

THE WALL STREET JOURNAL®

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WEDNESDAY, February 29, 2012

Family Helps Fund Research on Rare Disease

By MELANIE GRAYCE WEST

To Andrea and Philip Marella, advances in research for Niemann-Pick Type C disease cannot happen fast enough.

The Marellas, who live in Greenwich, Conn., are the parents of two children with Niemann-Pick Type C. It is a genetic metabolic disorder that is sometimes referred to as a kind of childhood Alzheimer's. Their daughter, Dana, 18, was diagnosed with NPC in 2002. Their 12-year-old son, Andrew, was diagnosed in 2004. Their two other children don't have the disease.

About 10 years ago, the Marellas created the Dana's Angels Research Trust, otherwise known as DART, to raise awareness about NPC and funds for research. They started out with events at schools and grew to an annual benefit concert that attracts some 1,500 people. This year's concert, to be held in Stamford, Conn., is May 5 and will feature a performance by Natalie Cole.

Over time, the Marellas have raised some \$2.3 million for research.

Mr. Marella, an attorney who works in the entertainment industry, says that part of their success is by making the appeal so personal. "It is also a more effective way to get awareness out," he says. "You put your family's face on this effort."

The Marellas can point to their two children and talk about the advances and differences in treatment and medication. Dana, who receives treatments to slow the progression of the disease, is now confined to a wheelchair and unable to talk, but still attends a special school and enjoys music and "loves to hold hands," says Mrs. Marella.

Andrew, who was diagnosed earlier and started immediate treatments, is "our miracle," says Mrs. Marella. He will start playing flag football next month.

"The horror is that your child is born perfect, other than a large spleen or an extended spleen," said Mrs. Marella. "She went from a young child who could run, play and chat to a young lady who is in a wheelchair."



Philip and Andrea Marella

Money raised goes toward a collaborative research effort called Support of Accelerated Research (SOAR). The group is comprised of a number of NPC organizations, foundations and families. The goal is to create a drug, or mix of drugs, that can serve as a treatment and move quickly through a clinical trial.

The Marellas say that much of the research on NPC also benefits research on cholesterol and lipid metabolism, HIV and diseases like Tay-Sachs. The advances to date are also because of the number of dedicated families who have banded together to fund combined research and trials, says Mr. Marella.

"Researchers have told me that because of the focus and parents, there isn't another genetic disease which has had as fast and as successful progress."