SPRING 2025

DYSCOURSE

NEWS FROM THE FAMILIAL DYSAUTONOMIA FOUNDATION

NEW LIVING ARRANGEMENTS IN THE FD COMMUNITY

TIKUN THERAPEUTICS IS ON A ROLL!

TOP 7 REASONS TO BE SEEN AT THE NYU DYSAUTONOMIA CENTER

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.

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On the Cover

Elisabetta Morini, PhD, Asst. Prof, Center for Genomic Medicine, Mass General Research Institute and Assistant Professor of Neurology, Harvard Medical School, in the lab with Senior Research Scientist, **Krishnakanth ("KK") Kondabolu, PhD**. (photo credit **Susan Slaugenhaupt**) To read more about Dr. Morini's work, visit page 2.

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 Spring 2025

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

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

Designed by: **Albulena Prelvukaj**

MESSAGE FROM THE PRESIDENT AND THE EXECUTIVE DIRECTOR

Though FD may be a club no one wants to join, we never forget how fortunate our community is to have so many dedicated scientists, researchers and clinicians working with us, identifying effective treatments that will ensure better and longer lives for our loved ones with FD.

We are delighted to call your attention to the **2024-2025 Year in Review**, a booklet recently prepared by the NYU Dysautonomia Center summarizing the remarkable progress made on the many treatment and research initiatives currently underway. You can view this publication on the Foundation's website at www.familialdysautonomia.org/researchbook.

We are certain that you will be encouraged to learn about the work in progress at the NYU Center and in laboratories across the world, that aim to slow disease progression, ease some of the most distressing symptoms of FD, and improve overall quality of life for people living with the disorder.

While there is much uncertainty about the availability of government funding for scientific research in this country going forward, we are encouraged to know that individuals studying and working on FD remain committed to continuing their life's work. Likewise, the Foundation is committed to helping find and secure alternative sources of support to ensure that this essential research continues. It will take a village!

We want to acknowledge **Horacio Kaufmann MD** and **Alejandra Gonzalez-Duarte MD**, Director and Co-Director of the NYU Dysautonomia Center, and their team, for compiling this year's comprehensive overview of FD research. We also recognize and thank **Dr. Frances Lefcort** and **Dr. Adrian Gilbert**, co-chairs of the Foundation's Scientific Advisory Board, who oversee, coordinate and advise on FD research, as well as **Adam Sachs**, who has not only taken on a leadership role with the SAB, but also during 2024 led an initiative to establish a new entity, **Tikun Therapeutics**, whose objective is to aggressively drive drug development for FD.

Please enjoy reading this year's research booklet–and join us in offering our gratitude to the many individuals whose ongoing efforts are making a tremendous impact on the long-term well-being of our loved ones.

Sincerely,

Faye Ginsburg

Lanie Etkind

Scan QR code to view booklet

FOUNDATION AWARDS TWO RESEARCH GRANTS TO EARLY-STAGE CAREER INVESTIGATORS

For an ultra-rare disorder, the familial dysautonomia community has been blessed over the years with a disproportionate number of scientists and researchers committed to identifying effective treatments, many of whom have dedicated their entire careers to studying FD. At a recent scientific meeting in New York, leaders of the Scientific Advisory Board realized that, nearly a quarter century after the discovery of the gene that causes FD, it is imperative to encourage and inspire the next generation of FD researchers.


Elisabetta Morini, PhD

Drawing from the **Clare and Philip Wexler Research Fund**, during the spring of 2024, the Foundation announced to the greater scientific community a Request for Proposals (RFP) for Early-Stage Career Investigators, offering a grant of $60,000 for a project that showed potential to reach clinical evaluation within three years, ideally for "new treatments or therapies that would improve the lives of FD patients or modify the progression of the disease."


Christiano Alves, PhD

The Scientific Advisory Board evaluated several Letters of Interest and invited full proposals from three candidates. Of those, two proposals scored highly, and the Foundation was able to award not one, but two, $60,000 grants. One grant was awarded to **Drs. Elisabetta Morini** and **Christiano Alves** at Harvard MGH to support "A One and Done Therapeutic Strategy to Correct ELP1 Splicing Defect in familial dysautonomia." A grant was also awarded to **Dr. Anil Chekuri** at Schepens Eye Research Institute to support "Development of a Gene Therapy Approach to Rescue RGC Loss in Familial Dysautonomia." The FD Foundation is deeply grateful to the **Kopelman Foundation** for underwriting the Clare and Philip Wexler Research Fund, and we look forward to reporting progress on these studies in future issues of the newsletter.


Anil Chekuri, PhD

GRATITUDE TO:

- We are grateful to the **Joe Namath Foundation** for their renewed grant of $7000 to support neurological research.
- Our heartfelt appreciation to **the Slomo and Cindy Silvian Foundation** for their $15,000 grant to fund mental health services through the **Stevie Schwartzberg Mental Health Counseling Program**.
- Our special thanks to the **Knobel family** of The Villages, FL, who donated their RV to the Foundation.


The Knobel family


RV donated by the Knobel family

DONATE YOUR VEHICLE

If you have an old vehicle and don't want to deal with it, just donate it to the Foundation. The process is simple. Cars, Trucks, RVs, Boats, Motorcycles are accepted.

1-866-628-2277

www.famdys.org/donate-vehicle

NEW LIVING ARRANGEMENTS IN THE FD COMMUNITY

Recently, several members of the FD community moved into new housing situations and are enjoying newfound growth and independence in stable, reliable and caring environments.

This past July, **Kevin** and **Jason Gross**, brothers from West Lebanon, New Hampshire, moved out of their mom **Bobbi's** home into a brand new supported, two-bedroom apartment, in a warm and homey building called Spruce House in Hanover, New Hampshire. The local nonprofit that manages this residence is called Visions for Creative Housing Solutions. Visions oversees three local sites, each housing up to 12 adults. According to Bobbi, Jason and Kevin now live in the newest one that opened last summer, which was built with an elevator and is totally accessible." Visions' mission is to provide residential options that meet the individualized needs of each adult. Each resident lives in their own two-bedroom or studio apartment, each with their own staff. Adds Bobbi, "It's been an amazing experience for both of them!"

Kevin (l) and Jason Gross preparing Tiramisu for everyone with Ernesto, one of their DSP's, in the kitchen at Spruce House

According to Kevin (age 42): "For me, it was not a hard transition. I knew ahead of time what was going to happen and I'm learning new things all the time. Since we moved, we've been able to go out almost every day, with the help of our Mentors/DSP's (Direct Support Professionals). Some days we might shop, on other days we will head out to a local fitness center to exercise, and other days we go to Dartmouth men's hockey games when they're playing at home."

Kevin added, "I also volunteer through Global Campus, which is a program I've been helping to organize. Classes are offered to the residents from all three Visions houses." In December, Kevin hosted a class at a local library focused on the movie Airplane, "to share how funny it was." Kevin said, "When I'm feeling down, this movie always cheers me up, so I asked people in the audience what they like to watch to make themselves feel happier, and I received great feedback." Kevin elaborated by saying that in January, someone taught a class about 'online safety,' securing a guest speaker. The following month, a presenter talked about why she enjoys horror movies. So each class offers something different to learn about. Kevin enjoys scheduling these classes which take place at nearby libraries.

Kevin also is employed (per diem) at a local hospital. He works as a "Standardized Patient" in Dartmouth Medical Center's "Patient Safety Training Center," where he plays different "patient roles" - each one designed to help train new healthcare professionals. Kevin notes, "Because I have poor eyesight and poor balance, I was selected to appear in a training video that demonstrated to new healthcare professionals how to deal with a specific situation." In the video, Kevin was the "patient," in an enactment of a scenario that was designed to train people on procedures to follow when there is a chemotherapy spill. Kevin explained that because he can't see well and he uses a transport chair, the professionals being trained needed to learn not only how to take care of the chemo spill, but also how to properly address the needs of a patient with disabilities at the same time.

Jason, who, at almost 50 years old, is the oldest resident at Spruce House added, "All of the helpers (DSP's) here have been amazing! They assist us with showering, preparing food, doing our laundry, going out in the community, shopping, and keeping our apartment clean. It's the first time we've lived in our own apartment and are paying our own rent, so it's a whole new experience. Sometimes we cook together. Usually we eat lunch in our apartment. At night, we typically go downstairs for family-style dinners with the other residents."

Kevin added, "The best parts have been our amazing mentors (DSP's) here, and the social aspect. I love hanging out with the other residents." Both brothers have forged new friendships with the other ten residents who range in age from 24 to 49 years old.

In addition, Kevin hosts a monthly game night with the other residents at Spruce House. Jason has been hosting a monthly movie night in the common room on the first floor, where everyone can take advantage of the big TV screen and new sound system there. He adds, "I love picking the movies, like Mel Brooks' Men in Tights, or Walt Disney's Country Bears."

Says Bobbi, "Clearly, Jason and Kevin are thriving in their new home. Their daily social interactions, both in the community and within their house, are a key factor in the success of the Visions model."

Just 3 hours away, north of the Green Mountains of Vermont is Montreal, Quebec, where 29-year-old **Morgan Asinowski** lives. He moved in October, into a CHSLD "centre d'hébergement et de soins de longue durée," which translates to "long-term care and housing center," referring to residential facilities in Quebec that provide housing and care for those with complex needs.

According to Morgan's dad **Nelson**, "They reorganized the health system here. This is an ongoing long duration care service that is government designed. Fortunately, one opened in a suburb near us in September targeting 21-64 year olds." Adds Morgan's mom **Florence**, "There are three floors with 24 rooms on each one. Currently they have one floor open and Morgan was the ninth resident to move in. It's an experiment, and if it goes well, they will open up the others." According to his parents, Morgan has been thriving there. Each resident has his own room with a handicapped accessible bathroom, a communal kitchen, and individualized food supplied to the residents. Explains Nelson, "They really cater to each person. Morgan eats all of his food minced and the staff presents it to him just the way he needs it. He can choose to eat in the dining room or in his own room, but he always likes to socialize with others during meals." The unit also offers a social room where people come together to participate in activities. Adds Florence, "Morgan has his own set of activities. There is music that he goes out for and that he also does on zoom and he often goes out on weekends for discussions. He's very proud of what he calls his apartment and feels a nice sense of independence. It's a well supported group and he seems to be very happy!"

Both parents are receiving amazing feedback from the staff about how he interacts with everyone and they are relieved that it has been a very pleasant transition. "For me as a mom, the first few weeks were nerve wracking but I see that they are good to him and that makes it easier for us. We also recognize the benefits of having our time alone together!" says Florence.

Morgan Asinowski in his new living space in a "centre d'hébergement et de soins de longue durée" in Montreal

Another interesting new living arrangement is that of **Sam Landau**, who moved into his own apartment in November, in the same building in which his parents, **Robin** and **Laurent**, live in Manhattan. Born in that same building and now 29-years-old, Sam is living independently for the first time and thriving. According to his sister **Bebe**, "Sam lives one floor down from my parents but he's become so much more independent. I'm so impressed with him!" Adds Sam, "I felt like it was time. I lived with my parents for long enough. I still see them daily and have dinner with them most nights but it's nice living on my own and planning my own schedule." Currently looking for a job, Sam plans to learn how to cook soon and loves his newfound feeling of self reliance. He notes, "It's nice to be able to pick my own TV shows and listen to my music. I feel more myself and think it was a great decision all around."

Sam Landau relaxing in his new NYC apartment

(image by **Rick Guidotti**, Positive Exposure)

FD "COVER GIRL" SAMANTHA GINSBURG MYERS Z"L

Thanks to the advocacy of the amazing activist photographer **Rick Guidotti** and his remarkable organization **Positive Exposure** (https://positiveexposure.org), **Samantha Ginsburg Myers z"l** graces the February 2025 cover of the journal *Helen: The Journal of Human Exceptionality*_(focused on the rare disease community) in honor of Jewish Disabilities Awareness, Acceptance and Inclusion Month. The issue also features an article about the FD Foundation.

Faye posted the cover image and links to the article on her Facebook Page on February 8, commemorating what would have been Sam's 36th birthday, commenting, "I can't think of a better way to remember her -- she would have LOVED the Barbie pink graphics and being able to tell people about her life with FD as her birthday gift. The Sam Effect lives on!..."

To read Sam's story visit
https://helenjournal.org/february-2025

To view the article visit:
https://helenjournal.org/february-2025/what-is-familial-dysautonomia

US-ISRAEL COLLABORATION CONTINUES WITH RESEARCH TALK BY DR. MIGUEL WEIL

Dr. Miguel Weil directs a research lab at Tel Aviv University where he has been studying the molecular and cellular pathways underlying neuronal death in FD. He also is the parent of a son with FD and has been close to the FD community for over 20 years. Together with his junior colleague, **Dr. Michal Shilian**, Dr. Weil was invited to present the latest data from his innovative research via Zoom to members of the Foundation's Scientific Advisory Board and some Foundation board members in March. There was keen interest in Dr. Weil's work, which is novel and promising, and a robust exchange of questions and answers followed their talk. Most importantly, because all FD scientists share the goal of finding therapeutics for FD, the more they can all work together towards understand the underlying pathways that dysfunction in FD, the more our cause is helped.


Dr. Miguel Weil


Dr. Michal Shilian

TIKUN THERAPEUTICS IS ON A ROLL!

There have been many promising updates to **Tikun Therapeutics** since last fall. The Public Benefit Corporation (PBC) is led by **Adam Sachs**, the proud father of 30-year-old **Justin** who has FD. Explains Adam, "Tikun's mission is to aggressively drive drug development for the FD community. We successfully achieved our first big milestone with FDA's recent approvals for both Orphan Drug (ODD) and Rare Pediatric Disease (RPD) Designations for both the small molecule splicing modulator and the gene therapy vector drug candidates to treat FD."

Following these approvals, Tikun issued a press release directed at both the biotech drug development and investment communities. Adam notes, "the press release had a very high click-through rate and generated numerous inquiries from potential investors. The PBC also launched its website: tikuntherapeutics.com."

Scan QR Code to visit Tikun site and read the press release

According to Adam, "our next priority is to submit a Small Business Innovation Research (SBIR) grant through the NIH." The team worked hard to meet the April deadline. **Dr. Frances Lefcort**, Tikun's Chief Science Officer, is the primary author and will serve as principal investigator. Adds Adam, "we couldn't access the SBIR as part of the (non-profit) FD Foundation but since we are a PBC we could apply." Phase one of this grant could lead to approximately $320,000 for the gene therapy vector development; and related subsequent grants, if awarded, could be worth several million dollars. He explains, "we are submitting our proposals and are very hopeful to receive funds we've never been able to target before."


Adam Sachs and Dr. Tod Woolf

Another exciting development is the addition of **Dr. Tod Woolf** to Tikun's Scientific Advisory Board (SAB). Says Adam, "Tod is a personal friend who I've known since middle school. He's a very accomplished scientist in our field and he agreed to be an active Tikun consultant. He has been a trusted scientific resource for me since Justin was born and we are very fortunate to have him and be able to tap into his considerable technical and business expertise."

Dr. Woolf is the Executive Director of the Technology Ventures Office at Beth Israel Deaconess Medical Center in Boston and is also on the faculty at Harvard Medical School. At Beth Israel, Dr. Woolf does all the licensing and collaborations with industry professionals. Tod has 25+ years' experience as an entrepreneur and leader in research, business development and technology transfer. He founded the biotechnology company Sequitur and cofounded RXi Pharmaceuticals, now a public company. "Tod is a great fit for the Tikun SAB, adding his considerable experience in molecular and cell biology and impressive track record of success in business development in support of the FD patient community," adds Adam.

"Adam and I were roommates at Michigan," notes Dr. Woolf. "I was studying biotech and Adam was studying oceanography but we both ended up in biopharma and we have now merged our careers. With my experience developing drug research in RNA therapeutics, and more specifically on splice modulation, I can provide technical assistance on clinical trials and the early stages of developing drug and licensing agreements."

Adam and Dr. Woolf developed a pitch deck to take to potential philanthropists and are optimistic about raising money for the FD Foundation to develop future treatments. "It's coincidental that we both became biotech people and now I'm in the RNA space and a leader in gene therapy. FD is such a rare disease, but we have an amazing team especially given how few patients there are," states Dr. Woolf.

Adam is now looking to add additional members to Tikun's Scientific Advisory Board. "Tod is the first from outside of our FD circle and we are proud to have him and really appreciate his time and generosity. We have such strong momentum going on right now. There are a lot of very talented people committing their time and energy and I'm optimistic we can get this done."

FD Hangouts

Michelle Schwab facilitates our popular FD Hangouts on Zoom, providing an opportunity for people who have FD to meet and socialize with one another virtually.

- ORANGE GROUP (40S-60S) - TUESDAY AT 7:30PM ET (WEEKLY)
- GREEN GROUP (30S)- WEDNESDAY AT 8:00PM ET (BIWEEKLY)
- YELLOW GROUP (20S)- WEDNESDAY AT 7:00PM ET (BIWEEKLY)
- BLUE GROUP (TEENS) - WEDNESDAY AT 7:00PM ET (BIWEEKLY)

If you are interested in participating in a hangout group and would like to register, please contact Michelle at info@famdys.org.

Virtual Game Nights

Hosted on Zoom by the FD Social Committee

Game Nights are open to all. No registration is needed.

If you are interested in participating please contact Michelle at info@famdys.org.

Game Night 7:30 PM ET	22 MAY	Game Night 7:30 PM ET	26 JUN	Game Night 7:30 PM ET	24 JUL
Game Night 7:30 PM ET	29 AUG	Game Night 7:30 PM ET	23 SEP	Game Night 7:30 PM ET	28 OCT

For additional dates in 2025 and beyond, please contact the FD Foundation at info@famdys.org or visit https://bit.ly/fdhangouts

OUT AND ABOUT

Members of the "New York contingent" of our FD community came together to celebrate Frannie Cohen's 30th birthday at Serafina in NYC in March. L to R: **Perry Goldberger**, **Sam Landau**, **Jackie Goldberg**, **Gabi Jassie**, **Becca Newman**, birthday girl **Frannie Cohen**, **Josh Kietz.**

At the "Mom's Table": L to R: **Barbara Kietz**, **Lisa Newman**, **Marsha Cohen**, **Carrie Pepkin**, **Robin Landau.**

At the "Dad's Table": top row L to R: **Roger Jassie,** and **Jeff Cohen**; bottom row L to R **Laurent Landau** and **Jeff Newman** (not pictured: **Steve Kietz**)

Please send us your photos for inclusion in the next issue of DYS/Course at info@famdys.org.

FOUNDATION ADDS NEW GROUP FOR BEREAVED SIBLINGS TO ROSTER OF EXISTING SUPPORT GROUPS

Back in 2023, **Matthew Hertzberg** started a caregiver group for FD families during his internship with the Dysautonomia Center when he was in graduate school. After becoming a licensed social worker, Matt stayed on with the FD Foundation and currently facilitates three different groups for various FD populations on Zoom. He is now preparing to launch a fourth group this Spring for Bereaved Siblings, whether they lost a sibling 20 years ago or just recently.

In addition to the FD Parent/Caregiver Support Group that meets monthly to discuss caring for loved ones with complicated medical issues, Matthew also hosts a Grief and Bereavement Group for parents that meets every other week.

The latest group, which launched in December 2024 and meets monthly, is the Sibling Support Group. According to Matt, "I really enjoy this new group since the siblings seem to get a lot out of it. We know the bonds that parents share as caregivers as well as young adults living with FD, but the siblings are now also able to connect and share their stories since it's never the one that's told first. I find them insightful, empathetic people because of their experiences, and they are really good at expressing themselves." The group ranges in age with the youngest at just 21 in college and the oldest in her early 40s. He adds, "The participants are definitely in tune with each other and often bounce ideas off of each other to solve real life issues." While this group is certainly lighter in tone than the other groups, it's a mix of socializing with opening up and being vulnerable. "We hope to get the next generation involved," notes Matthew. "Many are going into medicine or pursuing roles in mental health or occupational therapy since FD is part of their story. They are invested in it."

According to **Bebe Landau**, the 26-year-old sister of Sam Landau who is 29 and living with FD in New York City, "I've really enjoyed the Sibling Support Group sessions so far. It's so nice to be involved and the feeling of camaraderie is unparalleled. I could explain my feelings to a million people, but no one would really understand, whereas everyone in the group totally gets it." The group sometimes focuses on deep issues, but they also have light conversations as well to create an easy atmosphere. "I met someone I didn't even know existed which is interesting since it's such a small community," notes Bebe, who has since reached out to others to tell them about the group. Looking ahead, Bebe observes that sooner or later they may all have some level of responsibility for their siblings. "We may as well do it together."

FD PARENTS AND CAREGIVERS

Meets monthly on Wednesday - 7PM ET. 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

Meets every two weeks on Wednesday - 7PM ET. This group supports parents navigating those incredibly difficult first months—and years—following the loss of a child. All are welcome.

FD SIBLINGS

Meets monthly on Monday - 7PM ET. Recently launched; new members are welcome. No family member is unaffected by FD. The FD Sibling group offers a warm and compassionate forum for siblings to share their mutual experiences, work through common stressors and create lasting bonds.

BEREAVED SIBLINGS

Meets monthly on Monday - 7PM ET. Join others who have lost a sibling/cousin/close friend to FD. Gain strength and comfort from meeting others who share a lived experience.

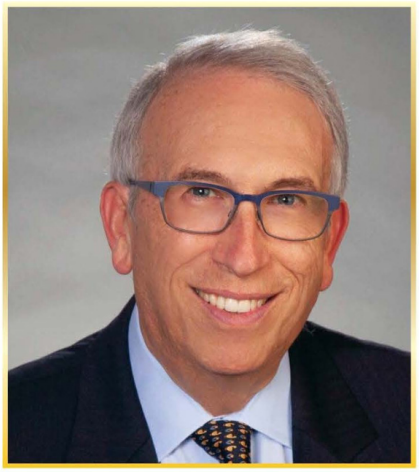
For more information on any of the FD support groups, please email Matt at matthewhertzbergmsw@gmail.com.

WE ARE KVELLING FOR...

Natalie and Frannie Cohen

- Mazel Tov to FD Foundation board member **Becky Sernovitz** and her husband **Larry Sernovitz** on the Bat Mitzvah of their daughter **Daniella** in April 2025.
- We are Kvelling for **Marsha and Jeff Cohen**, whose daughter **Natalie** (sister of **Frannie**) matched at Duke for her internal medicine residence starting in June!
- **Alejandra Gonzalez-Duarte, MD** was selected to be part of the scientific program at the American Academy of Neurology 2025 Annual Meeting presenting her work entitled, "Elongator Protein I as a Biomarker of Disease Severity in Familial Dysautonomia," at this top neurology meeting held April 2025 in San Diego, CA.

A GIANT AMONG MEN



HOWARD WEISER z"l

The FD community lost a "giant among men" when Foundation board member **Howard Weiser** passed away from a long illness on January 1, 2025. This year would have marked Howard's 50th year on the Foundation's board of directors, a truly unprecedented length of service, which also included a term as president. Equally extraordinary is that Howard's involvement with FD was completely altruistic, as he did not have a family member affected by the disorder. A generous donor, leader, mentor and advisor, Howard will be missed by all. We send our heartfelt condolences to Howard's beloved wife **Tova**, their children, **Chanie**, **Reuven** and **Rivka**, and to all his adoring grandchildren. The Foundation is planning a special tribute to Howard, and details will be announced later this year.

*"It's very hard to grasp that Howard is no longer with us; he was always so full of life and with a twinkle in his eye, and unfailingly kind and supportive to those dealing with the death of their beloved children and other losses. His devotion to Tikkun Olam –to repair the world –was evident in everything he did - his dedication to the FD community for 50 years was extraordinary, one of many causes he supported with care, intelligence, wisdom, and compassion. There is a Howard Weiser size hole in the universe that can't be filled but will always remind us to embrace Yetzer HaTov -our impulse to do good – as he did with such grace and energy." --**Faye Ginsburg**, President*

*"Howard was a friend and mentor to me and my family. His essence was חנון, a Hebrew word meaning giving generously and selflessly. FD was just one of the many causes to which he was devoted. He truly made the world a better place."--**Gerald Adler**, Director*

ARE YOU Forever FD DEVOTED?

You can invest in the future of the Familial Dysautonomia Foundation by including the Foundation in your estate plans. Especially during these turbulent financial times, making a planned gift requires no outlay of cash today, but asks you to consider how your funds will be disbursed at a future time. We invite you to:

Include a bequest to the Foundation in your will or trust: You can specify an amount or a percentage, a residuary or a contingency, ensuring that your loved ones are provided for as well.

Name the Foundation as a beneficiary (or partial beneficiary) of a bank account or investment account; life insurance policy; or IRA, 401K or other retirement plan: This can usually be done simply by completing a form and does not require an attorney.

Once you have included the Foundation in your estate plans, please let us know so we can acknowledge you as a member of our Forever Devoted Planned Giving Society.

Questions? Please contact Lanie Etkind, Executive Director at letkind@famdys.org or 212-279-1066.

A tax-wise tip for those aged 70 ½ and older: You can give up to $108,000 per calendar year to qualified charities (like the FD Foundation) directly from most IRA's (as can your spouse if you file jointly). The distribution counts towards your RMD, but the IRS does not treat it as income to you. Ask your financial advisor for details.

UNITED AIRLINES NYC HALF 2025

NEW YORK ROAD RUNNERS

THANK YOU TEAMFD

TEAM FD FAMILIAL DYSAUTONOMIA FOUNDATION, INC

for running the 2025 United Airlines NYC Half Marathon on March 16th and raising more than $11,000 to support the FD Foundation's mission!

CASEY KAPLAN
ran for Scott Fass

GARY KAPLAN
ran for Scott Fass

JUSTINE LEASE
ran for the FD Foundation

JESSICA SCHOEN
ran for Rebecca Newman

LILY SZAJNBERG
ran for Samantha Myers

Team in formation at press time

MEET THE RIDERS

TD FIVE BORO BIKE TOUR
NEW YORK / MAY 4, 2025

SCAN QR CODE TO SUPPORT TEAMFD

ADAM BERGENFELD
Riding for Jack Posnack

JAKE BERGENFELD
Riding for Jack Posnack

MECKY KUIJPERS
Riding for FD

THE RIGHT CALL: STRENGTHENING COMMUNICATION FOR BETTER CARE

At the NYU Dysautonomia Center, our commitment to exceptional care goes hand in hand with clear, effective communication. We receive a wide range of communications each day, from pharmacy and equipment requests to questions about symptoms, appointments, and administrative forms. Some are essential. Some are urgent. Others are better directed through alternative channels. But what all of them have in common is that they take time—and when communication is misdirected, delayed, or repeated unnecessarily, it can slow down care, increase frustration, and in some cases, jeopardize health outcomes. When unnecessary or misdirected calls and emails flood the system, they reduce our ability to deliver timely care to everyone who needs it.

Behind the Scenes: What You Might Not See

Every day, our team manages time-intensive responsibilities that directly impact patient care. Here's a closer look at what happens behind the scenes:

- **Medication Prior Authorizations.** Each FD patient typically requires at least three ongoing prior authorizations—for controlled medications such as Valium, for respiratory care, and for any other issue, including gastrointestinal or other therapies. Each request can take over two hours to complete. This includes submitting initial forms, answering insurer questions, reviewing patient charts, writing letters of medical necessity, and sometimes filing appeals. These steps must be repeated every time coverage is denied or requires renewal.
- **Appointments and Transportation Arrangements.** Coordinating appointments for patients with complex medical needs, out-of-town travel, or special equipment is often a logistical challenge. Transportation alone can take several hours to arrange, especially when coordinating with insurance, transportation vendors, and caregivers to ensure both safety and accessibility.
- **Medication Refill Requests.** A single "urgent" refill request often results in three separate phone calls: the first to let us know the refill is urgent, the second to confirm it was submitted, and the third to report it hasn't been received—despite having been informed that processing takes 48 hours. Many of these medications are already listed for automatic monthly refills, and repeated calls do not accelerate the process.

Emergencies: When Seconds Count

The most important reminder we can offer is this: **In a medical emergency, do not call or email the Center, call 911 immediately.** Emergency medical services are trained and equipped to act right away—something no outpatient clinic, no matter how experienced, can do over the phone.

Once the emergency is under control, our role begins. We coordinate follow-up care with families, caregivers, local physicians, and specialists. We are here to guide next steps and support recovery. But we are not an emergency response service, and emergency treatment should never be delayed while trying to reach us.

Finding the Right Channel

To ensure that we respond quickly and appropriately, please use the following guidelines when contacting us:

- **MyChart.** Use this platform for all non-urgent clinical questions, prescription refill requests, follow-up messages, and document uploads. MyChart is secure, HIPAA-compliant, and directly connected to your medical record. It allows your request to reach the right person efficiently.
- **Email.** Reserve email for administrative needs only—such as letters of necessity, insurance documentation, pharmacy forms, or transportation coordination. Please note that email is not monitored in real-time and should never be used for clinical questions or emergencies.
- **The On-Call Phone Line.** This line is available outside of regular business hours for urgent—but not emergency—medical issues. Calls are recorded and reviewed to ensure quality and accountability. While response times may vary, when used appropriately, these communication tools allow us to stay organized, respond effectively, and deliver the high standard of care you've come to expect from the Center.

We're In This Together

We recognize the effort and vigilance it takes to care for someone with FD. Our team is here to support you every step of the way—but we can only do that well with your partnership. As the volume and urgency of daily requests continue to grow, it's more important than ever that every interaction—whether for a medication refill or a clinical concern—is routed efficiently and safely.

A NEW LENS ON CARE: THERMAL IMAGING FOR EARLY INFLAMMATION DETECTION IN FD

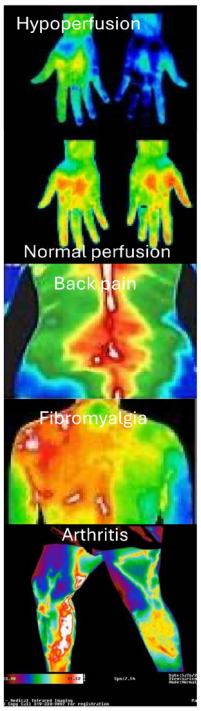
At the NYU Dysautonomia Center, thermal imaging is now part of routine annual visits for patients with Familial Dysautonomia (FD)—a population in which classic signs of disease often go unnoticed due to impaired pain and temperature sensation.

A persistent challenge in FD care is identifying occult inflammation. Autonomic crises are often triggered by underlying infections or inflammatory processes that progress silently, without producing obvious or recognizable clinical signs. In other cases, lab tests may reveal elevated inflammatory markers—such as ESR or CRP—without a clear source. To address this gap, the team at the Center turned to thermography, a technology that offers a non-invasive way to visualize physiologic changes, including inflammation and infection.

Once primarily used in industrial and security settings, thermography gained renewed clinical relevance during the COVID-19 pandemic, when infrared thermal screening became commonplace for fever detection. This widespread exposure accelerated its adoption in medicine, sparking research and integration into a variety of specialties.

Today, thermography plays a role in the diagnosis and monitoring of numerous conditions:

- In rheumatology, it helps detect early joint inflammation in arthritis.
- In diabetic care, it is used to monitor peripheral circulation and prevent foot ulcers.
- In vascular medicine, it helps identify ischemic areas.
- In oncology, it has been explored as a supplementary tool for detecting abnormal metabolic activity, especially in breast cancer.



Its ability to visualize temperature fluctuations in real time—without radiation or physical contact—makes thermography a valuable addition to clinical workflows.

For FD patients, this technology offers a critical new tool. Because these individuals may not feel pain or discomfort, inflammatory sources such as dental abscesses, joint infections, or soft tissue inflammation can go unnoticed, and still trigger severe autonomic episodes. When combined with laboratory findings, thermographic scans can help localize the source of inflammation and guide timely clinical intervention.

During clinic visits, the team performs scans of the mouth, hands, feet, thorax, abdomen, and pelvis. These high-resolution temperature maps allow the team to:

- Detect localized thermal abnormalities suggestive of inflammation or poor circulation
- Track changes over time to assess disease progression or stability
- Evaluate treatment response and intervention efficacy
- Gain broader insight into autonomic and vascular function in FD
- Document objective thermal data for integration into the patient's medical record

This initiative was made possible thanks to the generous support of the Familial Dysautonomia Foundation, which helped the Center acquire the thermographic equipment. The device's WiFi connectivity enables seamless transfer of images into electronic medical records, supporting real-time documentation and longitudinal analysis.

Looking ahead, the Center plans to analyze thermographic data over time to identify emerging patterns, improve understanding of how inflammation contributes to autonomic instability, and develop personalized strategies for early detection, prevention, and care in FD.

Dr. Alejandra Gonzalez-Duarte shared: "By integrating thermographic technology into our clinical practice, we are significantly enhancing the level of care provided to FD patients.This aligns with our mission to improve health outcomes through advanced diagnostics and patient-centered approaches."

UPDATES FROM THE DYSAUTONOMIA CENTER AT NYU LANGONE

TOP SEVEN REASONS TO BE SEEN AT THE NYU DYSAUTONOMIA CENTER

When is the last time you scheduled an annual visit at the Dysautonomia Center? If it's been longer than a year, we hope you will consider making an appointment. Here's why:

1. Monitoring Changes: Annual follow-ups are essential to keeping track of the many challenges that come with familial dysautonomia (FD), such as autonomic instability, breathing issues, or digestive problems, which can be easy to miss without regular check-ins.

2. Collaborative Care: At the NYU Dysautonomia Center, we work closely with doctors from other specialties. This ensures care isn't limited to one perspective but incorporates a variety of expert opinions for a well-rounded approach.

3. Personalized Approach: We focus on creating treatment plans that fit each person's individual needs, preferences, and lifestyle, making care as effective and patient-centered as possible.

4. Ongoing Innovation: We're always exploring new treatments and tracking their outcomes. When something works well, we can apply it to others, and if there are side effects, we adjust accordingly.

5. Research Opportunities: Patients have the option to participate in research protocols focused on disease-modifying therapies. These opportunities allow those interested to contribute to advancing treatments and benefit from cutting-edge developments in FD care.

6. Learning From Experience: We follow many patients annually and have an enormous data bank where all the information can be tracked and analyzed. By understanding what helps each patient, or group of patients, we can improve care strategies and use those insights to benefit others with FD.

7. Enhancing Quality of Life: This combination of teamwork, innovation, personalized care, and research ensures patients stay healthier, manage their symptoms better, and enjoy an improved quality of life.

"I would like to highlight that things are changing at the Dysautonomia Center. We now have a new team fully dedicated to listening to patients and working with them to guide their care, rather than making decisions for them. We're more open to collaboration than ever before, making sure everyone's voice—patients, families, and other doctors—is heard and respected. We don't push our ideas over others but work together to find the best approach for each person."

Dr. Alejandra Gonzalez-Duarte, Co-Director of the NYU Dysautonomia Center

Platinum Transparency 2025

Candid.

FD Foundation is proud to have earned a 2025 Platinum Seal of Transparency from Candid (formerly Guidestar).

The Platinum Seal signifies that the organization has met Candid's rigorous standards for transparency and accountability. The Platinum Seal is the highest level of recognition awarded by Candid, and only a small percentage of US-based non-profit organizations have been recognized with it. To view our work visit https://bit.ly/Candidfd25

On **Rare Disease Day**, February 28, 2025, members of the FD community "showed their stripes" in creative ways, helping to raise awareness for familial dysautonomia and the 300 million people worldwide living with rare diseases.

DID YOU KNOW? IRS RAISES ABLE ACCOUNT DEPOSIT LIMIT FOR 2025

Did you know? As of January 2025, individuals with disabilities can accrue more funds than ever before in a special type of account that allows people to save without jeopardizing their access to Medicaid and other government benefits.

Contributions to ABLE accounts can total up to $19,000 for 2025, an increase from the limit of $18,000 in 2024. This change comes after the IRS raised the federal gift tax limit. Since the cap on annual deposits for ABLE accounts is tied to that figure, it increased as well.

What is an ABLE account?

The ABLE (Achieving a Better Life Experience Act) act became federal law in 2014 and aims to ease financial strains faced by individuals with disabilities by making tax-free savings accounts available to cover qualified disability expenses. Through ABLE accounts, people with disabilities can save up to $100,000 without sacrificing their eligibility for Social Security and other government benefits. (Medicaid can be retained regardless of how much is in the account.) Funds saved in an ABLE account can be used to pay for qualified disability expenses such as education, health care, transportation and housing. The account earns interest tax-free. Individuals with disabilities who are employed can save some of their earnings so long as they do not contribute to a retirement plan and meet other requirements.

For more information, contact the ABLE National Resource Center at https://www.ablenrc.org. We recommend that you consult your financial advisor to determine if an ABLE account is an appropriate option for your family.

HOST A FUNDRAISER TO BENEFIT FD

If you're celebrating a birthday, anniversary or wedding, consider inviting friends and loved ones to donate to FD in your name instead of giving a gift, or set up a campaign yourself on your social media accounts to collect donations. Whether you are tech savvy or not, 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:

Kim Donadio
Jayme Madisyn
Barrie Rappaport
Carla Rosenberg
Joyce Sanders
Jess Schoen
Mike Zucker

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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 2025 MEMBER PLATINUM

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DYS/COURSE 2025- 1

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

Register Today!