



NEWS RELEASE

Omeros Reports Significant Improvement in Overall Survival Data in OMS721-Treated Patients with Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy

2018-02-15

-- Greater than 16-Fold Improvement in Survival Compared to Historical Control --

SEATTLE--(BUSINESS WIRE)--Feb. 15, 2018-- Omeros Corporation (NASDAQ: OMER) today announced new results from the company's ongoing Phase 2 study of OMS721 evaluating patients with hematopoietic stem cell transplant-associated thrombotic microangiopathy (HCT-TMA). The data demonstrate an increase in median overall survival in HCT-TMA patients treated with OMS721 compared to a matched historical control (347 days vs. 21 days, respectively, by Kaplan-Meier analysis; $p < 0.0001$ by log-rank test). Historical control data are typically used for comparison when it is impractical or unethical to include a placebo arm in a clinical trial. In addition to and consistent with the survival data reported today, updated assessments of platelet count, lactate dehydrogenase (LDH) and haptoglobin – all markers of TMA activity – continue to demonstrate clinically meaningful and statistically significant improvements in the HCT-TMA patients treated with OMS721.

A total of 19 HCT-TMA patients have been treated to date with OMS721, 18 in the ongoing study and one patient under a compassionate use protocol. An historical control that best matched the OMS721-treated population was identified from the literature. The literature reference selection criteria were those studies that specified: (1)

individual patient data (required for analysis), (2) adult and/or adolescent populations, (3) allogeneic stem cell transplant recipients only, and (4) no or partial response to immunosuppressive regimen modification. Overall median survival demonstrated greater than 16-fold improvement in survival in the OMS721-treated group ($p < 0.0001$).

Markers of TMA activity in study participants, specifically mean platelet count, mean LDH, and mean haptoglobin, continue to demonstrate statistically and clinically significant improvements following OMS721 treatment. At the end of protocol-allowed treatment, the mean platelet count (normal range: 150,000 – 400,000 $\times 10^6/\text{mL}$) increased from 18,100 $\times 10^6/\text{mL}$ at baseline to 52,300 $\times 10^6/\text{mL}$ ($p = 0.017$). The mean LDH (normal range: 125-220 U/L) decreased from 591 U/L at baseline to 250 U/L ($p < 0.001$). The mean haptoglobin (normal range: 14-268 mg/dL) increased from 8 mg/dL at baseline to 141 mg/dL ($p = 0.003$). Mean creatinine remained stable at approximately 120 $\mu\text{mol/L}$ (normal range: 63-104 $\mu\text{mol/L}$) but a majority of patients had co-existing conditions for which they were receiving nephrotoxic medications. Other serious co-existing conditions included graft versus host disease (GvHD), cytomegalovirus and human herpes virus 6 infections, prior sepsis, diffuse alveolar hemorrhage, and residual underlying malignancies.

OMS721 has been well tolerated and no safety concerns have been identified. The most commonly reported adverse events were diarrhea and neutropenia. Four deaths occurred during the study: one due to progression of acute myeloid leukemia, two due to neutropenic sepsis, and one due to acute renal and respiratory failure. Only one of these deaths – the acute renal and respiratory failure – was considered “possibly drug-related” because an association could not be definitively ruled out by the investigator. These are common complications of HCT. The other three deaths were deemed not to be related to OMS721.

Earlier data from this study have previously been presented at the 2017 combined annual meetings of the Center for International Blood & Marrow Transplant Research and the American Society for Blood and Marrow Transplantation, the 2017 annual meeting of the European Society of Blood and Marrow Transplantation (EBMT), and the 2017 EBMT Crash Course on Diagnosis and Treatment of Noninfectious Complications after HCT. Two HCT-TMA case reports were presented independently of Omeros at EBMT meetings. One patient was an adolescent girl whose course was complicated by diffuse alveolar hemorrhage (DAH) and who did not tolerate eculizumab treatment but responded well to compassionate use OMS721 treatment. She was able to discontinue all hemodialysis as well as all platelet transfusions – prior to treatment with OMS721, she was receiving hemodialysis thrice weekly platelet transfusions. The second was a study patient who had a difficult post-transplant course, including steroid-resistant GvHD and cytomegalovirus infection. He developed TMA that did not respond to conservative measures and had co-existing GvHD with multiple neurological complications and was unable to walk. Following OMS721 treatment, his TMA and GvHD resolved and his neurological complications improved. He was able to return to work and his neurological status has continued to improve.

“As evidenced by the published literature, this is a population with an extremely high mortality rate and a disorder for which there is no approved therapy, and the improvement in survival in these patients with OMS721 is compelling,” stated Rafael Duarte, M.D., Ph.D., 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. “Thrombotic microangiopathy following stem cell transplantation is increasingly being recognized as part of a spectrum of endothelial cell injury syndromes caused by the transplantation as well as by the post-transplant medications and complications. These complications include GvHD and diffuse alveolar hemorrhage. Seeing improvement in overall survival and TMA markers combined with resolution of GvHD and diffuse alveolar hemorrhage in critically ill patients indicates the role that the lectin pathway plays in these syndromes and the wide potential of OMS721 in stem cell transplantation.”

In the Phase 2 HCT-TMA clinical trial, patients receive weekly OMS721 treatments for four or eight weeks, at the discretion of the investigator. To be eligible for enrollment, HCT-TMA patients are required to be adults with post-transplant TMA persisting 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 severe co-existing conditions, and mortality rates have been reported to be as high as 100 percent.

“Hematopoietic stem cell transplantation is a potentially curative and life-saving medical procedure but is far too often complicated by thrombotic microangiopathy, for which serious cases carry an unacceptably high mortality rate,” stated Gregory A. Demopoulos M.D., chairman and chief executive officer of Omeros. “The improvement in overall survival in such a seriously ill patient population is compelling. We look forward to working with regulatory agencies to make our drug broadly available to transplant patients as quickly as possible.”

Omeros is scheduled and expects to meet with the U.S. Food and Drug Administration and with the European Medicines Agency to discuss the most expeditious path to approval for OMS721 in HCT-TMA.

About HCT-TMA

Thrombotic microangiopathy is a potentially life-threatening complication of HCT. Approximately 20,000 HCT procedures are performed in the U.S. annually, and TMA is reported to occur in approximately 10 to 25 percent of HCT patients. Although the kidney is the most commonly affected organ, HCT-TMA is a multi-system disorder and can also manifest clinically in the lungs, gastrointestinal tract and central nervous system. Reported mortality in patients with multi-organ involvement is greater than 90%. Even in patients who survive acute episodes, HCT-TMA increases the risk for chronic kidney disease and end-stage renal disease.

About Graft-versus-Host Disease

Graft-versus-host disease is a common complication of HCT. Both acute and chronic forms exist and result from donor immune cells recognizing the recipient patient as foreign tissue. This triggers an immune response against the recipient patient. Acute GvHD occurs in up to 50% or more of patients who receive allogeneic transplants. Acute GvHD most commonly targets the skin, gastrointestinal tract, and liver, but can also affect the kidney, eye, lung, and blood cells. Chronic GvHD occurs in approximately 40% of patients who receive allogeneic transplants and most commonly affects the skin, liver, eye, gastrointestinal tract and lungs. Both acute and chronic GvHD are related to significant morbidity and mortality.

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, 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," "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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 9, 2017. 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.

View source version on businesswire.com: <http://www.businesswire.com/news/home/20180215005588/en/>

Source: Omeros Corporation

Cook Williams Communications, Inc.

Jennifer Cook Williams, 360-668-3701

Investor and Media Relations

jennifer@cwcomm.org