Pregnancy Enhanced Tracking with Neonatal and Infant Assessment (PETUNIA): Design of a Safety Study for Nipocalimab in Generalized Myasthenia Gravis

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Key Takeaways

- **PETUNIA is a global enhanced pharmacovigilance pregnancy study designed to obtain real-world safety data on nipocalimab exposure immediately before and during pregnancy**
- **Insights gained from this study will support clinical decision-making for pregnant individuals with gMG**

Background

Generalized myasthenia gravis (gMG) is a rare autoantibody-mediated disease that frequently affects people of childbearing potential[1–3]

In the context of gMG, the neonatal fragment crystallizable receptor (FcRn) maintains high levels of immunoglobulin G (IgG) including pathogenic IgG and extends the half-life of IgG; therefore, FcRn inhibition constitutes a targeted approach for treatment of gMG[4–6]

Nipocalimab is an FcRn blocker approved in the U.S., Europe, and many other countries globally for the treatment of gMG[7,8]

- However, its safety has not been studied in pregnant individuals with gMG
- Because FcRn is expressed on placental synctiotrophoblasts and mediates maternal-fetal IgG transfer, pregnancy surveillance is important to characterize potential effects on immunity in the offspring[9]

While single-product prospective pregnancy registries are often used to evaluate safety in a postmarketing setting, they have limitations including recruitment and retention challenges and failure to deliver timely data-driven evidence, especially in the setting of rare disease[10–12]

An enhanced pharmacovigilance study design that leverages routine reporting of pregnancy exposure and is paired with structured procedures, targeted follow-up questionnaires (TFUQs), and rigorous processes for data entry and quality control can overcome these limitations[12]

Objective

Pregnancy Enhanced Tracking with Neonatal and Infant Assessment (PETUNIA) aims to determine the prevalence of pregnancy, maternal, and infant outcomes after maternal exposure to nipocalimab immediately before or during pregnancy using a novel enhanced pharmacovigilance approach

Methods

Study Design

- PETUNIA[a] is a global, non-interventional, single-arm study using an enhanced pharmacovigilance approach that aims to estimate the prevalence of pregnancy outcomes, maternal complications, and neonatal and infant health outcomes among individuals exposed to nipocalimab immediately before or during pregnancy (**Figure 1**)
- The study is conducted in the postmarketing setting and captures prospective and retrospective reports of nipocalimab exposure immediately before or during pregnancy through the sponsor's global safety database, from the time of first approval (April 29, 2025) through 2036
- Eligible cases must have documented exposure to at least one dose of nipocalimab immediately prior to (≤2 weeks before the last menstrual period) or during pregnancy (**Figure 2**)
- Using structured TFUQs, reports are followed through the end of pregnancy and corresponding live births are assessed during the first year of life (**Table 1**)
  - TFUQs are to be completed by healthcare providers (HCPs) and/or patients who report pregnancy exposure
  - When reports are made by pregnant individuals, their partners, or other non-HCP informants, authorization to contact their HCP is sought

[a]*ClinicalTrials.gov registration in progress; NCT number pending*

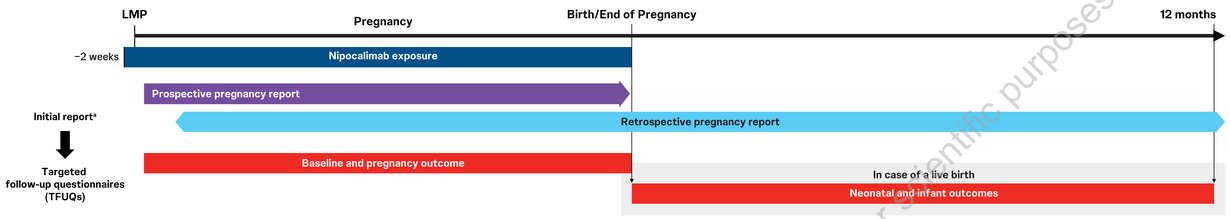
Endpoints

- Primary endpoints include pregnancy outcomes and maternal complications (fetal growth restriction, hypertensive disorders of pregnancy, placental abruption, small for gestational age), serious infections in neonate and infant, and major congenital malformations (**Table 2**)
- Secondary endpoints include live birth, spontaneous abortion, stillbirth, preterm birth, intrapartum hemorrhage, postpartum hemorrhage, fetal death, neonatal death, and infant death (**Table 2**)

Data Analysis

- The projected sample size is ≥162 total pregnancies with 50 prospectively reported cases with known pregnancy outcomes expected over a 10-year period from regions where nipocalimab is approved and prescribed
- All analyses are stratified by prospective and retrospective initial pregnancy reporting, with prospectively reported pregnancies constituting the primary analysis
  - Retrospective reports are analyzed separately in a secondary analysis to minimize potential selection bias
  - Prospective and retrospective case classifications are defined separately for each of the study objectives to reflect the timing at which different outcomes may become clinically apparent (**Figure 3**)
- Proportions of nipocalimab-exposed pregnancies, mothers, or neonates/infants for each endpoint are calculated with 95% confidence intervals using exact methods
- Prevalence estimates are contextualized using the prevalence of these outcomes observed in the overall gMG population and general population of pregnant individuals

Figure 1. *Study design*



*Pregnancy reports will be defined as prospective or retrospective based on the timing relative to the pregnancy and testing performed prior to the initial report and will vary based on study objective.
LMP=*last menstrual period.*

Figure 2. *Key inclusion and exclusion criteria*

Inclusion Criteria

- Documented exposure to ≥1 dose of nipocalimab any time immediately before (≤2 weeks before LMP) or during pregnancy in the postmarketing setting

Exclusion Criteria

- Nipocalimab was discontinued >2 weeks before LMP
- Indirect report (by someone other than the pregnant individual or HCP) for which the pregnant individual or HCP cannot be identified based on the information provided
- Lack of sufficient reporter contact information for follow-up
- Cases occurring in an interventional setting (i.e., clinical trials)
- Paternal-only exposure to nipocalimab during the partner's pregnancy

HCP=*healthcare provider,* ***LMP***=*last menstrual period.*

Table 1. *Key variables to be collected in the TFUQs*

Baseline	Pregnancy Outcome	Neonatal and Infant Outcomes
• Maternal information • Current pregnancy information, including prenatal testing • Exposure to medications and other substances during current pregnancy • Maternal obstetric history • Maternal history of MG • Maternal medical history, including comorbidities • Paternal history	• Course of pregnancy (pregnancy and MG complications) • MG disease activity during pregnancy • Exposure to medications and other substances during current pregnancy • Outcome of pregnancy	• Infant status • Infant medical history, including vaccination history • Neonatal intensive care unit admission • Outcomes including MCM[a] and serious infection • Maternal status • Maternal MG disease course and exposure to medication during lactation • Postnatal growth deficiency • Neurodevelopmental delay

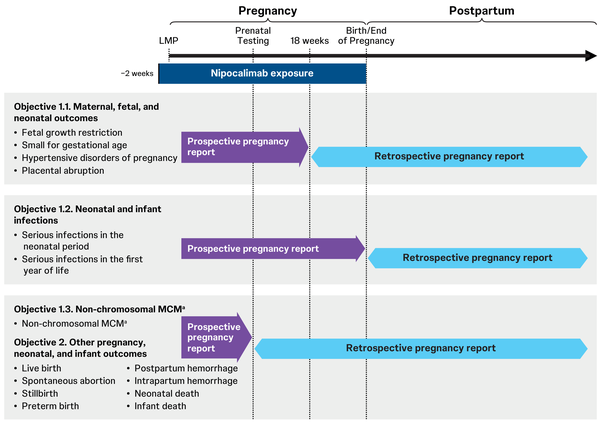
[a]*A congenital abnormality that requires medical or surgical treatment, has a serious adverse effect on health and development, or has significant cosmetic impact.*
MCM=*major congenital malformation,* ***MG***=*myasthenia gravis,* ***TFUQ***=*targeted follow-up questionnaire.*

Table 2. *Primary and secondary endpoints*

Objective	Endpoints
Primary	
1.1. Estimate the prevalence of **maternal complications and fetal and neonatal outcomes** among people exposed to nipocalimab during pregnancy	• Fetal growth restriction • Hypertensive disorders of pregnancy (i.e., pre-eclampsia; gestational hypertension; hemolysis, elevated liver enzymes, and low platelets [HELLP] syndrome; and eclampsia) • Placental abruption • Small for gestational age
1.2. Estimate the prevalence of **neonatal and infant serious infections** among people exposed to nipocalimab during pregnancy	• Serious infections in the neonate (0–27 days) • Serious infections in the first year of life
1.3. Estimate the prevalence of **non-chromosomal major congenital malformations**[a] in pregnancies of people exposed to nipocalimab during pregnancy	• Non-chromosomal major congenital malformations[a]
Secondary	
2. Estimate the prevalence of **other pregnancy outcomes, neonatal outcomes, and infant outcomes** among people exposed to nipocalimab during pregnancy	• Live birth • Spontaneous abortion • Stillbirth • Postpartum hemorrhage • Intrapartum hemorrhage • Preterm birth • Neonatal death • Infant death

[a]*A congenital abnormality that requires medical or surgical treatment, has a serious adverse effect on health and development, or has significant cosmetic impact.*

Figure 3. *Prospective and retrospective case classification definitions for primary study objectives*



*A congenital abnormality that requires medical or surgical treatment, has a serious adverse effect on health and development, or has significant cosmetic impact.
LMP=*last menstrual period,* ***MCM***=*major congenital malformation.*

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