HEALTH & WELLNESS

What it's like when your baby has a rare blood disorder

A condition called fetal and neonatal alloimmune thrombocytopenia (FNAIT) can be deadly in newborns. That's why Johnson & Johnson is passionate about developing a treatment for the disorder.

WRITTEN BY BARBARA BRODY | NOVEMBER 02, 2023

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When Alicia McGrew gave birth to her first child 11 years ago, the doctor declared that her new son was "perfect." When her husband, Patrick, inquired about the tiny red dots covering the baby's skin and then asked why he was bleeding so much after a standard vitamin K shot, the delivery nurse told him not to worry.

The doctor who performed their son's circumcision the next day, however, noted that there had been more bleeding than expected during the procedure. He told the McGrews he would order a complete blood count (CBC), just to confirm that everything was fine.

After the CBC test was administered, Alicia McGrew was waiting for the green light to pack her things when a team from the neonatal intensive care unit (NICU) rushed into her room and said they had to take her son immediately. Confused and panicked, she followed them to the NICU, and like any new mom, began peppering them with questions. "I had no idea our baby was in danger until a nurse snapped at me, and said, 'Look, we're trying to save his life!' " recalls McGrew.

After things calmed down, she learned that her baby's platelet count had dropped precariously low. Platelets are small blood cells that are crucial for clot formation. Without adequate platelets, a prick from a needle can bleed profusely; a mild injury—say, banging your hip into the car door, or, in the case of a newborn, making your way through the birth canal—can result in a life-threatening internal hemorrhage.

A normal platelet count is between 150,000 and 400,000. By the time the McGrew baby's platelet count was checked, he was at 10,000. Three hours later, after he was rushed to the NICU, he was down to 8,000. "They told us it was a miracle he was alive," says McGrew.

After getting two whole blood transfusions and three infusions of intravenous immunoglobulin (IVIg) to increase his platelet count, he was discharged from the hospital days later. McGrew was still fuzzy on what exactly had happened—and hadn't been given any official diagnosis—but she was mostly relieved to be going home with a healthy baby. It wasn't until she became pregnant with her second child that she started Googling. She landed on a website (naitbabies.org) and a private Facebook group for families impacted by a rare condition called fetal and neonatal alloimmune thrombocytopenia (FNAIT).

The McGrews' firstborn received transfusions through his umbilical line.

As she soon learned, FNAIT, often just called NAIT (pronounced "nate"), stems from a mismatch between a pregnant person's platelets and their fetus. "FNAIT is a rare, complex, potentially life-threatening disorder," says Katie Abouzahr, M.D., Vice President, Autoantibody Portfolio and Maternal Fetal Immunology Disease Area Leader, Janssen Pharmaceutical Companies of Johnson & Johnson. Around one in 1,000 births are impacted by the disorder, and most families don't know about it until a baby is born and starts showing symptoms. Treating FNAIT can be tricky. That's why scientists at Johnson & Johnson are working to develop better options.

"In the past few years, several seminal papers in the field have been published, and there are a number of physician-investigators who have dedicated their careers to understanding FNAIT and finding treatments that will lead to healthier babies," says Paige Meizlik, M.D., M.S., Director of Clinical Research, Maternal Fetal Immunology, Janssen. "We've been working closely with key experts to do just that."

Read below for five things to know about FNAIT.

1.

It's caused by a mismatch between a pregnant person's platelets and those of the baby.

Most people know there are different blood types, but it turns out that platelets—small blood cells that are responsible for clotting—also come in different types. Platelets are typed according to the specific substance (antigen) that sits on the surface of each platelet cell; 98% of people have platelets with the HPA-1a antigen.

An embryo inherits a combination of genes from both parents that determine many of the baby's features—including platelet type. If the fetus' platelets contain an antigen from a parent that the pregnant person lacks, conflict ensues: When blood from the baby crosses the placenta and comes in contact with the pregnant person's blood, the pregnant person's body forms immune system proteins called alloantibodies.

"Maternal alloantibodies can then bind to and destroy the fetus' platelets," Dr. Abouzahr explains.

2.

FNAIT can lead to serious disability or death.

In some cases, the mismatch in platelet type causes only mild problems. Petechiae (red or purple pinpoint-sized dots on the skin) and purpura (larger red and purple spots) might be the extent of it. But if a fetus or newborn has been overloaded with maternal alloantibodies and can't flush them out fast enough, the consequences can be severe.

Some babies with FNAIT suffer hemorrhages, often in the gastrointestinal tract or brain.

Any type of internal hemorrhage can be fatal, and surviving doesn't always equal good health. "About 20% to 70% of babies with a brain bleed will have lifelong neurological deficits," says Dr. Meizlik.

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>
> Vice President, Autoantibody Portfolio and Maternal Fetal Immunology Disease Area Leader,
> Janssen Pharmaceutical Companies of Johnson & Johnson

3.

Most parents of babies with FNAIT don’t realize their baby is in danger.

Because FNAIT is rare, most people—including many medical professionals—aren’t aware of it. According to the National Organization of Rare Disorders, FNAIT is not routinely screened for during pregnancy, so it’s believed to be underdiagnosed.

McGrew had a total of four FNAIT pregnancies, and her children are doing well today. But getting the right help was a struggle. After learning about FNAIT, she says she encountered an OB/GYN and even a hematologist who had never heard of it. Fortunately, she found several maternal-fetal specialists who were able to help her.

4.

Current treatments are risky and time-consuming and can be invasive.

Another problem: Most labs aren’t equipped to run the tests needed to diagnose FNAIT. Once FNAIT is on your radar from a previous pregnancy, the parents’ blood platelets are often sent out to a specialized blood center to be genotyped and compared. In McGrew’s case, her husband was deployed earlier in the second pregnancy so the doctors had to perform an invasive amniocentesis in order to test the fetus in utero.

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Assuming a pregnant person knows they are carrying a baby at risk for FNAIT, the current standard treatment involves administering IVIg to protect the developing fetus. IVIg contains antibodies from a collected pool of plasma from donated blood. Although IVIg can be effective in preventing the pregnant person’s immune system from destroying a baby’s platelets when there is a mismatch, it hasn’t been approved by the U.S. Food and Drug Administration for this purpose.

“IVIg has not been studied in a rigorous clinical trial, and it is used off-label without a proven dosing recommendation,” says Dr. Abouzahr. “Additionally, there are significant safety, tolerability and access challenges associated with it.”

McGrew learned about such challenges firsthand while receiving treatment during her second, third and fourth pregnancies. She recalls that receiving IVIg meant being hooked up to an IV for up to 12 hours twice a week for a significant portion of each of those pregnancies.

McGrew also had bad reactions to certain formulations of IVIg. She was willing to deal with headaches and nausea, but one type of IVIg dropped her own red blood cell count so low that she developed severe anemia and had to be rushed to the hospital for transfusions. With her fourth pregnancy, she found a brand of IVIg that worked well without causing complications, but it has since been discontinued. If she were to get pregnant again, her doctor told her, it would no longer be an option.

5.

New treatments are on the horizon.

Today, people who find out they have a FNAIT pregnancy can be given IVIg, but as McGrew’s experience illustrates, it’s not a simple remedy. Exactly how IVIg works for FNAIT isn’t fully understood, though it likely prevents the pregnant person’s body from producing antibodies that attack the fetus’ platelets or reduces the number of these antibodies that cross the placenta. One major catch: It simultaneously suppresses the pregnant person’s own immune system.

“New treatment options are needed that move beyond broad immunosuppression and instead focus on selectively addressing antibodies—including the pregnant person’s alloantibodies—while maintaining the wider immune function of both the pregnant person and their growing fetus,” says Dr. Abouzahr. “Johnson & Johnson is committed to pushing the boundaries of science and building on the rapidly evolving understanding of the immune system to deliver innovative and transformational approaches for immune-mediated rare diseases, including those driven by autoantibodies or alloantibodies, like FNAIT.”

In the meantime, McGrew advises people to heed their instincts and act accordingly. “Trust your gut, ask questions and, if needed, be ready to fight for your baby’s health.”

Alicia and Patrick McGrew with their boys (from left) Aidan, Colin, Declan and Killian.