



NEWS RELEASE

Omeros Corporation Reports Updated Results from Narsoplimab HSCT-TMA Clinical Trial and Highlights from Recent Clinical and CMC Meetings with FDA

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– Results Surpass FDA-Agreed Efficacy Threshold –

SEATTLE--(BUSINESS WIRE)-- Omeros Corporation (Nasdaq: OMER) today reports an update on clinical data from its pivotal trial of narsoplimab in the treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA), markedly exceeding the FDA-agreed threshold for the primary efficacy endpoint. While an overview of preliminary data submitted to FDA was made public on December 4, 2019 in a press release from the company, all patients have now completed treatment and trial enrollment has been closed. Narsoplimab is Omeros' human monoclonal antibody targeting mannan-binding lectin-associated serine protease 2 (MASP-2).

In recent meetings with FDA focused on clinical as well as chemistry, manufacturing and controls (CMC) data, FDA confirmed important aspects of Omeros' rolling Biologics License Application (BLA) for narsoplimab in HSCT-TMA. The BLA continues on its clear path to completion.

The efficacy threshold agreed with FDA, the updated results from the 28-patient trial, and highlights of the recent FDA meetings are the following:

Primary Endpoint

- 15% is the FDA-agreed efficacy threshold for the primary endpoint (i.e., the complete response rate [CRR]) in the clinical trial
- The CRR for the study population, and the lower limit of the 95 percent confidence interval (95% CI), significantly exceed the efficacy threshold:
 - 54 percent CRR (95% CI = 34 percent to 72 percent, p-value < 0.0001) in patients who received at least one dose of narsoplimab
 - 65 percent CRR (95% CI = 43 percent to 84, p-value < 0.0001) in patients who received the protocol-specified narsoplimab treatment of at least 4 weeks of dosing
- As described in the December 4, 2019 press release, the FDA-agreed primary endpoint (the CRR) is the proportion of patients who fully achieve a rigorous set of response criteria, requiring both improvement in HSCT-TMA laboratory markers (platelet count and lactate dehydrogenase [LDH] levels) and improvement in clinical status comprised of organ (renal, pulmonary, gastrointestinal and neurological) function and transfusions (platelet and red blood cells). The full response criteria are provided below.

Secondary Endpoints

- The 100-day survival (defined as survival from the day of HSCT-TMA diagnosis) is 68 percent in all treated patients, 83 percent in patients who received at least 4 weeks of narsoplimab treatment as specified by the protocol, and 93 percent in patients who responded to narsoplimab treatment. Experts familiar with the pivotal trial data would expect a 100-day survival rate of less than 20 percent in the trial population.
- Preliminary results of the laboratory secondary efficacy endpoints (change from pre-treatment baseline for each laboratory value) continue to demonstrate meaningful improvement and meet statistical significance in platelet count, LDH and haptoglobin (p < 0.01 in all treated patients).

Safety

- The most commonly reported adverse events in the trial were diarrhea, nausea, vomiting, hypokalemia, neutropenia and fever – all common in stem-cell transplant patients.
- Six deaths occurred during the trial. These were due to sepsis, progression of the underlying disease, and graft-versus-host disease, all common causes of death in this patient population.

The treated population had multiple high-risk features that portend a poor outcome, including the 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 mortality rate, with 93% of them having multiple risk factors.

“The efficacy and safety data from the pivotal trial with narsoplimab are encouraging,” said Miguel-Angel Perales,

M.D., Deputy Chief of the Adult Bone Marrow Transplantation Service and Director of the Adult Stem Cell Transplantation Fellowship at Memorial Sloan Kettering Cancer Center. "Given the trial's stringent response criteria across laboratory markers and organ function, the complete response rate seen with narsoplimab is remarkable, as is the 100-day survival. There currently is no approved treatment for HSCT-TMA. Current therapy is generally limited to supportive care and withdrawal of drugs critical for GVHD prophylaxis. Not only could narsoplimab become central to the treatment of HSCT-TMA, it might well allow us to maintain that needed GVHD prophylaxis."

Complete clinical trial data will be presented by Dr. Perales later this month at the Annual Meeting of the European Society for Blood and Marrow Transplantation in Madrid.

Recent FDA Meeting Highlights and CMC Updates

- FDA confirmed that the number of HSCT-TMA patients enrolled is sufficient for the BLA's filing and review for approval. FDA agreed to stopping enrollment.
- FDA requested near-term manufacturing dates for narsoplimab so that FDA's pre-approval inspections could be scheduled.
- FDA and Omeros' reached agreement on CMC requirements for stability data and release assays.
- Omeros elected to accelerate the manufacturing schedule for a one-time set of five narsoplimab process validation and commercial lots. These lots were successfully manufactured by Omeros' manufacturing partner Lonza, satisfy the BLA requirements and can be used for commercial sale following approval.

"The non clinical sections of our BLA have been submitted, our CMC campaign is progressing well with process validation and commercial lots already manufactured, and our pivotal trial is complete," stated Gregory A. Demopulos, M.D., chairman and chief executive officer of Omeros. "The efficacy threshold agreed with FDA reflects both the primary endpoint's stringent response criteria and the poor outcomes expected in the patients enrolled in our trial. Of course, we're very pleased that the response rates and confidence intervals seen with narsoplimab are well above that efficacy threshold. We look forward to continuing to work closely with regulators to make the drug commercially available to transplanters and their patients in the U.S. and internationally as quickly as possible."

In addition to its HSCT-TMA program, Omeros is enrolling its narsoplimab Phase 3 clinical trials for immunoglobulin A (IgA) nephropathy and atypical hemolytic uremic syndrome (aHUS). Narsoplimab has been granted, for both HSCT-TMA and IgA nephropathy, FDA's breakthrough therapy designation as well as orphan drug designations from FDA and the European Medicines Agency. The drug also holds FDA's fast-track designation for aHUS.

Primary Efficacy Endpoint

To be considered a responder, a patient must achieve the primary endpoint of complete HSCT-TMA response

defined by improvement in laboratory markers and improvement in clinical status.

Laboratory Markers

- Criteria for improvement in laboratory markers are LDH less than 1.5 x upper limit of normal AND improvement of platelet count measures:
 - For patients with baseline platelet count $\leq 20,000/\mu\text{L}$, response requires tripling over baseline platelet count, a post-baseline platelet count $> 30,000/\mu\text{L}$, and freedom from platelet transfusion
 - For patients with baseline platelet count $> 20,000/\mu\text{L}$, response requires: an increase in platelet count by $\geq 50\%$, a post-baseline platelet count $> 75,000/\mu\text{L}$, and freedom from platelet transfusion

Clinical Status

- Criteria for improvement in clinical status requires at least one of the following:
 - Renal response – requires $> 40\%$ reduction in creatinine, or normalization of creatinine and $> 20\%$ reduction in creatinine, or discontinuation of renal replacement therapy
 - Pulmonary response – requires extubation and discontinuation of ventilator support, or discontinuation of non-invasive mechanical ventilation (continuous positive pressure ventilation)
 - Gastrointestinal response – applicable only to patients with biopsy-proven gastrointestinal HSCT-TMA and requires improvement in gastrointestinal function as determined by the Mount Sinai Acute GVHD International Consortium (MAGIC) criteria
 - Neurological response – requires improvement in reversible neurological conditions (e.g., cessation of seizures), or stabilization of irreversible neurological conditions (e.g., stability of neurological deficits following stroke without further deterioration or subsequent strokes)
 - Freedom from transfusion – only applicable if patient was undergoing transfusion at baseline

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,” “can,” “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 for the year ended December 31, 2019, filed with the Securities and Exchange Commission on March 2, 2020. 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.

Dr. Miguel-Angel Perales has received compensation from Omeros for advisory services.

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