



**EMILY'S
ENTOURAGE**

IMPACT REPORT

2019 FISCAL YEAR

July 22, 2020

To our incredible Entourage,

In 2011, Emily's Entourage (EE) was born from a collision of hope and urgency. We were seeing unprecedented progress towards transformative new treatments for 90% of the Cystic Fibrosis (CF) population with the most common CF genetic mutation, and we quickly realized that progress for the remaining 10%, including those with nonsense mutations, was severely lagging. Not only were we concerned by the limited number of projects in the pipeline for nonsense mutations, but also by the challenges to actually get new projects in the queue, the lack of focus in the area, and the slow pace of therapeutic development.

Since its inception in 2011, we have built a "foundation of the future," shepherding in a new era of medical research and drug development. Led by patients, families, and communities with unique expertise and a vested interest in fast progress, EE takes a disruptive, entrepreneurial approach to identifying, advancing, and financing high-risk, high-reward initiatives. We have laid the groundwork for rapid drug discovery and development and cultivated a community of the world's top CF scientists and biopharmaceutical partners. We believe deeply in harnessing collaboration between patients and families, academia, and industry to progress therapeutics through the pipeline and bring life-saving new treatments to patients fast.

We are proud and grateful beyond belief to share the work of EE through December 31, 2019 in our inaugural impact report. Since its founding, EE has funded over \$4 million in research through our *Catalyst for the Cure* campaign, strategic investments, pilot grants, and independent grants. Our progress is only possible because of the belief, passion, and generous support of our endlessly committed Entourage.

As we take a moment to reflect on our achievements through the 2019 fiscal year and express our heartfelt appreciation for all those involved, our sights are firmly set on the future. Our early concerns about the final 10% being left behind became a reality when, on October 21, 2019, the revolutionary triple combination therapy drug, TRIKAFTA, received FDA approval for 90% of the CF population. The work of EE is more urgent now than ever before as the outlying 10% still has nothing. We continue to live with the same killer disease that CF has always been as we watch our CF compatriots being airlifted to safety, our hearts bursting with pure happiness for them, but desperate too, desperate to join them.

From the start, our vision at EE has been to create a future where 100% of the CF community can live long, healthy lives—no mutations or people left behind. We remain deeply committed to making that vision a reality in record speed. We are only able to do so with the continued support and partnership from our mighty Entourage. From the depths of our heart, thank you.

With hope, energy, and profound gratitude,

Emily Kramer-Golinkoff
Co-Founder of Emily's Entourage



“ Our vision at EE has been to create a future where 100% of the CF community can live long, healthy lives—no mutations or people left behind. ”

FUNDING TOMORROW'S BREAKTHROUGHS

We are funding the breakthroughs of tomorrow by generating new and innovative ideas that dive head first into high-risk, high-reward research areas that have the potential to reach patients in the near to mid-term. To date, EE has provided over \$4 million dollars to fund 17 research projects across the globe. The projects that we fund advance our two-fold scientific strategy. We seek breakthroughs—and cures—at the genetic level, including gene therapy, RNA editing, and readthrough or restoration of the CFTR protein. We also pursue approaches that “buy time,” serving as a bridge therapy to sustain people with CF until transformative advances are developed.

Importantly, while the goal of EE is to develop breakthroughs for individuals with nonsense mutations of CF, many of our funded projects have the potential to be scaled to far larger populations. A number of EE's funded research projects are mutation-agnostic, meaning they could benefit the whole CF community regardless of one's mutation. In addition, while nonsense mutations are relatively rare in the CF population, they are the cause of roughly 12% of all genetic diseases, which means some of the therapeutic approaches EE is advancing have the potential to benefit roughly 30 million people with other genetic diseases caused by nonsense mutations, including certain types of muscular dystrophy, inherited blood disorders, and cancer.

RESEARCH HIGHLIGHTS

17

CATALYST FOR THE CURE
GRANTS AWARDED

5

MUTATION-AGNOSTIC
PROJECTS FUNDED

\$6.25

MILLION IN FOLLOW-ON
FUNDING SECURED

1

CF GENE THERAPY
COMPANY LAUNCHED



In our third and fourth rounds of grant funding, EE funded research teams at Yale University, Georgia Tech, the University of Rochester, and Case Western Reserve University. These projects focus on:

- Developing bacteriophage viruses for the treatment of CF-associated, antibiotic-resistant MRSA infections
- Measuring the effectiveness of CF therapeutics in development to speed drug discovery
- Using nanoparticles to deliver nucleic acids to the lungs, facilitating the development of various therapeutic approaches lacking a mechanism for delivery
- Developing murine models with the W1282X mutation and distributing to laboratories to test new therapeutic strategies

Through our grant program and other strategic investments, we have supported a diversified pipeline that spans the therapeutic development spectrum.

RESEARCH AND DEVELOPMENT PIPELINE


RESOURCE
DEVELOPMENT

2
PROJECTS

→ 
TRANSLATIONAL
RESEARCH

11
PROJECTS

→ 
CLINICAL
RESEARCH

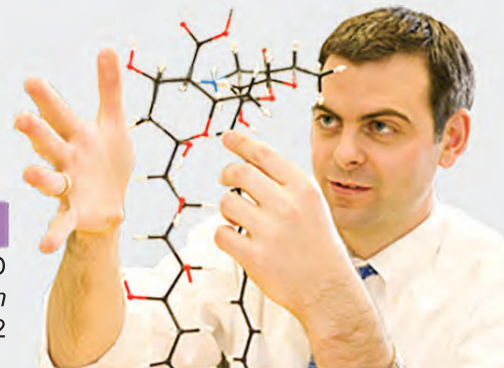
4
PROJECTS



“Emily’s Entourage is so much more than a funding mechanism—it’s a revolution in the making, the manifestation of citizen-science, a moon-mission for our time, and a tremendously inspiring and powerful engine for disruptive innovation.”

GRANT RECIPIENT

Martin Burke, MD, PhD
University of Illinois at Urbana-Champaign
Catalyst for the Cure Campaign, Round 2



FOSTERING A RESEARCH NETWORK DEVOTED TO NONSENSE MUTATIONS

For decades, nonsense mutations of CF took a backseat to research on the most common CF mutation, F508del. By building academic and industry collaborations, we have raised the profile of nonsense mutations of CF among researchers and the biopharmaceutical industry, staunchly advocating for prioritization given the urgent unmet need and untapped potential.

Raising the profile of nonsense mutations of CF has required laying a strong groundwork by investing in resources, tools, and infrastructure that were previously lacking in the field and slowing drug discovery and development. EE has collected cells and funded projects to develop validated cell and animal model systems and made them available to investigators and companies around the world. Additionally, by building a patient registry of individuals with nonsense mutations of CF, EE has developed the ability to connect individuals with vital research opportunities and partner with companies to expedite clinical trial recruitment and enrollment.

While tools and resources are critical to progress, ultimately the power of the network lies in its people and relationships. Through our 2018 scientific symposium, *Therapeutic Development for Nonsense Mutations: The Final Frontier of CF*, and annual reception at the North American Cystic Fibrosis Conference (NACFC), we focus on bringing together researchers and companies, developing a growing research network, and championing rapid development of therapies for people with nonsense mutations of CF. We also continue to cultivate critical connections and conversations through speaking engagements at top biotech and pharmaceutical companies and conferences throughout the year.

In developing our research program, we have engaged scientific leaders from within the CF field as well as encouraged top researchers and companies from outside the CF space to apply promising ideas to nonsense mutations of CF. This expanding network of scientists and industry leaders can then leverage our funding opportunities and resource repository to rapidly advance their research projects and contribute to the field. By combining the brain power of these leaders, removing operational barriers, and providing funding and easily accessible resources, we reduce the hurdles to progress and can speedily catalyze transformative changes for those living with nonsense mutations of CF.



CATALYZING RESEARCH AND COLLABORATION

340

INDIVIDUALS ON
NONSENSE MUTATION
PATIENT REGISTRY

16

INSTITUTIONS WITH
CELLS DONATED AND
FACILITATED BY EE

55

COMPANY MEETINGS,
AND SPEAKING
ENGAGEMENTS IN 2019

90+

CF RESEARCHERS
AND FAMILIES AT EE'S
NACFC RECEPTION

CREATING A FOUNDATION OF THE FUTURE

What differentiates EE from traditional organizations is our laser focus on our end goal—breakthroughs and a cure for nonsense mutations of Cystic Fibrosis—and our agile, entrepreneurial approach to getting there as fast as possible. We view our role in the biomedical landscape as not only responding to good ideas that scientists and companies propose, but actually generating the roadmap, facilitating exchanges of information through connections, and creating innovative pathways to expeditiously advance promising opportunities. The shift in the role of a patient advocacy organization has required the development of new business models and funding mechanisms for biomedical research, that position EE as a primary driver of drug development, including venture philanthropy.

A prime example of the power of EE's strategic and entrepreneurial venture philanthropy investments is the success of Spirovent Sciences (formerly Talee Bio), a biopharmaceutical company focused on gene therapy for CF. EE conceived of the idea, gathered the scientific community to explore promising therapeutic approaches, contracted Militia Hill Ventures to form and manage the company, and provided the seed funding to officially launch Spirovent in 2016 and develop two mutation-agnostic, potentially curative gene therapy candidates for CF. In early 2019, the CF Foundation committed \$4.5 million toward the development of Spirovent's gene therapy candidates. In late 2019, Spirovent was acquired by Sumitomo Dainippon Pharma of Japan with commitment to continue to develop the two gene therapy programs, a testament to their scientific promise.

Our focus on rapidly advancing promising opportunities extends to our grant program as well. By providing early funding to de-risk innovative ideas that traditional funders would not typically support, our grant recipients generate key data that allow them to secure substantial follow-on grants from major funding outlets, thereby speeding potential time to market. As of December 2019, our funded researchers have received over \$6.25 million in follow-on funding. Our goal is to take chances on high-risk, high-reward projects and remove the operational roadblocks that have previously delayed progress so that the only thing standing between us and breakthroughs is science.



“Emily’s Entourage has helped Vertex think about how to help all the patients with CF beyond the 90%. They helped to motivate us in a way that is very deep. It’s beyond the science; it is on a personal level.”

CORPORATE PARTNER

Paul Negulescu, PhD
Senior Vice President, Vertex Pharmaceuticals

VENTURE PHILANTHROPY IN ACTION

Fund Novel,
High-Risk
Research

Develop
Strong Scientific
Expertise

Bridge
Institutions
and Ideas

Build a
Patient
Community

IN 2016, EE PROVIDED SEED FUNDING
AND CONNECTIONS TO LAUNCH



WHICH HAS SINCE BEEN ACQUIRED BY
SUMITOMO DAINIPPON PHARMA OF JAPAN

“In CF, there’s still a very important part of the population that isn’t being treated. Emily’s Entourage is thinking about innovative, new solutions and putting them in the mind of scientists and biopharma.”

STRATEGIC INVESTMENT PARTNER

Joan Lau, PhD, MBA
CEO, Spirovant Sciences



MAKING NOISE FOR THE FORGOTTEN 10%

There has been trailblazing progress in the world of CF, but that progress stops short of helping 10% of CF patients with rare mutations, including nonsense mutations. Racing against time, EE has become a thought leader in the CF, rare disease, and patient-led research space, with invitations to speak at prestigious conferences and regular coverage in major media outlets.

From coast to coast, we advocate the development of therapies for the outlying 10% of the CF community. Some of our speaking engagements and meetings in 2019 include:

- Facebook’s Health Expert Stakeholder Meeting (Menlo Park, CA)
- PRA Health Sciences Rare Disease Day (Blue Bell, PA)
- Translate Bio’s Rare Disease Day and CF Awareness Month (Lexington, MA)
- Health Datapalooza (Washington, DC)
- University of Pennsylvania’s Master of Bioethics Graduation Keynote (Philadelphia, PA)
- BIO International Convention (Philadelphia, PA)

Our work has also been featured in a broad range of media outlets including:

- *The New York Times*
- *The Skimm*, the mega popular daily online newsletter
- PharmExec.com, an industry outlet for pharmaceutical and biopharmaceutical executives
- *Rare Revolution*, a digital magazine dedicated to rare diseases
- *Science Magazine*, a peer reviewed academic journal of the American Association for the Advancement of Science
- *STAT*, an online health and life-sciences publication

In June 2019, we were humbled to be chosen as a featured partner for the Chan Zuckerberg Initiative’s *Rare As One Project*, which develops a shared infrastructure to lower the barriers for patient-led research and facilitates cross-community learnings. The inclusion of EE in this project is a testament to our transformative work in the rare disease space and groundbreaking model to move research forward faster and more efficiently.

GETTING THE WORD OUT



15,350+

FOLLOWERS ON
SOCIAL MEDIA

26

MENTIONS IN
PRESS IN 2019

The New York Times
Science the Skimm’ STAT



FUNDRAISING HIGHLIGHTS IN 2019

\$1M+

RAISED BY GALA
AND ANNUAL DRIVE

193%

INCREASE IN #34FOR34
BIRTHDAY CAMPAIGN

960+

TOTAL TICKETS SOLD
FOR EENY AND GALA

3,015

UNIQUE DONORS
IN FISCAL YEAR

FUELING AN ENTOURAGE THAT NEVER RESTS

From intimate parlor meetings and marathon teams to invigorating galas, the ferocious enthusiasm of this Entourage is truly breathtaking and humbling. The 8th Annual *Evening with Emily's Entourage Gala* in December 2019 raised a record-breaking \$840,000, featuring powerful speeches, an illuminating performance, and the debut of the 2020 campaign video. The 4th annual EENY in May 2019, co-chaired by the founders of Eesho, a community-minded events company, kicked off CF awareness month with nearly 500 attendees and an electrifying immersive art experience, raising \$235,000.

Finally, 58 runners laced up as part of Team EE in the 2019 Philadelphia Marathon and 2019 Blue Cross Broad Street Run. This represented a 52% growth for Team EE in 2019.

“Emily’s Entourage gives us unbridled hope that the intersection of research and patient advocacy can transform the drug development landscape not just for CF but also for other rare diseases. By doing things in ways that have not been done before, Emily’s Entourage is creating meaningful collaborations to try to find breakthroughs and cures more quickly.”

LEADERSHIP TEAM MEMBER

Robert Adelson
Managing Partner, Osage Partners



2019-2020 CAMPAIGN VIDEO

Production Company
Storyed Studios

Art Direction and Illustration
Andrea Amanda

Director and Producer
Joanna Rudnick

Contributing Producer
Lindsey Rosin Passman

Animation and Editing
Adam Kennedy, Resolution
Workshop

Music Courtesy of
Score a Score





PARTNER ORGANIZATIONS

EE is proud to partner with visionary organizations making transformative change in health care.

CORPORATE PARTNERS



PATIENT ADVOCACY PARTNERS



NO ONE SHOULD BE LEFT BEHIND

EMILY'S ENTOURAGE LEADERSHIP

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For more information or to get involved,
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