

# Drug trial for rare fetal blood disease shows promise for less invasive approach

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Data from a new investigational drug that could alter the standard treatment for a rare blood disease suggests it has the potential to delay or prevent anemia and the need for intrauterine blood transfusions in babies who are at high risk for the condition, known as Hemolytic Disease of

the Fetus and Newborn (HDFN). Results of the Phase 2 clinical trial of the drug nipocalimab were published today in [The New England Journal of Medicine](#).

HDFN is a serious condition in which the blood types of the mother and her [fetus](#) do not match, potentially causing life-threatening anemia in the baby. The current standard for treating HDFN requires an average of four ultrasound-guided intrauterine blood transfusions during the pregnancy. Complications with transfusion include [fetal death](#), premature rupture of membranes and preterm birth.

"If further studies support using nipocalimab to treat HDFN, it will make treating the fetus in these pregnancies safer and easier for pregnant moms," said maternal fetal medicine specialist and lead study investigator Kenneth Moise Jr., M.D. Moise is a professor in the Department of Women's Health at Dell Medical School at The University of Texas at Austin and co-director of the Comprehensive Fetal Care Center, a clinical partnership between Dell Children's Medical Center and UT Health Austin, the clinical practice of Dell Med.

Called the UNITY study, the research involved following 13 [pregnant women](#) who had either experienced a fetal loss or needed early intrauterine transfusions during a previous pregnancy due to HDFN. DNA tests indicated their current fetus was at high risk of also having HDFN. Participants received intravenous nipocalimab between 14 and 35 weeks of gestation during pregnancy.

More than half the participants in the study (54%) had a live birth at or after 32 weeks without needing a transfusion. Some did not need a [transfusion](#) even after birth. None of the babies developed a dangerous HDFN condition called fetal hydrops, a condition linked to a lower survival rate for babies wherein large amounts of fluid collect inside the fetus.

For HDFN, nipocalimab works by halting the transfer of antibodies across the placenta, preventing the attack on the fetus's red blood cells and lowering the amount of antibodies in the mother's bloodstream.

"Nipocalimab is the only drug in development with the potential to treat a variety of alloimmune diseases that affect the fetus such as fetal/neonatal alloimmune thrombocytopenia and immune-mediated congenital heart block," Moise said. Nipocalimab also has the potential to treat a wide spectrum of autoantibody diseases such as [rheumatoid arthritis](#) and myasthenia gravis, he said.

In late 2023, Johnson & Johnson, the sponsor of the UNITY Phase 2 study, initiated a Phase 3 pivotal trial of nipocalimab in HDFN called AZALEA. Researchers began enrolling pregnant individuals earlier this year who are at risk for severe HDFN and have experienced the condition in a prior pregnancy to further assess the efficacy and safety of nipocalimab.

The AZALEA trial is a [randomized controlled trial](#) that is being conducted in maternal fetal centers around the world. Moise is the lead investigator of the Phase 3 trial in Central Texas.

**More information:** Nipocalimab in Early-Onset Severe Hemolytic Disease of the Fetus and Newborn, *New England Journal of Medicine* (2024). [www.nejm.org/doi/full/10.1056/NEJMoa2314466](http://www.nejm.org/doi/full/10.1056/NEJMoa2314466)

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