cscf | Children's Sickle Cell Foundation, Inc.

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The White House
President Barack Obama
1600 Pennsylvania Avenue NW
Washington, DC 20500

March 31, 2014

Dear Mr. President:

When my son, Jonathan, was born with sickle cell disease (SCD) in 2000, they told me that a cure could come in his lifetime, maybe within the next 20 years. Though there is promising research, we are still hoping for a cure. During his first 6 years of life he had numerous hospitalizations for pneumonia, acute chest syndrome and unpredictable, excruciatingly painful, vasoocclusive crises that are managed with strong medicines like Morphine. *Currently, there is only one FDA approved drug therapy to prevent these unpredictable episodes of pain, Hydroxyurea (HU).* It is because of this drug that since starting HU at 6 years of age, Jonathan has only had 1 acute chest syndrome episode, 1 emergency room visit due to sickle cell and will celebrate his 14th birthday on May 3, 2014. ***He is Living Well with Sickle Cell® while we wait for a universal cure for sickle cell disease.***

Though inexpensive and readily available, only 20% of those persons who are eligible to take HU, have access to it. HU Therapy includes a treatment regimen that may present barriers for some with monthly clinic visits for blood draws (e.g. transportation). In Pennsylvania, we are working through the PA Sickle Cell Providers Network to increase awareness and access to HU. We have the support of our state legislators who responded in 2013 by expanding the current line item, adding a new line for Health Education and Awareness for Hydroxyurea. We are confident that keeping children and adults with SCD healthy in PA will result in lower utilization of emergency room visits, a decrease in hospitalizations and length of hospitalizations when they are necessary. This means a healthier SCD population that is stronger, employed and contributing in PA. ***With more than 70% of our families dependent on Medicaid as their primary health insurer, there is a good financial reason for getting this drug to everyone who is eligible and more importantly, it is what is needed to help them to start on their journey to Living Well with Sickle Cell®.*** Unfortunately, this is one among many of the challenges that affect the lives of children and adults living with SCD.

There are approximately ***100K persons with sickle cell disease in the United States of America*** and millions worldwide who are suffering, waiting for a CURE for this chronic, often debilitating and fatal disease. There are children with SCD who are suffering with pain every day, pushing through pain to attend school or play. The average lifespan of a person with SCD is 45 for women and 47 for men, though I have personally witnessed the loss 5 children under the age of 25 and the

physical and emotional devastation that this disease causes in our families. ***There is much to be done before we have a CURE.***

I am writing to you because you are the only one that can help. The last time a President made history for persons with sickle cell disease was back in 1972, when President Nixon signed the Sickle Cell Anemia Control Act of 1972. (Public Law 92-294) This was a huge step towards the CURE for SCD.

> *"The National Sickle Cell Anemia Control Act, which I am today signing, follows the course that we have charted. These actions make clear, I believe, the urgency with which this country is working to alleviate and arrest the suffering from this disease. "* President Nixon

The Comprehensive Sickle Cell Centers were in place until 2008 when the National Heart, Lung and Blood Institute of the National Institutes of Health made a decision to defund the Centers and since then, have proceeded to insufficiently fund sickle cell research through what they have called a realignment of sickle cell disease research that does not reach the levels of funding set forth in the Sickle Cell Anemia Control Act of 1972.

A second bill;S.874/H.R.1736 (aka Talent Bill) passed and became the Sickle Cell Treatment Demonstration Act of 2004. It was reauthorized in 2009 and is administered through the Health Resources and Services Administration. A companion law to the SCACT of 1972, it focuses on the care and treatment. With current appropriations at only $10,000,000 per year, spread over only 7 to 9 funded sites in the country; the sickle cell community is suffering from a type of poverty that has continued for decades, yet the number of persons with SCD is increasing. I have enclosed supporting documentation with this letter.

As a mother, I wonder why sickle cell disease has remained underfunded for so long and why there is so little support when curing it holds such promise for curing other genetic diseases as well. I hope that you will partner with us as we ask the tough questions in order to get to the real solutions that will benefit all persons living with SCD so that they can live and contribute greatly to the good of everyone in this great nation.

What we are asking you to do is to look into sickle cell disease funding according to the law, both historically and currently. Consider the following steps that will fulfill the aforementioned laws and provide appropriate resources for the CARE of persons with SCD and for research for the CURE:

1. Increased appropriations beyond the current levels for the Health Services and Resources Administration to expand to the new regional model for the Sickle Cell Treatment Demonstration Act to provide **care and education** for persons living with sickle cell disease.

2. Appropriations retroactive to 1972 directed to the National Institutes of Health to restore the Comprehensive Sickle Cell Centers and research coordinating center to find a **cure** for sickle cell disease.

3. Support for the Centers for Disease Control to work on ***expanded surveillance and an appropriate registry*** for all persons with sickle cell disease so that we know how many persons have sickle cell disease and where they live in order to provide access to care and research.

We call this Care2Cure™ and are doing our part to raise care and research funds from the community. ***We need your help to put federal support for sickle cell disease care and research back on track.*** If sickle cell disease remains underfunded according to the law and this poverty persists, children and adults with SCD will continue to suffer and eventually succumb to the complications of SCD resulting in early death. ***My greatest fear is that a cure won't come in time for Jonathan, and it will be my turn to feel the pain of losing my son to sickle cell disease.*** Please help save my son from this fate and save my heart, this pain.

Respectfully,

Andrea M. Williams, BA
Founder and Executive Director

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