Stories of Hope: The CIRM Stem Cell Four

At our 2016 CIRM December Board meeting, we invited four people to speak about their experience participating in CIRM-funded stem cell clinical trials. We’re calling them the CIRM Stem Cell Four, and their inspirational stories remind us that the ultimate goal of funding stem cell research is to find treatments that can cure patients with unmet medical needs. We hope that the voices of these individuals will motivate others who are suffering from diseases that have no cures.

**Brenden Whittaker**

At the age of one, Brenden was diagnosed with X-linked chronic granulomatous disease (X-CGD), a rare, genetic disease that causes the immune system to malfunction leaving it unable to fight off certain bacterial and fungal infections which, over time, can be life-threatening. Hospitalized hundreds of times over the years Brenden almost died on a couple of occasions and had part of his lung and liver removed due to repeated infection.

On December 13, 2015, Brenden was cured thanks to an experimental procedure, developed by Dr. Don Kohn, that removed some of his own blood stem cells; they were then genetically modified to remove the gene that causes the problem, and then re-infused to him. The modified blood stem cells created a new blood system and a healthy immune system capable of fighting off infections.

Now that Brenden is cured of his disease, he is getting ready to go back to college full-time. “I want to get into pre-med, go to med school and become a doctor. All the experience I’ve had has just made me more interested in being a doctor. I just want to be in a position where I can help people going through similar things to what I experienced.”

**Evangelina Padilla-Vaccaro**

Evangelina was diagnosed shortly after birth with severe combined immunodeficiency (SCID); it’s also known as “bubble baby” disease because in the past, children were kept in a sterile plastic bubble to protect them from getting sick. It’s a rare – and often deadly -- genetic disorder that meant she had no functioning immune system, leaving her vulnerable to infections. Many children with this condition die within the first year of life.

Evangelina was cured as part of a clinical trial at UCLA run by Dr. Don Kohn. She underwent a stem cell transplant that took her own blood stem cells, genetically re-engineered them, and returned them to her body. These re-engineered stem cells created a new blood and immune system.

Angelina can now play outside and attend parties with her twin sister Annabella. Her parents Alysia and Christian are overjoyed that both their children are healthy. “I get to look at my kids the way other parents should be looking at their children all the time, but until you are put in that position you never appreciate it, says Alysia. “We take things for granted. We all do. I know I do, particularly when the twins drive me crazy, but I also know how very precious this is.”

Alysia could not hold back her emotions at the December Board meeting and said through tears, “Thank you for keeping my family complete.”

Read more about Evangelina’s story on the Stem Cellar blog.
People like Karl, Jake, Evangelina and Brenden and their families are the reason why stem cell research moves forward. Their brave efforts to advance stem cell treatments through clinical trials will help our agency attain our mission and hopefully one day, fund a treatment that develops into a cure.
Special thanks to David Jensen for coining the term "California Stem Cell Four" and writing about these patients on his blog California Stem Cell Report.

Source URL: https://www.cirm.ca.gov/our-progress/stories-hope-cirm-stem-cell-four