

**STATEMENT ON AMYLOIDOSIS**

**BY**

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**SUBMITTED TO**

**THE SUBCOMMITTEE ON LABOR, HEALTH AND HUMAN SERVICES,**

**EDUCATION AND RELATED AGENCIES**

**COMMITTEE ON APPROPRIATIONS**

**U.S. HOUSE OF REPRESENTATIVES**

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Mr. Chairman,

I appreciate the opportunity to submit testimony to the Committee on the life-threatening disease amyloidosis. I ask that you continue to include language in the Committee's report for fiscal year 2013 drawing attention to amyloidosis.

In previous testimony before this Committee, I described my efforts to combat the life-threatening disease primary amyloidosis. I have obtained treatment for amyloidosis and am in remission. Now I want to devote time to use my experience to help others.

Amyloidosis, which is often fatal, leads to heart, kidney, liver and other organ failure. Amyloidosis can literally kill people before they know what hit them. Left untreated, there is an average survival from the time of diagnosis of about 15 months.

Thousands of people die because they were diagnosed too late to obtain effective treatment. Thousands of others die never knowing they had amyloidosis. The small number of those with amyloidosis who are able to obtain treatment face the challenges of high dose chemotherapy and stem cell replacement or organ transplantation.

Less than one percent of the U. S. population has been identified to have this disease. Many feel that the disease is vastly underdiagnosed, especially in the African-American community.

### **AMYLOIDOSIS**

Amyloidosis occurs when cells malfunction and produce proteins that deposit in organs, such as the heart, kidney and liver. These misfolded proteins clog the organs until they no longer are able to function—sometimes at a very rapid pace.

In addition to primary amyloidosis, there are also cases of inherited or familial amyloidosis and secondary or reactive amyloidosis. All three types of amyloidosis, left undiagnosed or untreated, are fatal.

There is no explanation for how or why amyloidosis develops and there is no known cure.

### **AMYLOIDOSIS TREATMENT**

Boston University School of Medicine and other centers for amyloidosis treatment have found that high dose intravenous chemotherapy followed by stem cell replacement, or rescue, is an effective treatment in many patients. Abnormal bone marrow cells are killed through high dose chemotherapy and the patient's own extracted blood stem cells are replaced in order to improve the recovery process.

I am part of a clinical trial and have gone through this procedure twice.

The high dose chemotherapy and stem cell rescue and other new drugs have increased the remission rate and long term survival dramatically. However, many patients with these diseases still die from them.

### **RESEARCH, DIAGNOSIS AND TREATMENT**

Researchers are poised to begin to develop targeted treatments that will specifically attack the amyloid proteins. Additional funding for research and equipment is needed to accomplish this task. Only through more research is there hope of further increasing the survival rate and finding treatments to help more patients.

Timely diagnosis is also of great concern. Although I was diagnosed at a very early stage of the disease, many people are diagnosed after the point that they are physically able to undertake treatment.

Early treatment is the key to success. More needs to be done in this area to alert health professionals to identify this disease.

### **CURRENT INITIATIVES**

Through the leadership of this Committee and the further involvement of the U. S. Government, a number of positive developments have occurred.

- The National Institutes of Health has substantially increased its interest in amyloidosis. The NIH participates in meetings and symposiums and works closely with organizations doing research and outreach on amyloidosis.
- There has been increased basic and clinical research at the Boston University Amyloid Treatment and Research Program: a model for the disease is under development; factors that cause protein misfolding are being identified; and new clinical trials are underway.

- Increased Federal funding for research, equipment and treatment has been another important element. This is essential to speed the pace of discovery for basic research.

### **REQUEST FOR FISCAL YEAR 2013**

Mr. Chairman, I ask that the Committee take the following actions to help address this deadly disease.

- First, include language in your report identifying amyloidosis as an important concern and encouraging more research to find a cure.
- Second, continue to encourage the Centers for Disease Control and the National Institutes of Health to educate the American public and medical profession on the need to diagnose this disease at an early stage.

The United States Congress and the Executive branch working together are essential to finding a cure for and alerting people to this terrible disease.

I ask for your support in helping me turn what has been my own life-threatening experience into hope for others.

Thank you for your consideration.