



Belle

Motto: Saving Belle & Abby

Age: 7

Champion Teammate: Ryan Whalen

HS Team: Hutto - Hyde Park Cheer

When Belle was three, she began showing some developmental delays in her gross and fine motor skills. Whole Exome Sequencing revealed that Belle had Niemann-Pick Disease Type C1, a genetic disease that is so rare that there are only 500 diagnosed cases of NPC1 in the world and only 100 in the US. Shortly after receiving Belle's results, their geneticist Dr. James Gibson suggested that the Andrews have their two-year-old daughter, Abby, tested. They were devastated again when Dr. Gibson confirmed that Abby also had NPC1. NPC1 is a progressive, neurodegenerative disease (childhood Alzheimers) where the lysosomes of all cells of the body accumulate toxic amounts of lipids, causing progressive damage to the brain, spleen, liver, and other tissues. If left untreated, Belle and Abby will

likely eventually suffer from seizures, dementia, ataxia, and cataplexy. As the disease progresses, children diagnosed with NPC1 require feeding tubes because of difficulty swallowing, are confined to wheelchairs, are unable to speak or recognize loved ones, and die in adolescence. Currently, a clinical trial being conducted by Vtesse and the National Institute of Health (NIH), to evaluate a drug called VTS-270 for its effectiveness in treating NPC1. To date, there is no FDA-approved drug to slow the progression of NPC1. Belle and Abby are part of this trial. In the meantime, the Andrews family continues to hope this clinical trial results in the FDA approval of VTS-270, so their daughters and other afflicted children can begin to get some relief from this insidious disease.



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