



NEWS RELEASE

## Omeros Corporation Reaches Agreement with FDA on OMS721 Phase 3 Trial Protocol for IgA Nephropathy

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-- Also Initiates OMS721 Phase 3 Program in HCT-TMA --

SEATTLE--(BUSINESS WIRE)--Jan. 3, 2018-- Omeros Corporation (Nasdaq: OMER) today announced that it has reached agreement with the US Food and Drug Administration (FDA) on Omeros' protocol for its Phase 3 clinical trial evaluating OMS721 in patients with IgA nephropathy (IgAN). Patient enrollment is expected to begin early next month.

The single Phase 3 trial is a randomized, double-blind, placebo-controlled trial in patients at least 18 years of age with biopsy-confirmed IgAN and with 24-hour urine protein excretion greater than 1 g/day at baseline on optimized renin-angiotensin system (RAS) blockade. Patients will receive an initial 12 weekly intravenous doses of study drug; additional weekly dosing can be administered for partial responders and relapsers. The primary endpoint, which could suffice for full approval, is reduction in proteinuria at 24 weeks after the start of dosing. The trial will employ an adaptive design that will allow intra-trial adjustment in sample size. For purposes of safety and efficacy assessments, the initial sample size for the proteinuria endpoint is estimated at 140 patients in each of the treatment and placebo groups. This will include a subset of patients with high levels of proteinuria (i.e., equal to or greater than 2 g/day) at baseline, and full approval could also be obtained if a substantial improvement is seen at

24 weeks in this subset of patients alone. The trial design will allow assessment for either full or accelerated approval at 24 weeks based on proteinuria results either (1) across the general population of study patients or (2) in the high-proteinuria subset of patients. In the event that the primary endpoint at 24 weeks results in accelerated approval from FDA, change in estimated glomerular filtration rate (eGFR) will be assessed at approximately three years after the start of dosing. The initial sample size estimate for the eGFR endpoint is approximately 160 patients per group and also will be adjustable under the study's adaptive design.

FDA granted both breakthrough therapy designation and orphan drug designation for OMS721 in IgAN. Omeros' applications to the European Medicines Agency (EMA) for orphan drug status and for eligibility to the Priority Medicines (PRIME) program for OMS721 in IgAN are pending.

Omeros has also initiated its Phase 3 program for OMS721 in hematopoietic stem cell-associated thrombotic microangiopathy (HCT-TMA) and intends to amend the ongoing Phase 2 protocol following discussion with FDA and/or EMA to transition the protocol into a Phase 3/pivotal trial. In the third quarter of 2017, Omeros submitted a preliminary breakthrough therapy designation request to FDA for OMS721 in HCT-TMA. Based on the data in that submission, FDA requested that Omeros submit a full application for breakthrough therapy designation for OMS721 in HCT-TMA following collection of additional information on the patients in the Phase 2 clinical trial. Omeros intends to submit the full application for breakthrough therapy designation by the end of this month. Enrollment in the Phase 2 trial has continued pending initiation of/conversion to the pivotal trial. Omeros also recently applied for eligibility to EMA's PRIME program for OMS721 in HCT-TMA. FDA has already granted orphan designation for OMS721 in the prevention (inhibition) of complement-mediated TMAs, which includes HCT-TMA.

The company's Phase 3 program in aHUS is also ongoing in both the US and Europe. The single-arm, open label, Phase 3 trial is targeting approximately 40 patients for EMA approval and US accelerated approval with 80 patients required for full approval in the US. Dosing consists of an initial IV loading followed by daily subcutaneous dosing. The FDA has granted OMS721 fast track designation in aHUS as well as orphan designation for OMS721 in aHUS and other complement-mediated TMAs.

"We're pleased to have received agreement from FDA for our OMS721 Phase 3 protocol in IgA nephropathy," said Gregory A. Demopoulos, M.D., chairman and chief executive officer of Omeros. "Final preparations to begin the trial can now be completed and enrollment is expected to open in early February. To our OMS721 Phase 3 programs in IgA nephropathy and aHUS, we have recently added a third Phase 3 program in stem cell transplant-associated TMA. We look forward to ongoing discussions with both FDA and EMA regarding the HCT-TMA Phase 3 trial as well as breakthrough and PRIME designations. In the meantime, enrollment in the Phase 2 HCT-TMA trial has progressed with patients expected to have a very high mortality rate doing well on OMS721."

## 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," "likely," "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.

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