



Health Canada Approves UPLIZNA® (inebilizumab for injection) for the Treatment of Neuromyelitis Optica Spectrum Disorders (NMOSD)

-- First and only anti-CD19 B-cell-depleting monotherapy for the treatment of adult patients with NMOSD who are anti-aquaporin-4 immunoglobulin G seropositive (AQP4-IgG+) --

-- NMOSD is a devastating autoimmune disease of severe and recurrent central nervous system attacks which can result in blindness, paralysis and death --

TORONTO – Jan. 16, 2024 – Horizon Therapeutics plc, now part of Amgen, announced that on December 15, 2023, Health Canada approved UPLIZNA® (inebilizumab for injection) as a monotherapy for the treatment of adult patients with NMOSD who are AQP4-IgG+. This rare autoimmune disease is caused by inflammation in the central nervous system, resulting in severe and recurrent attacks that can lead to permanent disability, such as vision loss and paralysis.¹ An estimated 1,000 people in Canada live with the disease.^{2,3,4}

“Today’s approval of UPLIZNA marks a significant milestone for adults living with NMOSD in Canada, bringing a new, targeted treatment option to those living with this devastating disease,” said Matt McCarthy, general manager, Canada, Horizon. “Just a single NMOSD attack can have a life-altering impact, including pain, debilitation and irreversible vision loss. We are committed to bringing new medicines to people living with rare and challenging diseases around the world, and today’s announcement is an exciting milestone in that effort.”

Health Canada based its approval of UPLIZNA on results from the N-MOMentum pivotal trial ([2014-000253-36](#)), the largest NMOSD clinical trial to date. UPLIZNA demonstrated a significant reduction in the risk of an NMOSD attack with only two infusions per year, following the initial loading doses. Additionally, 87.6% of patients in the AQP4-IgG+ group remained relapse-free during the six-month period post-treatment.⁵ UPLIZNA also demonstrated an acceptable safety profile.

People impacted by NMOSD live with unpredictable attacks; 90% will experience repeat attacks within five years of an initial attack.⁷ Damage is caused when CD19+-expressing B-cell lymphocytes (plasmablasts and some plasma cells) secrete AQP4-IgG, triggering an escalating autoimmune reaction. Depletion of CD19+ B-cells has proven effective at halting inflammation, lesion formation and astrocyte loss. UPLIZNA offers a unique mode of action developed specifically to deplete CD19+ B-cells and prevent attacks.^{5,6,7}

“NMOSD has historically been misdiagnosed for multiple sclerosis (MS), which can delay proper treatment and worsen outcomes, especially if treated with MS drugs,” said Mark Freedman, director, Multiple Sclerosis Research Unit, Professor of Neurology, University of Ottawa, Dept of Medicine, Senior Scientist, the Ottawa Hospital Research Institute. “We can now accurately diagnose NMOSD, which is distinctly different from MS and warrants specific treatment. UPLIZNA is an important new treatment option that provides physicians and patients living with NMOSD a therapy with proven efficacy, a favorable safety profile and a twice-yearly maintenance dosing schedule.”

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“We at The Sumaira Foundation are excited to see new, clinically proven therapies becoming available to NMOSD patients in Canada that give them additional options for treatment and management of this rare but serious condition,” said Sumaira Ahmed, an NMOSD patient, founder and Executive Director of The Sumaira Foundation. “Many patients have benefitted from access to these therapies in the US, Europe and around the world. As a patient living with this rare disease for ten years, I know first-hand how important it is to have multiple treatment options available. As a patient advocacy leader, I have seen how they can transform the lives of fellow patients.”

UPLIZNA was approved by the U.S. Food and Drug Administration (FDA) in June 2020, by the Japanese Ministry of Health, Labor and Welfare in March 2021, by the European Commission (EC) in April 2022 and by the Brazilian Health Regulatory Agency (ANVISA) in December 2022.

For additional information about UPLIZNA, please see the [product monograph](#).

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.^{9,10} Approximately 80% of all patients with NMOSD test positive for anti-AQP4 antibodies.¹¹ 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.¹²

Anti-AQP4 autoantibodies are produced by plasmablasts and plasma cells. These B-cell populations are central to NMOSD disease pathogenesis, and a large proportion of these cells express CD19.¹³ 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.¹²⁻¹⁴ Loss of vision, paralysis, loss of sensation, bladder and bowel dysfunction, nerve pain and respiratory failure can all be manifestations of the disease.¹⁵ Each NMOSD attack can lead to further cumulative damage and disability.^{16,17} NMOSD occurs more commonly in women and may be more common in individuals of African and Asian descent.^{18,19}

About Horizon

Horizon, now part of Amgen, 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.

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