ECD GLOBAL ALLIANCE

A DECADE OF IMPACT

ACCOMPLISHMENTS & A PLAN FOR PROGRESS

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Kathy Brewer, ECDGA Founder & President

ABOUT THE ECD GLOBAL ALLIANCE

For most people, an experience with Erdheim-Chester disease begins with a frustrating, frightening search for answers. This was certainly true for ECD Global Alliance founder Kathy Brewer and her late husband, Gary. At the time of his death, Gary had been ill for 20 years and very ill for three. He had symptoms of kidney disease and, in fact, underwent a kidney transplant. But it was not until his autopsy that Kathy learned Erdheim-Chester was the true cause.

This is the challenge with a very rare disease. When a condition afflicts only an estimated 1,000 people worldwide, few physicians have the familiarity required to diagnose it, particularly when it can manifest with symptoms ranging from bone pain and weight loss to problems with vision and balance. (Among many body systems, ECD can affect the pituitary and adrenal glands, the lungs, the membrane around the heart, the kidneys, and the brain.)

However, the ECD story is remarkably different today than it was in 2008, and the reason is the ECD Global Alliance. Started by patient caregivers who found each other through the internet, the Alliance has grown from six families to almost 700, and it has changed the outlook for ECD patients dramatically.

Today, most ECD patients can control the progression of the disease with one of several treatments now in use. Many have gone from facing a typical life expectancy of 3-5 years to managing symptoms as they carry on with their lives.

Established a network of Care Centers at leading medical institutions internationally, each with a cadre of specialists prepared to diagnose and treat ECD.

Launched a patient registry to build an archive of data on ECD and partnered with the NIH to recruit patients for a natural history study—key to building an understanding of the progression of the disease.

Created a support network of families and brought members of the international ECD community together for a series of annual conferences—the first opportunity for patients to meet each other and for many to talk with doctors specializing in ECD.

Helped make possible the work in gene therapy that led to FDA approval of a drug effective in treating ECD.

PATIENT PERSPECTIVE

Without the support of patients and families, this organization would not thrive or even exist. The gratitude of patients expressed for the ECD Global Alliance has kept our mission clear and steadfast.

> **"I NEVER FELT MORE ALONE IN MY LIFE. AM HAPPY TO HAVE ECDGA TO SHARE AND LEARN FROM OTHERS AND TO SOMETIMES READ ABOUT GOOD NEWS AND ABOUT OTHERS WHO CARE. IT HELPS TO KEEP MY SPIRIT UP. THANK U!"**
>
> - Anonymous Patient

Our medical community is more than a directory network.

A Miracle Baby

Alice, a young nurse in Norway, welcomed her first-born child into the world on September 27, 2017, when she was 38 years old. The remarkable part of the story is that Alice had been sick since 2005 and received an ECD diagnosis in 2011. Alice attended the first ECDGA annual event in 2013 using walking sticks and physical assistance from family members. Based on what she learned at that event, Alice was able to advocate with her physicians in Norway to begin targeted treatment. Through lots of hard work and therapy, Alice regained her independence and surprisingly she was able to give birth to a healthy baby girl, something she didn't know was possible!

A Ray of Hope Amid the Sorrow

In mid-2018, a young professional was diagnosed with ECD, almost simultaneously with his wife experiencing a miscarriage of their first child. It was a most difficult time as he struggled to accept the unexpected turns his life was facing. Now, thanks to his resolve and recent advancements in treatments, he and his wife have two healthy children and their family is thriving.

Regaining Control

Hugh, an attorney, and judge began to be short-winded and knew something was wrong. After undergoing open-heart surgery and then experiencing a heart attack, a single physician who knew about ECD made the diagnosis that saved his life. Hugh initially believed the diagnosis was a death sentence so he retired and got his affairs in order. Then he found the ECD Global Alliance and made a couple of trips to an ECD Care Center. As a result, treatment began that he calls “literally a miracle.” He now practices law again as part of his pro bono court work.

PLANS FOR PROGRESS

Despite the Alliance’s successes, significant challenges remain. Diagnoses of ECD can still take too long, and based on the view of medical professionals, it remains significantly underdiagnosed—meaning some patients never learn the true source of their symptoms. In addition, many do not have access to the treatment they need, and current therapies can entail serious side effects. To address these challenges, the ECD Global Alliance has developed a strategic plan to guide its progress over the next five years. In fact, it’s developed three separate plans, each targeting a key priority and ready for funding and implementation. All while expanding the reach and establishing sustainable resources.

Awareness & Education

Educate more medical personnel to dramatically improve the timeliness of ECD diagnoses

Patient Support

Empower and strengthen a community of informed and connected patients, caregivers, and physicians

Research Program

Enhance collaborative research with a patient-centered approach

Expand & Establish

Expand to begin serving the entire adult histio community

Establish long-term sustainability for the ECD Global Alliance in order to continue serving the community for decades to come

THE AWARENESS & EDUCATION PLAN

The Goal:

Shorten the time to diagnosis and ensure that no patient goes undiagnosed by increasing awareness of ECD among doctors. Data on the current time to diagnosis is not definitive, but one recent study cites a mean of 4.2 years from the onset of symptoms. We believe diagnosis within a year is a realistic goal. Achieving it will mean patients can start treatment before the onset of organ damage—critically important.

Action Steps:

- Produce a series of webinars and live online forums for medical professionals —building on the success of a pilot webinar already completed, addressing a line-up of specific treatment-related topics, and reaching physicians worldwide without the need for travel.

- Plan a presence at 3-4 medical specialty conferences annually—sharing information, meeting practitioners, and having physicians from ECD Care Centers make presentations, in order to bring ECD to the attention of doctors in the full range of specialties that may encounter the disease.

- Plan ECD-focused grand rounds events—at 5-6 academic medical centers each year, reaching doctors at locations across the U.S. and internationally through live patient case presentations by visiting experts from ECD Care Centers.

- Ensure ECD is accurately featured in leading digital diagnostic systems—interactive tools used by physicians struggling with difficult-to-diagnose cases.

The Investment:

These low-cost/high-impact initiatives mainly require staff time, travel expenses for staff and physicians, and honoraria for speakers. To fulfill these initiatives annually over the next three years, the estimated budget is $225,000-$250,000.

> **“The Global Alliance is exemplary—really, really advocating, reaching out to doctors, getting people out of their siloes and connected.”**
>
> **- An ECD Care Center physician**

MEDICAL
PROFESSIONAL
REACH

1,357

THE PATIENT SUPPORT PLAN

The Goal:

Ensure that once diagnosed, all patients have access to answers, a support network, and treatment, whenever possible through an ECD Care Center. Ensure that none face the disease frustrated and alone. Just as we hope to reduce the time to diagnosis for ECD, we plan to cut the time it takes for families to receive needed information and connect to Care Centers.

Action Steps:

- Expand Alliance-sponsored patient and caregiver networking—which brings ECD families from around the world together virtually and helps link new and experienced patients in mentoring relationships.

·Introduce patient navigation services—with the addition of a Patient Navigator to the Alliance team, a professional who will help ensure access to accurate answers and guide families in accessing treatment and identifying medical and financial resources.

·Launch patient and family webinars—sharing key information and providing affirming support for patients, their caregivers, and those grieving a loss.

The Investment:

In addition to increased staff time and webinar production costs, the key expense will be a new salary line for the Patient Navigator. We will begin by hiring and training one part-time support assistant and then expand to one full-time or two part-time positions. To fulfill these roles successfully over the next three years, the estimated budget is $225,000-$250,000.

> **“Before ECDGA, there was no central place to treat the disease, or get information, no organization. You would meet a doctor offering to treat you and find out they had never treated one ECD patient.”**
>
> **- Caregiver for an ECD Patient**

FAMILIES SERVED
748

THE RESEARCH PLAN

The Goal:

Double the number of researchers focusing on ECD, help promote coordination among these researchers to better align their efforts with key priorities, and promote new multi-center studies so that patients can participate without traveling to distant sites. It is impossible to specify the outcomes this work will yield, but progress over the past decade indicate it is not unreasonable to expect advances in understanding the causes and basic science of ECD, tracing its impacts on different organ systems, and advancing toward new treatments and diagnostics.

Action Steps:

- Increase Alliance investment in pilot studies—with yearly seed grants that will bring new researchers into the field and help them take their work to the proof-of-concept stage and quality for third-party funding. (The Alliance has awarded pilot grants and found these have led to grants from other sources, but we have not had the budget to make these awards regularly.)

- Launch new targeted research grants—that will provide multi-year support for projects in areas of focus identified by the ECD medical and patient communities as top priorities.

- Restart and expand ECDGA's annual medical symposium (paused due to COVID)—an event that has proven its impact in catalyzing research collaboration and accelerating the pace of progress.

The Investment:

With a goal of dedicating $50,000 to pilot studies and a minimum of $100,000 to fund grant proposals for targeted research each year over the next three years, the estimated budget is $720,000-$750,000.

> **"There is so much that has been accomplished in the last decade from a science perspective and a human perspective, and very little of that does not have the Alliance's fingerprints on it."**
>
> **- An ECD researcher**

RESEARCH
FUNDING
GRANTED

>750k

Hosting the 2019 Annual Patient Gathering in Italy allowed patients from nine surrounding countries to attend.

COUNTRIES SUPPORTED

65

What about the family?

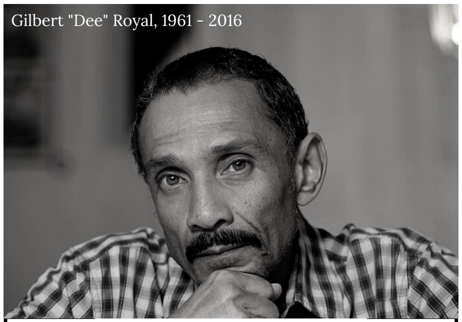
“My father just recently got diagnosed with Erdheim Chester disease and passed away because it was misdiagnosed as we know it’s so rare. My 3 siblings and I would like to know more about this disease and if we should get tested for it. Is it hereditary?”

A Daughter's Loss

“[ECD] Took my mum 6 years ago - to see the progress made in terms of diagnosis & treatment is fabulous. We had to fight (& fail) for treatment in the UK 6 years ago and diagnosis then was purely by chance. We definitely knew more than the doctors at the time."

Grateful & Hope-Filled

“When my Dad underwent treatment for Erdheim-Chester Disease the doctors knew less than we did about what it was or how to help and insurance companies refused coverage for treatments as anything was considered “experimental” at the time. Thanks to amazing advocates like Erdheim-Chester Disease Global Alliance so much has changed in the last few years; ECD has a name, doctors are more informed, families have more support, and insurance companies finally acknowledge and pay for treatments previously denied without the need for appeals!”



Gilbert "Dee" Royal, 1961 - 2016

> “
>
> **HAD DEE BEEN DIAGNOSED, IT WOULD’VE NOT ONLY HELPED HIM TO UNDERSTAND WHAT WAS GOING ON INSIDE HIS BODY, BUT GIVEN HIM THE CHANCE TO FIGHT.**

A CALL TO ACTION

The story of the ECD Global Alliance is inspiring on multiple levels. It is a story of lives saved and suffering prevented. It is a story of personal grief and loss transformed into something wonderfully positive. And, it is a case study of a high-impact organization, with the potential to emerge as a model for peers.

In its remarkable first decade, the ECD Global Alliance has proven its ability to set and meet ambitious goals, and it’s not stopping now. With your support, we will increase awareness of ECD and speed diagnosis, dramatically improve support for patients, and accelerate research. This is an opportunity to make a philanthropic investment offering an exceptional return—and this is the time to do so. Please join the ECD Global Alliance in advancing our vitally important work.