Johnson&Johnson

Media contact:
Bridget Kimmel
Mobile: (215) 688-6033
bkimmel@its.jnj.com

Investor contact:
Raychel Kruper
investor-relations@its.jnj.com

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Johnson & Johnson's nipocalimab granted U.S. FDA Fast Track designation to reduce the risk of fetal neonatal alloimmune thrombocytopenia (FNAIT) in alloimmunized pregnant adults

FNAIT is a rare disease that occurs when a pregnant person's immune system attacks fetal platelets, resulting in the risk of internal bleeding, which can be life threatening to the fetus or newborn

The Phase 3 FREESIA program is underway and nipocalimab is the only investigational therapy currently reported to be in clinical development for the treatment of FNAIT

SPRING HOUSE, Pa. (March 26, 2024) – Johnson & Johnson announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation (FTD) for nipocalimab to reduce the risk of FNAIT in alloimmunized[a] pregnant adults during their current pregnancy. FNAIT is a rare and severe condition that occurs when the immune system of a pregnant person mistakenly attacks platelets in a developing fetus. This immune response can lead to impaired clotting ability and bleeding, posing a significant risk to the fetus or newborn.[1] Nipocalimab, an investigational monoclonal antibody targeting FcRn, is the only investigational therapy currently reported to be in clinical development to address the needs of alloimmunized pregnant individuals at risk of FNAIT.

The FDA's Fast Track program is designed to expedite development and review timelines of drugs that demonstrate the potential to treat serious conditions and address unmet medical needs for serious or life-threatening conditions. Fast Track designation supports close communication between the FDA and sponsor with the aim of delivering new therapeutics to patients more quickly.

"Receiving Fast Track designation for nipocalimab in FNAIT underscores the urgency to address the unmet need for safe, effective, and targeted treatments to prevent FNAIT, a condition that could carry severe health consequences and even be fatal for the fetus or newborn," said Katie Abouzahr, M.D., Vice President, Autoantibody and Maternal Fetal Immunology Disease Area Leader, Johnson & Johnson. "We are committed to applying our decades of immunology leadership to pioneer innovative approaches to transform treatment for patients and their families affected by FNAIT and other alloantibody-driven diseases of pregnancy."

Johnson & Johnson is conducting research and development for nipocalimab, an FcRn blocker, to address the significant unmet need in reducing the risk of FNAIT. Nipocalimab is believed to work by blocking the transfer of immunoglobulin G (IgG) alloantibodies from pregnant individuals to their babies through the placenta while not suppressing the broader immune systems of the pregnant individual or developing fetus.[2] Johnson & Johnson is also proceeding with two Phase 3 trials focused on FNAIT. Nipocalimab was granted orphan drug designation by the U.S. FDA for FNAIT in December 2023.

Nipocalimab is additionally being studied in hemolytic disease of the fetus and newborn (HDFN), another alloimmune disease of pregnancy with a similar disease mechanism, often referred to as the red blood cell counterpart to FNAIT.[3] After Phase 2 safety and efficacy results from the UNITY trial, Johnson & Johnson is additionally proceeding with Phase 3 trials focused on HDFN. [4]

Editor's Notes:

a. Alloimmunized: an immune response to foreign antigens upon exposure to genetically different cells or tissues

About Fetal Neonatal Alloimmune Thrombocytopenia (FNAIT)

Fetal neonatal alloimmune thrombocytopenia (FNAIT) is a rare and potentially life-threatening alloimmune condition in which a pregnant person's immune system develops antibodies against fetal or newborn platelet antigens, leading to thrombocytopenia (low platelet counts in the fetus or newborn).[1]

FNAIT can result in severe bleeding complications for a fetus or newborn and is characterized by organ bleeding in the gastrointestinal tract, lungs, or eyes, and may result in lifelong disability or death.[1] If a severe bleed occurs in the

brain, termed intracranial hemorrhage (ICH), death or life-long neurologic effects could occur.[1] ICH occurs in up to 26 percent of untreated pregnancies with FNAIT.[5]

There are no approved targeted therapies for FNAIT management. FNAIT is not routinely screened for during pregnancy and firstborn children with FNAIT are often only diagnosed postnatally.[1]

About HDFN

Hemolytic disease of the fetus and newborn (HDFN) is a rare disease (and in its severe form, ultra rare) that arises in pregnancies with maternal-fetal incompatibility in certain red blood cell types.[6] Alloantibodies produced by the maternal immune system against fetal red blood cells cross the placenta during pregnancy and attack fetal red blood cells causing fetal anemia or persist after birth in the neonate to cause neonatal hyperbilirubinemia and anemia.[Error! Bookmark not defined.] The symptoms of HDFN can range from mild jaundice, to neurotoxic hyperbilirubinemia in the newborn, to life-threatening fetal anemia requiring invasive intervention.[7] The potential for in utero onset at an increasingly earlier GA with increasing risk of severe outcomes may occur with each incompatible pregnancy due to pregnancy-related alloimmunization.[8] Currently there are no non-surgical interventions approved for pregnancies at high risk for severe HDFN.[Error! Bookmark not defined.] Pregnancies affected by severe HDFN may necessitate repeated intrauterine transfusions (IUTs), which are invasive, technically complex surgical procedures performed by specialists at specialized medical centers, and these procedures are associated with an increased rate of fetal mortality and premature birth.[9,10,11] The most difficult to treat cases of HDFN are early onset severe HDFN (EOS-HDFN) that develops at ≤24 weeks gestational age (GA) and results in significant fetal/neonatal morbidity and mortality. According to the *American Journal of Obstetrics and Gynecology*, in the U.S., it is estimated that up to 80 of every 100,000 pregnancies are affected by HDFN each year.[12]

About Nipocalimab

Nipocalimab is an investigational, high-affinity, fully human, aglycosylated, effectorless, monoclonal antibody that aims to selectively block FcRn to reduce levels of circulating immunoglobulin G (IgG) antibodies, including autoantibodies and alloantibodies that underlie multiple conditions.[13] Nipocalimab is the only FcRn blocker being studied across three key segments in the autoantibody space: Rare Autoantibody diseases (e.g., generalized myasthenia gravis in adults and children, chronic inflammatory demyelinating polyneuropathy, warm autoimmune hemolytic anemia, and idiopathic inflammatory myopathies); Maternal Fetal diseases mediated by maternal alloantibodies (e.g., hemolytic disease of the fetus and newborn and fetal and neonatal alloimmune thrombocytopenia); and Prevalent Rheumatology (e.g., rheumatoid arthritis, Sjögren's disease, and systemic lupus erythematosus).[14,15,16,17,18,19,20,21,22] Blockade of FcRn has the potential to reduce overall IgG including pathogenic alloantibody levels while preserving immune function without causing broad immunosuppression. Blockade of IgG binding to FcRn in the placenta is also believed to prevent transplacental transfer of maternal alloantibodies to the fetus.[8,23]

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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Janssen Research & Development, LLC and Janssen Biotech, Inc. are both Johnson & Johnson companies.

Cautions Concerning Forward-Looking Statements

This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding product development and the potential benefits and treatment impact of 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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