

Gateway to a Cure Newsletter

Volume 5, Issue 1 • Spring 2013

Inside This Issue

<i>Art, Autos & Amyloid: The Healing Power of Art</i> By Emilia Allen	1
Amyloidosis Foundation Updates	2
President's Corner	3
<i>A Suffering Heart</i> By Paula Scanland	5
Open Clinical Trials	5
<i>My Ten Years With Amyloid Mouth Misery</i> By Patricia Pinchin	6
First Annual Joe LaGuardia 5k Run/Walk	8

We now have our very own QR code! Just scan the square below with your smart phone to be directed to our webpage!



Visit our website:
www.amyloidosis.org

You're Invited!

Please Join Us For the 2013 Amyloidosis Foundation Metro Detroit Benefit:
Art, Autos & Amyloid
Theme: The Healing Power of Art

On May 16, 2013, the Amyloidosis Foundation will be hosting an evening fundraising event, the Metro-Detroit Benefit, to further support ground-breaking medical research, education of patients and the medical community as well as patient advocacy programs. Held in the beautiful atrium of one of Detroit's premiere luxury hotels, The Inn at St. John's, the benefit will include a gourmet strolling dinner, decadent desserts, open bar, live entertainment, a silent auction, and a brief informative presentation of our philanthropic activities.

This year's theme is the "Healing Power of Art" and will feature various forms of artwork by amyloidosis patients and others whose lives have been touched by this horrible disease. Their artwork serves as a means of exploration into the deeply personal battle with this rare and devastating disease. Pieces of their artwork, done in all mediums ranging from oil to watercolor to glass mosaic, will be available for bidding in our silent auction, alongside the work of professional artists. In addition to the artwork, the silent auction will boast prizes that reflect the Motor City. Most importantly, the benefit provides an opportunity for those whose lives have been touched by amyloidosis to come together as a community, and offer their support of a future where a cure is possible.

Event co-chair, Therese Antonelli lost her father, Thomas Antonelli to amyloidosis on February 29, 2013; he was only 66 years old. This event is a way for Therese to honor her father and bring attention to this

*Continued on page 4
Continued on page 4*

Amyloidosis Foundation Updates

Rare Disease Day 2013: Research Holds Promise

Rare Disease Day is internationally recognized as the last day in February. Which is most fitting, as like the diseases it brings awareness to, the date is uncommon. This year, the Amyloidosis Foundation was able to celebrate Rare Disease Day through community and advocacy events. Emilia Allen, of our development team attended the 2013 Rare Disease Day conference held at the National Institute of Health (NIH) located in Bethesda, Maryland. At the two day conference, spanning February 28th through March 1st the foundation was provided with the unique opportunity to hear updates from and engage with the government leaders of such organizations as the NIH, Food & Drug Administration (FDA), Social Security Administration (SSA), National Center for Advancing Translational Sciences (NCATS), and Defense Advanced Research Projects Agency



Rare Disease Day

is the result of collaborative efforts many federal departments. If successful, the "organ on a chip" program will tremendously aid in the research and development of new medications because it will alter the current way that we test drug efficacy. Instead of experimenting on animals, which along with ethical concerns often times does not prove effective in humans, or on cultured cells which do not reflect the complexities of a human body, the "organ on a chip" offers more. For example, with just enough cardiac tissue to mimic the function of a human heart, researchers will be able to judge a drug's ability to rid the cardiac muscle of disease. Government officials hope that one day, this innovative program will not just be limited to a single organ but will be expanded to include whole body systems so that a drug's impact can be measured holistically. This has many exciting implications for amyloidosis and supports our belief that research paves the way to improved treatments and a cure. **AF**

Support Group Spotlight

Tennessee Support Group

Date & Time: April 20, 2013 from 11am to 2pm

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: April 20, 2013 from 10am to 2pm

Location: Stanford University Medical Center, The Cancer Center, 875 Blake Wilbur Drive, Stanford, CA

Room: Conference Rooms, second floor

Speaker: Ron Witteles, MD

Coordinator: Dena Heath

For more information email:
info@amyloidosis.org

Get Involved!

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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President's Corner



"No problem can stand the assault of sustained thinking."

— *Voltaire (Francois-Marie Arouet), French author, wit and philosopher (1694-1778)*

This is an unofficial motto of the foundation - it speaks to one of our core initiatives, to find a cure for amyloidosis of all types.

This quote speaks to the very reason the foundation created the research grant program, of which many of you have so generously supported in the past. The foundation is one of the most active grant providers, other than the National Institute of Health, for all systemic amyloidosis. Since the grant program began in 2004, we have awarded more than half a million dollars to various research centers around the world. We look forward to continuing to award these grants and truly appreciate the generosity you bestow on the grant program. My thanks to all.

Mary E. O'Donnell
President

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'Hello Amy' Music Release

Hello, Amy

I didn't expect you here this soon

You're not due for quite a while at least that's what I assumed



The 'Hello Amy' project began as a song from musician, artist, and amyloid patient Dylan Duncan's debut album *What to Keep & Let Go* and has evolved into a collaborative musical movement to benefit amyloidosis research and awareness. The album features the work of twelve other artists, including Dylan's original piece.

When asked about the song's message and the personal significance it holds, Dylan explains: "It holds a stern message against the very unwelcomed guest of amyloidosis, a disease that has plagued my family for many generations and that still takes its hold on many people around the world, including myself and sister".

100% of the proceeds benefit the Amyloidosis Foundation's research program. The 'Hello Amy' cd may be purchased for \$10 by visiting www.amyloidosis.com/support or by calling the foundation at 1-877-AMYLOID. AF

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Art, Autos & Amyloid continued.

often misdiagnosed and debilitating disease. The funds raised from this event, combined with other donations, will help the Amyloidosis Foundation continue to work towards our mission to increase education and awareness of amyloidosis within the community leading to earlier diagnosis and improved treatment.

The generous support of our many advocates and friends has helped the foundation maximize our research grant funding. To date, the Amyloidosis Foundation has funded fourteen Junior Research grants at \$492,000, three Senior research grants at \$187,000 and forty-nine travel grants at \$95,860 for the International Amyloid Symposium. Your gifts and attendance at the Metro Detroit Benefit are greatly appreciated by the Amyloidosis Foundation but even more so by the patients and family members who benefit from the foundation's services.

At right you will find many ways to be involved with the Metro Detroit Benefit and we ask, on behalf of the amyloidosis community that you consider attending or making a gift. Please contact the foundation at any time to learn more. We look forward to hearing from you!

AF

Get Involved With the Metro Detroit Benefit!



EVENT DETAILS

When: Thursday, May 16, 2013

Cocktail Reception: 6:30pm

Dinner & Program: 7:15pm

Where: The Inn at St. John's, Plymouth Michigan

Benefit Attire: Casual Cocktail

Cost: \$100.00 per person

Please visit amyloidosis.org/events to register!

SPONSORSHIP OPPORTUNITIES

There are many costs associated with coordinating this event and we are looking for sponsors to help underwrite these expenses thus enabling us to maximize our research grant funding. In exchange, sponsors receive many benefits depending on the amount of their gift.

Diamond Sponsor (\$5000+): Name recognition on event educational literature. Includes all benefits listed below with name recognition accentuated.

Platinum Sponsor (\$1000):

- Name recognition on all event material to include: All pre and post event announcements, publicity, and marketing.
- Name recognition in newsletter
- Name recognition on event website with active link to website
- Prominent banner at entrance and exit of event
- Full page advertisement in the event program
- Name recognition on table literature
- Continual name mention during event
- Opportunity to distribute marketing material or to provide product sampling
- Speaking presence or exhibit opportunity at the event

Gold Sponsor (\$500):

- Prominent name recognition on the key sponsors signage at the entrance of event
- Name recognition on event website
- Half page advertisement in the event program
- Name recognition on table literature
- Name mentioned at beginning and end of event
- Opportunity to distribute marketing literature or to provide product sampling

Silver Sponsor (\$250):

- Prominent name recognition on the key sponsors signage at the entrance of event
- Half page advertisement in the event program
- Name mentioned at the beginning and end of event

Student/Patient Sponsorship (\$100):

- Name mentioned in the event program
- Name mentioned at the beginning and end of event

Other Sponsorship Opportunities:

Valet Sponsor (1) - \$2,000.00; Bar Sponsor (1) - \$1,500; Information Table Sponsor (1) - \$500; Food Station Sponsor (4) - \$500; Educational Sponsor (3) - \$500; Quarter page event program advertisements (15) - \$50

SILENT AUCTION DONATION

Can you help us fill out our silent auction? We are looking for gifts of artwork, jewelry, wine, "experiences" or trip packages, collectables, and gift baskets. These donations are tax-deductible as well. Please email Emilia at callen@amyloidosis.org for more information regarding silent auction donations. The deadline for silent auction submissions is May 2, 2013.

STUDENT/PATIENT SPONSOR

The Amyloidosis Foundation is an international organization providing services, and supporting research across the world. Because of this, many of our loyal donors are unable to attend events due to location and scheduling conflicts. The Foundation has created a unique sponsorship program for our donors to connect with other members of the amyloidosis community by allowing them to gift a patient, family member of a patient, or a student the opportunity to attend the benefit in their place.

TAX DEDUCTIBLE

The Amyloidosis Foundation is a 501 c3 organization and most donations are eligible for tax-deduction. If you would like to attend the event, sponsor a patient, family member, or student to attend in your place, donate a piece of artwork or have any questions regarding the event please contact Emilia at callen@amyloidosis.org.

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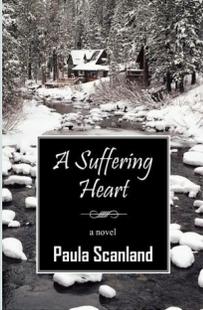
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A Suffering Heart: Author Shares Healing Power of Writing

By Paula Scanland



My husband, Robert Zimmerman, died of cardiac amyloidosis on Christmas Eve 2005. The previous two years had been frustrating for him since none of the doctors we visited believed that something was wrong. Every test they did indicated he was just out of shape. The actual diagnosis came less than two months before he passed away and right after his third cardiac catheterization. By that time, the disease had progressed so far that he only had time for one round of chemotherapy.

Exactly thirteen months after his passing I sat at my computer and began to write. My goal was to honor his life by trying to raise awareness of amyloidosis and to bring attention to the fact that I believe it is often misdiagnosed. The book was a way for me to come to grips with his loss. Although it is a work of fiction, it was inspired by certain aspects my life as well as the strong network of family and friends who continue to support me.

Telling our story in a more entertaining than factual way was a healing tonic for me. While Bob was alive he always supported whatever endeavor I attempted and I felt his spirit with me throughout the entire writing and publishing process. I hope that my inclusion in the novel of information about this terrible illness gives someone else the idea to investigate it further. If it can save one life, I will be satisfied. AF



For more information on Paula and her novel, *A Suffering Heart*, please visit: www.paulascanland.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

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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My Ten Years With Amyloid Mouth Misery *By Patricia Pinchin*

I had my dream job as the Modern Languages' Consultant for Dorset Education Authority when amyloidosis made me unable to continue. I live in Poole, England with Patrick, my wonderful husband who cares for me. I have a married son and daughter and two young grandsons.

It all began in March 2003 when, aged 55, I noticed a lumpy feeling around my upper right back teeth. My dentist checked for abnormalities but found nothing. Referral to a Maxillo-facial Consultant also led no-where. I told him that, in addition to the lumpiness, I had a powerful salty taste in the saliva flowing from my upper palate. "Stress," he said, my jaw being rather stiff, and advised physiotherapy. My physiotherapist did observe abnormal hard lumps in my right cheek, which I now know was a marker. After an evening cinema visit in December, my husband exclaimed, "Whatever have you done to your eyes?" In the mirror, I was astonished to see two enor-

mous racoon-like deep purple bruises encircling my eyes. More "eye episodes" ensued at intervals. Why? In December 2004, my General Practitioner referred me to a Consultant Haematologist, "just in case," and said I should take a photo showing my bruised eyes to the appointment.

In 2005, armed with my racoon eyed photo, I saw Dr. Bell, a Haematologist at my local hospital in Poole. From my GP's referral letter and the photo, he concluded "Your symptoms are a classic presentation of amyloidosis, a very rare, complex, and serious disease. You

"Your symptoms are a classic presentation of amyloidosis, a very rare, complex, and serious disease."

need to have a blood test but in the meantime don't search the internet because you will scare yourself". Ignoring his "don't," I Googled amyloidosis. Aghast at the severity and incurability of the disease, I did not understand much of the terminology, and put it temporarily at the back of my mind. At my next haematology consultation, my blood test result showed

that my free light chains were indeed elevated, which clinched a diagnosis of amyloidosis.

Dr. Jack, now my permanent Haematologist at Poole, re-explained everything again to Patrick and me.. He said that I would be referred to the

National Amyloidosis Centre (NAC) in London, where I would have a special SAP scan—which could detect where masses of amyloid in the major organs had deposited. I understood little, was dazed, and devastated. "One positive," said Dr. Jack "was that I was very lucky. Until about 5 years previously no treatment had existed for amyloidosis whereas now there was more hope."

At the NAC, to our great relief, my scan proved that there was no amyloid involvement in my organs. One of the NAC's doctors diagnosed MGUS (monoclonal gammopathy of uncertain significance). In 2007 a lump on my upper arm was

investigated by ultrasound and diagnosed as unusual lipoma. By 2008, the salty taste had



Author, Patricia Pinchin with grandson Wilf, age 3

become so overbearing that I had to retire. The arm lump had also increased in size. During a CT scan, a conscientious radiologist read my file and noted my amyloidosis diagnosis. A biopsy confirmed the lump was an amyloid as did biopsies of other lumps in my breasts. I had further multiple lumps all over my upper body but none were troublesome. However, the ones in my sub-mandibular glands "could be associated with the salty taste." I began treatment to lower the excess of amyloid producing free light chains in 2008, with a three month course of dexamethasone. In 2009, I proceeded to four cycles

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Amyloid Mouth Misery cont.

of CDT but stopped the thalidomide after a rash during the first cycle. Both resulted in no clonal response. On my next visit to the NAC, Dr. Wechaleker noting that my abnormal protein was IgM, the same as for lymphoma, prescribed the lymphoma chemotherapy R-CVP (Rituximab, Cyclophosphamide, Vincristine, Prednisolone). His diagnosis was nodular cutaneous amyloidosis (amyloid in the skin/soft tissue). Back at Poole, Dr. Jack explained that he would have to make a special application to the NHS trust for me to have R-CVP. After an unsuccessful first request, eventually I had eight cycles with a good clonal response. Three years later, my free light chains are just above normal. Chemotherapy worsened the already powerful sore salty taste in my mouth. Multiple amyloid lumps lined my cheeks and lips and I often had painful ulcers on my tongue. I attempted to find other Maxillo-facial Consultants who could help me with more advanced treatment. Amyloid was risky to remove surgically because of its vascular nature and the nodules

are diffuse. I “was lucky not to have macroglossia (amyloid swelling in the tongue).” Further research proved that “amyloid in the soft tissue rarely changes over time.” I was in despair; as I now knew it unlikely that I would get better.

Amyloidosis had robbed me of life as I knew it. Activity and conversation worsened my symptoms so normal relationships with friends and family, social activities, days/evenings out, travel, cycling, gardening, holidays, everything I loved, became things of the past. My hard to endure symptoms are not understood, so no-one was able to empathise. My Doctors are sympathetic, but with my light chains stable and my disease non-life threatening, I am of little concern.

The impact of omnipresent, obscure throbbing mouth discomforts, which are thought to be nerve damage caused by amyloid, has been most distressing. Management by drugs for neuropathic disorders provides minimal relief. They leave me fazed and often give me

blurred vision though the sedative effects enable me to sleep. My teeth decay repeatedly because of amyloid blocking the normal flow of protective saliva. Chatting with family and friends is of necessity reduced, with a heart breaking emotional effect. I have to prevent myself from crying other-

wise purple bruises encircle my eyes. I cannot be the wife and mother of my former self or the

fun, active, and avid story telling grandmother to my grandsons that I had always hoped to be.

I remain optimistic that the anti-amyloid drug undergoing NAC clinical trials may become a reality and work for me. In the meantime, my occupations have become uncharacteristically sedentary and solitary. I try to stay cheerful and positive for the sake of my cherished family and live my life as best I can. Supporting the wonderful work of the NAC by helping with publicity for an inspirational fundraiser has been therapeutic as is “meeting” other sufferers in the virtual world. Awareness of the

disease is gathering momentum and I hope to be further involved in this. I urge all affected by amyloidosis to donate to funds for research. In the UK and globally, research alone holds the key to discovering the cure. **AF**

“I remain optimistic that the anti-amyloid drug undergoing NAC clinical trials may become a reality and work for me”

The Amyloidosis Foundation greatly appreciates contributions to our newsletter, such as this one by Patricia Pinchin. By sharing and learning from the experiences and trials of those patients and family members whose lives have been impacted by amyloidosis, we can come together as a community and offer mutual support.

We welcome all kinds of newsletter contributions and are always looking for different ways to connect with each other.

Please email Emilia at ecallen@amyloidosis.org to express your interest!

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First Annual Joe LaGuardia Amyloidosis Foundation 5k Run/Walk

Pictured on the right is a billboard in Syracuse, New York advertising the **First Annual Joe LaGuardia 5k Walk/Run**. The race will be held at the Inner Harbor in Syracuse, NY on Tuesday, May 14, 2013; the one year anniversary of Joe LaGuardia's passing. Joe LaGuardia was a devoted community leader and loving family man as well as a talented executive who elevated the stature of the New York State Fair to unprecedented heights; known now as "The House that Joe Built".

Race start time is 5:30pm and will be followed by live music, family activities, and refreshments. Joe LaGuardia Online early bird registration is \$25 now through April 22nd. Day of race registration is available as well for \$35.00. Please visit,

www.josephlaguardia.com for more details.

AF



Excerpt from Carol LaGuardia's blog:

"Joe's last request of me when we were in Boston before he passed was to please try to get everyone in our community or wherever they may live to get involved in raising money for this cause...**Research and Awareness into this dreadful disease.** He wanted nothing for himself or his family... it is for others. (That's how Joe lived, always caring and doing things for others, never expecting anything in return)". AF

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 research 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. Thank you!



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 callen@amyloidosis.org

We look forward to hearing from you!

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