



NEWS RELEASE

Omeros Corporation Streamlines Path to Biologics License Application for OMS721 in HSCT-TMA Following Meeting with FDA

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Patient Data from Existing Single-Arm Study Will Form the Basis for BLA; No Historical Control Required

SEATTLE--(BUSINESS WIRE)--Feb. 14, 2019-- Omeros Corporation (Nasdaq: OMER) today announced that, based on a recent meeting with the U.S. Food and Drug Administration (FDA), the company has streamlined the required work and further advanced its preparations for its Biologics License Application (BLA) for OMS721 in the treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA). OMS721 holds FDA's breakthrough therapy designation as well as orphan drug designations from FDA and the European Medicines Agency (EMA) for HSCT-TMA. OMS721 is the company's Phase 3 human monoclonal antibody targeting mannan-binding lectin-associated serine protease-2 (MASP-2), the effector enzyme of the lectin pathway of the complement system.

Agreements reached during the recent FDA meeting are the following:

- A response-based analysis is the most appropriate and expeditious assessment for inclusion in an OMS721 BLA for the treatment of HSCT-TMA. Criteria for this primary-endpoint analysis were proposed by Omeros and discussed with FDA and are expected to be finalized in the near term. Overall and 100-day survival will now be secondary endpoints.

- Using the response-based analysis as the primary endpoint eliminates the need for a historical control, and Omeros is no longer conducting its chart review-based historical-control data collection.
- Patient data from the existing single-arm OMS721 HSCT-TMA trial will form the clinical basis for the BLA, and Omeros will collect additional data from patients currently in the trial for inclusion in the BLA.
- FDA will consider not only accelerated approval but also regular (full) approval for OMS721 in HSCT-TMA, with the determination based on the submitted data.
- A rolling BLA submission is appropriate for OMS721 in this indication. Omeros plans to submit first the nonclinical sections, which were completed late last year.

“We appreciate FDA’s collaborative approach to the development of OMS721 for patients with stem cell transplant-associated TMA. We now have an even clearer and more streamlined path to submitting our BLA, saving time and crucial resources as we advance toward regulatory review and approval,” stated Gregory A. Demopoulos, M.D., chairman and chief executive officer of Omeros. “There’s no FDA-approved treatment for stem cell transplant-associated TMA, and severe cases progress rapidly with a very high mortality rate. We have seen in our ongoing clinical trial the life-saving effects of OMS721 in these patients, a good number of whom had not only TMA but also other components – such as graft-versus-host disease and diffuse alveolar hemorrhage – of broader endothelial-injury syndrome. We remain committed to working closely with FDA and European regulators to make OMS721 available as quickly as possible to patients who need it.”

Omeros plans to meet with European regulators and expects to harmonize the marketing authorization application (MAA) for OMS721 in HSCT-TMA in Europe with the U.S. BLA. Omeros has received positive feedback from EMA’s Pediatric Development Committee on its Pediatric Investigational Plan for OMS721, a required element for an MAA, and intends to meet in the second quarter of this year with the rapporteurs assigned to the company’s application.

In addition to its HSCT-TMA registration program, Omeros is enrolling its OMS721 Phase 3 clinical trials for immunoglobulin A (IgA) nephropathy and atypical hemolytic uremic syndrome (aHUS). OMS721 also holds FDA’s breakthrough therapy designation for IgA nephropathy, FDA’s and EMA’s orphan drug designations for IgA nephropathy, and FDA’s fast-track designation for aHUS.

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. Gene-targeted MASP-2-deficient mice and humans with MASP-2 gene polymorphisms that affect MASP-2 serum levels and MASP-2 functional activity are generally healthy with no obvious adverse phenotype.

Phase 3 clinical programs are in progress for OMS721, Omeros' lead MASP-2 inhibitor, in hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA), in immunoglobulin A (IgA) nephropathy, and in atypical hemolytic uremic syndrome (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 HSCT-TMA, orphan drug status for the prevention (inhibition) of complement-mediated thrombotic microangiopathies, for the treatment of IgA nephropathy and for the treatment of HSCT-TMA, and fast track designation for the treatment of patients with aHUS. The European Medicines Agency has granted orphan drug designation to OMS721 for treatment of primary IgA nephropathy and for treatment in HSCT.

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 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,” “prospects,” “should,” “slated,” “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, the ability for OMIDRIA to obtain separate reimbursement as part of CMS’ OPPS, 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 8, 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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