



NEWS RELEASE

Omeros' Narsoplimab Pivotal Trial Data Shared in Oral Presentation at Annual European Hematology Association Congress

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SEATTLE--(BUSINESS WIRE)--Jun. 12, 2020-- Omeros Corporation (Nasdaq: OMER) today announced that the results of its pivotal trial of narsoplimab in the treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA) were shared during an oral presentation at the virtual edition of the 25th **Congress of the European Hematology Association (EHA)**. The EHA oral presentation, entitled Narsoplimab (OMS721) for the Treatment of Adult Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy was delivered by Alessandro Rambaldi, MD, Professor, Department of Oncology and Hematology-Oncology at the University of Milan and Head of the Hematology and Bone Marrow Transplant Unit at ASST Papa Giovanni XXIII in Bergamo, Italy.

"I was excited to present narsoplimab data demonstrating that targeted upstream inhibition of the lectin pathway of complement – specifically MASP-2 – is a promising treatment strategy for HSCT-TMA," said Professor Rambaldi. "Narsoplimab demonstrated a 54 percent complete response rate in all patients and a 65 percent complete response rate in patients receiving at least 4 weeks of narsoplimab treatment. In this population of very sick patients, those treated with at least one dose of narsoplimab and the responder group showed remarkable 100-day survival rates of 68 and 93 percent, respectively. We also saw marked and clinically significant improvements in laboratory markers and organ function. HSCT-TMA is a devastating post-transplant complication with high morbidity and mortality. We look forward to having this new treatment option available soon for our patients."

There is no approved therapy or standard of care for HSCT-TMA. HSCT-TMA is a systemic, multifactorial disorder caused by endothelial cell damage induced by conditioning regimens, immunosuppressant therapies, infection, graft-versus-host disease, and other factors associated with stem cell transplantation. Recent reports in both adult and pediatric allogeneic stem cell transplant populations have reported an approximately 40-percent incidence of TMA following stem cell transplantation. At least one high-risk feature is 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 chronic organ injury and other conditions can persist.

The detailed clinical trial data presented underscore the importance of narsoplimab as a potentially significant advance in the treatment of often-fatal HSCT-TMA. The data include:

- 54 percent complete response rate in all patients (n = 28) and 65 percent complete response rate in patients receiving at least 4 weeks of narsoplimab treatment (n = 23)
- 100-day survival rate of 68 percent among all patients receiving any dose of narsoplimab
- 83 percent 100-day survival in patients receiving at least 4 weeks of narsoplimab treatment
- 93 percent 100-day survival among complete responders
- Statistically and clinically significant improvements from baseline in platelet count (p = 0.001), lactate dehydrogenase (LDH) (p = 0.008), and haptoglobin (p < 0.001)
- A well-tolerated safety profile: no infusion side effects reported in patients receiving narsoplimab; most common adverse events were nausea, vomiting, diarrhea, hypokalemia, neutropenia, and fever – all common in this patient population; 6 patients died during the study, again all due to causes common in HSCT with most early in the course of narsoplimab treatment

A high-risk population was enrolled in this pivotal single-arm open-label trial of patients with HSCT-TMA. High-risk features included persistence of HSCT-TMA despite modification of immunosuppression (which was a criterion for trial inclusion), graft-versus-host-disease, significant infections, non-infectious pulmonary complications and neurological findings.

The pivotal trial protocol specified that patients receive narsoplimab intravenously once weekly for 4 or 8 weeks, with a 6-week follow-up period. As presented today, the FDA-agreed primary endpoint is a response-based composite measure requiring improvement in laboratory markers of TMA (platelet count and LDH) and improvement in clinical status (i.e., organ function [renal, pulmonary, gastrointestinal, or neurological] and transfusion burden). The FDA-agreed efficacy threshold for the primary endpoint is 15 percent. Secondary endpoints were survival and changes in laboratory TMA markers. Submission of a rolling Biologics License Application for marketing authorization is underway and completion is expected next quarter.

Dr. Rambaldi's slide presentation can be viewed at <https://www.omeros.com/scientific-publications/>.

About Narsoplimab

Narsoplimab, also known as “OMS721,” is an investigational human monoclonal antibody targeting mannose-binding lectin-associated serine protease-2 (MASP-2), a novel proinflammatory 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.

In September 2019, Omeros announced that the FDA had agreed with the company’s proposed schedule for the rolling submission of its Biologics License Application (BLA) for narsoplimab in the treatment of HSCT-TMA. The rolling submission enables Omeros to submit sections of the BLA as they are completed, which may accelerate the time to approval; the FDA can review sections as they are submitted rather than wait to begin its review until the entire BLA has been submitted. Omeros anticipates completion of the BLA submission process next quarter.

In addition to the BLA for HSCT-TMA, narsoplimab is in Phase 3 clinical programs for narsoplimab 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.

About Omeros Corporation

Omeros is a commercial-stage biopharmaceutical company committed to discovering, developing and commercializing small-molecule and protein therapeutics for large-market and orphan indications targeting inflammation, 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. Omeros also 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.

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 (SEC) on March 2, 2020, as supplemented by its Quarterly Report on Form 10-Q filed with the SEC on May 11, 2020 and subsequent filings with the SEC. 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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