



## A Patient's Story

# Clinical trial gives autistic patient his “first win”

When Debora Moritz enrolled her 10-year-old son, Griffin, in a clinical trial at Cincinnati Children's Hospital Medical Center in October 2008, the stakes couldn't have been higher.

Griffin, who is autistic and non-verbal, suffers from tuberous sclerosis complex (TSC), a rare genetic disease that causes noncancerous tumors to grow throughout the body. Griffin suffered from severe seizures and facial and kidney tumors. Recently he had developed subependymal giant cell astrocytomas, or SEGAs in his brain. The SEGAs were causing hydrocephalus or fluid on the brain. Unless the pressure on his brain was alleviated, Griffin would die.

Griffin wasn't considered a good surgical candidate, so his neurologist in Scottsdale, Ariz., sent the boy's MRI films to Cincinnati Children's where researchers were testing a drug called everolimus to treat SEGAs. The researchers promptly responded: Griffin was a perfect candidate for the trial.

For Debora, deciding whether or not to join the trial was excruciating. Other than the medication, the components of the trial were all procedures Griffin had been through before: blood draws, MRIs and EEGs. But Debora worried: If the drug failed, surgery was Griffin's only alternative and they would have wasted precious time.

In the end, she says, “We rolled the dice.”

Traveling cross country with an autistic, seizure-prone child for clinic visits was challenging and Griffin

experienced mouth ulcers and slightly elevated cholesterol as side effects of the medication. But Debora noticed improvements as well. After only a few weeks the bumpy red tumors on his face were less angry and he seemed calmer.

Debora didn't get a chance to see if the medication was shrinking Griffin's SEGAs, however, until February 2009 when they flew to Cincinnati for his 15-week clinic appointment, which included an MRI.

The results were fantastic: Griffin's SEGAs had shrunk by 30 percent. “It was his first win,” Debora says, her voice catching with emotion.

Throughout the remainder of the trial Griffin and other participants continued to show improvement. Results were so impressive that in October 2010, the U.S. Food and Drug Administration granted accelerated approval of everolimus for TSC patients with SEGAs.

Today Griffin's SEGAs are 60 percent smaller than when he entered the trial and his seizures are much less frequent and severe. The lesions on his face have largely cleared, the tumors in his kidneys have stopped growing and the 13-year-old has become more independent.

And Debora? Like many mothers, she's posted her kid's picture on Facebook. The only difference: the pictures are “before” and “after” MRIs of Griffin's brain.

“I tell people, ‘This is my kid's brain’ and ‘This is my kid's brain on drugs,’” she says happily. It's her way of promoting clinical research.



# What is informed consent?

**I**nformed consent is the process of making sure volunteers understand the clinical trial so they can consent or agree to participate. It is the first step in the trial process for every volunteer.

The informed consent form is a detailed document that describes the trial, outlines your rights and responsibilities as a participant and provides key contact information.

The research team uses this form to help explain the study. The form is often very complicated. Be sure to ask any questions you might have. The study staff should go through each item on the form with you and provide you with a copy of the form to keep. You can use the form to talk to your family members, doctor and others about the study.

The informed consent form describes:

**Eligibility requirements.** Inclusion and exclusion criteria are meant to keep participants safe and make sure the most appropriate people are involved in a trial. Depending on factors like your age, gender, medical condition and treatment history, you may or may not be allowed to participate in the trial.

**Your responsibility.** The form spells out exactly what is required of you, including procedures you will receive and how often you will visit the clinic. Many trials require you to complete 'homework', such as recording your temperature or keeping a food diary. It's important to consider all these responsibilities before agreeing to participate.

**Potential discomfort.** The document will tell you about any known discomforts -- such as injections, blood draws or dietary restrictions-- that are part of

the trial and warn you about known and unknown side effects of the study drug.

**What you receive.** The informed consent form will tell you what you will receive in exchange for volunteering, such as free medical treatment or financial compensation for your participation. Not all clinical trials provide compensation.

**Your rights.** As a research participant you have:

- The right to understand the purpose, benefits, risks and side effects associated with the trial.
- The right to ask any questions and discuss any concerns with the research staff at any time during the trial.
- The right to withdraw from the clinical trial at any time.

The informed consent document is NOT a binding contract or a legal obligation for you to participate. Even after you sign the form you can decide to leave a trial at any time. Participation is always voluntary: you should never feel pressured to join or stay in a trial.

The informed consent process is a key way of ensuring researchers and volunteers share a mutual understanding of the purpose and "ground rules" of a trial and an important way to help volunteers understand their rights.