Ruxoprubart (NM8074) Scores FDA Orphan Drug Designation for Paroxysmal Nocturnal Hemoglobinuria (PNH) Treatment!

CLEVELAND, OHIO, UNITED STATES, February 12, 2024 /EINPresswire.com/ -- NovelMed today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to Ruxoprubart, an alternative pathway (AP) blocker anti-Bb antibody, for the treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH) (https://www.accessdata.fda.gov/scripts/opdlisting/oopd/detailedIndex.cfm?cfgridkey=627318). FDA's recognition of Ruxoprubart as an orphan drug for PNH underscores its potential to fulfill a crucial need for individuals grappling with this disease condition.

Ruxoprubart is currently undergoing a Phase II trial in treatment-naïve PNH patients, which aims to evaluate the safety and efficacy of the drug and bringing us one step closer to providing an effective and innovative therapy for PNH patients in USA and around the world.

"We are delighted by the FDA's decision to grant orphan drug designation to Ruxoprubart, underscoring the pressing need for innovative therapeutic solutions for patients with PNH," says Robert Bard, VP Regulatory Affairs. This ODD approval marks a significant advancement in the therapeutic landscape with Ruxoprubart 's unique ability to selectively block the alternative pathway (AP) while preserving the classical pathway required for clearing infections in PNH patients.

ORPHAN DRUG DESIGNATION (ODD) −−−

The ODD from the US FDA is a special designation granted to facilitate the development and assessment of prospective new medications. The FDA's Office of Orphan Products Development bestows orphan status upon drugs in development for the treatment, diagnosis, or prevention of rare diseases or conditions impacting fewer than 200,000 people in the US. This Designation aims to offer various advantages to drug developers in order to bolster the creation of innovative medications. These advantages 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.

RUXOPRUBART (NM8074) −−−

Distinguishing itself from Iptacopan (Fabhalta) by exhibiting no affinity for Factor B, our lead candidate Ruxoprubart selectively binds to protein Bb of the alternative pathway. This humanized anti-Bb monoclonal antibody serves as a potent blocker of the Alternative Pathway.

Having successfully completed the phase I trial in healthy volunteers with expected outcomes, Ruxoprubart has progressed to a phase II trial, focusing on treatment-naïve PNH patients in a multi-dose regimen. Notably, the Anti-Bb molecule marks a pioneering biologic undergoing testing in treatment-naïve PNH patients. FDA has also approved the initiation of Phase II trials for multiple indications, including C3 Glomerulopathy (C3G), Atypical Hemolytic Uremic Syndrome (aHUS), and ANCA vasculitis (AAV) with trials to be conducted in the United States. Overseas regulatory approvals cover trials for PNH for the adult population and aHUS, extending to adult and pediatric population.

PAROXYSMAL NOCTURNAL HEMOGLOBINURIA (PNH) −−−

The dysregulation of the Alternative Pathway (AP) is widely recognized as a pivotal factor influencing the course of PNH disease. Classified as an orphan disease, current PNH treatment options remain limited and imperfect. Current sanctioned treatments either hinder infection clearance or induce hyperlipidemia. PNH is characterized by low PNH-RBC clone size, hemolysis, hemoglobinuria, low hemoglobin levels, elevated LDH, and numerous other symptoms that can lead to chronic issues, including premature death if left untreated. Chronic symptoms and multiple organ damage are also prevalent in PNH patients.

While FDA-approved anti-C3, C5, and Factor B drugs exist for PNH treatment with potential symptom management, they are not without significant side effects. The imperative for additional treatment options for PNH, both effective and well-tolerated, remains recognized, with the potential to improve the disease outcome.

ABOUT NOVELMED −−−

NovelMed is dedicated to pioneering novel biologics for diverse complement-mediated diseases. As the first to conceptualize and validate an anti-Bb antibody for chronic complement-mediated and complement-associated disorders, NovelMed advances its leading product candidate through clinical trials, primarily focusing on hemolytic and renal disorders.

"Rooted in a commitment to creativity and compassion, NovelMed aims to deliver transformative therapies reshaping the treatment landscape for rare disease patients," says Dr. Rekha Bansal, Chief Executive Officer. Positioned as a leader in developing Alternative Pathway (AP)-targeted therapies, NovelMed is dedicated to tackling debilitating diseases in hematology, ophthalmology, nephrology, dermatology, and neurology.

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

PNH, a hemolytic disorder resulting in a low PNH-RBC clone size, increased RBC transfusions, elevated LDH values, and decreased hemoglobin values, remains an orphan disease with limited treatment options.

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

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