

Gateway to a Cure Newsletter

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Amyloidosis and Agent Orange By Staff Writer

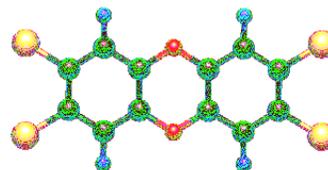
On May 7, 2009 cardiac AL Amyloidosis was recognized by the Secretary of Veterans Affairs as having a "positive association between exposure to herbicide agents" this allowed cardiac AL amyloidosis to be established as a presumptive disease associated with 2,3,7,8-tetrachlorodibenzo-p-dioxin (TCDD) exposure. Below is a brief introduction to Agent Orange exposure and information on how Veterans may enroll to receive the benefits that they have earned.

* * * * *

Introduction

Between the years of 1962 and 1971 the United States Government dredged various parts of Vietnam, Cambodia, Laos, and South Korea with several herbicides intended to defoliate

the countries' vegetation. By killing the plant life, the United States military was able to minimize guerrilla acts and deprave opposing forces of food. However, not enough testing was done on the potential negative effects of these herbicides and the scope of their deadly impact is still being unveiled today.



Chemical structure of 2,3,7,8 Dioxin (TCDD)

The most notorious of these herbicides is nicknamed, "Agent Orange" and was also the most widely used. The specific combination of the chemicals in Agent Orange produced an unforeseen by-product called 2,3,7,8-tetrachlorodibenzo-p-dioxin (TCDD). Dioxin TCDD is a pollutant and exposure to high levels of it has been linked to serious health conditions in

many animal studies. As concerns about the harmful effects of Agent Orange began to arise, the United States Congress passed Public Law (PL) 102-4, The Agent Orange Act of 1991 in an effort to garner information on the health effects of

the herbicides sprayed during the Vietnam War.

The Law required that a comprehensive evaluation

of scientific and medical research regarding Dioxin TCDD and Agent Orange exposure be completed every two years, with the findings updated and published every two years. It was not until 2006 that the committee charged with reviewing the scientific and medical publications on Agent Orange exposure, recognized an *continue to page 4*



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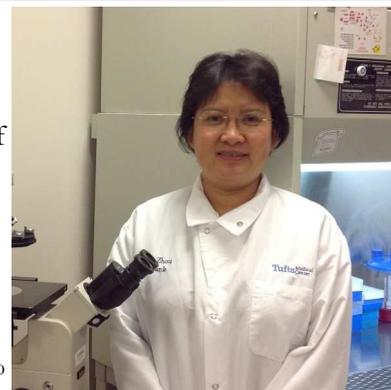
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Amyloidosis Foundation Updates

2013 Senior Research Grant Recipient

Ping Zhou, MD, PhD -Tufts Medical Center

Off the shelf Lambda Light Chain Knockdown Therapy - Despite the advances of stem-cell transplant and proteasome inhibitor therapy, systemic AL amyloidosis (AL) remains a lethal disease for patients who die of AL within 3 to 4 years of diagnosis, representing about half of newly diagnosed patients. We need to develop targeted therapies that directly and rapidly eliminate production of toxic amyloid-forming light chains in order to improve patient outcomes and survival. We will extend our work developing an off-the-shelf lambda light chain specific gene silencing agent that interferes directly, specifically and promptly with production of the protein that causes 80% of cases of AL - the lambda light chain protein. We have shown in plasma cells from patients with AL lambda type, that our agent markedly and rapidly reduces lambda light chain production. This grant will allow us to extend our pre-clinical study of this agent in an additional 20 patient specimens and in a mouse model.



Dr. Ping Zhou in her lab at Tufts Medical Center.

2013 Junior Research Grant Recipient

Jennifer Ward, PhD - Boston Medical Center



Dr. Jennifer Ward in her lab at Boston Medical Center

Tetracycline Analogs for Treatment of Amyloidosis - Primary systemic AL (amyloid light chain) amyloidosis is caused by the light chain (LC) of an antibody that misfolds, aggregates and forms amyloid fibrils that deposit throughout the body. Targeting the plasma cells which produce antibodies with chemotherapy has been successful at stopping progression of disease but there still remains no approved therapy against the amyloid fibrils that remain in tissues. We have built upon studies with other amyloidogenic proteins to demonstrate that the tetracycline antibiotic doxycycline can disrupt light chain AL amyloidosis fibrils isolated from human tissue. We propose to treat transgenic mice with doxycycline to determine if doxycycline can break up the amyloid deposits and we will determine the time and dose needed. We will test if doxycycline treated fibrils are toxic to cardiac and renal cells. We believe that successful treatment of AL amyloidosis will be achieved using a combination of therapies targeting both the plasma cell and the fibrils themselves.

Calling All Committee Members!

This is an exciting year for the Amyloidosis Foundation and we want you to be a part of it! We are looking for passionate individuals with innovative ideas to serve on our "Involvement Committees". The best part is that all correspondence will be done on-line or via phone conferences so members can be located any where in the world! We look forward to hearing from you!

For more information email:
callen@amyloidosis.org

Teleconferences

Teleconferences are a convenient and efficient way for physicians to educate and update a large patient community. The next teleconference will be **March 7, 2013 with Dr. Vaishali from 1:00pm to 3:00pm EST**. Recordings of past teleconferences are available on the Foundation's website. We highly encourage teleconference attendance.

For more information email:
info@amyloidosis.org

Upcoming Medical Conferences

American College of Cardiology (ACC)

Dates: March 9th—March 11th, 2013

Location: San Francisco, California

This will be the ACC's 62nd annual Scientific Session and Exposition and is one of the most highly attended and respected events in cardiovascular medicine.

For more information email:
info@amyloidosis.org

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President's Corner



For some 2012 has been a trying year: the loss of a loved one, a new diagnosis, or the reemergence of amyloidosis. Whatever your experience has been with the disease, I hope the Amyloidosis Foundation was able to provide you with the resources and support you needed.

2013 holds lots of promising developments for the amyloid community. Drugs for amyloidosis are in various stages of clinical trials, the Foundation awarded two new research grants, and our awareness and educational programs are rapidly expanding. Please know that none of these programs would be in existence without your generous help.

On behalf of all us here at the Amyloidosis Foundation I'd like to wish you and your family a happy new year!

Mary E. O'Donnell
President

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Make Your Resolution!

Make your New Years resolution with the Amyloidosis Foundation! Now is the perfect time to incorporate the Foundation into your yearly planning and as a part of your life! Whether it be volunteering at a Foundation event, donating a silent auction item, joining a committee, participating in our involvement program or even "liking" our Facebook page and providing newsletter feedback - We value our community and want to stay in touch with you!

Support Group Spotlight

Tennessee Support Group

Date& Time: February 20, 2013 from 3:00pm to 5:30pm

Location: Vanderbilt Clinic of The Vanderbilt University Medical Center

Room: 2703

The Tennessee support group meets monthly in The Vanderbilt Clinic on both Wednesdays and Saturdays

Coordinator: Charlotte Haffner

For more information email:
info@amyloidosis.org

Support Group Spotlight

Northern California Support Group

Date& Time: February 2, 2013 from 10am to 2pm

Location: Kaiser, Walnut Creek 1425 South Main Street

Room: Oak 1& Oak 2

Speaker: The Binding Site has updated information on FLCs and may be able to join us.

Coordinator: Dena Heath

For more information email:
info@amyloidosis.org

Providing our patients and their families with the support they need is very important to the Foundation. The expansion of our support group program is one of the ways that we are working to meet this need. Please contact the Foundation to learn more about our support groups or express interest in coordinating the formation of a new one.

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Agent Orange

association with AL amyloidosis and the pollutant.

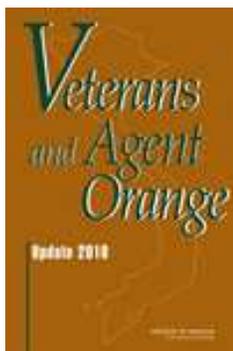
Association

The Department of Veteran Affairs considers AL amyloidosis to be a presumptive condition associated with Agent Orange exposure. When a disease is considered to be presumptive that means that it was expected to develop under normal conditions.

As it applies to the VA: there are certain diseases that are accepted as to have resulted from a veteran's service - should they meet the time and location criteria. .

The *Veterans and Agent Orange: Update 2010* lists AL amyloidosis as a non-neoplastic health condition and places it in the same chapter as multiple myeloma due to the commonalities between the two diseases. The Update 2010 states that both conditions, "share several biological features, most

notably clonal hyperproliferation of B-cell-derived plasma cells and production of abnormal amounts of immunoglobulins" (*Institute of Medicine*, p.409). It is through these commonalities - and the difficult to explain statistic that 10%



Veterans and Agent Orange: Update 2010 is the ninth in a series of congressionally mandated studies that review and evaluate scientific evidence regarding the health effects of herbicide exposure in Vietnam.

to 15% of multiple myeloma patients also have AL amyloidosis; though there is currently little research to pinpoint exactly what the specific

correlation is - that has allowed veterans who suffer from AL

amyloidosis to receive benefits and compensation. This presumptive consideration allows for a Veteran not to have to prove an explicit connection between their AL amyloidosis and military service. Due to the presumptive service connection, the VA assumes that a relationship between AL amyloidosis and Agent Orange exposure exists based on other

qualifying criteria such as dates of service and location.

Compensation & Benefits

Although AL amyloidosis is presumptive, a Veteran must still submit a claim to receive consideration for disability compensation. Eligible veterans may receive the following benefits:

1. **Agent Orange Registry Health Exam** - is free and voluntary to Veterans who have been exposed to Agent Orange.
2. **Health Care Benefits** - encompassing a full range of services (must first enroll in VA's healthcare system)
3. **Disability Compensation** - monthly payments for service-connected or service-related conditions.
4. **Dependents' Benefits** - children of Vietnam Veteran's who have suffered birth defects may be eligible for healthcare, monetary

monetary compensation or vocational training.

3. Survivors' Benefits

- spouses, children and dependent parents may be eligible to receive benefits following the death of a Veteran.

It is important to note that each claim is assessed individually and there are many factors that are considered when providing Veterans with the benefits and services that they have earned.

How to Submit a Claim

The VA has now streamlined the process of submitting claims by allowing for their processing to be done online. This new approach has allowed for the VA to dramatically decrease its turn-around time and award compensation in a more efficient manner. The following page lists a detailed account of helpful resources and contact information for veterans interested in filing a claim to receive the benefits and services earned in selfless service to the United States. **AF**

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Agent Orange done.



Department of
Veterans Affairs

Veterans with Questions about Agent Orange Exposure should utilize the following contacts:

- VA's Special Issues Helpline: **1-800-749-8387**
- VA benefit's information: **1-800-827-1000**
- To locate your nearest VA medical center visit www.va.gov/directory

Miles That Matter



Eli, Kim, Kristy and Tammy finish in style at Key West, Florida after their 1417 mile bike ride which began in Suffolk, Virginia

An incredible labor of love is the only thing to call Eli, Kim, Kristy, and Tammy's 1417 mile bike ride from Suffolk, Virginia to Key West, Florida. They began on September 27, 2012 and finished on October 15, 2012. They rode to raise awareness of amyloidosis and to support Tammy's mother, Judy who is in need of a kidney transplant due to complications from the disease. The Foundation is so honored to have such wonderful ambassadors—we are very proud of you. To read about their journey please visit their blog at <http://milesmatter.blogspot.com/>



Open Clinical Trials

The list of clinical trials that are currently accepting patients is not limited to the ones listed, in fact there are over twenty open clinical trials for amyloidosis around the world. The Foundation does not support one clinical trial over another but does adamantly advocate for patient participation in clinical trials. A sustained and enhanced clinical research effort is what will be behind any medical progress in amyloidosis. More information on other clinical trials and their locations can be provided by contacting the Foundation or visiting www.clinicaltrials.gov AF

Drug or Name of Study: Bendamustine and Dexamethasone in Patients With Relapsed AL Amyloidosis; Phase II

Sponsor: Columbia University

Collaborator: Cephalon

Principal Investigator: Suzanne Lentzsch, MD

Location: Columbia University (more locations will open soon)

Drug or Name of Study: Study of Oral MLN9708 in Adult Patients With Relapsed or Refractory Light Chain Amyloidosis

Sponsor: Millennium Pharmaceuticals, Inc.

Location: Various locations within the United States and Internationally

Coming Soon

Drug or Name of Study: Carfilzomib

Principal Investigator: Adam Cohen, MD

Location: Fox Chase Cancer Center, Philadelphia, PA (more locations to come)

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Reflections on the 6th Annual New York Benefit By Charlotte Haffner

Charlotte Haffner, a long-time friend of the foundation shares her experience as a guest at the 6th annual New York City Benefit for the Amyloidosis Foundation. Supporting research and actively participating in the Amyloidosis Foundation's events is important to Haffner not only because she believes in the foundation's mission but also is an amyloidosis patient. In the summer of 2008, she was diagnosed with primary AL amyloidosis. Her story is a unique one full of promise: Charlotte was the first patient to receive both a heart transplant and a stem cell transplant at Vanderbilt University Medical Center. Due to her trials and triumphs with the disease, Ms. Haffner has become a titan in the Amyloidosis community. She volunteers on behalf of the Amyloidosis Foundation at medical conferences, coordinates the amyloidosis support group at Vanderbilt University Medical Center and has made numerous press appearances to generate awareness of amyloidosis. Here are her thoughts on the 6th annual New York City Benefit:

September 26th 2012 my dear friend Beverly Murrey and myself boarded a plane for New York City. We were going to help support the Amyloidosis Foundation at the 6th annual New York City Benefit for the Amyloidosis Foundation. Beverly had never been to New York before so I really wanted this trip to be a lot of fun for her.

Shortly after we arrived I called Uria Espanoza, who is not only the chairwoman of the NYC Benefit but also the Secretary of the Amyloidosis Foundation's board of directors, to let her know that we had landed safely. Uria suggested that we meet downtown for dinner - she was just as nice as I thought she would be! Over dinner, she asked me to say a few words at the benefit about my experience with amyloidosis and my vision of the future for amyloidosis patients. I was really honored by her request.

The following morning, we woke and started the big day with a run through Central Park. It was beautiful outside and that set the tone for the

entire day. I loved every moment of it and was really looking forward to attending the benefit at the New York City Athletic Club.

Later that evening when we arrived at the venue (a beautiful building brimming with old world charm) we were greeted by Uria and mingled with the other guests. We met some new friends and spent time with old ones. I was really impressed by the silent auction! It had everything from a diamond necklace to gift certificates to some of New York's top restaurants.

When it came time for me to give my speech I told everyone about my battle with Amyloidosis and how it took so long to get the correct diagnosis. I expressed how thankful I was to have gotten to Vanderbilt and in the hands of Dr. Joe Fredi. Dr. Fredi, a cardiologist, recognized right away that my heart failure was caused by Amyloidosis. I spoke about my treatment journey and how fortunate I was to have met Dr. Doug Sawyer, chief of the Division of Cardiovascular Medicine at Vanderbilt. Dr. Sawyer had just come to



Photo Courtesy of Vanderbilt University Medical Center newsletter (2008)

Vanderbilt from Boston where he had been involved in a 10 year study of amyloidosis. Things have really worked out well for me, but more often it does not. My goal now is to become an advocate for education and research for this terrible disease; of which there is no known cause and no known cure. We must all work together, patients and doctors. Amyloidosis is a terrible and personal disease that effects everyone in a different way. Education is the key to early diagnosis, improved treatments and a higher quality of life for amyloidosis patients. I would like to offer many thanks to the wonderful doctors I have, and to the wonderful ones I have met. You make a difference. **AF**

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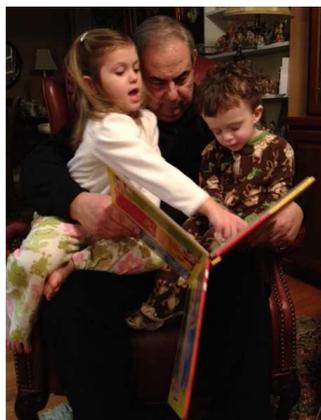
1st Annual “Joe LaGuardia” Amyloidosis Foundation 5K Run/Walk *By Lori Harrington*



Joe LaGuardia, will always be remembered as a passionately driven pioneer and dedicated community member who elevated the stature of the New York State Fair to unprecedented heights; known now as “The House that Joe Built”.

Please join us for our **1st Annual “Joe LaGuardia” Amyloidosis Foundation 5K Run/Walk** to be held in Syracuse, New York at the Syracuse “**Inner Harbor**” on **May 14, 2013**. (the first anniversary of Joe’s passing from this dreaded disease). The purpose of this fundraising event is to raise awareness of this rare disease and to raise money for research so that a cure can be found. Before Joe was diagnosed with Amyloidosis in November of 2011, he had retired as Executive Director of Lakefront

Development Corporation. One of Joe’s responsibilities with Lakefront was to oversee the activities that took place at the “Inner Harbor”. Joe loved his position at Lakefront and was passionate about how this area would help in the growth and development of Syracuse and the community in which he lived and grew up. Joe was a respected leader in the New York State Fair and Entertainment Industry. Joe has earned honors and recognition by The Entertainment Buyers Association, The Academy of Country Music and The International Association of Fairs and



Joe reads with his grandchildren, Giada and Anthony

Expositions where he received the highest honor in the industry when he became a Certified Fair Executive in

1994. Joe was very active and generously devoted his time to many community organizations such as, The Syracuse Sports Hall of Fame, The Syracuse Sports Corporation, The Central NY Arthritis Foundation, Elmcrest Childrens Center, The Syracuse Convention and Business Bureau, The Syracuse Chiefs and many others. The center of Joe’s universe was his family. Joe was the greatest husband, father, papa and friend. Again, this event will take place on **May 14th, 2013** - the first anniversary of Joe’s death -and will have a donation fee of \$35. There will be a live band, activities, food and of course, fun. But most of all, you will be able to help raise awareness of AMYLOIDOSIS and the research efforts into this deadly disease. In the Syracuse area, there are a number of people fighting this disease as well as those who have fought bravely to survive and did not. So we ask all to walk, run, and support our efforts in the research of AMYLOIDOSIS. Information regarding the upcoming Run/Walk can be found www.josephlaguardia.com and also the Amyloid-



Joe’s wife, Mrs. Carol LaGuardia and his daughter, Mrs. Lori LaGuardia Harrington attend the International Entertainment Buyers Association (IEBA) Hall of Fame induction ceremony for Joe LaGuardia.

osis Foundation website. Please sign up there and educate yourself on this disease. We look forward to meeting new faces. **AF**

Date: May 14, 2013
Registration: 4:00pm
Race Start: 5:30pm
Location: Inner Harbor- Syracuse, New York
Website: www.josephlaguardia.com
Contact: joelaguardia-benefit@gmail.com

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The Amyloidosis Foundation's 2013 Metro-Detroit Spring Benefit

The Amyloidosis Foundation will be hosting a benefit for the amyloidosis community in the metro-Detroit area on **May 2nd, 2013**. Several physicians and prominent members of the amyloid community will be in attendance to support the first-ever Foundation benefit in the Detroit region. The benefit will include dinner, music, door prizes and silent auction. The theme will focus on the healing power of art. Many of the silent auction items will be pieces of artwork created by patient artists. If you would like to donate a piece of artwork or other silent auction item, participate on the benefit's planning committee, or attend the event please contact the Foundation. As details of the benefit become finalized invitations will be circulated. We hope to see you there! **AF**



Dylan Duncan (1979-)

Sanibel Island . 2012

Oil on canvas

We Need Your Support!

“Individual commitment to a group effort — that is what makes a team work, a company work, a society work, a civilization work”,
Vince Lombardi.

It is the individual commitment of our donors and supporters to the Amyloidosis Foundation that allows us to provide our many services: ranging from patient support to re-search grants to education of the medical community.

It is because of your generosity that we are able to continue to work towards finding a cure.



What Did You Think?

The Amyloidosis Foundation welcomes your feedback! We are always looking for letters, articles or any suggestions that you may have to make this publication better!

If you have something that you would like to share with the Amyloidosis Foundation community, please contact us at info@amyloidosis.org

We look forward to hearing from you!

The *Gateway to a Cure* newsletter is a publication of the Amyloidosis Foundation and is distributed as a service of the Amyloidosis Foundation with the understanding that the Foundation is not engaged in rendering medical advice or other professional medical services. The Amyloidosis Foundation is a 501(c)(3) tax exempt non-profit organization.

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