



## What's happening in the community?

### ECD GLOBAL ALLIANCE

*Jan/Feb/Mar Newsletter*

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## RESEARCH NEWS

### Great Study Results on Cobimetinib

BRAF-negative patients have responded very well to cobimetinib (a MEK-inhibitor). The article below documents the results of the Memorial Sloan Kettering clinical trial. This is great news for the ECD community, be sure to share with your care team! Thank you to all the patients and doctors for their efforts towards this success!

[READ ARTICLE](#)

[TRIAL DETAILS](#)

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### Registry for Patients with Erdheim-Chester Disease - Still Recruiting

Memorial Sloan Kettering Cancer Center (MSK) serves as the trusted custodian of a centralized database for ECD patient information and tissue biopsies. The stored data will be available to investigators to allow ECD to be more readily studied. The registry is designed to answer questions and provide information for investigators to find effective treatments.

MSK has enrolled 120 participants so far. Thank you to those who have dedicated their information and time to this project. Please consider joining this project to help ECD research.

[LEARN MORE](#)

The FIRST paper using data from the above ECD Registry was just published entitled, "A scale for patient-reported symptom assessment for patients with Erdheim-Chester disease."

**KEYPOINTS:** (1) Collaboration among clinical experts, behavioral research ethodologists, and patients can yield valuable symptom-assessment tools. (2) Patients with ECD possess widely varied and unappreciated symptomatology.

Share this publication with your care team!

Thank you to our donors, this is your dollars at work! Thank you to the patients, we know your time and energy are precious and this could not be accomplished without you!

### SYMPTOMS PUBLICATION

## 3D culture of Erdheim-Chester disease tissues unveils histiocyte metabolism as a new therapeutic target

As a result of the 2015 ECDGA-funded research project, the first paper on the use of the bioreactor technology for ECD was published in the Annals of the Rheumatic Diseases. The work of the Milan Research Group has had a impactful role in the ECD community. Meet their team July 12, 2019 at the ECD event in Milan, Italy at IRCCS San Raffaele Scientific Institute Vita-Salute San Raffaele University! [Read more...](#)

## In Case You Missed It:

NEW Clinical Drug Trial in Houston, TX

[Dabrafenib and Trametinib in Treating Patients With Erdheim Chester Disease With BRAF V600 Mutations](#)

## Medical Awareness

ENDO Expo  
2019

New Orleans,  
LA

Press  
Release

At this annual endocrinology event, leaders in endocrine practice and hormone research share their latest breakthroughs that advance science and shape the future of patient care. The ECD Global Alliance participated in this exhibition to generate awareness of ECD, communicate the recent research landscape and new treatment opportunities for patients, and facilitate the growth of the ECD Global Alliance medical professional network among interested physicians and scientists.



Kathy Brewer with Dr. Caroline Davidge-Pitts (Mayo Clinic)



Dr. Fady Hannah-Shmouni (R) educating visitor about ECD.

Thank you to Dr. Fady Hannah-Shmouni, National Institutes of Health, and Dr. Carine Courtillot, University Hospitals Pitié Salpêtrière in Paris, for creating endocrinology-relevant content to provide. Also, thanks to Abigayle Brewer, our design volunteer, for the design of the informational materials provided at this event.



Thank you to the booth team for volunteering your time and providing expertise about ECD to physicians and interested booth visitors. Team (pictured L to R): Dr. Juvianee Estrada-Veras, ECDGA Board Member, Kathy Brewer, ECDGA President, and Nancy Feely, Johns Hopkins Medicine.

## Annual Patient Event in Italy

### What:

The purpose of the Patient and Family Gathering is to unite patients and family members, as well as increase their knowledge and understanding of Erdheim-Chester Disease.

### When:

Jul 11, 2019 @ 6 - 8 PM

Jul 12, 2019 @ 9 AM - 5PM

### Where:

IRCCS San Raffaele Scientific Institute in Milan, Italy

### Who:

This event is open to the entire ECD community of patients, caregivers, and ECD medical professionals, but we encourage international patients to take advantage of this rare location. Invite your doctor!

### How to register:

To register in English, follow this link: <https://conta.cc/2Ew6dQ1>. The early registration fee of \$100 will be extended to April 15!

**For assistance with the English form**, please respond to this email with your language preference to be put in contact with one of our volunteers.

**Order your event T-shirt by April 17 to ensure you receive it before your trip!**



ORDER NOW

## Rare Disease Day (RDD) Highlights

Carol Lundin delivered educational material to local health institutions and spoke with the care teams about ECD. She also interviewed with the local news to share her journey: [North Sea Resident Refuses To Let Rare Disease Stop Her From Enjoying Life](#)



Richard Mortz, a French member, visited his local rally for RDD. He met other advocates, shared about ECD and submitted a [poem](#). [RDD Video in French](#)



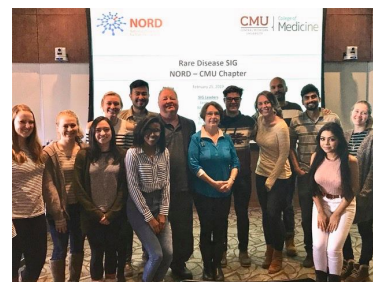
Shannon, daughter and caregiver to her mom Joanne, raised RDD/ECD awareness and provided education at her local YMCA.



Janet Bunge shared her amazing story of courage and strength with the ECDGA! [Read her story here.](#)



Joe Lofaro dedicated his time gaining support from the state of Tennessee and the city of Martin to officially recognize Erdheim-Chester Disease and the rare disease community, sharing his story for local news coverage, and organizing a fundraiser to benefit the ECDGA! [See his news interview here.](#)

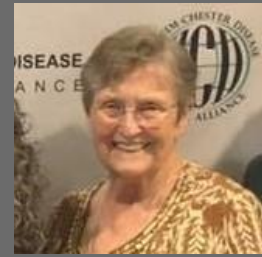


Central Michigan University invited John DeWitt and Amy Teare, an amazing ECD couple, to give a 45-minute presentation about ECD. They shared about the challenges of diagnosis and support with this rare disease, as well as a Q&A following. This couple was also responsible for the state of Michigan proclaiming Rare Disease Day.

Rare Disease Day State Proclamations (with an emphasis on ECD) were awarded in Tennessee, Louisiana, and Michigan.

### VOLUNTEER BIRTHDAY CORNER

Happy Birthday to one of the first of many ECDGA volunteers, Mrs. Bridget Gates! Your willingness to help the team in whatever is needed has been a tremendous contribution to the day-to-day productions and overall service of the ECDGA. We appreciate you!!



**THANK YOU** to each of our members for making the ECDGA possible!

*Together we will achieve great things for all affected by ECD worldwide!*

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