Beyond the Trial:

Impacting FNAIT Awareness

versiti™
Clinical Trial Services

How Versiti helped advance global understanding of FNAIT through innovation in hematology and prenatal diagnostics.

Preventing FNAIT before it begins requires more than scientific rigor—it demands translational agility. When an emerging therapy program from a global biotechnology organization sought to identify and protect rare at-risk pregnancies in real time, Versiti combined its hematologic and immunogenetic expertise to transform a complex global study into actionable insights and validated diagnostics.

Executive Summary

Versiti provided services to support a multi-phase clinical program aimed at preventing Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) through early identification of at-risk pregnancies and prophylactic intervention. After an initial diagnostic vendor could not meet program needs, Versiti assumed and rapidly stabilized the testing workflow, enabling high-volume maternal screening across global sites. Versiti implemented a multi-step testing algorithm, developed and operationalized a research-use-only cell-free fetal DNA (cffDNA) assay during active enrollment, and provided ongoing scientific and logistical problem-solving. Although the interventional phase concluded early, the program advanced understanding of FNAIT and produced an assay now undergoing clinical validation for routine diagnostic use.

FNAIT Background

Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) is a rare condition in which maternal alloantibodies directed against fetal platelet antigens cross the placenta and cause fetal and neonatal thrombocytopenia. The most common antigen involved is HPA-1a, and FNAIT typically occurs when an HPA-1a negative mother (HPA-1b/b genotype) carries an HPA-1a positive fetus. FNAIT occurs in an estimated 1 in 800 to 2,000 births, though incidence varies based on population-level HPA-1a allele frequency[1]. When alloimmunization occurs, clinical outcomes can include intracranial hemorrhage, neurologic impairment, or fetal loss.

Engagement History

A global biotechnology partner sought to develop a prophylactic therapy administered by approximately 16 weeks of gestation to prevent alloimmunization and thereby prevent FNAIT before it occurs. This required a partner capable of identifying rare at-risk pregnancies, supporting complex reflex testing workflows, and managing high-volume maternal screening across multiple global collection sites on a tight timeline.

Versiti was uniquely positioned to support this work. Investigators at the Versiti Blood Research Institute (VBRI) were among the first to define the HPA-1a antigen at the DNA level, and Versiti's clinical laboratories serve as a national reference center for platelet immunology and esoteric hematologic testing.

The natural history study was initiated through a large CRO, but the initially outsourced diagnostic testing vendor was unable to meet program needs. The biotech organization and their CRO partner required immediate transition of the testing workflow. Versiti assumed responsibility in 2021, with first sample receipt in October 2021 marking the start of operational collaboration.

The biotechnology partner described the collaboration as transparent and steady from the outset, noting that Versiti's scientific leadership and operational teams approached the work with clarity, direct communication, and shared ownership of results.

Natural History Screening

The natural history study was designed to identify at-risk women and observe pregnancy outcomes without therapeutic intervention. The study was initially scoped to include up to 30,000 pregnant women across U.S. and European sites, with sample processing centralized at Versiti. A total of 14,346 women were ultimately screened.

Versiti implemented a multi-step screening algorithm consisting of:

- HPA-1 genotyping
- HLA-DRB3*01:01 typing
- Anti-HPA-1a antibody detection
- Non-invasive fetal HPA-1a determination via cell-free fetal DNA (cffDNA)

At the outset of the study, Versiti did not yet have a clinically validated cffDNA assay for fetal HPA-1a typing. The natural history study provided the specimen volume required to rapidly develop and operationalize a research-use-only version of the assay for use within the screening workflow, while refining assay performance parameters in real time. This allowed cffDNA testing to be incorporated during active enrollment. Building on research testing performed throughout the study, the assay is now undergoing formal clinical validation for routine diagnostic use.

Phase II Interventional Study

Based on progress during the natural history phase, the biotechnology partner initiated a Phase II interventional study in Europe. Screening and confirmation needed to occur within a 10–14-week gestational window, requiring rapid site coordination, dual blood draws for cffDNA confirmation, and expedited sample routing to Versiti laboratories for sample processing and testing. Using Versiti Clinical Trial Services' central laboratory infrastructure, testing was completed under these tight turnaround requirements.

Versiti processed 148 cases in this phase before the study was discontinued following administration of the investigational therapy in the first participant, when drug levels were undetectable in maternal plasma, preventing informed dose adjustment and further enrollment.

Scientific and Operational Agility in Clinical Trials

Versiti's contribution extended beyond testing execution. In a related male immunization model, unexpected antibody signals suggested possible alloimmunization. Versiti scientists identified that the observed signal likely reflected circulating therapeutic antibody rather than endogenous antibody formation. Within approximately one month, Versiti developed and validated a novel assay to distinguish drug from alloantibody, enabling accurate interpretation of pharmacodynamic data.

Additionally, Versiti validated a refrigerated cold-chain shipping protocol in under two months, replacing a frozen shipment requirement, maintaining assay integrity while reducing cost and logistical complexity across thousands of shipments. Regular operational governance ensured continuous alignment on turnaround times and testing volumes.

Outcomes and Legacy

Although the interventional trial concluded earlier than anticipated, the collaboration yielded multiple advances:

- Screening of 14,000+ pregnancies, representing one of the largest systematic FNAIT-focused maternal cohorts described to date.
- Development, deployment, and ongoing clinical validation of the fetal HPA-1a cffDNA assay.
- Demonstration of Versiti's capacity to solve emergent scientific questions under real-time trial conditions with agility.

Versiti's biotechnology partner reflected on the impact of the work:

> "Not every study ends the way we hope scientifically, but what we achieved here shifted global understanding of FNAIT. The natural history work meaningfully advanced recognition of this rare condition in clinical and research communities, helping to clarify who is at risk and how to identify them earlier in pregnancy. Even when therapeutic outcomes don't proceed as planned, the awareness and evidence generated can still move the field forward and ultimately benefit patients and families affected by rare diseases."
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> –Vice President and Head of Development Operations

This collaboration highlights the distinct role Versiti plays within the clinical trial ecosystem: a partner equipped to combine esoteric hematologic science, immunogenetic assay development, and operational execution under real-world trial conditions—capabilities not commonly available through standard central laboratories or CROs.

References

1. Epidemiology and management of fetal and neonatal alloimmune thrombocytopenia. de Vos TW, Winkelhorst D, de Haas M, Lopriore E, Oepkes D. Transfus Apher Sci. 2020;59:102704. doi: 10.1016/j.transci.2019.102704.

About Versiti

Versiti provides expert support for a diverse range of clinical trial needs. Our comprehensive approach includes logistics, assay and biomarker development, IRB services, central laboratory services, blood products for research, and more. Our depth of expertise across the spectrum of blood health and clinical trials ensures nimble, high-quality, efficient and reliable results. We are your partner throughout every stage of your clinical or academic research.

To learn more about Versiti's clinical trial services and biomaterials for research, visit versiticlinicaltrials.org, or contact us at clinicaltrials@versiti.org.

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