designation to OMS721 for the treatment of patients with high-risk hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA), specifically those patients who have persistent TMA despite modification of immunosuppressive therapy. This is the second breakthrough therapy drug designation for OMS721, which last year received the designation from FDA for the treatment of Immunoglobulin A (IgA) nephropathy. OMS721 is Omeros' lead human monoclonal antibody targeting mannan-binding lectin-associated serine protease-2 (MASP-2), the effector enzyme of the lectin pathway of the complement system.

"TMA is an increasingly common complication following stem cell transplantation and is devastating when conservative treatment of immunosuppressive modification fails," stated Rafael Duarte, M.D., Ph.D., F.R.C.P.(Lon), Associate Professor, Head of Hematopoietic Transplantation and Hemato-oncology Section, University Hospital Puerta de Hierro Majadahonda, Madrid, Spain, and Secretary of the European Society for Blood and Marrow

Transplantation. "Patients with HSCT-TMA who cannot undergo or do not respond to modification of immunosuppressive therapy are at very high risk of death from this complication. Currently, there is no approved treatment for HSCT-TMA, which remains one of the most pressing therapeutic needs in the field of HSCT. The impressive effect on survival and other results seen in OMS721-treated patients are quickly apparent following initiation of OMS721 therapy and can't be explained by other factors. The HSCT community looks forward to the drug's broad availability for our patients."

Breakthrough therapy designation was granted based on data from Omeros' Phase 2 clinical trial evaluating OMS721 in patients with high-risk HSCT-TMA. To be eligible for enrollment in the clinical trial, HSCT-TMA patients are required to be adults with post-transplant TMA persisting for at least two weeks following immunosuppressive regimen modification (conservative treatment) or more than 30 days post-transplant. This population was chosen to represent a population at risk for poor outcomes, including mortality. These patients often have serious, life threatening co-existing conditions, and mortality rates have been reported to be as high as 100 percent. As reported previously, the estimated median survival for OMS721-treated patients was an order of magnitude greater than that for a matched historical control ($p < 0.0001$). Further analysis of the data examined 100-day mortality, an important endpoint previously used as an approval endpoint in HSCT. That analysis also showed that OMS721-treated patients had improved survival relative to the historical control (53% vs 10%; $p = 0.0002$). As previously reported, biomarkers of disease (i.e., mean platelet count and mean levels of lactate dehydrogenase and haptoglobin) demonstrated statistically significant improvement. Study patients also showed substantial improvement in red blood cell and platelet transfusion requirements. Other serious co-existing conditions in the patients treated with OMS721 included graft versus host disease (GvHD), cytomegalovirus and human herpes virus 6 infections, prior sepsis, diffuse alveolar hemorrhage, and residual underlying malignancies.

"I have treated several stem-cell TMA patients with OMS721 and seen marked and unexpected improvements that can't be otherwise explained," stated Professor Alessandro Rambaldi, from the University of Milan and Director of Department of Hematology and Oncology, Azienda Ospedaliera Papa Giovanni XXIII. "Most notable was a deteriorating patient with co-existing GVHD, TMA and neurological disability that confined him to bedridden hospitalization. Following treatment with OMS721, his TMA resolved quickly and his neurological status progressively improved, allowing him to leave the hospital and return to part-time work. This effect on TMA was observed in the absence of other specific treatments. The improvement seen in my patients has convinced me that lectin pathway inhibition by OMS721 is a scientifically sound and highly promising treatment strategy for a range of disorders associated with endothelial cell injury that commonly occur following stem cell transplantation."

FDA's breakthrough therapy designation enables expedited development and review of a drug candidate for the treatment of a serious or life-threatening disease. Preliminary clinical evidence indicating that the drug may demonstrate substantial improvement over existing therapies is required. Benefits of breakthrough therapy

designation include the eligibility for priority review of the application and rolling submission of portions of the application. FDA works closely with the company to provide guidance to determine the most efficient route to approval.

“High-risk TMA following hematopoietic stem cell transplant carries an extremely high mortality rate, and no treatments are approved for this devastating disorder,” stated Gregory A. Demopoulos, chairman and chief executive officer of Omeros. “We appreciate FDA’s recognition of the potential for OMS721 to improve outcomes – most importantly survival – for these patients, and we look forward to working closely with the Agency to accelerate the development and approval of OMS721.”

Persistent thrombotic microangiopathy is a life-threatening complication of HSCT. Approximately 20,000 HSCT procedures are performed in the U.S. annually, and TMA is reported to occur in approximately 10 to 25 percent of HSCT patients. Reported mortality in high-risk patients is greater than 90%.

OMS721 is also being evaluated in ongoing Phase 3 clinical trials in IgA nephropathy and atypical hemolytic uremic syndrome. Across all clinical trials with OMS721, the drug has been well tolerated and no safety concerns have been identified.

About Omeros’ MASP Programs

Omeros controls the worldwide rights to MASP-2 and all therapeutics targeting MASP-2, a novel pro-inflammatory protein target involved in activation of the complement system, which is an important component of the immune system. The complement system plays a role in the inflammatory response and becomes activated as a result of tissue damage or microbial infection. MASP-2 is the effector enzyme of the lectin pathway, one of the principal complement activation pathways. Importantly, inhibition of MASP-2 does not appear to interfere with the antibody-dependent classical complement activation pathway, which is a critical component of the acquired immune response to infection, and its abnormal function is associated with a wide range of autoimmune disorders. MASP-2 is generated by the liver and is then released into circulation. Adult humans who are genetically deficient in one of the proteins that activate MASP-2 do not appear to be detrimentally affected by the deficiency. OMS721 is Omeros’ lead human MASP-2 antibody.

Phase 3 clinical programs are in progress for OMS721 in atypical hemolytic uremic syndrome (aHUS), in immunoglobulin A (IgA) nephropathy and in hematopoietic stem cell transplant-associated thrombotic microangiopathy (HCT-TMA). Also, two Phase 2 trials are ongoing. One is continuing to enroll IgA nephropathy patients and has already generated positive data in patients with IgA nephropathy and with lupus nephritis; the other is enrolling and has reported positive data in patients with HCT-TMA and in patients with aHUS. OMS721 can be administered both intravenously and subcutaneously, and Omeros expects to commercialize each formulation

of OMS721 for different therapeutic indications. In parallel, Omeros is developing small-molecule inhibitors of MASP-2. Based on requests from treating physicians, Omeros has established a compassionate-use program for OMS721, which is active in both the U.S. and Europe. The FDA has granted OMS721 breakthrough therapy designation for IgA nephropathy and for high-risk HSCT-TMA, orphan drug status for the prevention (inhibition) of complement-mediated thrombotic microangiopathies and for the treatment of IgA nephropathy, and fast track designation for the treatment of patients with aHUS.

Omeros also has identified MASP-3 as responsible for the conversion of pro-factor D to factor D and as a critical activator of the human complement system's alternative pathway. The alternative pathway is linked to a wide range of immune-related disorders. In addition to its lectin pathway inhibitors, the company is advancing its development of antibodies and small-molecule inhibitors against MASP-3 to block activation of the alternative pathway. Omeros has initiated the manufacturing scale-up process of its MASP-3 antibodies in preparation for clinical trials.

About Omeros Corporation

Omeros is a commercial-stage biopharmaceutical company committed to discovering, developing and commercializing small-molecule and protein therapeutics for large-market as well as orphan indications targeting inflammation, complement-mediated diseases and disorders of the central nervous system. The company's drug product OMIDRIA® (phenylephrine and ketorolac intraocular solution) 1% / 0.3% is marketed for use during cataract surgery or intraocular lens (IOL) replacement to maintain pupil size by preventing intraoperative miosis (pupil constriction) and to reduce postoperative ocular pain. In the European Union, the European Commission has approved OMIDRIA for use in cataract surgery and other IOL replacement procedures to maintain mydriasis (pupil dilation), prevent miosis (pupil constriction), and to reduce postoperative eye pain. Omeros has multiple Phase 3 and Phase 2 clinical-stage development programs focused on: complement-associated thrombotic microangiopathies; complement-mediated glomerulonephropathies; Huntington's disease and cognitive impairment; and addictive and compulsive disorders. In addition, Omeros has a diverse group of preclinical programs and a proprietary G protein-coupled receptor (GPCR) platform through which it controls 54 new GPCR drug targets and corresponding compounds, a number of which are in preclinical development. The company also exclusively possesses a novel antibody-generating platform.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, which are subject to the "safe harbor" created by those sections for such statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "likely," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar

expressions and variations thereof. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Omeros' actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with product commercialization and commercial operations, unproven preclinical and clinical development activities, regulatory oversight, intellectual property claims, competitive developments, litigation, and the risks, uncertainties and other factors described under the heading "Risk Factors" in the company's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 1, 2018. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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