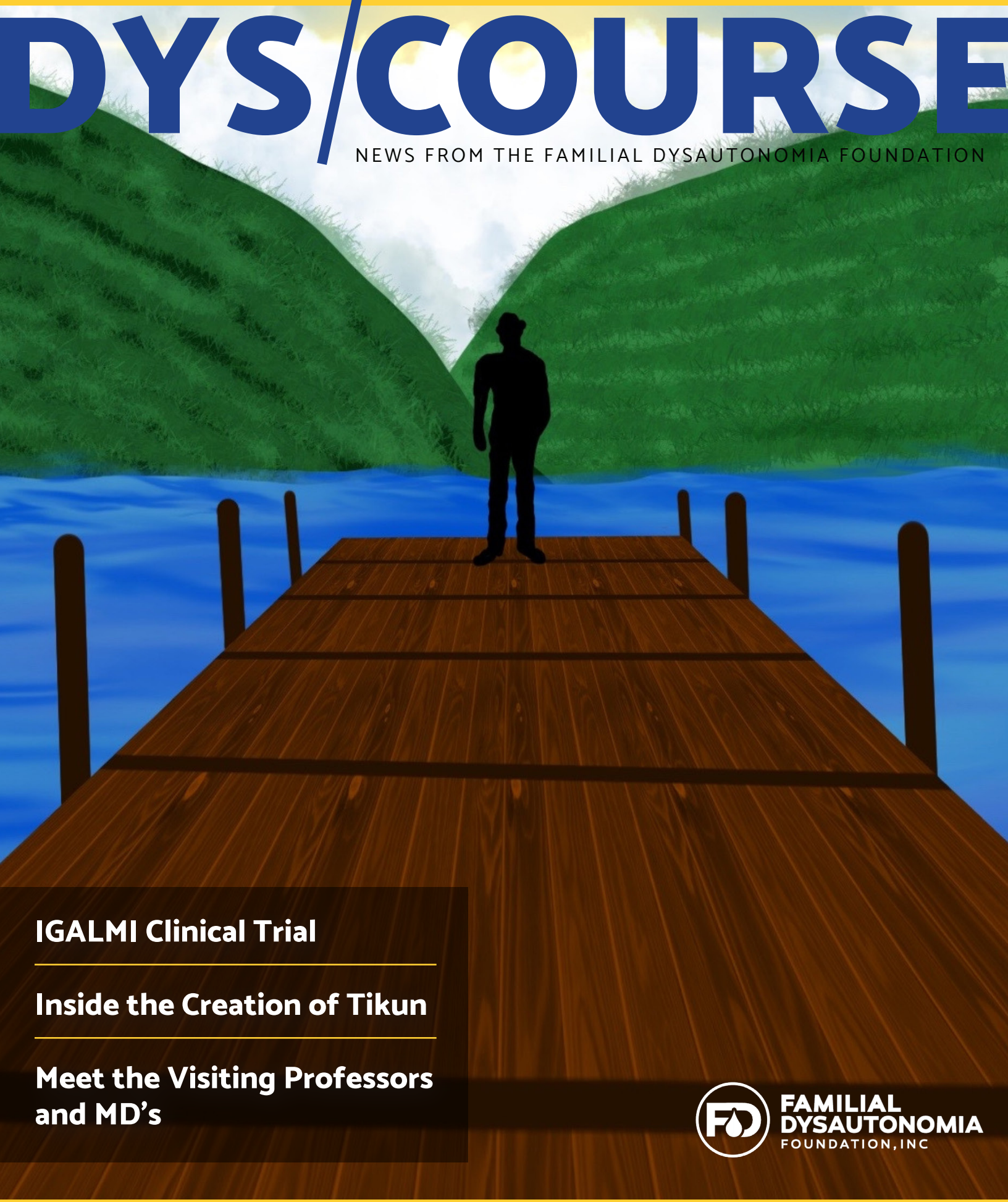
FALL 2024

DYS/COURSE

NEWS FROM THE FAMILIAL DYSAUTONOMIA FOUNDATION



IGALMI Clinical Trial

Inside the Creation of Tikun

Meet the Visiting Professors and MD's

FAMILIAL DYSAUTONOMIA FOUNDATION, INC

The FD newsletter was endowed with a gift in memory of Elaine Jamie Lipson, whose love of life will remain forever in our hearts.

FD Foundation Board of Directors

Faye Ginsburg, President
Steven S. Fass, Secretary
Allan Cohen, Treasurer
Ed Baranoff, VP
Jeffrey Goldberger, VP
Steven Kietz, VP
Laurent Landau, VP
Lisa Newman, VP
Paul Wexler, VP
Gerald Adler, Director
Daniel Landau, Director
Gregg Meyers, Director
Rebecca Sernovitz, Director
Jennifer Sonenshein, Director
Brian E. Stillman, Director
Howard Weiser, Director

Staff

Lanie Etkind, Executive Director
Albulena Prelvukaj, Director of Fundraising and Communications
Julia Winer, Development Operations Associate

On the Cover

"Man on Dock" by **Sam Landau**. This submission was awarded **first place** in the **2024 FD Day Art Competition**. In describing his work, Sam said, "I was inspired to draw this on the iPad when I saw a similar image of a boy standing on a dock in the rain.

If you have an image you'd like to share for a future issue of DYS/COURSE, please send it to info@famdys.org

DYS/COURSE Fall 2024

Written by: **Lisa Denburg**, **Alejandra Gonzalez-Duarte**, **Lanie Etkind** and **Margarita Grobocopatel**

Edited by: **Faye Ginsburg** and **Lanie Etkind**

Designed by: **Albulena Prelvukaj**

MESSAGE FROM THE PRESIDENT AND EXECUTIVE DIRECTOR


Faye Ginsburg


Lanie Etkind

Dear Families, Friends, and Supporters:


Horacio Kaufmann

Photo by Rick Guidotti

You are likely reading this newsletter right around Thanksgiving, a time of year when we count our blessings. This seems like an appropriate opportunity for us to acknowledge and recognize **Horacio Kaufmann, MD** for 18 "chai" years of leading the Dysautonomia Center, first as Co-Director and then as Director. When Dr. Kaufmann joined the Center as Co-Director in 2007, working alongside **Dr. Felicia Axelrod**, we knew we were incredibly fortunate to be able to recruit a top neurologist and a world-renowned expert in autonomic disorders to join our community. Since becoming Director of the Center upon Dr. Axelrod's retirement in 2015, Dr. Kaufmann has ushered in a new era in FD care and research that has had a remarkably positive impact on those living with FD. Some highlights include the following accomplishments:

- Through **expert medical care** provided under Dr. Kaufmann's supervision, patients who once had a 50% chance of reaching age 5, now have a 75% chance of living to 40 years old—and beyond.
- Dr. Kaufmann has both mentored and gained invaluable knowledge from the brilliant young physicians who have trained at the Dysautonomia Center over the years through the **Visiting Professors Program** he established and oversees.
- Dr. Kaufmann was instrumental in developing and implementing the **Telemedicine** initiative, which has helped bring the essential FD medical care provided by the Center virtually to patients who are unable to travel in person to NYC.
- After years of Dr. Kaufmann (and many others) working behind the scenes, this summer not one, but TWO transformative **clinical studies** were launched at the Dysautonomia Center. 1) Clinical research on IGALMI (a new form of Precedex) promises to provide a way to treat FD crisis successfully at home; and 2) the launch of a disease-modifying genetic treatment that could slow progression of FD, carried out with the support of the n-Lorem foundation. Preliminary findings are extremely encouraging with both these studies. We look forward to sharing outcomes with the community as soon as they are available.
- Like Dr. Axelrod before him, Dr. Kaufmann has had the forethought to prepare the next generation of FD clinicians by adding the wonderful and talented **Dr. Alejandra Gonzalez-Duarte** to the Center team.

We invite you to join us in paying tribute to Dr. Kaufmann for 18 "chai" years of extraordinary service to our community in a special section in the 2025 FD Journal. Watch for more information in the Journal brochure coming to you by mail and email in early December.

Sincerely,

Faye Ginsburg

Lanie Etkind

INSIDE THE CREATION OF TIKUN

Many thanks to **Adam Sachs** who recently led the creation of a Public Benefit Corporation (PBC) called **Tikun Therapeutics** to aggressively drive drug development for the FD community. The proud father of 30-year-old Justin who has FD, Adam received the **2024 FD Champion Award** during FD Day this year.

With a 30-year history working in the biotechnology and pharmaceutical industries, Adam is now consulting, giving him the flexibility and the opportunity he'd been looking for to help the FD community. He is a long-time member of the Dysautonomia Foundation's Scientific Advisory Board (SAB) and is now working closely with other leaders of Tikun (which means "to restore or repair" in Hebrew). "We are so fortunate to have such talented and dedicated researchers on board with us," he says.

Tikun
"to restore or repair"
in Hebrew

Since Tikun is a PBC, a wholly owned subsidiary of the Dysautonomia Foundation, "we now have the opportunity to apply for grants and seek investments that are simply unavailable to non-profits, opening up new potential funding paths for us," explains Adam. As a small company, he plans to apply laser focus and assemble scientific, operational and regulatory experts to help gain approval for the three promising drugs that the FD community has been nurturing for years. He adds, "I left the last SAB meeting very optimistic about these drugs and where they were in their development, but it was clear that if we kept trying to raise funding through the currently available channels, it would be extremely challenging."

There are presently three initiatives underway including gene therapy, a small molecule and an ASO (antisense oligonucleotide). All three act to raise the levels of the fully intact ELP1 protein (the protein which, when truncated, leads to the multitude of FD symptoms) and will require multi-million-dollar investments to bring to fruition. Says Adam, "We've seen compelling safety and efficacy data in animal models. We now need funds for ongoing drug development studies and subsequent human clinical trials to get us across the finish line for final drug approval." Adam hopes that securing investments from new sources will drive these programs to a happy conclusion where these drugs will be available to the FD community at the lowest possible cost.

Justin and **Adam Sachs**

Over the past six months, Tikun has been focused on applying to the FDA for Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) designations for two of the three drugs. "Gaining these designations from the FDA provides invaluable benefits as we pursue our drug development. Orphan drug designation is particularly important because we will receive additional market exclusivity upon approval, gain tax credits for clinical trial costs, and it allows our company to bring the drugs to market more efficiently." The exciting news is that Tikun recently learned that it received ODD and RPD designation approvals for both the gene therapy and small molecule drugs. They are also working to secure the necessary licenses and sub-licenses from both industrial and academic partners to ensure full access to any approved drugs for the FD community.

"The hope with these drugs is to reduce and better manage FD symptoms and slow disease progression. We are all working so diligently on this because we want to help our kids. I feel so fortunate that I'm now in a position to lead Tikun. I've been looking for such an opportunity to help the FD community as we move forward with a great sense of purpose and urgency."

Observes **Frances Lefcort**, Co-Chair of the Foundation's SAB, "*We are grateful to our colleague Adam Sachs, a critical partner and leader within the SAB, for revitalizing and promoting the research efforts of the FD community. Recognizing the impact and potential for the promising research advances being pioneered by our scientists and clinicians, Adam has focused and directed our efforts into establishing this business framework for developing our therapeutics. We are hopeful that Tikun will lead us into the promised land for FD research, enabling us to realize our goal of translating FD treatments to the clinic and facilitating their accessibility to all those in the FD community.*"

TIKUN THERAPEUTICS

For more information visit tikuntx.com

FD DAY

SUNDAY, SEPTEMBER 15, 2024

The FD Foundation, together with the NYU Dysautonomia Center, hosted FD Day on Sunday, September 15. This was the Foundation's 39th Annual FD Day, our fifth year held virtually, and the first time FD Day took place in the fall. Here are some highlights:

Speakers and topics during the Clinical Session included:

- **Horacio Kaufmann, MD**, speaking about ASO disease-modifying treatment and the current n-lorem clinical study;
- **Alejandra Gonzalez-Duarte, MD**, discussing clinical trials and clinical care;
- **Mikhail Kazachkov, MD**, reviewing lung care in FD;
- **Lily Armstrong, LPC**, discussing emotional well-being and the Stevie Schwartzberg Mental Health Counseling Program; and
- Brief talks about FD around the world by international physicians including **Maru Briseno, MD** (Mexico); **Bat El Bar Aluma, MD** (Israel); **Valeria Iodice, MD** (UK); and **Margarita Grobocopatel, MD** (Argentina).

Speakers and topics during the Scientific Session included:

- **Dr. Frances Lefcort**, discussing the latest FD eye research;
- **Dr. Anil Chekuri**, reporting updates on gene therapy; and
- **Dr. Sue Slaugenhaupt**, providing an overview of her small molecule work.

Congratulations to our 2024 DYStinguished Award Winners: **Emily Koltai, Adam Margolis** and **Pyper Stillman**.

We salute all those who participated in the 2024 Art Competition, and especially the award winners: **Keshi Taryan-Kigel** (3rd place); **Gabi Jassie** and **Eli Berkovitz** (tied for 2nd place); and **Sam Landau** (1st place).

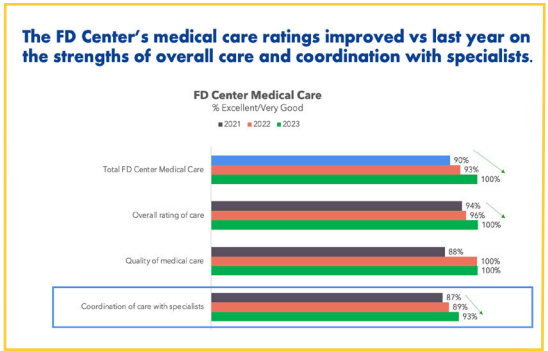
The Foundation presented the 2024 FD Champion Award to **Adam Sachs**. (See also, article about Adam's work forming Tikun, a public benefit corp. subsidiary of the FD Foundation, on page 2 of this issue of DYS/Course).

Several hundred members of our worldwide community joined our virtual FD Day live on September 15, but if you missed it, or wish to re-watch, the full program, as well as segmented recordings, are available for viewing at www.famdys.org/fdday24, or by scanning the QR code.

Thank you to our FD Day Sponsors

THIRD ANNUAL PATIENT AND FAMILY SATISFACTION SURVEY SHOWS FAVORABLE RESULTS FOR DYSAUTONOMIA CENTER

Many thanks to all the families who participated in the third annual patient and family satisfaction survey of the Dysautonomia Center that was conducted this past summer. We continued to receive favorable results regarding the Center after assessing the responsiveness, quality, and effectiveness of the services provided. Talking confidentially to more than 50 families across the country and in Canada, we discovered what's working, what needs improvement and listened to concerns and suggestions for the future. We hope we fostered additional connections and ensured that families felt heard and understood. Special thanks to **Barrie Rappaport**, who is not only an FD mom, but also a nationally known market research expert who organizes our annual market research study.



FD FOUNDATION NOW OFFERS MANY DIFFERENT SUPPORT GROUPS – WHICH ONE IS RIGHT FOR YOU?

FD PARENTS AND CAREGIVERS

Meets monthly on Wednesday evening 7PM Eastern. All are welcome. This group provides a safe space for parents and caregivers to speak candidly about their experiences loving and caring for a child with FD.

BEREAVED PARENTS

This group formed recently to support parents navigating those incredibly difficult first months—and years—following the loss of a child. All are welcome. Currently, meeting every two weeks on Wednesday evening at 7PM Eastern. Participating parents have shared that, although painful, it has been very beneficial for them to stay connected with the FD community that has been so vital to their lives and the lives of their children.

FD SIBLINGS

This group is currently in formation. Meeting day/time is to be determined. No family member is unaffected by FD. Its impact is felt big and small within the family unit. As part of the FD Foundation's NextGen initiative, the forthcoming FD Sibling group will offer a warm and compassionate forum for siblings to share their mutual experiences, work through common stressors, and hopefully create lasting bonds.

BEREAVED SIBLINGS

This group is currently in formation. Meeting day/time is to be determined. Join others who have lost a sibling/cousin/close friend to FD. Gain strength and comfort from meeting others who share a lived experience.

All of the support groups listed above meet virtually on Zoom and are facilitated by **Matthew Hertzberg**, LMSW. To learn more, or to join a group, please contact Matt at matthewhertzbergmsw@gmail.com

According to board member and FD dad **Steve Kietz**, who is the annual patient satisfaction survey sponsor, "100% of the research participants would recommend the FD Treatment Center to others, up from previous years." He adds, "I was really pleased to see that 79% of the research participants have their local doctors contact the Center, another increase over last year. We are seeing high satisfaction with response time from the Center and are now up to 93% satisfaction rates with the coordination of specialists."

Another encouraging data point the gleaned from the survey was that 9 out of 10 respondents follow the latest in FD research and treatments, highlighted in this newsletter, before the Foundation's website and though the FD Center. Social engagements such as FD Hangouts and Game Nights seem to be a big hit among respondents, and we would love to have more participants. For the teen and young adult programs, we had several requests for movie nights, online book clubs and karaoke events and we are looking into launching more initiatives down the road.

We were especially pleased to note that the ease of making an appointment and after hours care scores improved once again in 2023. Notes Steve, "We learned that 87% found their calls returned promptly. We are very gratified to have such a renowned Treatment Center for our community, and we appreciate the way they handle patient requests. We expect to conduct this survey annually as it's a good way to keep our community up to date with patient satisfaction.

#FDMATCH 2024 CAMPAIGN

FDMatch24 On Track to Meet Goal

FD Match has become one of the FD Foundation's key annual fundraisers. Proceeds support essential programs funded by the Foundation such as:

- Medical care at the Dysautonomia Foundation;
- Telemedicine Program, offering remote check-ups and medical consultations;
- Scientific and clinical research to identify effective treatments for FD;
- Programs offering social and emotional support, such as Hangouts, Game Nights, Mental Health Counseling, Support Groups, Bereavement Groups and more;
- Oxygen concentrator loan program; and so much more!

At press time, the campaign has raised $154,720 and is poised to meet its goal of $250,000.

We are grateful to our challenge donors, whose leadership generosity enabled us to double the value of every gift received:

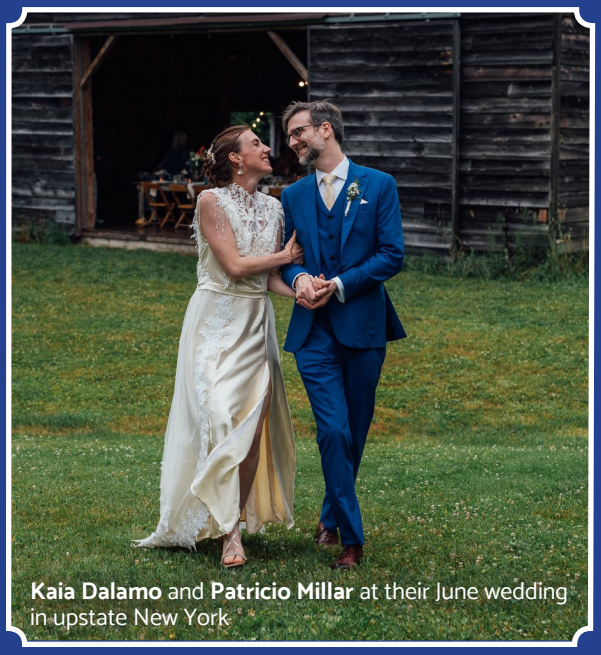
Annette Applebaum Charitable Trust
Baranoff Family
Allan Cohen
Faye Ginsburg & Fred Myers
Solange Landau
Newman Family
David Steiner
Barbra Waldfogel and family
Anonymous

We are also grateful to the following fundraising individuals and teams:

Robin Landau
Mike Zucker
Susan LeVine
Alyson Brenner
Barrie Rappaport
Steve Kietz
Jennifer Sonenshein
Rebecca Sernovitz
Team The Landaus
Team Kelly Brotman & Susan LeVine
Team Josh Kietz
Montreal Chapter of Dysautonomia

KVELLING:

- Mazel tov to NP **Kaia Dalamo** and MD **Patricio Millar**, our colleagues from the NYU Dysautonomia Center, on their marriage on June 29, 2024 in Long Eddy, NY



Kaia Dalamo and **Patricio Millar** at their June wedding in upstate New York

- Congrats to Foundation board VP **Paul Wexler**, who earned Honorable Mention for the Lifetime Achievement Award in the 2024 Invest in Others Awards. This nomination was evaluated based on Paul's leadership, dedication, contribution, inspiration, and impact. Paul was an active stimulus for positive change and demonstrated an entrepreneurial vision for the Foundation–elements at the heart of the Lifetime Achievement Award. The Familial Dysautonomia Foundation received a $2,000 donation from **Invest in Others Charitable Foundation** in recognition of Paul's work!

- We couldn't be prouder of **Mara Clawson**, who was selected to participate in the Artists-in-Residence Program at the acclaimed Byrdcliffe Arts Colony in Woodstock NY during September.

Mara Clawson painting en plein air during her fall residency at Byrdcliffe

- Mazel tov to **Eddie** and **Freddi Baranoff** on marriage of their son **Nathaniel** to **Lillie Baranoff** on 9/25/24 in Brooklyn, NY.

- Did you know that there have been at least 14 scientific papers published about familial dysautonomia since 2023? To view the full list, visit www.famdys.org/resources. Congratulations to all of our researchers for your continued efforts to uncover the mysteries of FD!

Michelle Schwab and Nick Bavaro

- Best wishes to **Jose Martinez, MA**, who retired from his role as Senior Clinical Trials Manager on September 30, 2024 after fifteen years as a member of the research staff at the NYU Dysautonomia Center. We will miss you, Jose!

Jose Martinez

- Mazel tov to **Michelle Schwab**, who facilitates our FD Hangout Groups and Game Nights, for becoming engaged to her longtime boyfriend **Nick Bavaro**.

WHAT'S YOUR PASSION? WHY NOT PUT IT TO WORK TO BENEFIT THE FD FOUNDATION?

Many family members and friends ask us how they can make a difference in the world of FD. You would be surprised at the impact one person (or family) can have when it comes to raising funds to sustain the Foundation's critical work. We encourage members of our community to take a personal interest, passion or milestone event and put it to work in support of the FD Foundation's mission. The Foundation team will even provide logistical back-up and support for your initiative. Listed below are just a few examples of past fundraisers that friends and families have hosted on behalf of the Foundation:

- Bar Night
- Benefit Concert
- B'nai Mitzvah project
- Endurance Event (Distance Run, etc.)
- Golf Outing
- Group Spin Class
- Ladies Card Game Event
- Silent Auction
- Social Media Campaign

In this issue of Dys/Course, we spotlight **Mike Zucker**, dad of Sarah, who is an avid cyclist. For the last five years, Mike has organized a challenging distance ride for himself and a group of intrepid friends, inviting others to donate to the Foundation in support of their efforts. This fall, for Mike's 5th Annual FD Cycling Tour, he plotted a course through the White Mountains of New Hampshire, involving a 70-mile ride and 4700 feet of vertical climb. Equally impressive, Mike's 2024 FD Cycling Tour raised nearly $9,000 for the FD Foundation!

"We have a great time finding challenging new rides for the Cycling Tour," Mike said. "And the financial support that we get from family and friends each year is both incredible and humbling."

What's YOUR passion? Why not put it to work to benefit the FD Foundation? Give us a call (212-279-1066) or send us an email (info@famdys.org). We can't wait to hear your ideas!

Mike Zucker (l) and his friend **Mike Pomerantz** (r) at the summit of their 5th Annual FD Cycling Tour, September 2024

GRATITUDE TO:

- **Mike Zucker** and friends for their annual Bike Ride to raise funds and awareness (see article above)
- **Jake Walker**, who, on September 14, participated in the Rocky Branch Challenge, a 50K "ultra marathon" held in Belmont, North Carolina. This was Jake's third athletic event to heighten awareness and raise funds for FD. Jake successfully brought in more than $3000 in honor of his cousin Josh Kietz and all those living with FD.
- **Old Westbury Golf and Country Club** held their annual charity day this summer. Thanks to the **Wexler**, **Schlau** and **Haberman** families and their very generous friends, the FD Foundation received a gift of more than $10,000.
- **Melany Sommer RN** for organizing a team ("For Pete's Sake") to participate in Philadelphia's annual Rocky Run on November 9, in honor of **Peter Sonenshein**, for whom she provides nursing care.


Jake Walker and his family

Team shirts for the **Rocky Run**

A GREAT DAY OF GOLF

Golfers and non-golfers alike enjoyed a fabulous day at the Glen Oaks Club in Old Westbury, New York on May 28th, 2024. The **27th Annual FD Golf Classic** raised more than $300,000 to support the work of the FD Foundation. We are especially grateful to our four co-chairs, **Paul Wexler**, **Steve Fass**, **Steve Kietz** and **Rachel Schlau**, and to long-time event sponsor **The Kopelman Foundation**.

LOOKING BACK ON A TURNING POINT MOMENT FOR THE FOUNDATION

During a recent meeting, Foundation secretary **Steve Fass** informed the board of the passing of his friend **Theodore (Teddy) Leb** over the summer. Mr. Leb's passing reminded Steve of a turning point moment for the Foundation, which he went on to describe. (One of the longest-serving members of the board, Steve also functions as an unofficial historian of the Foundation.)

Steve recalled, "The year was 1988, I was president, and the FD Foundation was trying to raise $1 million to create an endowed chair at NYU. That was a lot of money back then, and we weren't sure how we were going to accomplish this, but we were determined to make it happen. Mr. Leb owned a car dealership in Lawrence, NY called Auto Europa. Our daughters were good friends and Teddy wanted to do something philanthropic to help the FD Foundation. He agreed to donate a luxury car at cost for the FD Foundation to auction off, as well as to host a special cocktail reception in his showroom."

Steve reflected that the event was a huge success! "The Foundation was able to sell 100 tickets for $1000 each. At the event, each ticketholder received an individual car key. The holder of the key that opened car was the winner who would then have the choice of taking home one of four luxury cars. The lucky winner was **Henry Opatut** whose daughter had FD, but Henry generously declined the car and let the FD Foundation keep the entire $100,000 in profits."

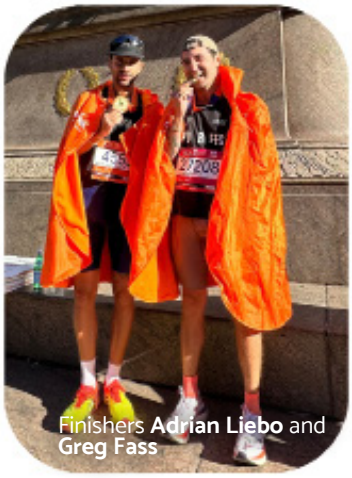
Adds Steve, "When I came up with the idea for this fundraiser, everyone thought I was out of my mind. Yet without Teddy's generosity and the momentum we built from this event, we might not have that NYU endowed chair today. This story just goes to show the long-term impact that one person can have on the trajectory of our Foundation's mission."

The FD Foundation remains grateful to Teddy Leb for his generosity and his vision. We honor his memory and send our heartfelt condolences to his family.

TEAM FD RUNS THE 2024 TCS NYC MARATHON

Six members of TEAM FD ran the TCS NYC Marathon on Sunday, November 3rd. The Foundation's cheering section gathered at Mile 23 on 5th Avenue to encourage our runners, which included a mix of first-timers and veteran marathoners. Together Team FD raised over $32,000 for FD Foundation.

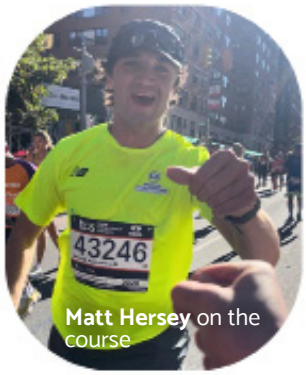
At the After Party: L to R: **Sam Landau, Matt Butrimowitz, Josh Franklin, Josh Kietz, Matt Hersey**



Finishers **Adrian Liebo** and **Greg Fass**

At the Mile 23 Cheering Section: **Albulena Prelvukaj, Lanie Etkind, Brian Levine** and **Lucy**

Bebe Landau cheers for **Matt Hersey**



Matt Hersey on the course

Josh Franklin and his family at the After Party

Are YOU Forever FD EVOTED ?

JOIN OUR LEGACY SOCIETY TODAY!

Over 60 percent of American adults lack an estate plan. We hope you are not one of them. But if you are, this is an urgent reminder that we all need to prepare for life's inevitable twists and turns. And if you do have a plan in place, but haven't updated it in more than five years, now might be a good time to take another look. Estate Planning is about drawing up the most important documents of your life: those that protect your interests and your loved ones.

It's never too early to give yourself peace of mind by ensuring that you have a thoughtful estate plan in place. An estate plan can include a will or a trust, or both. It can detail beneficiary designations, like those you created for your bank account, retirement account or life insurance. And it should include powers of attorney for your finances and your healthcare decisions, in the event you become incapacitated.

An estate plan is also a smart way to shape your legacy and support the causes you care about, including the Familial Dysautonomia Foundation. You can make a gift directly from your will or explore other planned gift options that may be a better fit for your situation. You can even make your gift in honor or in memory of a loved one. With a simple plan to shape your legacy, you can make an incredible difference in the FD Foundation's efforts to ensure longer and better lives for people living with familial dysautonomia.

Some ways to Leave a Legacy to the FD Foundation:

- Include a **bequest** to the Foundation in your will or trust. You can specify an amount, a percentage, a residuary (what's left after other gifts are distributed), or a contingency (based on certain events).
- Name the Foundation as a beneficiary (or partial or contingent) of a **bank account**, an **investment account** or a **life insurance policy**.
- Include the Foundation as a beneficiary of your **IRA, 401K or other retirement plan.**

Some of these options are simple and do not even require the services of an attorney. Of course, we always recommend you consult with your financial advisory to determine what options make the most sense for you and your family.

We encourage you to notify us once you've taken steps to include the FD Foundation in your estate plans so we can officially welcome you as a member of the Forever Devoted Society.

For more information about Forever Devoted, please contact Lanie Etkind, Executive Director, at letkind@famdys.org or 212-279-1066.

Good at doing good!
We've earned a
FOUR-STAR RATING
from
Charity Navigator

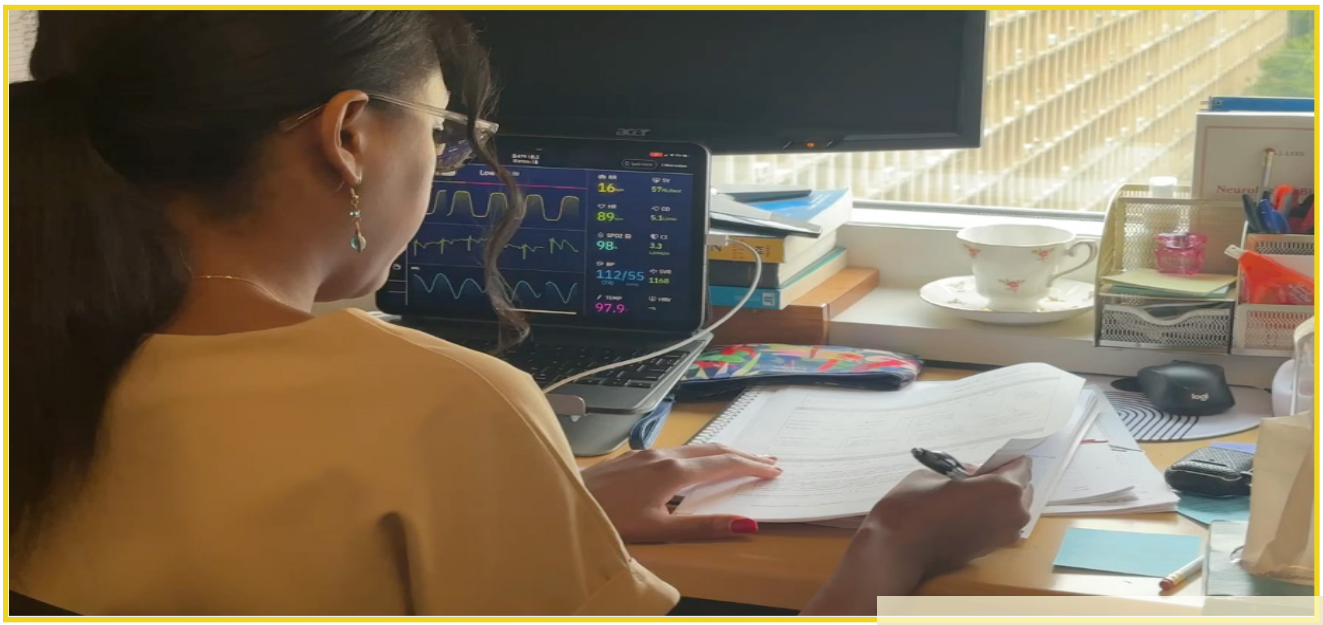
What is a 4-star rating from Charity Navigator?

For the tenth year in a row, the FD Foundation has been awarded a four-star rating by Charity Navigator. This prestigious rating is granted to organizations that achieve a score of 90 or higher in Charity Navigator's assessment, indicating an "Exceptional" status. Among the thousands of nonprofits evaluated by Charity Navigator, only one in four receives a four-star rating, which reflects a high standard of rigor, responsibility, and dedication to transparency.

IGALMI Clinical Trial: A Fast, At-Home Solution for Managing Autonomic Crises in Familial Dysautonomia

The IGALMI clinical trial for Familial Dysautonomia (FD) is testing a new, easier way to treat dangerous autonomic crises, which can happen suddenly and make people very sick. Right now, the best treatment for these crises is a drug called dexmedetomidine, but it must be given through an IV in the hospital, often leading patients to the ICU or emergency room when a crisis can't be stopped at home.

So far, six people with FD are currently enrolled in the trial and have already tried IGALMI. The overall experience has been very positive, with patients and caregivers noting the rapid relief provided by the medication with no side effects. After the first dose of IGALMI, patients usually experience drowsiness, with a slowed heart rate and reduced blood pressure, making the crisis symptoms less severe within about 30 minutes. In most cases, the symptoms disappear completely within two hours.



Nurse Practitioner **Andreana Barnett** is remotely monitoring a patient's vital signs using advanced monitoring technology as part of the trial.

IGALMI is a new form of this medicine that dissolves under the tongue, allowing it to be absorbed fully into the body. This fast absorption means the medication can start working quickly, helping patients stop the crisis before it worsens. Crises often cause patients to retch or vomit making absorption of medications difficult. Even those using a G-tube for medications can experience delayed bowel movements, which makes other treatments less effective. IGALMI, which is absorbed under the tongue, bypasses these issues, and works more efficiently.

The trial uses cutting-edge devices to monitor more than 45 different body functions, including heart rate and oxygen levels, giving doctors detailed insights into how the patient's body responds to the treatment. During active crises, patients and caregivers also receive live support from a physician at the NYU Dysautonomia Center, guiding them through the event in real time.

At the NYU Dysautonomia Center, we understand how stressful and disruptive crises can be for both patients and their families. Our goal is to demonstrate that IGALMI is a safe and effective treatment option for managing crises at home. If the trial is successful, we aim to make IGALMI a standard part of every FD household's medicine cabinet, helping to reduce hospital visits. To achieve this, we are committed to providing strong clinical evidence to regulatory agencies to support the approval of IGALMI as a key treatment for FD.

Regardless of the frequency of crises–whether they occur seldom or regularly–anyone interested in participating in the trial should contact the NYU Dysautonomia Center for more information.

UTILIZING THE AUTONOMIC CRISIS CLINICAL SCALE AND CRISIS TRACK MANAGER FOR ENHANCED CRISIS MANAGEMENT IN FAMILIAL DYSAUTONOMIA

Autonomic crises can severely disrupt blood pressure, heart rate, and other bodily functions, making it crucial to have reliable information for both immediate care and long-term treatment planning. At the NYU Dysautonomia Center, we have developed the Autonomic Crisis Clinical Scale to provide an objective, standardized way to measure the intensity, onset, and resolution of autonomic crises in people with Familial Dysautonomia (FD). This scale allows patients, caregivers, and healthcare providers to track and manage crises more effectively with clear, data-driven insights.

To complement this, we have created the Crisis Track Manager, accessible via smartphone, iPad, or computer. This user-friendly app allows patients and caregivers to quickly log crisis details—such as onset, duration, triggers, and medications—simply by clicking boxes. It generates a PDF summary that can be saved and used to track patterns, compare crises over time, and share with healthcare providers for informed decision-making.

By documenting each crisis, patients and caregivers create a valuable record that helps anticipate and manage future events. For example, it allows caregivers to adjust plans or activities based on the severity of the crisis, decide whether to continue or pause medications, and determine whether to stay at home or seek further medical care. In parallel, the NYU Dysautonomia Center can use real-time data from multiple crises to refine treatment plans and analyze trends across patients, improving care for the entire FD community.

These tools empower users to better manage crises and help healthcare providers deliver more personalized, data-driven treatments. We encourage all FD persons and caregivers to start using these resources to improve crisis management and long-term outcomes.

To access the Crisis Track Manager, use the following link: https://redcap.link/ACS

The same link can be accessed as often as needed, and each use will generate a new entry that will then be saved for your records.

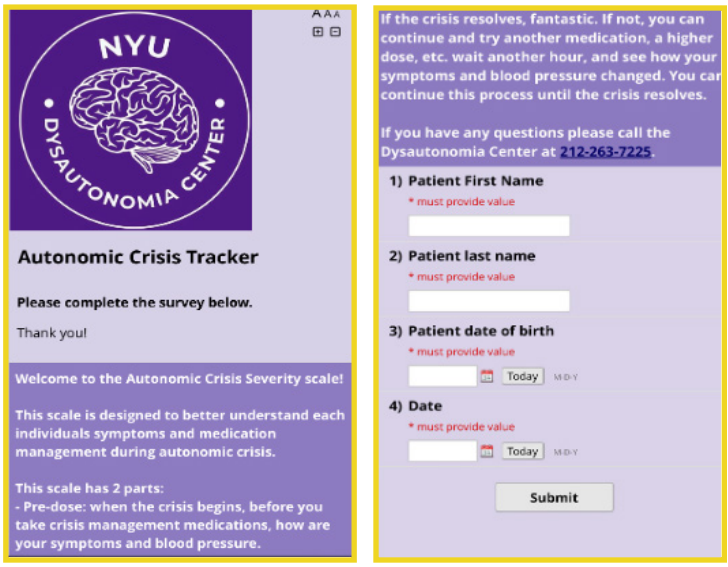
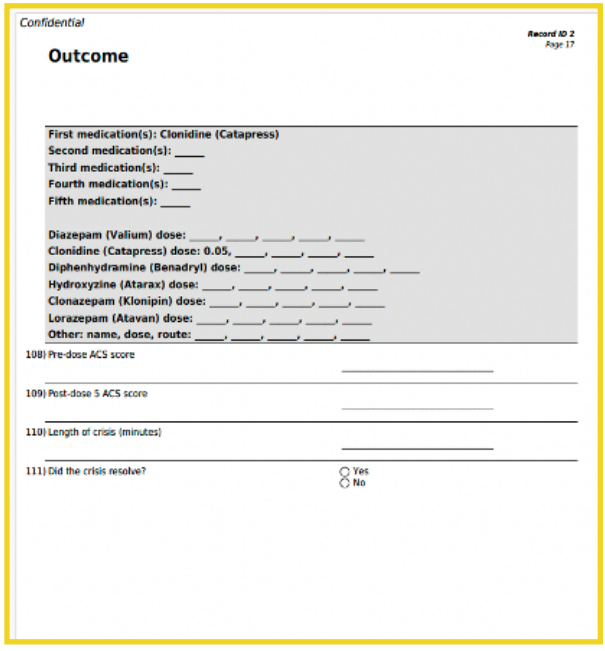


Image of the screen that you will see when adding the information:



Example of the PDF document you will generate at the end of the crisis:

UPDATES FROM THE DYSAUTONOMIA CENTER AT NYU LANGONE

MEET THE VISITING PROFESSORS AND DOCTORS

Name: **Mostafa Elsheikh**
Home Country: **Egypt**
Specialty/Field of Expertise: **Neurology**
Role at the Center: **Volunteer**
Length of stay: **7 months up to now**
What Has Been the Most Rewarding Aspect of Working Here?: **It has given me the opportunity to see some rare conditions that I am unlikely to encounter in the future.**
What's One Thing You'll Take Back to Your Own Practice?: **The importance of teamwork is the most relevant thing that I have been taught here.**

Name: **Joel Gutierrez Gil**
Home Country: **Cuba**
Specialty/Field of Expertise: **Electrophysiology of neuromuscular disorders. Clinical Neurophysiology**
Role at the Center: **Visiting professor; Observer; Educator on electrophysiology of nerves**
Length of stay: **1 month**
What Has Been the Most Rewarding Aspect of Working Here?: **Connecting with valuable collaborators. Participation in high-quality research studies.**
What's One Thing You'll Take Back to Your Own Practice?: **Teamwork is essential. There is a lot we can do for patients with rare and orphan neurological disorders such as FD.**

Name: **Anabella Cecilia Gomez**
Home Country: **Argentina**
Specialty/Field of Expertise: **Neurology**
Role at the Center: **Observer**
Length of stay: **2 months**
What Has Been the Most Rewarding Aspect of Working Here?: **Patients trust us and tell us their joys and main concerns. We dedicate time to get to know them and listen to them. Therefore, a really nice patient-doctor relationship develops. This is one of the nicest things in medicine.**
What's One Thing You'll Take Back to Your Own Practice?: **The teamwork in order to achieve a holistic approach to the patients and the opportunity to discuss the cases and think about the diagnosis with the colleagues.**

Name: **Juan Carlos Lopez Hernandez**
Home Country: **México**
Specialty/Field of Expertise: **Neuromuscular**
Role at the Center: **Visiting professor carrying out comprehensive autonomic evaluations of all patients**
Length of stay: **Six months**
What Has Been the Most Rewarding Aspect of Working Here?: **Understanding autonomic symptoms and the approach to patients with dysautonomia.**
What's One Thing You'll Take Back to Your Own Practice?: **Being able to recognize the main diagnosis associated with dysautonomia, like FD and autonomic failure in neurodegenerative diseases.**

Name: **Margarita Grobocopatel Marra**
Home Country: **Argentina**
Specialty/Field of Expertise: **Medical graduate pursuing Neurology**
Role at the Center: **Research Scholar**
Length of stay: **1 year**
Key Lesson Learned During Your Time at the Center: **It is not what you are treating, but who you are treating.**
What's One Thing You'll Take Back to Your Own Practice?: **When building relationships with people, whether it's your colleagues or people under your care, listening generally holds more value than talking.**

Name: **Daniel Rebolledo**
Home Country: **México**
Specialty/Field of Expertise: **Parkinson's and Movement Disorders**
Role at the Center: **Post-Doc Research Fellowship**
Length of stay: **1 year**
What Has Been the Most Rewarding Aspect of Working Here?: **A comprehensive learning about FD patients. Comprehensive could be a limited word to describe the amazing scientific field the patients offer us. However, the human values I have been learning are remarkable to me. The most recent one has increased my empathy and compassion to give my best in their care and treatment.**
What's One Thing You'll Take Back to Your Practice?: **To be a better version of myself and give my best to treat the patients.**

Rebecca Newman (l) and Sarah Zucker (r) enjoying a summer day during Rebecca's visit to Sarah's home in Rhode Island in August.

Frannie Cohen visited Paris with her family in October. Bien Fait, Frannie!

"OUT AND ABOUT"

Members of the NYU Dysautonomia Center represented the FD community at the 2024 Positive Exposure Gala in NYC in October and posed with one of photographer Rick Guidotti's favorite photos of Samantha Myers. From L to R: Margarita Grobocopatel MD, Mecky Kuijpers, Kaia Dalamo, Alejandra Gonzalez Duarte MD and her daughter Ana, Lee-Ann Lugg, Andreana Barnett, Daniel Rebolledo Garcia MD and Anabella Gomez MD.

Please send us YOUR photos to include in the next issue of DYS/Course!

WAYS TO SUPPORT FD

1

SET IT... AND FORGET IT!

BECOME A MONTHLY FD DONOR!

Did you know you can pre-set monthly donations to the FD Foundation by credit card or ACH bank payments?

Contact the Foundation and we'll help you get started.

www.famdys.org/donate

212-279-1066

2

DONATE YOUR VEHICLE

CARS // TRUCKS // MOTORCYCLES BOATS // RVS // CAMPERS

www.famdys.org/donate-vehicle

1.866.628.2277

3

Double Your Donation!

Many employers will match charitable gifts made by their employees. Check to see if your employer participates.

Tax ID / EIN: 13-6145280

www.famdys.org/double-donation

212-279-1066

4

Donate Appreciated Stock

This is an excellent tax strategy, as the IRS allows you to deduct the current value of the stock rather than its original cost. Please contact the Foundation for more information.

www.famdys.org/donate/stock

212-279-1066

5

PLAN YOUR TRIP WITH US

Just CALL and say " **FD sent us ...**"

Then K&E Travel gives you a great price, keeps track of your booking, and after your travels, K&E sends the FD Foundation a check equal to 50% of their commissions.

K&E Travel

www.ketravel.com

561-966-9808

HOST A FACEBOOK FUNDRAISER TO BENEFIT FD

If you're celebrating a birthday, anniversary or other milestone event, consider inviting friends and loved ones to donate to FD in your honor instead of giving a gift, or set up a campaign yourself on Facebook to collect donations. Whether you are tech savvy or new to Facebook, we are happy to help you set this up! Contact the FD Foundation at info@famdys.org for more information.

Thanks to everyone who held a Facebook Fundraiser for us recently:

Andrew Cohen

Nicholas Kitts

Fred Myers

Kim Olin

Joyce Sanders

Breanna Seymour

Annie Sunderland

Chava White

FOLLOW US ON:

famdys.org

@Famdys

@famdys

@famdysfoundation

@FamDys

@FamDys

FAMILIAL DYSAUTONOMIA FOUNDATION, INC
315 WEST 39TH STREET, #701
NEW YORK, NEW YORK 10018

ADDRESS SERVICE REQUESTED

NON PROFIT
US POSTAGE
PAID
WHITE PLAINS, NY
PERMIT NO 825

A copy of our latest annual report may be obtained from the FD Foundation (315 W. 39th St., Ste. 701, New York, NY 10018) or from the New York State Attorney General's Charities Bureau (28 Liberty Street, 15th Floor, New York, New York 10005).

The Familial Dysautonomia Foundation is a 501(c)(3) non-profit organization dedicated to funding treatment of, research on and awareness about familial dysautonomia, a rare, life-threatening, Jewish genetic disease that affects the autonomic nervous system.

NORD · 2024 MEMBER · PLATINUM

Platinum Transparency 2024 Candid.

Charity Navigator ✦✦✦✦ FOUR-STAR

DYS/COURSE 2024- 2

Your support means a lot to us. Please don't forget to use the enclosed envelope!