New Analysis Finds UPLIZNA® (inebilizumab) Effective Among European Populations with Neuromyelitis Optica Spectrum Disorder (NMOSD)

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-- Data being presented at the European Academy of Neurology Congress demonstrates consistent reductions in NMOSD attacks and disability scores across populations --

DUBLIN--(BUSINESS WIRE)--Jun. 24, 2022-- Horizon Therapeutics plc (Nasdaq: HZNP) today announced new findings from a post hoc analysis of the N-MOmentum Phase 3 pivotal trial of UPLIZNA supporting the medicine's efficacy in Europeans living with NMOSD. These data are being presented during the 8th Congress of the European Academy of Neurology (EAN), June 25-28 in Vienna.

UPLIZNA received marketing authorization from the European Commission (EC) on April 25, 2022 and is the first and only targeted CD19+ B-cell-depleting monotherapy proven to reduce attacks in adult patients with NMOSD who are anti-aquaporin-4 immunoglobulin G seropositive (AQP4-IgG+). This post hoc analysis compared attack rates, disability-related outcomes and safety among 50 trial participants from the European Union (EU) (including participants from Bulgaria, Czech Republic, Estonia, Germany, Hungary and Poland) versus 163 non-EU participants.

"People living with NMOSD in Europe need novel treatment options that have been shown to reduce attacks that can cause irreversible and debilitating damage, such as vision loss and paralysis," said Friedemann Paul, M.D., study author and head, Clinical Neuroimmunology Research Group, NeuroCure Clinical Research Center, and head, Clinical and Experimental Neuroimmunology, Experimental and Clinical Research Center, Charité–Universitätsmedizin Berlin and Max Delbrueck Center for Molecular Medine, Berlin, Germany. "With UPLIZNA, physicians have a treatment option that can be given twice a year after initial dosing to help prevent NMOSD attacks by specifically targeting CD19 B-cells, which play a central role in the pathogenesis of the disease."

Key analysis findings:[1]

- Participants in the EU who were treated with UPLIZNA experienced fewer attacks (12.5%) compared to those treated with placebo (30%), sharing similar results with non-EU participants receiving UPLIZNA (10.7%) or placebo (45.2%).
- No significant differences in Expanded Disability Status Scale (EDSS) worsening were found between participants in the EU (15%) versus non-EU participants (14.9%).
- Fewer NMOSD-related hospitalisations were reported among those receiving UPLIZNA compared to those treated with placebo (mean, EU: 1.0 vs 2.0; non-EU: 1.0 vs 1.33).

"The UPLIZNA pivotal trial is the largest in NMOSD and clearly demonstrates the merits of targeting CD19 B-cells, including plasmablasts and plasma cells, to provide broad, deep and durable B-cell depletion," said Karl Boegl, M.D. Ph.D., executive director, EMEA regional medical affairs lead, Horizon. "We believe these data provide treating physicians with greater certainty that a targeted monotherapy like UPLIZNA can be a valuable option for the treatment of NMOSD patients in Europe."

About Neuromyelitis Optica Spectrum Disorder (NMOSD)

NMOSD is a unifying term for neuromyelitis optica (NMO) and related syndromes. NMOSD is a rare, severe, relapsing, neuroinflammatory autoimmune disease that attacks the optic nerve, spinal cord, brain and brain stem.[2,3] Approximately 80% of all patients with NMOSD test positive for anti-AQP4 antibodies.[4] AQP4-IgG binds primarily to astrocytes in the central nervous system and triggers an escalating immune response that results in lesion formation and astrocyte death.[5]

Anti-AQP4 autoantibodies are produced by plasmablasts and some plasma cells. These B-cell populations are central to NMOSD disease pathogenesis, and a large proportion of these cells express CD19.[6] Depletion of these CD19+ B-cells is thought to remove an important contributor to inflammation, lesion formation and astrocyte damage. Clinically, this damage presents as an NMOSD attack, which can involve the optic nerve, spinal cord and brain.[5,7] Loss of vision, paralysis, loss of sensation, bladder and bowel dysfunction, nerve pain and respiratory failure can all be manifestations of the disease.[8] Each NMOSD attack can lead to further cumulative damage and disability.[9,10] NMOSD occurs more commonly in women and may be more common in individuals of African and Asian descent.[11,12]

About UPLIZNA

For information about UPLIZNA for Europe, please view the Summary of Product Safety Characteristics.

About Horizon

Horizon is a global biotechnology company focused on the discovery, development and commercialization of medicines that address critical needs for people impacted by rare, autoimmune and severe inflammatory diseases. Our pipeline is purposeful: We apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives. For more information on how we go to incredible lengths to impact lives, visit www.horizontherapeutics.com and follow us on Twitter, LinkedIn, Instagram and Facebook.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential benefits of UPLIZNA in treating patients with NMOSD. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include, but are not limited to, risks regarding whether future data analyses or clinical evidence will be consistent with the analyses from the N-MOmentum clinical trial or Horizon's expectations. For a further description of these and other risks facing Horizon, please see the risk factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon undertakes no obligation to update or revise these statements, except as may be required by law.

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