



ECD Global Alliance 2016 Annual Newsletter

Together Towards Tomorrow

November 21, 2016



Patients, family members, and physicians attending the first European Patient & Family Gathering.

The ECD Global Alliance offers resources to support the undiagnosed.

Patient Diagnosis Confirmed Through ECD Care Center Network Referral

Care Center affirms Erdheim-Chester Disease and begins treatment after patient is referred by diagnosing physician.

“I had been told I had every disease under the sun. I was kind of skeptical until I went to see Dr. Janku.”

-Rusty on receiving his diagnosis



Rusty evangelizing since beginning treatment thanks to the ECD care center network.

Rusty had been sick for 15 years. The determination of his pulmonologist finally led to an Erdheim-Chester Disease diagnosis. The pulmonologist had only seen one case of ECD in his entire life as an intern. **Through research, the doctor located the ECD Global Alliance website and referred Rusty to Dr. Filip Janku, an ECD Care Center physician, at The University of Texas MD Anderson Cancer Center.**

“I had been told I had every disease under the sun. I was kind of skeptical until I went to go see Dr. Janku. What he did was to actually gather all of my biopsies, and with his team of pathologists and radiologists, they came to a definite diagnosis.

That’s when I was finally convinced.” Rusty recalls finally receiving his ECD diagnosis. “I was encouraged that [my wife and I] actually knew what was going on. We did some research and discovered that there was some treatment out there for it. Dr. Janku had given us some pretty encouraging news.”

Today Rusty reports he is on an off-label treatment with good results. He stays dedicated to moving forward in his battle against ECD. *Learn more about Rusty at <https://rustykahn.com/>.*

Discover how the Global Alliance helps those affected by ECD at www.erdheim-chester.org.

If you or someone you know has been helped by the ECDGA, please tell us! Email Liz.Anderson@Erdheim-Chester.org.

About the ECD Global Alliance

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“This website and community has given me such encouragement and great expectations, especially in light of very recent research. You are all so amazing and the community is a God-send! God bless you all.”

-Sister of an ECD Patient

Financial Status

As stewards of each donation given to the community, the ECDGA takes each transaction and hour of work very seriously. It strives to meet its budget so that the majority of contributions go directly to supporting families and funding Erdheim-Chester Disease research.

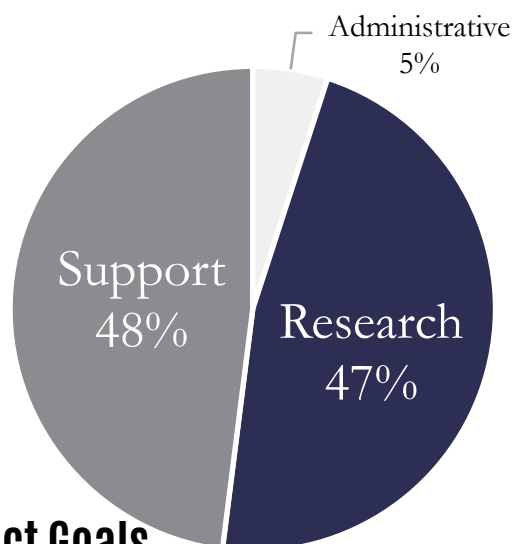
Our Mission

The ECD Global Alliance is dedicated to awareness and education, support, and research related to Erdheim-Chester Disease. Visit www.erdheim-chester.org/mission to learn all the ways the 501(c)(3) organization focuses its activities and programs to fight ECD and help those affected by the disease.



2016 Expenditures

* Year to date as of November 16, 2016



2017 Project Goals

Raise physician awareness of ECD at the 2017 US and Canadian Academy of Pathology meeting.	\$15,000
Facilitate educational webinars for those affected by ECD to learn about the disease, latest treatments and research, and ECDGA resources.	\$10,000
Raise physician awareness of ECD by attending specialty medical conferences.	\$15,000
Join ECD Care Center physicians together to collaborate on how to provide the best care for ECD patients through the care center network.	\$20,000
Support and facilitate ECD lectures given by ECD-knowledgeable physicians at medical institutions.	\$20,000
Funding Needs for 2017	\$80,000

In the year ahead the organization must fund ongoing commitments such as previously awarded grants, the ECD registry development, staff salaries, annual meetings for patients, families and medical professionals, and the awarding of a new research grant. **The execution of future projects is dependent on financial support.**



The ECD Global Alliance is Supported by You

The ECDGA relies heavily on private contributions from patients and their families and friends. These donations are supplemented with grants when awards are given, often on a sparse basis for small charities such as the Global Alliance.



Your Fundraisers Made It Possible

Thank you very much to the community members that raised awareness and money through fundraisers in 2016. Your commitment makes the ECDGA possible.



Running Vines Winery in Chesterton, IN hosted a fundraiser and awareness event in honor of ECD patient, Hank F. during Rare Disease Day weekend.

In Memory of Dee Royal



Razoo fundraiser in honor of ECD patient, Dee Royal. Erdheim-Chester Disease was confirmed after his passing. Friends and family raised funds to bring awareness and fund research for ECD.

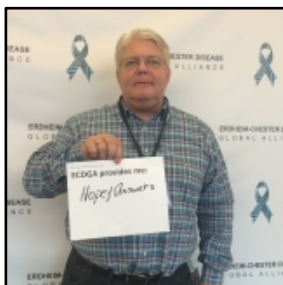


Razoo fundraiser in honor of ECD patient Mike Livings. His decade-long battle with ECD ended in April. Friends and family donated to the ECDGA in his memory.

In Loving Memory of Mike Livings

Razoo fundraiser by Joseph & Isis Gauzens to "help fund public awareness and research to give light to a very dark diagnosis"

Giving for Gauzens



Dine & Donate at Culver's

ECD patient, Ashley M. hosted two restaurant fundraiser events in Highland, IN. 10% of diner's meals went to support the ECDGA.

99% of 2016 funding was supported through private donations from ECD community members.



\$50,000

Dollars Raised in the
www.erdheim-chester.org
#ECDAWARENESS
for research
Fundraiser



Number of
ECD
Community
Fundraisers



fundECD.org

“I think the Gathering in France has been a success. New patients have been able to attend and get to know more about the ECDGA.”

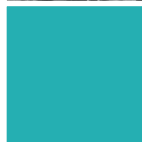
- Pål Feste, partner of an ECD patient

Support for Those Affected by ECD

The ECDGA website introduced 10 new language pages to support non-English speakers. Patients can now find information about the disease, treatment, and diagnosis in Chinese, German, Dutch, Spanish, French, Greek, Hebrew, Italian, Norwegian, and Portuguese.

Five new ECD Referral Care Centers joined the global care network. Washington University in St. Louis, MO and the Cancer Institute of São Paulo – University of São Paulo in Brazil join an elite group of hospitals willing and able to treat ECD. Centers that have treated less than five ECD patients are considered Emerging ECD Care Centers. New emerging centers include Mount Sinai Hospital in Toronto, Canada, The Seattle Cancer Center Alliance in Washington, and The American University of Beirut Medical Center in Lebanon.

The ECDGA planned and hosted the 4th annual international **ECD Patient & Family Gathering** in Paris, France. The first European event included 63 attendees from 17 different countries who heard from 16 physicians about ECD organ involvement, the use of scans to monitor the disease and its treatments, techniques to manage pain, and the latest research findings including treatment options.



Care center physician, Dr. Matthew Collin, visits with patient and his wife at the 2016 Patient & Family Gathering in Paris.

Awareness & Education

The 2nd annual ECD Awareness Week educated medical professionals, patients, caregivers, and the public about ECD through annual international events, sharing patient stories, fundraising, and researcher lectures at ECD care centers.


48 Countries
Represented
by the ECDGA



2016 ECD Medical Symposium co-host, Professor Julien Haroche from Paris, France presents his latest ECD research.

2016 ECD Medical Symposium guests in front of the ICM Auditorium at Hôpital Pitié-Salpêtrière in Paris, France





The 2016 Year in Review

Research

The ECDGA began the new year by awarding **\$50,000 to Italian researchers**, Marina Ferrarini, MD and Lorenzo Dagna, MD from San Raffaele Scientific Institute in Milan. The primary investigators are using the state-of-the-art cell culture system, the RCCS™ bioreactor, to understand how diseased cells in ECD patients accumulate and interact with neighboring healthy cells to fuel the disease.

The **World Health Organization declared ECD a histiocytic neoplasm**. This means the rare disease is now considered a slow-growing blood cancer that may originate in the bone marrow or a precursor cell. The March announcement represents a significant step toward improving support for sufferers.

The 4th annual international **ECD Medical Symposium** was held in Paris, France at Hôpital Pitié-Salpêtrière in September. The agenda included 28 presentations discussing treatment trial progress, model studies, case studies, basic science, pathology, and panel discussions regarding the ECD registry and possibilities of a future ECD Genome-Wide Association Study (GWAS). There were 64 medical professionals in attendance representing 10 different countries.

Progress continued on the setup of the **ECD patient registry** funded in 2015. This is possible through a collaboration between Memorial-Sloan Kettering Cancer Center and Newcastle University.

\$71,493
Dollars Dedicated to
Research in 2016

**Save
the
Date!**

**2017 International
Erdheim-Chester
Disease
Conferences**

**Medical
Symposium
October 26**

**Patient & Family
Gathering
October 27**

**New York, NY
USA**

403

Registered
Patients with
the ECDGA



The 2017 Year Ahead

Support for Those Affected by ECD
Host 5th Annual International ECD
Patient & Family Gathering

Continue to globalize through the development of a **European chapter**

Research

Implement the ECD Registry

Host the 5th Annual International
ECD Medical Symposium

Select and award a research grant with funds raised by the generous donors of the 2016 #ECDAwareness for Research campaign.

Awareness & Education

Educate pathologists about ECD at the March 2017 United States and Canadian Academy of Pathology (USCAP) meeting in San Antonio, TX.

“We are extremely lucky. We are seeing the effect of the ECD Care Center, and the teamwork involved in it. It came at the right time.”

– Kim Pape-Green,
wife of an ECD patient

Participating in a clinical trial can change the course of ECD.

Clinical Trials Inspire Hope

Bonnie is better today due to her trial participation.

“Unfortunately you do have ECD. And the patient that I had, has now passed away from it. And more unfortunate for you, we have nothing we can give you.”

- Bonnie's doctor on her confirmed ECD diagnosis

“Unfortunately you do have ECD. And the patient that I had, has now passed away from it. And more unfortunate for you, we have nothing we can give you.” Bonnie heard the diagnosing doctor's words as he confirmed she had Erdheim-Chester Disease.

Bonnie and her husband, John, worked through the ECD Global Alliance to discover what options were available to them. A clinical trial through the National Institutes of Health (NIH) in Bethesda, Maryland was accepting ECD patients who tested positive for the BRAF mutation to try a new off-label treatment for ECD.

Bonnie applied and was accepted into the trial that would go on for over a year. From her home in Washington state, every two to eight weeks, she and her husband flew across the United States in hopes of halting the debilitating disease. At first the trial was very difficult. Bonnie was severely sick and required lengthened stays at the hospital, but after a year Bonnie's ECD seemed to have slowed progression and in some places possibly ceased. Without participating in a clinical trial, Bonnie's fate may have been very different.

Share your experiences with The Mighty. They publish real stories by real people facing the real challenges of disability, disease and mental illness. Raise ECD awareness by contributing your story at <https://themighty.com/submit-a-story/>.

The
MIGHTY



Bonnie and her husband, John, smile today with their dogs because of Bonnie's courageous decision to join a treatment trial. Trying an experimental treatment ceased and reversed some of her debilitating symptoms.

Bonnie and John at the 2015 Patient & Family Gathering during her trial.

Learn about the trials that may help you at www.erdheim-chester.org/studies-trials/.

Resolve to Volunteer in 2017

“I witnessed all the frustrations, sadness, loneliness, exhaustion, and love during the years of trying to obtain a diagnosis. Now, there is help for patients and I am privileged to be able to volunteer.”

- Bridget Gates, ECD advocate and volunteer on why she volunteers with the ECDGA

Just a few of our selfless volunteers...



Dearbhaile D. Charles B. Simon D.

Thank you

In the New Year make a resolution to donate one hour of your time a week in support of those you love through the ECD Global Alliance. Visit <http://erdheim-chester.org/volunteer/> to find out some of the ways you can fight against ECD and support those affected by it.

ECD Care Centers Focus on the Best Care for ECD Patients

ECD Referral Care Center doctor comments on why the centers make a difference in patient outcomes.



“ECD Care Centers bring focused care by experts who are keenly interested in bringing the best care to ECD patients.”

-Dr. Kenneth L. McClain of Baylor College of Medicine at Texas Children’s Hospital



ECD Care Centers advise in treatment and proper care for those diagnosed with Erdheim-Chester Disease or looking to confirm an ECD diagnosis. The centers have committed teams of specialists knowledgeable about the ultra-rare disease. Dr. Kenneth L. McClain of Baylor College of Medicine at Texas Children’s Hospital explains, “The ECD centers bring focused care by experts who are keenly interested in bringing the best care to these patients.” Regardless of where a patient is located, their treating specialist can contact an ECD Care Center doctor. After a patient is

diagnosed, the centers are places patients and their treating physicians can turn to for guidance. Dr. McClain suggests that ECD patients “need to call for a referral and have their physicians communicate with the experts.”

There are 23 ECD Care Centers globally. The program officially began in October 2015 with 18 centers of excellence. In 2016, five new care centers joined the collaborative network.

For a complete listing and location map of ECD Care Centers visit <http://erdheim-chester.org/care-centers/>.



World Health Organization

Find out what the term "cancer" means for ECD patients.

Why Calling ECD a Blood Cancer Matters

The World Health Organization’s reclassification comes with new opportunities for ECD patients.

The term cancer evokes a plethora of ideas and emotions. In the case of those affected by Erdheim-Chester Disease it means an opportunity for increased support. In March of 2016, the World Health Organization (WHO) reclassified Erdheim-Chester Disease as a histiocytic neoplasm. This means the ultra-rare disease is now considered a slow-growing blood cancer. Currently, there are no FDA-approved treatments for ECD. All treatments are considered off-label. In addition, other than the ECD Global Alliance, there has been limited support for those diagnosed with Erdheim-

Chester Disease. **The updated WHO designation comes with hope that patients will be able to obtain off-label cancer treatments with less of a struggle from their insurance companies as well as gain support from cancer organizations.**

The reclassification is still very new and debate around the term cancer is still being discussed among ECD-knowledgeable doctors, but for now it provides a concrete way of describing and understanding this elusive and difficult disease.

You Can Support ECD Patients Today

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“Without research, progress will not be made towards first putting ECD in remission and eventually finding a cure. My wife and I contribute every month in the hope that that objective will be met in our son’s lifetime.”

- Lee Crump, monthly donor and ECD advocate
on why he gives to the ECD Global Alliance



*Make a
difference now!*

Your donations can
make the difference
between life and
death for ECD
sufferers and provide
much-needed support
for their families.

Your end-of-year giving starts with #GivingTuesday.
Support your loved one affected by ECD this holiday season.



Black Friday. Cyber Monday.
#GIVINGTUESDAY™
11.29.2016



Donate at www.fundECD.org or by sending a check to
ECD Global Alliance, P.O. Box 775, DeRidder, LA
70634.

Become a monthly contributor for as little as \$25 a
month to sustain support for those affected by ECD. Go
to www.fundECD.org, make a donation, and select your
sustainability level from the drop down bar.

Consider making a legacy contribution to help better
the lives of future ECD sufferers. Your loyal support will
enable the organization to plan assistance for those
affected by ECD into the future.

During this holiday season **make an end-of-year
contribution or encourage your loved ones to
support the organization in lieu of gifts** by writing a
letter or email discussing why contributing to the
ECDGA is important to you.

Contact your employer’s human resource department
about setting up a **company matching gift program**
benefiting the ECD Global Alliance.

Do your holiday shopping at AmazonSmile.com.
0.5% of your purchase will go to support the ECD
Global Alliance. Select the ECD Global Alliance as your
charity of choice and start shopping!

fundECD.org

The ECD Global Alliance | P.O. Box 775, DeRidder, LA 70634 USA | Support@Erdheim-Chester.org
www.Erdheim-Chester.org | Donations: www.fundECD.org

The ECD Global Alliance is a 501 (c)(3) organization

“What we have done for ourselves alone dies with us; what we have done for others and the world remains and is immortal.”

- Albert Pike

The work of the ECD Global Alliance is only possible due to the support, dedication, and inspiration from the ECD community. The ECD Global Alliance Board of Directors and staff thank all the patients, family members, medical professionals, volunteers, and donors who selflessly give their time, talent, and money to help us all. The organization would simply not exist without you. Many thanks for your continuous involvement in improving the lives of all affected by Erdheim-Chester Disease.



Contact Us

ECD Global Alliance

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Support@Erdheim-Chester.org

www.Erdheim-Chester.org | Donations: www.fundECD.org

Like us on [Facebook](https://www.facebook.com/ECDGA), follow us on [Twitter @ECDGA](https://twitter.com/ECDGA),
watch us on [YouTube](https://www.youtube.com/ECDGA), or connect with us on [RareConnect](https://www.rareconnect.org/ECDGA).

The ECD Global Alliance is a 501(c)(3) organization

ECD Facts

Erdheim-Chester Disease (ECD) is a rare, slow-growing blood cancer that may originate in the bone marrow or precursor cell.

- It is debilitating, underdiagnosed, and can result in organ failure unless successful treatment is found.
- Common early symptoms may include bone pain in legs and knees, fever, night sweats, weakness, fatigue, excessive thirst and urination, balance issues, lower back pain, bulging eyes/vision issues, shortness of breath and heart issues.
- ECD results in the overproduction of histiocytes, a type of white blood cell that normally fights infection and is created in bone marrow.
- The disease affects different organs in different people, yet it can affect virtually every organ system.
- Usually diagnosed by biopsy, scans (bone, PET, and MRI), and clinical symptoms.
- All treatments are off-label. These include targeted therapy, immunotherapy, chemotherapy, immunosuppressants, and steroids, all of which have been used with varying degrees of success.
- Breakthroughs identifying genetic mutations found in ECD patients are leading to targeted and effective treatments for patients.
- ECD research, with extremely limited funding, is at the forefront of science and “precision medicine,” illustrating that therapy targeting a specific genetic mutation can be effective, regardless of where cancer originates.