



Curing PI. Worldwide

Mr. Chairman and members of the Subcommittee, thank you for this opportunity. I am Vicki Modell, Co-Founder of the Jeffrey Modell Foundation. My testimony will request \$2.0 million for the HRSA Genetic Services Branch to save the lives of the 1 in 30,000 babies born each year with SCID. I will explain.

In 1987, my husband Fred and I established the Jeffrey Modell Foundation in memory of our son Jeffrey, who died at the age of 15 from complications of Primary Immunodeficiency.

Since its earliest days, our Foundation has worked in close collaboration with the NIH on Research and with CDC on a Public Awareness and Physician Education Program. In recent years, we have increased our efforts and resources to implement population based newborn screening for Severe Combined Immune Deficiency or SCID, working with CDC, HRSA, the states, and private industry.

This condition, often referred to as “Boy in The Bubble Disease”, is fatal in the first year of life if not diagnosed and treated early. Babies born with SCID appear completely

normal at birth, causing delayed diagnosis, until the babies are repeatedly hospitalized with life threatening infections, a nightmare for any family. But there is now a screening technology, called TRECS, which is more than 99% accurate and is inexpensive. A bone marrow transplant has a better than 95% success rate to cure this fatal disease, if identified in the first 3 ½ months of life.

The Secretary's Advisory Committee recently voted 26-0 to recommend that all infants in the U.S. be screened for this condition. We contacted every state to implement the Secretary's recommendation. But the states had significant budget problems. At the same time, we heard from too many grieving parents whose infants died because their state does not screen for this fatal disease. Their stories are tragic.

Fred and I knew we couldn't wait another day. With limited Foundation resources, one year ago, we offered "start-up" funds to all of the states for the assays, lab equipment, and educational materials for clinicians and parents. Almost immediately many states, including Georgia, responded that they would begin population screening for SCID in their states, if we would commit start-up funding.

The economic benefits are overwhelmingly persuasive according to peer reviewed scientific journals:

1. The cost of the test is \$4 per baby.

2. The cost of a transplant in the first few months of life is \$100,000.
3. If the baby is not screened and treated, the baby will develop overwhelming infections and hospitalizations in a pediatric intensive care unit, and the cost of care in the first year of life will be \$2-4 million, if the baby survives.
4. Three federal agencies: EPA, FDA, and Transportation estimate the value of a life saved to be \$7.7 million.
5. A newborn baby with SCID, that is screened and treated in the first 3 ½ months of life, generates more than \$64 dollars in contributions to society for every \$1 dollar we invest. 64 to 1! That's the economics!

And so, here is where we are... 2 years ago, there were 2 states screening for SCID. Today, 20 states are screening or piloting, and 23 states, including the state of Georgia, are prepared to begin as soon as we can help them with start-up funds. Together, those states represent 93% of the 4 million babies born annually in our country. We can finish the job and eradicate this disease NOW. In this rich and extraordinary country, why should it matter what state a baby is born in to live or die?

Our Foundation is not strong enough to finish the funding completely on our own and we cannot do this without you! This is a small investment with an outcome that is priceless!

I know, because I have held these babies and I have laughed with these babies...*and I have shared tears with others.*

Fred and I accept the reality that science and discovery did not come in time to save Jeffrey. But, we are dedicated and committed to working with you to save all of the Jeffreys in the future. Let's go forward together on this journey beginning now.

This can be an historic moment, and together, we will look back to this day, when we decided to eradicate this fatal disease that takes these beautiful babies from us and shatters their parents' hopes and dreams.

Mr. Chairman, thank you for what you do every day in service to our nation, and what we can do together to save more precious lives.