# ECD Global Alliance Organizational Status

*A nonprofit 501(c)(3) Organization*

September 2011

**Accomplishments:**

- Largest known group of ECD patients.
- Disseminates information via only website dedicated to sharing of ECD knowledge
- Provides mechanism for patients and family members to share experiences related to ECD and its treatments
- Supports research through financial support and patient/research communication.

**Inside this issue:**

## Work of the ECD Global Alliance

The mission of the ECD Global Alliance, a nonprofit donor-supported organization, is to support those affected by Erdheim-Chester Disease.

A tax-exempt status was granted the organization by the IRS in early 2010. Since that time the volunteers and members who make up the ECD Global Alliance have worked diligently to raise awareness of ECD, provide patient and family support, and promote research related to ECD.

The organization maintains a website dedicated to disseminating information regarding Erdheim-Chester Disease. The website is updated monthly with new information. In the first half of 2011 the website had over 4,000 unique visitors from almost 90 different countries.

Today the organization represents 100 patients/cases. It is with great sadness that we report, 19 of these patients have passed away. Patient representation comes from 16 countries. Although this may not seem like a large number of patients, it is the largest group of ECD patients known to exist. The existence of the group allows patients and family members to communicate with others who know what it is like to deal with the everyday challenges of living with ECD. In addition, it also allows researchers to have access to a patient population ready to help with their research.

Supporting research is crucial to the mission of the organization. This is accomplished by funding research projects and by acting as the communication conduit between researchers and patients.

There is much work to be done before we reach our intermediate goal of effective and rational ECD treatment options that have been scientifically proven. We will not stop until there is a cure.

## What Members Say about the ECD Global Alliance

The work of the ECD Global Alliance is possible because of generous personal donations. To all those who have donated money or volunteered their time to help, we say a BIG THANK YOU. We thought you might also want to hear directly what patients and their family members have to say about your support…

*I remain amazed by your attention and thank you for this.* - newly registered patient searching for information

*Your work is important and appreciated…it is good to know you are there.* - Wife of Patient

*I can see your efforts paying off, thank you.* - Patient

*Thanks for all you do!* - Patient

*The information on the ECD website is really helpful thank you so much.* - Daughter of patient

> *"The more money that is made available for research on rare diseases, the more researchers will be willing to devote their lives to that research."*
> *- Dr. Stephen Groft*
> *Director of the Office of Rare Diseases at the NIH*

## Organizational Challenges

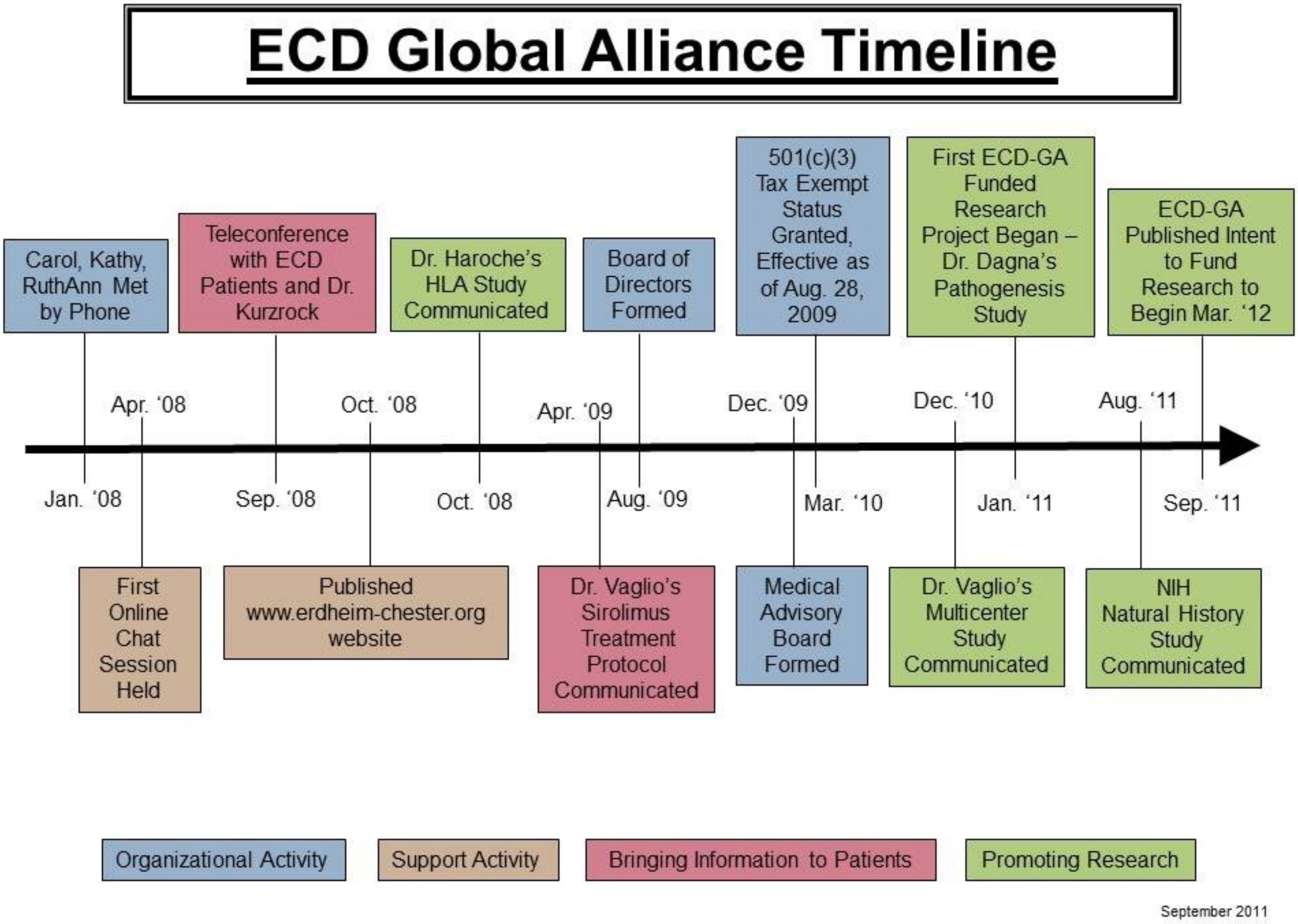
Bringing patients, resources and doctors together is at the core of the ECD Global Alliance mission. To do this, the organization must be visible. Patients must feel safe in registering with the organization and see value in staying connected to the organization. This requires an internet presence and continued communication with the patients to keep them engaged in the work of the organization.

Funds to support the organization and ECD research must be found. This requires constant communication with donors, fundraisers, and other funding bodies.

ECD-knowledgeable physicians must be aware of the organization and see the value of working with the organization. This requires being ever vigilant to learn and share valid data with physicians and patients.

Finally, effort must be made to raise awareness of the disease, especially within the medical system. This will help physicians to become interested in the disease and more patients will benefit from appropriate diagnoses and treatments. This is made extremely difficult as ECD is an 'unclaimed disease', meaning it is not treated by any one specific specialty.

You can imagine, this all takes time and resources. Thankfully there are some who unselfishly volunteer their hours and finances to make it all possible. We hope you might be one of those people. Thank YOU!



## Known ECD Studies at a Glance

| Title<br>Principal Investigator | Study Objective |
|---|---|
| Clinical and Basic Investigations into Erdheim-Chester Disease<br>Juvianee Estrada-Veras, M.D. , National Institutes of Health, Bethesda, MD USA | Natural history study designed to understand the disease, identify its cause, and classify it according to presentation and progression. The study does not include any modifications to patient treatment plans although follow on studies may address this. |
| Erdheim Chester Disease: Clinical Phenotype and Outcome. A Multicentre Survey<br>Augusto Vaglio, M.D. , Università degli Studi di Parma, Parma ITALY | Correlate the involvement of different organs with the response to therapy and the outcome. Expand the knowledge of ECD, allowing earlier diagnosis, better classification of the different disease forms, and improved prediction of the patient prognosis. |
| Developing a Comprehensive Genomic Database - Histiocytosis Research Laboratory<br>Carl E. Allen, M.D., Ph.D. and Kenneth McClain, M.D., Ph.D., Texas Children's Hospital, Houston, TX USA | Understand the basic causes of histiocytosis which hopefully will lead to more effective treatments. |
| HLA Study of ECD Patients<br>Julien Haroche, M.D., Ph.D., Hôpital Pitié-Salpêtrière, Paris FRANCE | Investigate the class I and class II HLA phenotypes of ECD patients to determine whether specific haplotypes are associated with an increased susceptibility to the disease or segregate with specific tissular involvement of ECD. |
| **Retrospective Review of Patients With ECD Treated With Interferon-alpha or imatinib mesylate**<br>Razelle Kurzrock, MD, FACP, M. D. Anderson Cancer Center, Houston, TX 77030 USA | Understand clinical characteristics, underlying biology, response to therapy and outcome of patients with Erdheim-Chester disease. |
| Pilot Study of Sirolimus with Prednisone as Treatment for ECD<br>Augusto Vaglio, M.D. , Università degli Studi di Parma, Parma ITALY | Assess whether (and if yes, to what extent) the combination of sirolimus and prednisone may prolong progression-free survival of ECD patients. |
| Hypoxia and inflammation in Erdheim-Chester disease microenvironment: insights on the pathogenesis and implications for therapy<br>Lorenzo Dagna, M.D., San Raffaele Scientific Institute, Milano, ITALY<br>(Funded by the ECD Global Alliance 2011 Grant) | Better define the pro-inflammatory microenvironment inside ECD lesions. To evaluate the possibility to interfere with the progression of ECD by means of specific inhibitors already available for human usage, in order to find new therapeutic options for the disease |
| A transcriptome analysis of the monocytes in Erdheim-Chester disease<br>Julien Haroche, M.D., Ph.D., Hôpital Pitié-Salpêtrière, Paris FRANCE | Perform a transcriptome analysis on a large international series of ECD patients in order to better understand the pathophysiology of the disease. |

## Financial Status of the Organization

The organization began 2011 with $83,500. Of this, $50,000 was used to fund Dr. Dagna's 2011 research to better define the pro-inflammatory microenvironment inside ECD lesions. Since the beginning of 2011, generous private donations made to the ECD Global Alliance total $142,450. Thanks to a strong force of committed volunteers, the expenses have been kept to an absolute minimum of $2,200.

The organization is currently planning to fund another $50,000 in research beginning in 2012. In addition, efforts are underway to hire the first employee of the ECD Global Alliance to help further develop the organization and ensure we are on a path that can be sustainable into the future.

The direction and accomplishments of the organization have been remarkable for a start up disease advocacy group that represents a small population of patients and families, yet it will never be enough until a cure is found.

The organization has been fortunate to have a generous benefactor who has provided financial support to allow the organization to build a foundation. However, the organization must begin to find a way to exist without this single source of support.

Every effort will be made to bring the right person onto the team to accomplish this mission. We hope that you will work with the organization to ensure that we will have the resources necessary to continue the search for better treatments and a cure. Together we can, and will, make a difference!

### Financial Status

Income

| | |
|---|---|
| 2010 Balance Forward | $83,500 |
| YTD 2011 Donations | $142,450 |

Costs

| | |
|---|---|
| YTD 2011 Expenses | $2,200 |
| 2011 Funded Grant | $50,000 |
| Planned 2012 Grant | $50,000 |

| | |
|---|---|
| Available Balance | $123,750 |

## Erdheim-Chester Disease Facts

*- Cause is unknown.*

*- Symptoms vary among patients. Some patients MAY have some of the following symptoms, or others:*

- *Pain in lower legs/knees*
- *General fatigue and feeling unwell*
- *Excessive thirst/urination*
- *Balance issues and/or slurred speech*
- *Kidney, heart, or vision issues*
- *Shortness of breath*

*- Patients normally wait years for a correct diagnosis.*

*- Diagnosis often requires multiple biopsies and scans.*

*- No standard, scientifically proven treatment.*

*- Unless successful treatment is found, organ failure can result.*

*- Few doctors have ever heard of the disorder. Fewer yet have ever seen or treated a case.*

*- Your involvement is needed to help find answers about this puzzling and debilitating disease.*

# ECD Global Alliance

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For more information -
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**Donations can be mailed to:**

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**Online Donations made at:**

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