Johnson&Johnson

NEWS RELEASE

Nipocalimab is the first and only investigational therapy granted U.S. FDA Breakthrough Therapy Designation for the treatment of adults living with moderate-to-severe Sjögren's disease

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The Breakthrough Therapy Designation (BTD) for investigational nipocalimab in Sjögren's disease, a prevalent autoantibody disease with no approved advanced therapies, is supported by results from the Phase 2 DAHLIAS study

A greater than 70 percent relative improvement in systemic disease activity at Week 24 was demonstrated in study participants on average who received nipocalimab 15 mg/kg compared to participants who received placebo

Nipocalimab was granted BTD in hemolytic disease of the fetus and newborn earlier this year, making this the second time Johnson & Johnson's nipocalimab has received this designation

SPRING HOUSE, Pa., Nov. 11, 2024 /PRNewswire/ -- Johnson & Johnson (NYSE: JNJ) today announced that the U.S. Food and Drug Administration (FDA) has granted nipocalimab Breakthrough Therapy designation (BTD) for the treatment of adults living with moderate-to-severe Sjögren's disease (SjD), a debilitating and chronic autoantibody disease with high prevalence, for which no approved advanced treatments are available.[1,2,3,4] Nipocalimab is the only investigational therapy to secure this designation in SjD. This regulatory milestone is the second time BTD has been granted for nipocalimab; the first was granted **in February** for the treatment of alloimmunized pregnant individuals at high risk of severe hemolytic disease of the fetus and newborn (HDFN).

"Today's announcement marks an important step forward in the continued research and development of nipocalimab, the first investigational FcRn blocker to demonstrate positive results in a Phase 2 study in adult

patients with moderate-to-severe Sjögren's disease," said Terence Rooney, Vice President, Rheumatology, Immunology Disease Area Leader, Johnson & Johnson Innovative Medicine. "With no treatments currently approved that may directly address the underlying cause(s) of the disease, innovation is critically needed to improve patient outcomes in Sjögren's disease. This milestone underscores our unwavering commitment to develop novel, transformational therapies that may help address significant unmet need for patients living with autoantibody-driven diseases."

The BTD is supported by data from the Phase 2 DAHLIAS study evaluating the efficacy and safety of nipocalimab for the treatment of adult patients with moderate-to-severe SjD and supports further evaluation of the investigational treatment through a Phase 3 study, which is underway. Data from the nipocalimab Phase 2 DAHLIAS study were **featured** in a late-breaking oral presentation (LBA0010) at the European Alliance of Associations for Rheumatology (EULAR) 2024 Congress and demonstrated the first-ever positive results of an investigational FcRn blocker as a potential targeted therapy in SjD.

The FDA grants BTD to expedite the development and regulatory review of an investigational medicine that is intended to treat a serious or life-threatening condition and is based on preliminary clinical evidence that indicates the drug may have substantial improvement in at least one clinically significant endpoint over available therapy.[5] Many patients living with SjD experience symptoms that interfere with daily activities and quality of life.[6] While SjD most frequently affects the glands that produce saliva and tears, more systemic symptoms called extraglandular manifestations are common and may impact multiple organ systems, including joints, lungs, kidneys, and nervous system.[7] Patients with SjD also have a high-risk of developing numerous associated conditions, including up to 20 times higher risk of developing B-cell lymphomas when compared to the general population.[8] Patients with high activity in more than one organ or disease domain have an increased mortality risk of up to five-fold.[8]

ABOUT SJÖGREN'S DISEASE

Sjögren's disease (SjD) is one of the most prevalent autoantibody-driven diseases for which no therapies are currently approved that treat the underlying and systemic nature of the disease.[9] It is a chronic autoimmune disease that is estimated to impact approximately four million people worldwide and is nine times more common in women than men.[3,10] SjD is characterized by autoantibody production, chronic inflammation, and lymphocytic infiltration of exocrine glands. Most patients are affected by mucosal dryness (eyes, mouth, vagina), joint pain and fatigue.[9] More than 50% of SjD patients have a moderate to severe form of the condition, and disease burden can be as high as that of rheumatoid arthritis or systemic lupus erythematosus and is often associated with impaired quality of life and functional capacity, and increased mortality risk.[1,3,7]

ABOUT DAHLIAS

DAHLIAS (NCT04969812) is a Phase 2 multicenter, randomized, placebo-controlled double-blind study to evaluate the effects of nipocalimab in participants with primary Sjögren's disease. DAHLIAS is a Phase 2 dose-ranging study for adults with moderately-to-severely active primary SjD who were seropositive for anti-Ro60 and/or anti-Ro52 IgG antibodies. 163 adults aged 18-75 were randomized 1:1:1 to receive intravenous nipocalimab at 5 or 15 mg/kg or placebo every 2 weeks through Week 22 and received protocol-permitted background standard of care. Safety assessments were conducted through Week 30.[11] The primary endpoint was change in baseline in the ClinESSDAI Score at Week 24. Select secondary endpoints included:

- Multiple organ system assessments:
  - European League Against Rheumatism Sjögren's Syndrome Disease Activity Index (ESSDAI) is a systemic diseases activity index designed to measure disease activity in patients with primary SjD. It is based on 12 domains including: constitutional, lymphadenopathy, glandular, articular, cutaneous, respiratory, renal, muscular, peripheral nervous system, central nervous system, and hematological.
  - Disease Activity Level (DAL) response is a reduction from baseline in disease activity level by at least 1 level in at least 1 clinESSDAI domain (e.g., articular, hematological, cutaneous, constitutional).
- Physician assessment:
  - The Physician Global Assessment of Disease Severity (PhGA) is recorded by the investigator, independent of study participants' assessment, on a scale with responses ranging from 0 ("No SjD activity") to 100 ("Extremely active SjD").
- Composite tools for clinical trial endpoints:
  - Sjögren's Tool for Assessing Response (STAR) is a composite responder index that includes all main SjD features, including systemic disease activity, patient-reported symptoms, tear gland item, salivary gland item and serology, in a single tool.
  - Composite of Relevant Endpoints for Sjogren's Syndrome (CRESS), a composite endpoint tool consisting of five complementary items: systemic disease activity, patient-reported symptoms, tear gland item, salivary gland item and serology, for use in trials of primary SjD.
- Patient reported outcomes:
  - European League Against Rheumatism Sjögren's Syndrome Patient-Reported Index (ESSPRI) is a patient-reported assessment of the severity of dryness, fatigue, and pain associated with primary SjD, in which patients report their symptom severity over the last two weeks on a numeric rating scale (NRS), ranging from 0 "No symptoms (dryness, fatigue or pain)" to 10 "maximal imaginable (dryness, fatigue, pain)".
  - Sjögren's Symptoms tool is a patient-reported assessment of the worst severity of their ocular, oral, and vaginal dryness and joint pain over the past 7 days on a 0 to 10 NRS, from 0 "No (specific symptom)" to 10, "Severe [specific symptom]".

ABOUT NIPOCALIMAB

Nipocalimab is an investigational monoclonal antibody, purposefully designed to bind with high affinity to block FcRn and reduce levels of circulating immunoglobulin G (IgG) antibodies, potentially without impact on other immune functions. This includes autoantibodies and alloantibodies that underlie multiple conditions across three key segments in the autoantibody space including Rare Autoantibody diseases, Maternal Fetal diseases mediated by maternal alloantibodies and Prevalent Rheumatology.[11,12,13,14,15,16,17,18,19] Blockade of IgG binding to FcRn in the placenta is also believed to prevent transplacental transfer of maternal alloantibodies to the fetus.[20, 21]

The U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) have granted several key designations to nipocalimab including:

- U.S FDA Fast Track designation in hemolytic disease of the fetus and newborn (HDFN) and warm autoimmune hemolytic anemia (wAIHA) in July 2019, generalized myasthenia gravis (gMG) in December 2021 and fetal neonatal alloimmune thrombocytopenia (FNAIT) in March 2024
- U.S. FDA Orphan drug status for wAIHA in December 2019, HDFN in June 2020, gMG in February 2021, chronic inflammatory demyelinating polyneuropathy in October 2021 and FNAIT in December 2023
- U.S. FDA Breakthrough Therapy designation for HDFN in February 2024 and for SjD in November 2024
- EU EMA Orphan medicinal product designation for HDFN in October 2019

ABOUT JOHNSON & JOHNSON

At Johnson & Johnson, we believe health is everything. Our strength in healthcare innovation empowers us to build a world where complex diseases are prevented, treated, and cured, where treatments are smarter and less invasive, and solutions are personal. Through our expertise in Innovative Medicine and MedTech, we are uniquely positioned to innovate across the full spectrum of healthcare solutions today to deliver the breakthroughs of tomorrow, and profoundly impact health for humanity.

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Cautions Concerning Forward-Looking Statements

This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding nipocalimab. The reader is cautioned not to rely on these forward-looking statements. These

statements are based on current expectations of future events. If underlying assumptions prove inaccurate or known or unknown risks or uncertainties materialize, actual results could vary materially from the expectations and projections of Janssen Research & Development, LLC, Janssen Biotech, Inc. and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in product research and development, including the uncertainty of clinical success and of obtaining regulatory approvals; uncertainty of commercial success; manufacturing difficulties and delays; competition, including technological advances, new products and patents attained by competitors; challenges to patents; product efficacy or safety concerns resulting in product recalls or regulatory action; changes in behavior and spending patterns of purchasers of health care products and services; changes to applicable laws and regulations, including global health care reforms; and trends toward health care cost containment. A further list and descriptions of these risks, uncertainties and other factors can be found in Johnson & Johnson's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and in Johnson & Johnson's subsequent Quarterly Reports on Form 10-Q and other filings with the Securities and Exchange Commission. Copies of these filings are available online at **www.sec.gov**, **www.jnj.com** or on request from Johnson & Johnson. None of Janssen Research & Development, LLC, Janssen Biotech, Inc. nor Johnson & Johnson undertakes to update any forward-looking statement as a result of new information or future events or developments.

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