Help Me Understand—Contemplating a Novel Therapy versus Waiting and Hoping

Beth Wakefield

ABSTRACT

A mother describes her experience during a hospitalization where her child was transferred to the pediatric intensive care unit. The child was not improving and the team wanted to try a novel therapy. This "Family Voice" article describes the mother's experience in a complex situation and with seemingly different opinions from the medical team.

In the summer of 2019, our seven-year-old daughter was unexpectedly hospitalized due to a dramatic increase in bleeding and bruising. She was born with a rare chromosomal difference that caused her to be medically complex, so we were quite familiar with the hospital as well as with working with many different careproviders. This hospitalization, however, was unlike any we had previously experienced.

She had been inpatient for a week on a typical medical unit when we were told she would have to transfer to the pediatric intensive care unit (PICU) at the other campus of the hospital's care system across town. This was a first for us, and we were quite shocked. The transfer was triggered by her new diagnosis of acquired thrombotic thrombocytopenic purpura (TTP). We were informed that this is a critical illness and needed a higher level of care. The hematologist suggested that her prognosis was good, since she was young and was doing so well in the past week. This was encouraging, but my husband and I were still quite frightened.

After a week in the PICU, undergoing daily plasmapheresis (a process in which unhealthy blood plasma is filtered out and replaced with healthy plasma or a substitute), high-dose steroids, and other typical TTP treatments, we were told by the medical team that she was not improving like they would have hoped by that point. Her platelets were next to nothing and not increasing. This was very discouraging because, despite her critical illness, she still seemed pretty close to her sweet and spunky self. They told us we would have to consider an additional, more novel treatment.

The hematologist explained that there was a new medication for TTP, just approved for use over the past year. The caveat was that it had only been approved for use in adults, and there was very little experience documented for pediatric administration. When we asked for more information, the hematologist was able to gather a couple of cases of use in older pediatric patients. Results were mixed in those cases, therefore not increasing our confidence in the medication.

We knew she couldn't maintain with such low platelets, but we kept hoping and praying that the next day would be the day they finally started to

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Beth Wakefield writes about her family's experience with making a complex decision about their daughter's care.

bounce back. But that unfortunately did not happen. We began to feel more pressure to try the new medication.

The stress was really starting to take a toll on all of us; compounded by lack of sleep and watching our child endure daily treatments and procedures with little to no improvement, I was reaching a breaking point. I no longer cared to get myself ready for the day or eat healthy meals. I did what I needed to survive, and in this case, that meant pajamas, Diet Coke, and Cheetos. I just wanted my little girl to get well and that was my primary focus. Everything else took a back seat.

We had heard subtle variations of opinions from the hematologists—depending on who was leading her case on a given day. One hematologist would suggest that improvements can take a while and she was doing okay for someone with TTP, while another would propose we consider starting the new medication soon. Some preferred well-tested therapies, while others were interested in trying more novel treatments. We were overwhelmed by the many discussions and tried to learn more about her illness and treatment options in order to keep up. At one point, I even wondered whether the new medication had been suggested out of medical curiosity or out of medical necessity. While we did understand and appreciate that each of the careproviders had their own perspective and communication style, it complicated our understanding of the severity of her situation. We wondered if we could wait a little longer to try the new medication, or should we have already started it three days earlier. We just didn't know. As parents we were stuck between two difficult choices: to use an experimental therapy or to wait and hope. Both options presented their own stressors and dangers.

It was decided that a care conference would be best, to discuss, as a group, the options in more detail, for the team to answer our remaining questions, and, ultimately, to make a decision on how to move forward. The careproviders attending the care conference included the PICU team, a pain and palliative provider—whom we had just added to our care team for this hospitalization—and the hematologist leading her case that week. Before the meeting, I asked our social worker if there was any way possible for us to get a second hematologist from the team to attend as well. We had already heard several opinions on her case, and felt it would really help us to come to a better understanding to have an additional hematologist involved in this discussion. Despite the difficulty of that request, they were able to have two hematologists attend the care conference. While they could tell us that the members of the team were on the same page, it was particularly helpful for us to see the individual perspectives come together and collectively explain why they felt we should move forward with the new medication. After a long, hard discussion, we agreed to move forward with the new treatment.

We are so grateful that we were able to work through this difficult decision with the help of our daughter's care team. That medication allowed her to finally turn a corner and quite rapidly make improvements. Because the team answered our questions, were patient with our processing, and went above and beyond typical care conference protocol to help us more fully understand the circumstances and medication, we were able to come to an agreement on a care plan that, I believe, may have saved our daughter's life.