



NEWS RELEASE

FDA Grants Orphan Drug Designation to Omeros' MASP-3 Inhibitor OMS906 for Treatment of Paroxysmal Nocturnal Hemoglobinuria

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-- Enrollment of PNH Patients Expected to Begin this Summer --

SEATTLE--(BUSINESS WIRE)--Jul. 29, 2022-- Omeros Corporation today announced that OMS906 has received orphan drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). OMS906 targets mannan-binding lectin-associated serine protease-3 (MASP-3), the key activator of the alternative pathway of the complement system. MASP-3 converts pro-complement factor D (pro-CFD) to mature CFD.

PNH is a rare, life-threatening disease characterized by red blood cell destruction, blood clots and impaired bone marrow function. Based on its mechanism of action as well as the pharmacokinetic/pharmacodynamic (PK/PD) profile shown in a completed Phase 1 study, OMS906 has the potential to offer a favorable safety profile and more convenient dosing than other drugs on the market or in development for PNH. Also, different than other enzymes in the alternative pathway targeted by competitors' agents, MASP-3 does not appear to be an acute phase reactant, meaning that the concentration of MASP-3 – and the effective dosing level of OMS906 – do not change in the setting of inflammation. Omeros continues to build a strong and exclusive intellectual property position around therapeutics targeting MASP-3.

“We are excited to begin clinical studies to demonstrate the efficacy of OMS906 in alternative pathway-related disorders,” said Gregory A. Demopoulos, M.D., chairman and chief executive officer of Omeros. “Based on our preclinical and Phase 1 trial data, we expect that OMS906 will perform well. With the potential advantages of MASP-3 inhibition and OMS906 – decreased infection risk, better dosing profile, and the ability to avoid ‘breakthrough’ disease seen with agents targeting acute phase reactants – we believe that OMS906 could become first-line therapy in alternative pathway disorders.”

FDA grants orphan designation to promote the development of a drug that is expected to have significant therapeutic advantage over existing treatments that target a condition affecting 200,000 or fewer U.S. patients annually. It qualifies a company for benefits that apply across all stages of drug development, including seven years of market exclusivity following marketing approval, tax credits on U.S. clinical trials, eligibility for orphan drug grants, and waiver of certain administrative fees.

Omeros recently completed a Phase 1 trial of OMS906 in healthy subjects and expects to begin enrollment this summer in a clinical trial assessing OMS906 in PNH patients who have had an unsatisfactory response to the C5 inhibitor ravulizumab. The company is also targeting efficacy data in populations of treatment-naïve PNH and C3 glomerulopathy patients by early 2023. Based on the results of the completed Phase 1 trial, administration of OMS906 is expected to be once monthly to once quarterly intravenously or subcutaneously.

About OMS906

OMS906 is an investigational human monoclonal antibody targeting mannan-binding lectin-associated serine protease-3 (MASP-3), the key activator of the complement system’s alternative pathway. The complement system plays a central role in inflammation and becomes activated as a result of tissue damage or microbial infection. Responsible for the conversion of pro-complement factor D to complement factor D, MASP-3 is believed to be the premier target in the alternative pathway – it has the lowest native circulating level and low relative clearance compared to the other alternative pathway proteins and, unlike C5 and C3 blockers, MASP-3 inhibition leaves intact the lytic arm of the classical pathway, important for fighting infection. Also, unlike other components of the alternative pathway, MASP-3 is believed not to be an acute phase reactant, which could provide a significant advantage to MASP-3 inhibitors, like OMS906, over other alternative pathway inhibitors. MASP-3 inhibitors are thought to have preventive or therapeutic effects across a broad range of diseases including paroxysmal nocturnal hemoglobinuria (PNH), hemolytic uremic syndrome (HUS), atypical HUS, traumatic brain injury, arthritis, wet age-related macular degeneration, ischemia-reperfusion injury, transplant-related complications and other immune-related disorders. Through its growing and exclusive intellectual property position, Omeros controls the use of MASP-3 inhibitors across a wide range of alternative pathway-related and other diseases and disorders.

About Omeros Corporation

Omeros is an innovative biopharmaceutical company committed to discovering, developing and commercializing small-molecule and protein therapeutics for large-market and orphan indications targeting immunologic diseases, including complement-mediated diseases and cancers related to dysfunction of the immune system, as well as addictive and compulsive disorders. Omeros' lead MASP-2 inhibitor narsoplimab targets the lectin pathway of complement and is the subject of a biologics license application pending before FDA for the treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA). Narsoplimab is also in multiple late-stage clinical development programs focused on other complement-mediated disorders, including IgA nephropathy, atypical hemolytic uremic syndrome and COVID-19. OMS906, Omeros' inhibitor of MASP-3, the key activator of the alternative pathway of complement, is initiating Phase 1b clinical programs in paroxysmal nocturnal hemoglobinuria (PNH) and complement 3 glomerulopathy (C3G). For more information about Omeros and its programs, visit www.omeros.com.

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," "objective," "plan," "potential," "predict," "project," "should," "slate," "target," "will," "would" and similar expressions and variations thereof. Forward-looking statements, including statements regarding the anticipated therapeutic advantages of Omeros' drug candidates, expectations regarding the initiation or continuation of clinical trials evaluating Omeros' drug candidates and the anticipated availability of data therefrom, 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, unanticipated or unexpected outcomes of regulatory processes in relevant jurisdictions, unproven preclinical and clinical development activities, events negatively affecting our financial condition or results of operations, regulatory processes and oversight, challenges associated with manufacture or supply of our investigational or clinical products, 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, 2022. 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 new information, future events or otherwise, except as required by applicable law.

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