



2014 Year End Report



ECD Global Alliance Overview

During the past year, hope has continued to grow for the Erdheim-Chester Disease community. The number of physicians and scientists involved in treating ECD patients and researching treatments has increased. Many BRAF-positive patients are doing well on BRAF-inhibitor treatments. Research is focused on finding better treatment options for BRAF-negative patients. ECD studies have continued to attract new patients and a new treatment trial was opened at the NIH. Efforts to raise awareness are growing stronger, with larger audiences being reached. The number of published medical journal articles about ECD was greater in 2014 than in any previous year. And, the ECD Community has continued to grow stronger and foster more interactions among patients and family members through the use of social media, chat sessions and the annual Patient and Family Gathering.

We are excited to tell you about some of the 2014 activities in this newsletter. We extend our sincere gratitude to everyone who has contributed to increasing hope for the community and helping the ECD Global Alliance thrive. Thanks are given to patients, family members, medical professionals, volunteers, and donors who have given their time, energy and donations in an effort to help us all. Our goal for 2015 is to keep the momentum going as we search for better and more targeted treatments for all and ultimately a cure for ECD.

ECD Board of Directors & Staff

Inside this issue

ECDGA Community Data	2
2014 Accomplishments	2
Planning for 2015	3
October 2015 ECD Event	3
Awareness & Fundraising ..	4-6
ECDGA Expenses	6
ECD Clinical Trials	7
ECD Publications	7
ECD Facts	8

Alone we are rare.

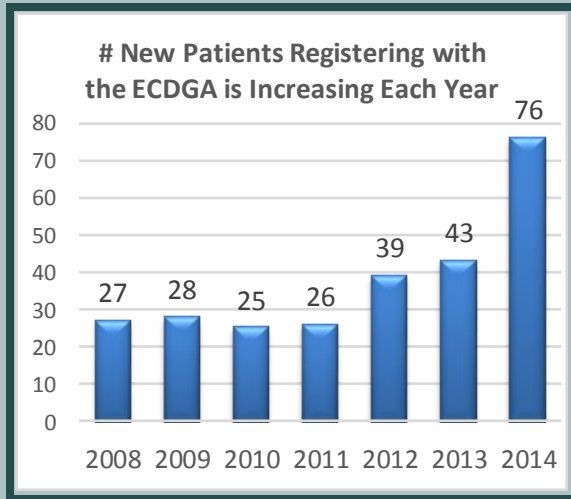
Together we are

strong.

- NORD

*(National Organization of
Rare Disorders)*

2014 ECDGA Community Data



Remembering and Hoping

We will always remember those who have lost their fight with ECD. Our prayers and thoughts are with their families. We will continue doing all we can to reduce the suffering that is caused by this dreadful disease.

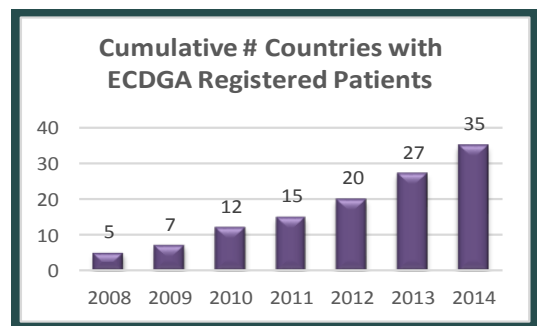
Effort is always underway to reach those who are suffering, but have not yet received a correct diagnosis.

And finally, we are committed to helping find better and more targeted treatments for all ECD patients. We are thankful for the breakthroughs we have seen, but we will not be satisfied until a cure can be found. We thank you for being part of that quest.

“Hope Beyond Borders”

2014 Accomplishments

- Partnered with the NIH to host the 2nd International ECD Medical Symposium held in September 2014, with 40+ medical professionals attending from 6 countries
- Planned and hosted the 2nd International ECD Patient and Family Gathering held in September 2014, with 78 patients and family members attending from 5 countries
- Brought together scientists and physicians who published the first peer-reviewed medical journal article providing a consensus view on the diagnosis, treatment and management of ECD (“Consensus guidelines for the diagnosis and clinical management of Erdheim-Chester disease”, published in BLOOD, 24 July 2014.)
- Partnered with leading research institutes to provide rapid information to patients regarding ECD studies and trials
- Raised over \$167,000 (US dollars) to support ECD research
- Provided an ECD patient and family support network through online chats, www.rareconnect.org, Facebook (<https://www.facebook.com/ErdheimChesterDisease>), and Twitter (<https://twitter.com/ECDGA>)
- Worked with the NIH to announce the opening of the new trial: “Dabrafenib and Trametinib in Treating Patients With Erdheim Chester Disease and BRAF Mutation”



Planning for 2015

Although great progress has been made in understanding and treating ECD, there is still much to do. We want to beat this disease and we won't give up until we are successful. But, we need YOUR help to accomplish the following goals in 2015:

- Plan and host the 2015 ECD International Medical Symposium and Patient/Family Gathering in partnership with MD Anderson in Houston, Texas, October 8—11, 2015. Please mark your calendars!
- Fund the development of an ECD patient registry to allow researchers access to the information they need to move forward with research without delay.
- Implement a redesigned ECD Global Alliance website that will work on all screen sizes and allow faster updates of information.
- Identify and support ECD Referral Centers of Care to help ECD patients more easily find quality and timely care.
- Provide support to patients and medical providers to help ease the burden of working with payers to gain approval for required treatments.
- Award ECD research grants to search for focused and effective treatments for all ECD patients.



ECD Event Scheduled for October 2015 in Houston, Texas

Each year the ECD Global Alliance plans and hosts an international event dedicated to sharing information about Erdheim-Chester Disease. Medical professionals and interested industry leaders from around the world come together during the one-day Medical Symposium to share the latest research findings. The following two days are dedicated to providing patients and their family/friends with the latest information and findings about ECD. It is also time for patients/families to interact with each other face-to-face. For many, it is the only opportunity they have to meet someone else with ECD.



2014 Patient and Family Gathering, Bethesda, MD

In 2015 the ECD event will be held in Houston, Texas. The Medical Symposium will be on October 8 and the Patient/Family Gathering will be from October 9—10. Plans are underway to host the 2016 event in Paris, France!

Awareness and Fundraising - Please Join In!

Many members of the ECD Global Alliance worked very hard in 2014 to increase awareness of ECD and raise funds for research. The creativity and diversity of these events were immense. The following are but examples of the many activities that have occurred around the world. Please join us in thanking the courageous and giving individuals and families who are working so hard to ensure ECD research can continue. Their efforts help us all.



May 6 was a call for the community to give to organizations they trust to tackle today's critical issues. Thank you ECD community for trusting the ECD Global Alliance.



Three friends, Anthony Edwards, Holly Spencer, and Jonathan Wareing, cycled from Johannesburg to Cape Town, South Africa to raise awareness of ECD. We are so proud of their achievement and showing us what a global community we really are.



The Kaline and Livings families brought awareness of ECD to the state of Florida as the Vero Beach High School Football team dedicated a winning district game to help in the fight against ECD. Go Fighting Indians!

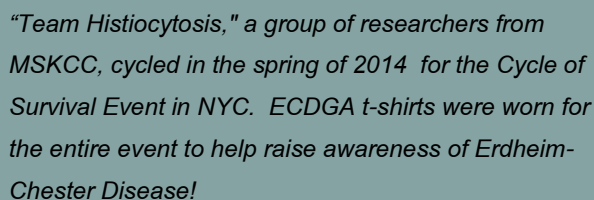
**You can help.
You can make a difference.**

Thank you for giving back and helping us support the ECDGA to fund research and find a cure!"

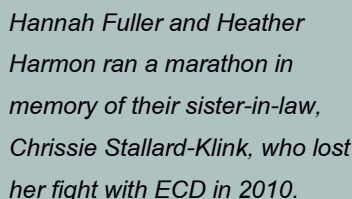
- ECD Caregiver

DawnMarie Crump showed that with a personal commitment, we can conquer big challenges as she completed a Spartan Race. DawnMarie's efforts raised over \$10,000 to support ECD research!

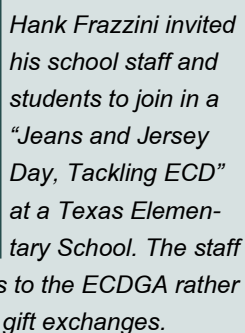




Unless someone like you cares a whole awful lot,
nothing is going to get better. It's not. ~Dr. Seuss



Barb Crump meets with ladies in her community every year for a workshop where they make pretty Christmas angel gifts for friends. This year Barb took the opportunity to explain something about ECD. As a result, the workshop participants made donations to the ECD Global Alliance.



"I've seen and met angels wearing the disguise of ordinary people living ordinary lives."
~Tracy Chapman

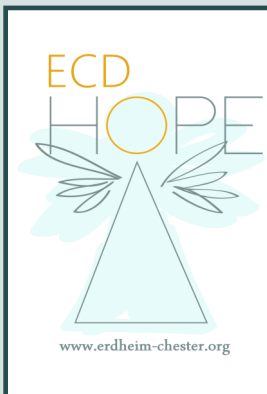


The Tuesday after the US Thanksgiving was declared Giving Tuesday and the ECD community showed their support. Thank you!

Awareness and Fundraising Continues!



DawnMarie is at it again! This time she has decided to have a Christmas party fundraiser, proving with this event that fundraisers don't have to be grueling and can be fun! Thank you for your dedication, DawnMarie!



The Jones Family kicked off the ECD Hope Angel Campaign and challenges everyone to participate. Take a video of yourself making an angel in the snow, sand, leaves or other media. Post it on Facebook challenging three friends to do the same and to donate \$20. If someone chooses to opt out of the ECD Hope Angel challenge, they can still participate by doubling their donation. Please post your angel!



The 2014 ECD Letter Campaign has seen many families sending letters to their friends and family explaining how to participate in the fight against ECD. Many have responded with generous donations.



During the 2014 Patient/Family Gathering, Bengt Thornberg challenged each ECD family to raise \$1,000 before the October 2015 event. He plans to throw all attendees a party if we meet the goal to reach \$241,000 in donations. Please help us meet this challenge!

ECDGA Expenses

The ECD Global Alliance takes great care to keep administrative costs as low as possible. The overhead of the organization is kept to a minimum by relying heavily on volunteers to manage the organization and to carry out its programs. Currently all organizational work is performed by one part-time staff member and a host of volunteers. However, the expenses associated with medical research are great. The organization's greatest expenses each year, by far, are funding research grants and hosting the annual Medical Symposium and Patient and Family Gathering.

Efforts are made to obtain grants to help the organization fund various programs. However, the reality is, almost all funding comes from private donors. Without donations the organization would be unable to carry out its mission. For this reason we are extremely grateful to all those who do what they can to help ensure necessary funds are available to allow ECD research to continue. Thank you!

Why are ECD Clinical Trials Important?

People with ECD often have difficulty gaining approval for the treatment they and their medical team believe will be best for them. One reason for this is because all ECD treatments are considered “off-label.” In the US, “off-label” means an FDA-approved drug is being used for a condition for which it has not been approved. This practice is not uncommon, especially in the rare disease and cancer worlds. However, health insurance companies (or other payers) closely scrutinize a drug that is to be used off-label and may refuse to pay for its use. They do this on the grounds that its use is “experimental” or “investigational.” To appeal a decision of this nature, a doctor will often send the insurer copies of peer-reviewed journal articles or other respected sources that support the off-label use. This takes time and is not always successful.

Drugs only become FDA-approved following a successful clinical trial that determines a treatment is safe and effective for a particular condition and/or group of patients. One of the challenges in performing a successful clinical trial for ECD is enrolling enough patients to complete a trial. Patients must qualify for a trial by meeting a set of inclusion/exclusion criteria.

Before enrolling in a clinical trial patients need to consider the personal risks and benefits to their health as well as any associated financial and time costs. Physicians and trial investigators are best prepared to help someone determine if they will qualify for a trial and if it is in their best interest to participate.

Two clinical treatment trials in the US are currently recruiting BRAF-positive ECD patients. One is for treatment with Vemurafenib at US centers in New York, Houston, and St. Louis. The other is for treatment with Dabrafenib and

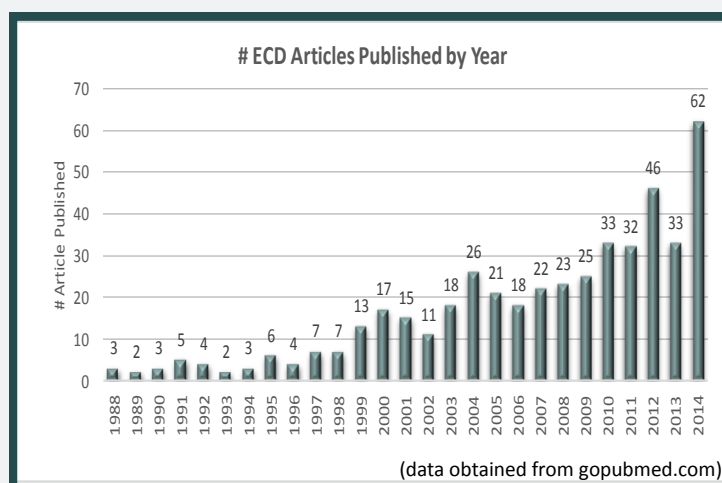
Trametinib taking place at the NIH in Bethesda. The ECD community is fortunate that these trials have been opened and it is hoped that patients will strongly consider enrolling in one of them if they qualify. Additional studies are also opened and can be found on-line at the ECDGA website.

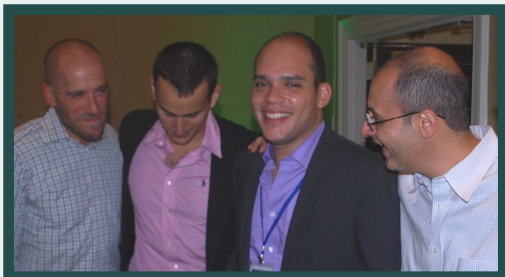
Scientists are also working hard to find targeted treatment options for BRAF-negative patients and it is hoped that new trials will open in 2015.

ECD Publications

The following chart shows the increase in number of medical journal articles that are being published world-wide about ECD. This is an indication of the growing interest and attention being given to learning more about ECD. With each article published more medical professionals will learn about the disease.

It is the hope of the ECD-GA that this will mean people are diagnosed sooner and our knowledge of the disease will grow exponentially, leading to more successful treatment options for all.





*Eli Diamond, MD, MSKCC;
Roei Mazor, MD, Sheba Medical Center;
Juvianee Estrada-Veras, MD, NIH;
Omar Abdel-Wahab, MD, MSKCC
at the 2014 ECD Medical Symposium*

Hold the Date!

2015 International ECD Medical Symposium

Date: October 8, 2015

Location: MD Anderson Cancer Center; Houston, TX, USA

2015 International ECD Patient & Family Gathering

Date: October 9-10, 2015

Location: Marriott Medical Center; Houston, TX, USA

"I would like to thank you again for the organization of this great meeting. It was helpful to develop new international collaborations on histiocytoses."

- Medical Doctor on attending the 2014 ECD Medical Symposium

Erdheim-Chester Disease Facts

An-ultra rare disease of unknown cause, affecting many different organs.

Characterized by excessive production of infection fighting cells, called histiocytes, that accumulate within multiple tissues and organs.

Involvement may include leg and arm bones, skin, tissues behind the eyes, lungs, brain, pituitary gland, kidney, abdominal cavity, the membrane surrounding the heart, and more rarely other organs. Each patient can have a different combination of organs involved.

Not currently categorized, but recent findings suggest a neoplastic or 'cancer-like' disorder.

Usually affects adults, but has been found in children.

Less than 500 cases have been documented in the world since it was first described in 1930 by Dr. Jakob Erdheim (Austrian) and Dr. William Chester (American).

Unless successful treatment is found, organ failure can result.



For more information -

Website: www.erdheim-chester.org

Phone: +1-337-515-6987

E-mail: support@erdheim-chester.org

Social Media:

<https://www.rareconnect.org>

<https://www.facebook.com/ErdheimChesterDisease>

Donations can be mailed to:

ECD Global Alliance

P.O. Box 775

DeRidder, LA 70634 USA

Online Donations made at:

<http://www.razoo.com/story/ECD-Global-Alliance>