



ECD Global Alliance

2015 Annual Newsletter

December 1, 2015

Together Towards Tomorrow



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2015 ECD Global Alliance Year-End Overview

Research breakthroughs, increased awareness and education, and improved patient support are some of the highlights of the 2015 year. There is much to cover in this year's end-of-year report. Some of the topics covered include the excitement and inspiration buzzing in the Erdheim-Chester Disease community over the introduction of new ECD Referral Care Centers and the ECD Patient Registry. The ECD Global Alliance organized the first-ever ECD Awareness Week, which garnered international support from ECD patients and their families and friends. In addition, research is showing that new treatments are helping BRAF-positive patients, while new trials are being opened to help BRAF-negative patients. Read on to learn more!



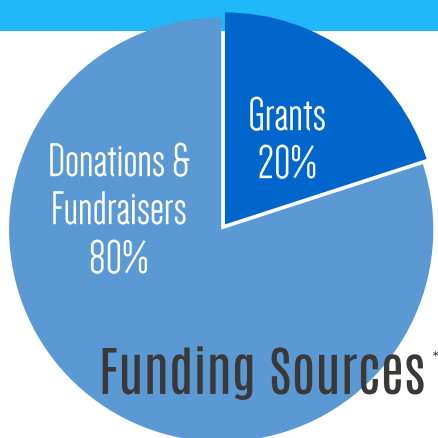
About the ECD Global Alliance

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"Thanks to the ECDGA, we are all in a much better place. Bless them." – chat room participant

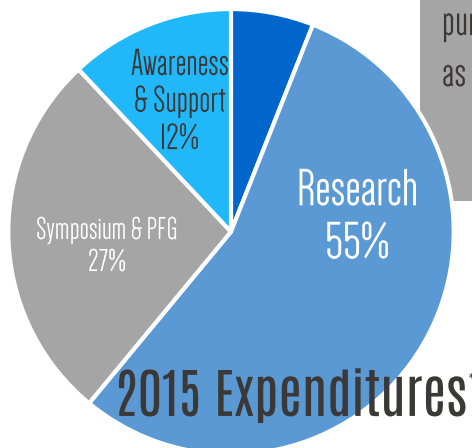
Financial Status

Exceptional financial stewardship is of the utmost importance to the ECDGA. The 501(c) 3 non-profit dedicates more than 90% of its funding for mission critical tasks. A host of volunteers and two part-time staff members perform the organization's work. Top organizational expenses include funding research and hosting international symposia and patient & family gatherings.



Funding Sources*

Overhead 6%



2015 Expenditures*

Our Mission

The ECD Global Alliance is dedicated to awareness, support, education, and research related to Erdheim-Chester Disease around the world.

Awareness facilitates the early diagnosis of the disease and stimulates concern and efforts to increase knowledge regarding ECD and treatments.

Support to lessen the burden to those affected by ECD. Support includes providing information, referrals, and one-on-one contact with others affected by the disease.

Education provided through the ECDGA website and published material, symposia, and patient & family gatherings for ECD patients, loved ones, medical professionals, and other organizations.

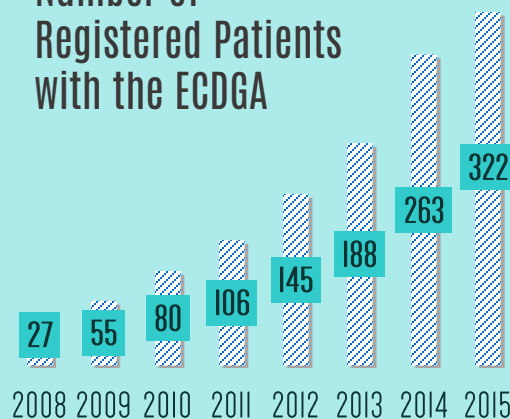
Research helps in understanding more about ECD and searching for more effective treatments, with the pursuit for a cure and/or prevention as the ultimate goal."

Organizational Data

The ECDGA members are a community of people that come together to fight this disease one day at a time.

Registration with the organization allows the community to have a stronger voice, provide accurate numbers of diagnosed patients and offers researchers access to more patients to enable research that can provide meaningful breakthrough opportunities.

Number of Registered Patients with the ECDGA



45
U.S. States Represented

42
Countries Represented



* Based off of estimated 2015 annual budget

The 2015 Year in Review

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"Awareness in Mason City has increased greatly, and we're so proud of that. We've had so many people ask us about ECD, especially after the article in the paper. The response has been amazing!" – Caregiver, Volunteer and Fundraising Organizer

Awareness & Education

Produced "A Leap Forward in Rare Disease Treatment" **film** to increase awareness of ECD and communicate research breakthroughs, made possible by a generous grant from Trovogene, INC

Organized the first **Annual ECD Awareness Week** to join together the ECD community to educate and generate awareness for medical professionals and public about Erdheim-Chester Disease

Support for Those Affected by ECD

Participated in **Give Rare Day**, a 24-hour fundraising campaign benefiting the ECD Global Alliance and ECD community on March 3, 2015. The organization was awarded a \$1,500 prize for highly engaged community involvement

Provided an improved **website** that offers up-to-date information for patients, families, and physicians on all device types

Planned and hosted the third International **ECD Patient & Family Gathering** held in October 2015, with 71 patients and family members attending from four countries

Identified the first **International ECD Referral Care Centers** to allow patients with ECD to access dedicated medical professionals who are knowledgeable about ECD treatments and committed to caring for ECD patients. Program commencement announced October 2015

Research

Awarded a two-year grant to Eli L. Diamond, MD at Memorial Sloan-Kettering Cancer Center and Matthew Collin, MB, BCh, DPhil from Newcastle University to create an **ECD patient registry** to better understand how the disease progresses and what treatment options work best under what conditions

Collaborated with The University of Texas MD Anderson Cancer Center in Houston, TX, USA to host the third International **ECD Medical Symposium** held in October 2015, with 34 medical professionals attending from six countries

Awarded two research **grants** to Eli L. Diamond, MD and Vaios Hatzoglou, MD both from Memorial Sloan-Kettering Cancer Center to better understand neurological issues in ECD patients and to Filip Janku, MD, PhD from The University of Texas MD Anderson Cancer Center and Omar Abdel-Wahab, MD from Memorial Sloan-Kettering Cancer Center to discover treatment opportunities for BRAF-negative ECD patients



Dr. Eli Diamond listens to patient and caregiver concerns at the Patient & Family Gathering Welcome Dinner.



ECDGA Global Excellence Award recipients pictured with ECDGA president, Kathy Brewer. From left to right, Juvanee Estrada-Veras, MD, Kathy Brewer, Amy Teare, volunteer, and Filip Janku, MD, PhD



"It always seems impossible until its done." – Nelson Mandela

In 2016, the ECDGA plans to capitalize on the momentum that has been achieved. The organization will work in an attempt to increase awareness of ECD within the medical community to increase earlier diagnosis of ECD, to reach more patients regardless of language spoken, and to fund more research to take advantage of the breakthroughs occurring in treatment options for ECD, no matter a patient's mutation status.



Awareness & Education

Support and facilitate presentations at medical institutions to increase medical community awareness and early diagnosis

Support to Those Affected by ECD

Provide website communication in multiple languages

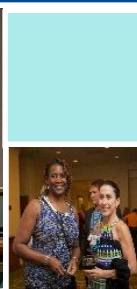
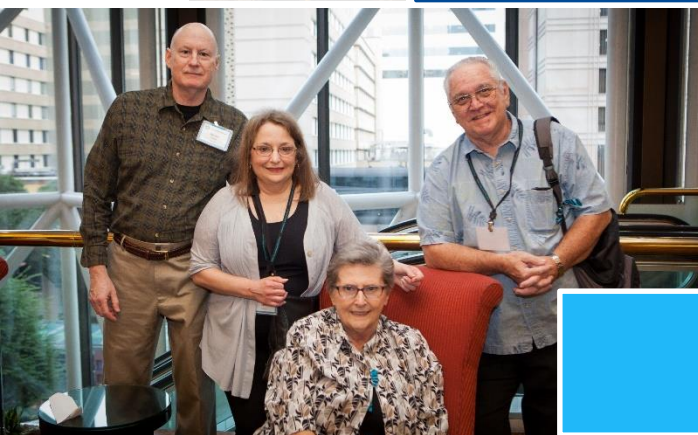
Continue the setup and development of ECD Care Centers to promote improved medical care for all ECD patients

Research

Fund ECD Patient Registry development and maintenance to better understand how the disease progresses and what treatment options work best under what conditions

Fund research to better understand neurological issues related to ECD

Fund research to further understand the genetic alterations of ECD patients and discover treatment options for all



Save the Date!

2016 International ECD Medical Symposium

September 15, 2016

Hôpital Pitié-Salpêtrière, Paris, France

2016 International ECD Patient & Family Gathering

September 16, 2016

Paris, France

ECD Care Centers & Patient Registry

Offer Better Opportunities for ECD Patients

Optimizing Patient Care with a Two-Pronged Approach

Darren has become mysteriously ill. Things that he once could do with ease, like play catch with his 13-year-old son, are overwhelmingly exhausting, leaving him with shortness of breath, and the pain in his legs and knees makes him feel as if he is twice his age. He visits his primary physician because he has lost 20 pounds in the last six weeks, has an unquenchable thirst, and awakens at night with abdominal and lower back pain. His doctor is perplexed. The physician runs tests, an incorrect diagnosis given, and Darren becomes sicker until finally after years of searching he discovers he has the ultra-rare condition, Erdheim-Chester Disease. Now, what happens?

Darren is a fictional ECD patient, but he exemplifies what many ECD patients face on their uncertain journey towards diagnosis and then once the diagnosis is found, the uncertain path towards treatment. The reality is overwhelming. How will these patients find help? Will their lives ever go back to normal? Is there any hope?

The answer is yes. In October 2015, the ECD Global Alliance announced the beginning of the ECD Care Center program and ECD patient registry at the organization's annual International Patient and Family Gathering in Houston, TX USA. The announcement delivers ECD patients hope that they will be able to find the best care possible. These opportunities will allow doctors to learn more and improve collaboration across institutions, with ECD patients being the winners as the knowledge base increases, bringing about better care for all patients.

What is an ECD Care Center and How Does it Help?

An ECD Care Center is an institution with a physician who has committed to leading a team of specialists to provide the best care to ECD patients. A Referral Care Center is one that has had five or more ECD patients treated at the medical center or by the lead physician.

The lead physician at each center has committed to providing formal or informal consultation on ECD cases at other centers. An ECD Emerging Care Center is an institution that is interested in caring for ECD patients but has not yet treated five or more ECD patients. Emerging Care Centers collaborate with Referral Care Centers in the treatment of all ECD patients until they become eligible to become a Referral Care Center. All care centers will work as a network, collaborating and sharing information to further the understanding of ECD and the best care for ECD patients *(continued on page 6)*.

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Number of Global Care Centers



The world's leading ECD-knowledgeable doctors gathered for a one-of-a-kind question and answer panel session during the 2015 ECD Patient & Family Gathering.

What is an ECD Patient Registry and How Does it Help?

The ECD Patient Registry is a database that compiles comprehensive data about the diagnosis, clinical management, and treatment outcomes of ECD patients worldwide. This collective information will be available to the ECD patient and scientific community to allow treatment recommendations to be made based on evidence and for the design of clinical trials. The registry is to characterize more fully the spectrum of ECD therapies, their toxicities, and their efficacy. The registry will not only reflect care but also come to shape it.

This registry will allow for retrospective comparison of multiple therapies across patients grouped by organ system, disease severity, and other pertinent information. Analyses like these hold promises for guiding evidence-based therapy for new ECD patients and the design of rational clinical trials based on the most rigorous available evidence.

Together Towards Tomorrow

The ECD Care Centers and Patient Registry are working together towards a more promising tomorrow for ECD patients. The combination of centers to treat the disease and centrally collecting patient outcomes, will serve as a prime opportunity to improve the treatments available to someone affected by Erdheim-Chester Disease.

For more information about either the ECD Care Centers or Patient Registry, please visit www.erdheim-chester.org or email questions to support@erdheim-chester.org.



ECD Research on the Forefront of “Precision Science” Clinical Trials Deliver Breakthroughs

Clinical trials are vital opportunities for patients to assist in finding potentially effective and FDA-approved treatments for ultra-rare diseases such as Erdheim-Chester Disease. In 2015, several clinical investigations offered hope in the understanding and treatment of ECD. Recapping the year's progress, several clinical trials provide encouraging strides in helping find better treatment options.

Findings from the Vemurafenib Basket Study, performed at Memorial Sloan-Kettering Cancer Center (MSKCC) in New York and published in the *New England Journal of Medicine*, found that vemurafenib had positive results for 43% of ECD patients (Hyman et al., 2015). The NIH Phase 2

therapeutic trial in Bethesda, Maryland opened in 2015 to investigate the safety, tolerability, and efficacy of two drugs, dabrafenib and trametinib, in the treatment of ECD. Ongoing studies are investigating the natural history of ECD to better understand how the disease progresses. Concurrently at Hôpital Pitié-Salpêtrière in Paris a separate investigation is evaluating the possibility of treatment interruption for those patients on a BRAF-inhibitor treatment. In addition, other trials have recently opened to investigate the use of Cobimetinib (MSKCC) and Lenalidomide (Dana-Faber Cancer Institute, Boston) as potential treatments for ECD. The progress of this year's trials are hopeful and encourage the continuation of more trials to help understand and treat ECD. On the horizon for 2016, additional trials

and studies are opening to ECD patients.

Gratitude goes to those doctors and researchers who make these trials possible, as well as the patients who enter them. Clinical trials help the entire ECD community as they can potentially lead to FDA approval of a treatment for ECD. With FDA approval, the treatment becomes an eligible drug for insurance approvals. Only with patient involvement in trials is this possible.

Reference: Hyman, D. et al. (2015). Vemurafenib in Multiple Nonmelanoma Cancers with BRAFV600 Mutations. *New England Journal of Medicine*, 373, 726-736. doi:10.1056/NEJMoa1502309

For more information on these trials and others like them, please visit <http://erdheim-chester.org/studies-trials/>

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Number of Trials Involving
ECD Patients

Help Support ECD Patients Today

"The value of man resides in what he gives and not in what he is capable of receiving."

— Albert Einstein

How do your
donations help?
*Let us count the
ways*

\$10 Hosts the www.erdheim-chester.org website for one month

\$25 Funds a Board of Directors Teleconference

\$40 Funds a Medical Advisory Board Teleconference

\$100 Hosts the www.erdheim-chester.org website for one year

\$150 Prints ECD pamphlets and posters for awareness events

\$500 Supports an awareness event at a medical conference

\$1,000 Supports travel and lodging for a patient to attend a Patient and Family Gathering

\$1,500 Pays the ECD Global Alliance insurance for one year

\$50,000 Funds an ECD-focused research project for one year



#ECDawareness

You Can Make a Difference
Make a Donation Today!

Visit <http://www.razoo.com/story/Ecd-Global-Alliance> or
place a check in the mail made out to the ECD Global Alliance
and mail to ECD Global Alliance, P.O. Box 775,
DeRidder, LA 70634 USA.



Thank You

"I can no other answer make but thanks, and thanks; and ever thanks." – William Shakespeare

The ECD Global Alliance Board of Directors and staff thank all the patients, family members, medical professionals, volunteers, and donors who have given their time, energy, and donations to help us all. The individual contributions of many have propelled the progress seen today in the ECD community. A sincere gratitude and appreciation to everyone involved with the forward motion in finding better treatments and hopefully one day, a cure.



Contact Us

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Support@Erdheim-Chester.org

ECD Facts

Erdheim-Chester Disease (ECD) is a debilitating, underdiagnosed rare condition. It is classified as a non-Langerhans histiocytosis and is increasingly described as an inflammatory myeloid neoplasia (blood cancer).

- Histiocytes, cells that normally fight infections, accumulate in tissue and organs.
- Affects different organs in different people, yet can affect virtually every organ system.

Common early symptoms may include bone pain in legs and knees, fever, night sweats, weakness, fatigue, excessive thirst & urination, balance issues, lower back pain, bulging of the eye/vision issues, rash, shortness of breath, and heart issues.

Usually diagnosed by biopsy, scans (bone, PET & MRI), and clinical symptoms.

Unless successful treatment is found, organ failure can result.

All treatments are off-label. These include gene inhibitors, immunotherapy, chemotherapy, immunosuppressants, and steroids, all of which have been used with varying degrees of success.

Recent breakthroughs identifying genetic mutations found in ECD patients are leading to targeted and effective treatments for a subset of 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.