ECDGA

ANNUAL REPORT 2020

ECD GLOBAL ALLIANCE

ECD Global Alliance

2020 Annual Report

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Board of Directors

The ECD Global Alliance Board of Directors are the backbone of the work behind the mission. Growing with five new board members early this year, new members energize and guide the organization to best support the ECD community into the future.

All board members embody the spirit of the community and bring talent, expertise, and energy to the table. The ECDGA is very fortunate to have them help lead the organization as we continue to strengthen the ECD community and search for better treatment options for all.

Linda Adams, PhD
Kathy Brewer, President
Jean Campbell
Mohammad Chowdhury
Juvianee Estrada-Veras, M.D.
Janet Froetscher
Paul C. Hendrie, MD, PhD
Glenn Padnick
Diane Schriner, Treasurer
David Smythe

READ MORE: https://erdheim-chester.org/boardstaff/

Walking with Hope

Story by Hugh Welborn, ECD Patient Since 2015

My name is Hugh Welborn. I have been married to my wife Linda for nearly 35 years, and we have two adult sons. I am now a retired attorney and judge. Linda is a retired school teacher and college professor. For exercise I walked about four miles every day, rain, snow, or shine. I was in great health and felt great - until I didn't.

About five years ago, I started getting short winded and knew something was wrong. After getting a heart exam, the doctors told me I had two severe blockages. I had open heart surgery. However, I never got much better over the next year. I went to doctor after doctor, but I kept getting worse. It got so bad that I couldn't walk two steps without getting out of breath. I developed an odd cough that wouldn't stop at times. I couldn't sleep lying down, so I tried to sleep sitting up in a cushioned chair. **I had no relief and was miserable. Finally, I had a heart attack.** An MRI showed a foamy substance covering my entire body and squeezing a heart artery, which caused the attack. **No doctor at the hospital had a clue** what this foamy substance was, but a new doctor suggested that I go to the medical university in Charleston. There, the doctors performed a biopsy and one doctor had studied Erdheim-Chester Disease (ECD), and he determined I had ECD.

I studied everything I could about ECD and **thought I had a death sentence coming very soon**. I got my affairs in order, retired from the bench and had a great doctor friend of mine refer me to MD Anderson Cancer Center (MDA) in Houston. I also called the ECD Global Alliance (ECDGA) and personally spoke with Kathleen Brewer late one night. She had the patience of Job and a kind demeanor.

I now had hope! After two trips to MDA, the doctors prescribed Mekinist (trametinib). In two days, I could breathe again! It was literally a miracle!

I now take Tafinlar (dabrafenib capsules) along with Mekinist. Although I am still retired, I do some pro bono legal work for the Family Court and help abused women in the courts for free as well. I am now back to walking every day, although not as fast or as far. I suffer from fatigue every day and take naps to get rid of it and my balance is impaired. I am going to Duke Medical Center since it is so much closer and they now have a program for ECD.

The ECD diagnosis was very scary, and I nearly died while the doctors tried to find out what I had. **I now appreciate life so much more, knowing how close I was to dying**. Honestly, it has changed my life for the better and I am so glad to be able to help others in my pro bono court work. Finally, thank God for Kathy Brewer and the ECDGA, because I know I am far from being alone in my walk with the diagnosis.

READ MORE PATIENT STORIES:
https://erdheim-chester.org/news-printed-stories/:

ECD Care Center Network

The ECD Care Center referral network includes 33 medical institutions worldwide.

HEAR FROM YOUR NETWORK PHYSICIANS:

- Dr. Razelle Kurzrock, UC San Diego Moores Cancer Center, San Diego, CA
"The ECD Care Center Network brings expert care to patients with an ultra-rare condition—ECD. We have extensive experience with ECD and are pleased to have helped identify some of the new treatments that have dramatically improved the outlook for patients with ECD."

-Dr. Ronald Go, Mayo Clinic, Rochester, MN
"Since 2017, the Mayo Clinic Histiocytosis Working Group, a multidisciplinary group of physicians studying histiocytic disorders, has been collaboratively treating patients with Erdheim-Chester disease. This practice model allows a more efficient way of patient care along with better integration of research and education."

- Dr. Michael Dickinson, Peter MacCallum Cancer Center, Melbourne, Australia
"At Peter Mac, we are working hard to improve access to ECD diagnostic tests and comprehensive care. We are relatively new in the field of ECD compared to other Global Alliance sites and we have learned over the last few years how very individual every patient's experience is with this disease compared to more common disease. The ECDGA is an impressive patient care and academic collaboration that keeps us all abreast of advances in this field."

- Dr. Douglas Ney, UC School of Medicine, Denver, CO
"The ECD Global Alliance has been instrumental in our patients' care. It serves as a source of education and support where so little good information exists. But most importantly, this network has allowed patients with a rare disease to find specialized care that can dramatically improve outcomes as well as quality of life. To have such a partner in care for these patients is a true privilege."

- Dr. Paul Hendrie, Seattle Cancer Care Alliance, University of Washington, Seattle, WA
"The Seattle Cancer Care Alliance participation in the ECD Care Centers has provided patients in the northwest United States with ECD and other histiocyte disorders, or suspected histiocyte disorder, a destination for evaluation, treatment and management of complications. For the patients this has provided early diagnosis, early access to treatment, and most importantly access to care givers who have experience with their disease. The increased histiocyte patient census in the clinic has provided learning experiences for physicians including trainees, nurses, other supportive services personnel resulting better familiarity with the disease and promoting interest in getting involved."

- Dr. Mohammad Atta, Johns Hopkins, Baltimore, MD
"When it comes to ECD, no one size fits all. At Johns Hopkins ECD care center, we treat each living person with her/his own peculiarities and we have the resources for it."

- Dr. Eli L. Diamond, Memorial Sloan Kettering Cancer Center, New York, NY
"At Memorial Sloan Kettering Cancer Center (MSK), we strive to provide high-quality, multidisciplinary, patient-centered care for patients with Erdheim-Chesterdisease and other histiocytic disorders. Our patients can participate in a variety of clinical trials or other research studies. We are proud to be a member of the ECDGA Care Center network and often share patients and research projects with other centers."

- Dr. Richard Riedel, Duke Cancer Institute, Durham, NC
"Being able to provide expertise, education, and hope to patients with ECD is incredibly gratifying. The Duke Cancer Institute has long been a leader in advancing care for patients with rare diseases. ECD is no exception. I am happy to partner with the ECD Global Alliance to provide the best care possible for patients affected by this disease. Advances are clearly being made and Duke is honored to be part of the process."

HOW DO PATIENTS BENEFIT FROM THESE CENTERS OF CARE?

- Michael Kaline, ECD Patient
"The ECDGA referred me to a care center in my state...a biopsy confirmed the diagnosis, finally I had an answer."

- Diane Schriner, Spouse to ECD Patient
"Luckily, we were quickly directed to an [ECD] care center doctor and were able to get information and treatment right away."

LEARN MORE ABOUT THE CENTERS NEAR YOU:

https://erdheim-chester.org/care-centers/

Research Updates

The ECDGA has funded over $700,000 in Erdheim-Chester Disease research.

2019 RESEARCH GRANT AWARD UPDATE

The study Pathophysiology of Neurodegenerative Erdheim-Chester Disease and Langerhans Cell Histiocytosis was chosen for a $50,000 award in the grant period of 2019, funding began in 2020. Dr. Nicole Coufal, Principal Investigator in San Diego, CA, offers an update to the project. *"This pilot project has allowed for a new area of research in my lab looking at neurodegeneration in ECD and LCH. Without this pilot grant neither would have been possible! I appreciate it very much. The work has been slowed down by COVID, but we are progressing!"*

2017 AWARD: Defining the Cell-of-Origin of Erdheim-Chester Disease, Dr. Benjamin Durham, MSK Pathologist

"The research expanded to other histiocytoses in addition to ECD. The research approach to ECD has also provided a blueprint for studying other rare disorders as well. The ECDGA also provides an international platform for sharing ECD and other histiocytosis research findings to the medical and scientific communities across the world. The funding and other support of the ECDGA has also led to major advancements in our clinical and functional molecular understanding, the molecular therapeutic options, and clinical management advancements for ECD and other histiocytic neoplasms during the past decade."

2015 AWARD: Tailoring Treatments for Erdheim-Chester Disease, Dr. Marina Ferrarini, Milan, Italy

"Techniques developed during the grant project are been exploited with other BRAF-mutated cancers (melanoma and in perspective LCH)...The ECDGA allowed me to perform my research, which otherwise would not have been possible, given the restricted availability of funding dedicated to research on rare diseases."

LEARN MORE ABOUT RESEARCH:
https://erdheim-chester.org/funding-research/

ECD Registry

NEW Study Progress & Findings, 7-page report: https://erdheim-chester.org/wp-content/uploads/2020/12/2020-Registry-Update.pdf

Patient registries help doctors and scientists make key advancements in the way we treat and care for people with rare diseases like Erdheim-Chester disease (ECD). By securely collecting a defined set of health and demographic information from patients with a specific condition, registries can give physicians insights into how diseases affect patients, what treatments are beneficial, and a disease's impact on patients' quality of life. Registries can also help doctors identify previously unknown symptoms or characteristics of a condition. The ECD Registry at Memorial Sloan Kettering Cancer Center (MSK) aims to improve the lives of patients with ECD worldwide. Through the generosity of the Erdheim-Chester Disease Global Alliance community, MSK has built a robust database that is transforming treatment recommendations and symptom monitoring. We are pleased to share a summary of our findings and future directions.

Currently, 161 eligible participants have enrolled in the study from various countries — the United States, Romania, Wales, Australia, the United Kingdom, the Philippines, Israel — and institutions, including MSK, MD Anderson, Columbia, Dana-Farber, Duke, Johns Hopkins, Mayo Clinic, Orlando Health, Stanford, Yale, Rush University, Cleveland Clinic, New York University, Broward Health, and the University of California in San Diego, Los Angeles, and San Francisco. Patients on the registry range in age from 18 years to 79 years. The average patient is 55 years old.

MSK researchers are also conducting a study to understand the experiences and needs for information and support among **ECD CAREGIVERS**. To learn more or join this study, please email neuhistiores@mskcc.org.

LEARN MORE ABOUT THE ECD PATIENT REGISTRY:
https://erdheim-chester.org/patient-registry/

Webinar Program

Delivering your dose of education & support straight from the sources.

Program Goals

- Identify ways to enhance the exchanges among patients, caregivers, and physicians.
- Create a medical hub of education (such as a virtual tumor board for histiocytic neoplasms).
- Align with other rare disease patient organizations to support and promote both research and the acquisition of expertise by the relevant physicians.

Meetings Ahead

- *For the Patient:* Monthly meetings will continue for social interaction. Also, how to manage care, finances, and more.
- *For the Caregiver:* Monthly to Bi-Monthly topics and discussion time will be offered.
- *For the Medical Professional:* Complex case studies, new breakthroughs in care and treatment, as well as general education are on the horizon.
- *For the Grieving:* A new group will begin in January, stay tuned for more.

WEBINAR SCHEDULE: https://erdheim-chester.org/webinars/

NEW: ECD Global Alliance Champions Program

Introducing ECDGA Champions! The ECD Global Alliance is committed to including the thoughts and knowledge of the ECD community in the development of programs, materials, and research for people with ECD and their families. The ECDGA Champions is our new go-to group for opinions and feedback on a variety of ECD topics. Members can participate at the level that suits them best. Additional opportunities may come up for members to apply to fill essential roles within committees and participate in other projects.

What is an ECDGA Champion? ECDGA Champions include people with Erdheim-Chester Disease and their family members. This group directly impacts programs and initiatives for the community by sharing their experiences and insights with the ECD Global Alliance. Because each person’s experience is unique, it is critical to hear from as many people as possible. Whether filling out a survey or participating in a focus group or committee, ECD Champion members are helping to shape efforts for the entire ECD community.

By signing up to be a member of the ECD Global Alliance Champions, you allow us to contact you for future opportunities. You can always say no if the timing isn’t right for you!

LEARN MORE & SIGN UP: https://erdheim-chester.org/ecd-champion/

Matching Gift Campaign

The $80,000 match campaign ends December 31!

Thanks to an $80,000 matching gift from a group of generous ECDGA Board members and benefactors – your donation's impact on our critical work will go TWICE as far in 2021. Thank you for your continued support to help the community continue on the path to a cure. Please take a moment to hear from our community members on why they give.

"When you have a family member with a devastating disease, you step up and do what you can." - Lee Crump, ECD Parent

Please watch our community video:

WHY I GIVE *3-minute video* https://www.youtube.com/watch?v=4DD-9sCmuAo

WHAT YOUR GIFT PROVIDES

- **More Research!** Funding research on Erdheim-Chester Disease in 2021 to continue the quest for better treatments is a priority.
- **Patient Navigation Support!** The ECDGA will add a staff position for a Patient Navigator who will serve as a point of contact for patients and caregivers to turn to for help and information about medical processes.
- **Increased caregiver support!** An increase in caregiver support will help caregivers feel less alone and provide them with tools to help cope with difficult situations.
- **Greater physician awareness!** Increasing physician awareness using innovative methods will help improve diagnoses rates and allow for

better treatment options, ultimately translating into better patient outcomes.

HOW TO DONATE

- Checks Payable to:
  ECD Global Alliance
  P.O. Box 775
  DeRidder, LA 70634
- Online:
  www.FundECD.org
- PayPal Giving:
  https://www.paypal.com/us/fundraiser/charity/1903749

2020 COVID-19 Erdheim-Chester Disease Patient Survey

Results from June Questionnaire

Ninety-six (96) ECD patients from seven (7) different countries responded to the COVID-19 survey conducted by the ECD Global Alliance. In addition to the survey, 11 of the 33 ECD Referral Care Centers submitted survey data for this research. From all the responses, only one ECD patient reports as having had COVID-19, with this occurring before widespread knowledge of COVID-19 risks and safety measures were known. Although the patient did require ICU care, we are happy to report recovery did occur. Since this survey was distributed this summer, two patients have reported having COVID to the ECDGA.

Unfortunately, becoming infected with the virus is not the only way this pandemic can affect patients. Fifty-six percent of responding patients have experienced impacts to their treatments and life as a result of the pandemic. This includes medical testing/follow up appointment delays (approximately 40%), treatment delays (approximately 12%), physical/occupational therapy delays (5%), and other social/economic impacts. In addition, centers reported some delays in new patient referrals, non-urgent follow-up appointments and procedures, and less frequently, treatment changes or delays.

Finally, one additional piece of good news came to light with the survey results. Approximately 1/3 of ECD patients have had at least one of their medical appointments converted from a face-to-face appointment to a virtual telehealth appointment. The ability for doctors and patients to participate in a telehealth appointment can help to reduce travel time and costs, improve remote monitoring of patients and potentially expand the number of patients who have access to ECD Care Centers in the future. Telehealth requires ongoing improvement in communication technology, government and payor buy-in, and greater reliance on local clinics for vital sign checks, labs and non-complex imaging studies. The ECD community is pleased that the use of telehealth appointments can ultimately allow more options for patients to receive the best medical care possible.

Thank you to Drs. Paul Hendrie, Eli Diamond and Juvianee Estrada-Veras for their work in making this survey possible for the ECD community.

THANKS TO YOU

We are grateful for your loyalty and dedication to those fighting Erdheim-Chester disease every single day. As we embark on another year together, we hope that you find new ways to connect and support one another.

SAVE THE DATES

- **National Rare Disease Day**
  February 28, 2020
- **ECD Awareness Week**
  September 13- 19, 2020
- **8th Annual Live Event**
  To be determined, stay tuned

Every day I am humbled by the courage and passion of the ECD community. I see patients, caregivers, medical professionals, researchers, volunteers, and donors who come together in ways that help not only individuals, but the community as a whole. Together we will continue the quest to obtain earlier diagnoses and treatments for all, as we search for a cure.

- ***Kathleen Brewer | President,*** +1.337.515.6987, Kathleen.Brewer@Erdheim-Chester.org

When I started as a staff member for the ECDGA in 2014, I had no idea that I was gaining a family, good friends, and daily doses of hope for humanity. But that is what I have found, and I am honored to be a part of the journey.

- ***Jessica Corkran | Executive Director,*** +1.337.502.8034, Jessica.Corkran@Erdheim-Chester.org

I am so excited to begin my journey with ECDGA. Since August, I have been able to meet so many wonderful people. This organization is truly family and it is encouraging to see how many are helped through its efforts. I look forward to meeting many more of you and participating in this worthwhile organization.

- ***Renita Page | New Staff Member***