



NEWS RELEASE

Omeros Reports Positive Data Across Primary and Secondary Endpoints in Pivotal Trial of Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy Patients Treated with Narsoplimab

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- 56 Percent of All Narsoplimab-Treated Patients Were Complete Responders by Primary Endpoint, Achieving Full Set of Response Criteria Agreed by FDA; 68 Percent Primary-Endpoint Complete Response Rate for Those Who Received Protocol-Specified Treatment of At Least 4 Weeks of Dosing –
- 100-Day Survival Across All Narsoplimab-Treated Patients Was 65 Percent; 93 Percent for Complete Responders –
- Statistically Significant ($p < 0.01$) Improvements in Platelet Count, LDH and Haptoglobin Across All Groups of Narsoplimab-Treated Patients –
- No Signal of Any Serious Safety Risk Observed in the Trial; Adverse Events Comparable to Those Commonly Seen in Stem-Cell Transplant –
- Conference Call and Webcast Today at 8:30 a.m. EST –

SEATTLE--(BUSINESS WIRE)--Dec. 4, 2019-- Omeros Corporation (Nasdaq: OMER) today announced positive data from its pivotal clinical trial of the company's novel investigational complement inhibitor narsoplimab in the

treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA), a frequently lethal complication of HSCT. These preliminary data were recently provided to FDA as part of the company's ongoing interactions with the Agency on the narsoplimab Biologics License Application (BLA). All safety and efficacy endpoints – including the composite primary endpoint and the secondary endpoints – are agreed with FDA. The reported data support a strongly positive benefit-risk balance.

The primary efficacy endpoint in this single-arm open-label trial of HSCT-TMA patients is the proportion of patients who achieve a highly rigorous set of response criteria that requires both improvement in HSCT-TMA laboratory markers and improvement in clinical status (organ function and transfusions). Patients who did not fully meet these criteria were considered non-responders. The secondary endpoints include survival rates and change from baseline in HSCT-TMA laboratory markers. Consistent with the pre-specified statistical analysis plan for the trial, the primary and secondary endpoints are assessed for (1) all patients who received at least one dose of narsoplimab and (2) patients who received at least 4 weeks of narsoplimab dosing. Patients enrolled in this trial had a high expected mortality rate. In severe cases of HSCT-TMA, mortality can exceed 90 percent.

Primary Efficacy Endpoint:

- 56 percent of all patients receiving at least one dose of narsoplimab achieved complete responder status (met full set of FDA-agreed response criteria).
- 68 percent of patients who received the protocol-specified narsoplimab treatment of at least 4 weeks of dosing achieved complete responder status.

Secondary Endpoints:

- 100-day survival following HSCT-TMA diagnosis for all patients receiving at least one dose of narsoplimab was 65 percent.
- 100-day survival following HSCT-TMA diagnosis for patients who received the protocol-specified treatment of at least 4 weeks of narsoplimab dosing was 81 percent.
- 100-day survival following HSCT-TMA diagnosis in the complete responder group was 93 percent.
- Substantial and statistically significant improvements in platelet count, LDH and haptoglobin were observed across all of the following groups:
 - All patients who received at least one dose of narsoplimab ($p < 0.01$ for each laboratory value)
 - Patients who received protocol-specified treatment of at least 4 weeks of dosing ($p \leq 0.002$ for each laboratory value)
 - Complete responders ($p < 0.001$ for each laboratory value).
- Hemoglobin increased across all groups and reached statistical significance ($p = 0.041$) in complete

responders.

- Creatinine also improved meaningfully in all patient groups but did not reach statistical significance given the use of nephrotoxic agents in trial patients.

Safety:

- No signal of any serious safety risk has been observed with narsoplimab in the trial.
- The most common adverse events seen in this trial were nausea, vomiting, diarrhea, hypokalemia, neutropenia and fever – all common in stem-cell transplant patients.
- 21 percent of patients died during the trial due to causes common in stem cell transplant, with no additional patients discontinuing for adverse events. The data from the patients who died were not excluded from any analyses.

The HSCT-TMA patient population enrolled in this trial had multiple high-risk features that portend a poor outcome. These include persistence of HSCT-TMA despite modification of immunosuppression (which was a criterion for entry into the trial), graft-versus-host disease, significant infections, non-infectious pulmonary complications and neurological findings. Patients in the trial had a high expected death rate, with 93 percent of them having multiple risk factors.

Patient enrollment in the pivotal trial has been completed. The details of the endpoints, including the response criteria agreed with FDA, and the number of patients in the trial remain confidential for competitive business reasons.

Last year the company reported data on 19 HSCT-TMA patients treated with narsoplimab on which FDA granted breakthrough therapy designation. The results reported today are even stronger. The response rate remains equally high at 56 percent, while the 100-day survival has improved from 53 percent to 65 percent.

“The response rate in this high-risk population would be expected to be 10 to 15 percent with a 100-day survival rate of less than 20 percent. The response rate and 100-day survival achieved with narsoplimab in this trial demonstrate an unprecedented effect in this condition,” said Rafael Duarte M.D., Ph.D., F.R.C.P., Associate Professor, Head of Hematology Department and Hematopoietic Transplantation Program, University Hospital Puerta de Hierro Majadahonda, Madrid, Spain, and Secretary of the European Society for Blood and Marrow Transplantation. “The other secondary endpoints are equally impressive. The data are consistent with my personal experience with narsoplimab. Patients with severe forms of HSCT-TMA have a dismal prognosis with no treatment currently available. I expect a treatment with this profile would be widely adopted for use in these patients and even lead to increased physician recognition of the disorder.”

Omeros reported the initiation of its rolling BLA in October. Narsoplimab, also referred to as OMS721, is Omeros' lead human monoclonal antibody targeting mannan-binding lectin-associated serine protease-2 (MASP-2) and has breakthrough therapy designation from FDA for this indication.

"The striking results seen in our pivotal trial are tremendously gratifying," said Gregory A. Demopoulos, M.D., chairman and chief executive officer of Omeros. "Our rolling BLA is underway – the nonclinical sections have been submitted and the data from this trial form the efficacy basis of the application. We continue to compile the remaining sections of the BLA and look forward to continued partnership with regulators to make narsoplimab widely available for the treatment of this devastating condition."

Data from this pivotal trial will also support the narsoplimab marketing authorization application for HSCT-TMA in Europe. The data are planned for publication and for presentation at international congresses in the first part of 2020.

In addition to breakthrough therapy designation from FDA, narsoplimab has orphan drug designation in both the U.S. and Europe for HSCT-TMA. Narsoplimab also has been awarded breakthrough therapy designation for immunoglobulin A nephropathy (IgAN), and Omeros has Phase 3 programs for narsoplimab ongoing in IgAN and in atypical hemolytic uremic syndrome (aHUS).

Conference Call and Webcast Details

Omeros' management will host a webcast and conference call to present data from its pivotal trial of narsoplimab in HSCT-TMA. The call will be held today at 8:30 a.m. Eastern Time; 5:30 a.m. Pacific Time. To access the live conference call via phone, please dial (844) 831-4029 from the United States and Canada or (920) 663-6278 internationally. The participant passcode is 3774209. Please dial in approximately 10 minutes prior to the start of the call. A telephone replay will be available for one week following the call and may be accessed by dialing (855) 859-2056 from the United States and Canada or (404) 537-3406 internationally. The replay passcode is 3774209.

To access the live or subsequently archived webcast and presentation materials on the internet, go to the company's website at www.omeros.com and select "Events" under the Investors section of the website. To access the live webcast, please connect to the website at least 15 minutes prior to the call to allow for any software download that may be necessary.

About Omeros Corporation

Omeros is an innovative biopharmaceutical company committed to discovering, developing and commercializing small-molecule and protein therapeutics for large-market as well as orphan indications targeting complement-

mediated diseases, disorders of the central nervous system and immune-related diseases, including cancers. In addition to its commercial product **OMIDRIA® (phenylephrine and ketorolac intraocular solution) 1%/0.3%**, Omeros has multiple Phase 3 and Phase 2 clinical-stage development programs focused on complement-mediated disorders and substance abuse. In addition, the company has a diverse group of preclinical programs including GPR174, a novel target in immuno-oncology that modulates a new cancer immunity axis recently discovered by Omeros. Small-molecule inhibitors of GPR174 are part of Omeros' proprietary G protein-coupled receptor (GPCR) platform through which it controls 54 new GPCR drug targets and their corresponding compounds. The company also exclusively possesses a novel antibody-generating platform.

About HSCT-TMA

Hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA) is a significant and often lethal complication of stem cell transplants. This condition is a systemic, multifactorial disorder caused by endothelial cell damage induced by conditioning regimens, immunosuppressant therapies, infection, GvHD, and other factors associated with stem cell transplantation. Endothelial damage, which activates the lectin pathway of complement, plays a central role in the development of HSCT-TMA. The condition occurs in both autologous and allogeneic transplants but is more common in the allogeneic population. In the United States and Europe, approximately 25,000 to 30,000 allogeneic transplants are performed annually. Recent reports in both adult and pediatric allogeneic stem cell transplant populations have found an HSCT-TMA incidence of approximately 40 percent, and high-risk features may be present in up to 80 percent of these patients. In severe cases of HSCT-TMA, mortality can exceed 90 percent and, even in those who survive, long-term renal sequelae are common. There is no approved therapy or standard of care for HSCT-TMA.

About Narsoplimab

Narsoplimab, also known as "OMS721," is an investigational human monoclonal antibody targeting mannan-binding lectin-associated serine protease-2 (MASP-2), a novel pro-inflammatory protein target and the effector enzyme of the lectin pathway of complement. 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. Omeros controls the worldwide rights to MASP-2 and all therapeutics targeting MASP-2.

Phase 3 clinical programs are in progress for narsoplimab in hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA), in immunoglobulin A (IgA) nephropathy, and in atypical hemolytic uremic syndrome (aHUS). The FDA has granted narsoplimab breakthrough therapy designations for HSCT-TMA and for IgA nephropathy; orphan drug status for the prevention (inhibition) of complement-mediated thrombotic microangiopathies, for the treatment of HSCT-TMA and for the treatment of IgA nephropathy; and fast track

designation for the treatment of patients with aHUS. The European Medicines Agency has granted orphan drug designation to narsoplimab for treatment in HSCT and for treatment of primary IgA nephropathy.

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,” “on track,” “plan,” “potential,” “predict,” “project,” “prospects,” “scheduled,” “should,” “slated,” “targeting,” “will,” “would” and similar expressions and variations thereof. Forward-looking statements, including statements regarding anticipated regulatory submissions, expectations regarding regulatory exclusivities, the timing and results of ongoing or anticipated clinical trials, and the therapeutic application of Omeros’ investigational product, 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, availability and timing of data from clinical trials and the results of such trials, 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, 2019. 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, whether as a result of any new information, future events or otherwise, except as required by applicable law.

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