The United States FDA Awards Orphan Drug Designation (ODD) to NM5072 for Treating Paroxysmal Nocturnal Hemoglobinuria

CLEVELAND, OHIO, UNITED STATES, April 15, 2024 /EINPresswire.com/ -- NovelMed today announced that the Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to NM5072, an AP blocker anti-Properdin antibody, for the treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH) (www.accessdata.fda.gov/scripts/opdlisting/oopd/detailedIndex.cfm?cfgridkey=835921).

NM5072 is being developed by for patients with PNH in the United States and Globally. The FDA grants ODD status to new medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

PNH is a rare, chronic, inflammatory, and hemolytic disease that occurs when PNH Red Blood Cells, breakdown within and outside the blood vessel, causing anemia, which can be fatal. In addition to PNH RBCs, other cells, including Neutrophils, Monocytes, and Platelets drive cellular destruction and inflammation, leading to long-term effects on the patient's health. If the disease remains untreated or partially treated, patients will continue to have anemia, potentially leading to chronic pain, fatigue, and other common symptoms of PNH.

Dr. Rekha Bansal, Chief Executive Officer, R&D, NovelMed, said: "PNH is a rare disease which involves a range of blood cells that contribute to debilitating symptoms for patients, including anemia, fatigue, and severe pain with ultimate shorter life span if remains untreated. The current standard of care treatments requires further improvements. We are hopeful that NM5072, with its unique mechanism of action that targets the top of the complement cascade, could become a potential new medicine to improve outcomes for these patients."

NM5072 is under regulatory review for multiple indications in the USA and overseas.

"We are thrilled by the FDA's decision, which highlights the demand for groundbreaking therapies for individuals grappling with PNH," comments Robert Bard, VP Regulatory Affairs. This Orphan Drug Designation approval represents a remarkable stride in therapeutic innovation, showcasing NM5072's distinct capability to selectively inhibit the alternative pathway (AP) while safeguarding the classical pathway essential for combating infections in PNH patients.

ORPHAN DRUG DESIGNATION (ODD) ---

The ODD from the FDA is a special designation granted to facilitate the development and assessment of new medicines. The FDA's Office of Orphan Products Development has granted ODD for NM5072 which is in development for the treatment, diagnosis, and prevention of rare diseases or conditions impacting fewer than 200,000 people in the US. This Designation offers various advantages which include the potential for market exclusivity lasting seven years upon FDA approval, eligibility for tax credits related to qualified clinical trials, waiver of application fees, reduced annual product fees, clinical protocol assistance, and the potential qualification for expedited development programs.

NM5072 (ANTI-PROPERDIN MONOCLONAL ANTIBODY) −−−

NM5072 is being developed by NovelMed as a potential first-in-class monoclonal antibody that selectively blocks the parts of the immune system that cause the disease, a key step that sits at the top of alternative pathway cascades. Blocking Properdin function is expected to block the lysis of PNH Red Blood Cells, which are implicated in the pathophysiology of anemia in PNH.
NM5072 is an investigation drug that selectively binds to protein Properdin of the alternative pathway. This humanized anti-Properdin monoclonal antibody serves as an efficacious blocker of the Alternative Pathway without blocking the part of the immune system required for clearing infections.
NM5072 has successfully completed the phase I trial in healthy volunteers with expected outcomes. This potent drug is in regulatory phases of development. The Anti-Properdin molecule is certainly a pioneering biologic with a much longer half-life. Regulatory preparations and review of Phase II trials for multiple indications, including C3 Glomerulopathy (C3G) and Atypical Hemolytic Uremic Syndrome (aHUS) are underway.

COMPLEMENT-MEDIATED DISORDERS −−−

The dysregulation of the Alternative Pathway (AP) stands as a pivotal factor influencing the trajectory of numerous acute and chronic rare diseases, spanning disciplines such as Hematology, Ophthalmology, Nephrology, Dermatology, and Neurology. Among these, Paroxysmal Nocturnal Hemoglobinuria (PNH) emerges as a prominent indication for complement blockers undergoing Phase II and III trials for FDA approval. This pivotal proof of efficacy study not only paves the way for future trials in diverse indications but also underscores the pressing need for novel therapeutic interventions.
In the context of PNH, the current armamentarium of treatment options remains limited and imperfect, often compromising infection clearance or inducing hyperlipidemia. PNH manifests with a spectrum of clinical features, including low PNH-RBC clone size, hemolysis, hemoglobinuria, reduced hemoglobin levels, elevated lactate dehydrogenase (LDH), and a plethora of other symptoms that can precipitate chronic complications, potentially leading to premature mortality if left untreated. Chronic symptoms and multisystem organ damage are recurrent in PNH patients.
While FDA-approved anti-C3, C5, and Factor B drugs provide potential for symptom management

in PNH treatment, they should be approached with caution due to their significant side effects. The imperative for additional treatment modalities for PNH, characterized by both efficacy and tolerability, remains unequivocally recognized, holding the promise of enhancing disease outcomes and improving the quality of life for affected individuals.

ABOUT NOVELMED ---

Dedicated to pioneering innovative biologics for a range of complement-mediated diseases, NovelMed stands at the forefront of conceptualizing and validating an anti-Properdin antibody for chronic complement-mediated and complement-associated disorders. Progressing its leading product candidate through clinical trials, the company primarily focuses on addressing hemolytic and renal disorders initially.
“Driven by a commitment to ingenuity and compassion, NovelMed is committed to delivering groundbreaking diagnostics and therapies that redefine the treatment landscape for patients with rare diseases,” said Dr. Rekha Bansal, Chief Executive Officer. Positioned as a frontrunner in the development of therapies targeting the Alternative Pathway (AP), NovelMed is devoted to addressing debilitating diseases across Hematology, Ophthalmology, Nephrology, Dermatology, and Neurology.

Our R&D team is currently engaged in the development of lead candidates aimed at identifying complement-mediated diseases. This synergistic strategy is geared towards broadening the spectrum of diseases that can be effectively treated using NM5072.

SEEKING PARTNERSHIP TO ADVANCE THE DEVELOPMENT OF RUXOPRUBART FOR RARE DISEASES ---

NovelMed is actively seeking licensing, investment, partnership, and acquisition opportunities to propel NM5072 through further development and approval across multiple rare disease indications in various countries. For more information about NM5072, please visit www.NovelMed.com/news.

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