

FALL 2025

DYS/COURSE

NEWS FROM THE FAMILIAL DYSAUTONOMIA FOUNDATION

**NEW HOPE FOR THE FD
COMMUNITY WITH ASO
THERAPY**

**CONVERSATION WITH LILY
ARMSTRONG, MENTAL
HEALTH COUNSELOR**

**UPDATE ON YOUNG
INVESTIGATOR RESEARCH**



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DYSAUTONOMIA
FOUNDATION, INC**

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On the Cover

“Practicing With a View” by **Mara Clawson**. Painting with acrylic on wood, Mara captured the beauty right outside her window during her art residency in Woodstock, NY. This was Mara’s submission for the 2025 FD Day Art Competition, and it was awarded First Place.

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

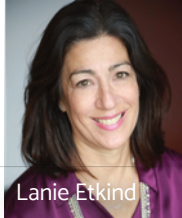
DYS/COURSE Fall 2025

Written by: Lisa Denburg, Alejandra Gonzalez-Duarte, Lanie Etkind
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Designed by: Albulena Prelvukaj

MESSAGE FROM THE PRESIDENT AND EXECUTIVE DIRECTOR



Faye Ginsburg



Lanie Etkind

Dear Families, Friends, and Supporters:

If you were able to join us live for FD Day on June 8, you could not help but feel encouraged by all the positive updates shared by the many clinicians and scientists stewarding FD research initiatives. There is meaningful progress being made on all fronts, and we are so grateful to all those who are working so hard to ensure longer and better-quality lives for people living with familial dysautonomia.

If you missed FD Day, we encourage you to watch the recorded presentations now available on the Foundation’s website at www.famdys.org/fdday25.

The entire Clinical Session of FD Day was devoted to sharing the exciting preliminary findings from the first patient enrolled in the ASO study. Drs. **Horacio Kaufmann** and **Alejandra Gonzalez-Duarte** are managing the study in collaboration with **Dr. Adrian Krainer** and the **n-lorem Foundation**, which developed the ASO for FD and is providing the drug for the study at no cost. (See pages 3-4 for more a detailed update.)

During the Research Session, we heard the latest updates from scientists exploring additional approaches to increasing ELP1 protein in FD neurons including:

Dr. Susan Slaughaupt, whose efforts to develop a **small molecule** therapeutic treatment continue to show promise,

Dr. Anil Chekuri, who carries on the **gene therapy** research initiated by **Dr. Frances Lefcort**, and **Drs. Elisabetta Morini** and **Christiano Alves**, who are pursuing a CRISPR (gene editing) method.

And finally, **Adam Sachs**, who was instrumental in founding **Tikun Therapeutics**, provided an update on this subsidiary of the FD Foundation, created solely to advance FD research.

We don’t want to miss the opportunity to add special thanks to our incredibly talented and dedicated Scientific Advisory Board, who keep us on track and ensure continued forward momentum, and especially to SAB co-chairs **Frances Lefcort**, **Adrian Gilbert** and **Adam Sachs** who give so generously of their time and talent. We are especially fortunate, as an ultra-rare disease community, that so many brilliant minds are working on solutions to the challenges presented by FD.

None of this important work could happen without the generous support of our community. We encourage you to participate in our fundraising initiatives, including the **Crowdfunding Campaign** now underway, and our upcoming **2026 Journal**, as we depend on your philanthropy to sustain all the Foundation’s work, including the essential research described above.

We wish you a Happy Thanksgiving and a peaceful and joyous Holiday Season,

Faye Ginsburg
President

Lanie Etkind
Executive Director

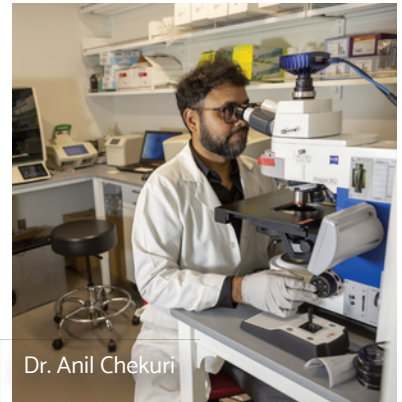
UPDATE ON TWO YOUNG INVESTIGATOR GRANTS UNDERWRITTEN BY THE CLARE AND PHILIP WEXLER RESEARCH FUND

Our readers may recall that in the last issue of Dys/Course (Spring 2025), we reported that the Foundation made a major investment in the future of FD Research. Thanks to the support of the **Clare and Philip Wexler Research Fund**, the Foundation provided grants to two early career scientists, encouraging their continued work in FD. We are delighted to share updates on their respective projects.

EXCITING PROGRESS IN GENE THERAPY

Dr. Anil Chekuri at Schepens Eye Research Institute in Boston is working diligently to support the development of a gene therapy approach to rescue retinal ganglion cell (RGC) loss and thereby improve vision in FD patients. Anil joined Scientific Advisory Board member **Sue Slaugenhaupt's** lab at Harvard Mass General as a postdoc in 2019 and then transitioned to a faculty member in 2020. "Dr. Slaugenhaupt was my mentor who introduced me to the world of FD," he explains. "Now I have my own lab, and with that comes federal funding." With a \$60,000 grant from the Clare and Philip Wexler Research Fund, Dr. Chekuri is currently fine-tuning a therapy to restore vision loss in FD patients.

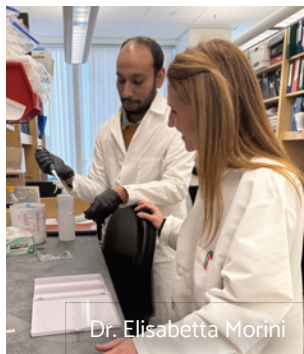
According to Anil, "One of the most devastating symptoms of FD is vision loss, which typically starts for people with FD in their teens, and continues to progress throughout their lifetimes. Since people with FD also have fragile balance, vision loss is even more debilitating." Progressive death of RGCs in FD leads to breakdown in communication between the retina and the brain in people with FD, and that's where Anil's lab comes into play. He explains, "We are now trying to rescue these RGCs through gene therapy. We've generated a vector to replenish the ELP1 protein in the retina that we are testing in my lab on mice." So far, they have discovered exciting results, with preliminary data showing that they can indeed rescue functional vision in these mice, which have the same visual dysfunction that mimics what is seen in FD patients. Adds Anil, "The FD mice showed improved visual function with administration of our gene therapy vector, and we have generated a key piece of data that we will use to file an FDA application for translating these findings for clinical use. Looking ahead, I am very optimistic!"



Dr. Anil Chekuri

A "ONE AND DONE" CRISPR APPROACH TO PREVENT BLINDNESS IN FD

After moving from Italy to Boston in 2010 as a visiting PhD student, **Dr. Elisabetta Morini** joined **Dr. Susan Slaugenhaupt's** lab in 2012, and she has been dedicated to FD research ever since. Now as an Assistant Professor of Neurology at MGH and Harvard Medical School, Dr. Morini is leading an ambitious project to create a permanent, one-time treatment for FD using cutting-edge gene editing technology, popularly known as CRISPR.



Dr. Elisabetta Morini

Supported by a \$60,000 Wexler Research Fund grant, her team is working to permanently fix the genetic mutation that causes FD by restoring the normal function of the ELP1 gene. "We've made great progress in the past few months," Dr. Morini shares. "We're testing our approach in human neuronal cells and in FD mouse models, focusing first on the retina with the goal of preventing or halting blindness."

Vision loss is one of the most devastating symptoms of FD, significantly impacting quality of life. There are currently no available treatments to stop this progression. Dr. Morini's project uses base editors (BEs), precision tools that "search and replace" the faulty letter in the DNA sequence, to correct the mutation at its source. Her team has already shown in cell models that fixing the mutation restores normal ELP1 splicing and protein function, and they are now testing whether this strategy can stop retinal degeneration in FD mice.

Her team recently welcomed the research technician **Samikshya Pokharel**, who is helping to accelerate this work. "Looking ahead, I'm confident that we can make a lasting difference," says Dr. Morini. "If we can prevent blindness, we can dramatically improve the lives of FD patients."

A NEW HOPE FOR FAMILIAL DYSAUTONOMIA: THE STORY OF THE FIRST ANTISENSE OLIGONUCLEOTIDE THERAPY

By Dr. Alejandra González-Duarte and the NYU Dysautonomia Center Team

A Dream Begins

For decades, doctors and families have dreamed of finding a way to treat Familial Dysautonomia (FD) at its root cause. FD is a rare genetic condition that affects the sensory and autonomic nerves – the ones that help us feel pain, control blood pressure, and regulate body temperature. At the heart of FD lies a faulty gene called ELP1, which prevents nerve cells from developing and surviving properly.

The idea behind the new treatment was simple but powerful: what if we could boost the production of ELP1? At first, the notion of treating FD with a genetic therapy seemed like science fiction. The disease is incredibly rare and developing a multimillion-dollar treatment for just a few patients scattered around the world required determination and creativity. Still, the development of antisense oligonucleotides (ASOs) – tiny, tailor-made pieces of genetic material that help the body read the ELP1 gene correctly – was becoming popular. It represented a new era: one where the disease could be addressed not just symptom by symptom, but at its genetic origin.

By then, the concept of restoring ELP1 had already taken shape in the laboratory. Pioneering work by Dr. Susan A. Slaugenhaupt and Dr. Frances Lefcort first demonstrated that it was possible to increase ELP1 expression in affected neurons – a discovery that opened the door to targeting the disease at its genetic root. Building on this foundation, their next generation of talented researchers, including Dr. Elisabetta Morini and Dr. Anil Chekuri, went a step further and showed that restoring ELP1 levels could rescue nerve-cell structure and function in FD mouse models.

Those results transformed what had once seemed a dream into a tangible path toward treatment: there was biological proof that at least some of the damage caused by FD might be reversible. In other words, if the right message reaches the neurons, they can begin to recover. This realization gave the scientific community the courage to take the next bold step – designing a special ASO for FD and testing it in humans.

Learning from Success: How SMA Paved the Way for FD

Dr. Adrian R. Krainer's laboratory first achieved a breakthrough in the treatment of Spinal Muscular Atrophy (SMA) by developing an ASO that corrected a genetic splicing defect to treat neurodegenerative diseases. Recognizing that a similar approach might apply to Familial Dysautonomia (FD), Dr. Horacio Kaufmann reached out to Krainer to explore the possibility of adapting this technology for FD. Their discussions evolved into a long-term collaboration aimed at using antisense therapy to restore the expression of the faulty ELP1 gene in FD.

Although SMA and FD are distinct diseases, they share important similarities that made this idea possible. Both are genetic disorders of the nervous system that begin in early life and cause the progressive loss of motor and sensory neurons—the specialized nerve cells responsible for movement and sensation. In both cases, the disease results from a splicing error in a critical gene that prevents cells from producing enough of the protein needed for neurons to survive.

Because Dr. Krainer's ASO therapy for SMA was remarkably successful, it offered a powerful model for FD. The principle was the same: if an ASO could help cells read the genetic message

correctly, it could increase the amount of normal protein and potentially restore nerve function. This biological similarity gave both teams the confidence that an ASO could be the key to changing the course of FD. To move from concept to action, they then engaged the n-Lorem Foundation, a nonprofit organization dedicated to developing personalized ASO therapies for ultra-rare diseases.

From that point forward, everything began moving at once. Biweekly meetings brought together the n-Lorem Foundation and the NYU Dysautonomia Center to align goals, share results, and keep the project advancing in step. Behind the scenes, multiple teams were working simultaneously: pre-clinical toxicology studies tested the ASO in FD mouse models to determine the safest and most effective dose and dosing interval, while others led the communication with the U.S. Food and Drug Administration (FDA) to navigate the regulatory process that would ultimately authorize the first administration of this ASO in a person with Familial Dysautonomia. At the same time, the clinical protocol was being developed in parallel, ensuring that every detail—from safety monitoring to outcome measures—was ready for translation from the laboratory to the clinic.

OMIA CENTER AT NYU LANGONE

The Journey of Patient One

When the protocol was finally ready, the next step was finding a volunteer. Everyone was excited, but there was hesitation too – nobody wanted to be the first patient, though everyone hoped to see that it worked. After careful discussions and an act of incredible courage, one family stepped forward. The case was presented to the U.S. Food and Drug Administration (FDA), which granted approval for this single-patient trial.

Every few months, the patient traveled to New York City, where the team at the NYU Dysautonomia Center carefully monitored every step of the process. Each visit meant days of testing – neurological exams, balance and thermal studies, tiny skin biopsies, hospital admissions, and operating room procedures under anesthesia for the intrathecal injection.

At first, the medication was given every two months, later every four. Slowly but surely, the effects began to appear. After nine months, the improvements were undeniable. The patient walked faster and steadier, moved his hands and feet with greater precision, and required fewer medications to control autonomic crises. Specialized testing confirmed better balance and coordination, even with eyes closed.

But the most astonishing finding came from the skin biopsies. When doctors compared samples taken before treatment with those taken nine months later, they saw something never observed before in FD – small nerve fibers growing in the skin of the thigh and leg. For a disease defined by nerve loss, this was nothing short of extraordinary. The patient slowly began doing more regular activities – attending camp, spending time with friends, and even beginning to think seriously about college.

Encouraged by these results, the FDA authorized the same process for a second patient, whose treatment is now underway. This next step is crucial: if both patients show similar improvements, it will confirm that the medication's effects are reproducible – a key step toward broader approval.

The journey, however, is far from over. Each case remains extraordinarily costly, involving hospital procedures, specialized testing, and rigorous regulatory oversight. To move forward, the Foundation and the medical team must raise one million dollars to treat at least ten patients – the minimum number required to even be considered for full approval of the medication for FD. This milestone is critical: it is the bridge between promise and access, between an experimental therapy and one that can reach every patient in need.

For the first time, there is real evidence that Familial Dysautonomia might be treatable – that its story can, at last, be rewritten. The first antisense therapy for FD is more than a medical milestone – it is a testament to perseverance and collective strength: to the researchers and doctors who refused to give up, to the families and friends who have stood by this cause for decades – giving their time, their resources, and their faith – and to the patients whose courage continues to guide us forward.

VISITING MD PUBLISHES A COMPREHENSIVE OVERVIEW OF FD RESEARCH

Dr. Margarita Grobocopatel Marra, a physician visiting from Argentina, published a paper in the July 2025 issue of Expert Review of Neurotherapeutics entitled, “Advances in the treatment of familial dysautonomia: what does the future hold?” Dr. Grobocopatel, whose residency at the Dysautonomia Center at NYU Langone was funded in part by a generous FD family, conducted a thorough search and review of publications for all types of studies regarding the genetic basis of FD and recent advances in the development of disease-modifying therapies. Her paper, written in collaboration with **Mechteld Kuijpers**, **Horacio Kaufmann, MD** and **Alejandra Gonzalez-Duarte, MD**, provides a comprehensive overview of current FD research including small molecule and genetic therapy, as well as the ASO clinical trial currently underway. To read the paper, visit <https://bit.ly/AdvancesinFD>.



Dr. Margarita Grobocopatel Marra

CONVERSATION WITH LILY ARMSTRONG, MENTAL HEALTH COUNSELOR

Hello! My name is Lily Armstrong, and I have been providing mental health counseling services to the FD community for over six years. I want to share a wonderful conversation I had with a bright and insightful young woman, Gabi Jassie. Gabi describes herself as a “creative soul who loves to help others express their inner light and find beauty in growth.” It was a gift to highlight one of the many bright voices in the FD community and hear about her strategies and challenges managing her mental health.



Gabi, what made you initially seek counseling services?

“I initially sought counseling services when I was younger because I used to believe I had to suppress my feelings. Experiencing emotions felt overwhelming, and I struggled to cope with them. It wasn’t until I was in school, preparing for tests, that I began to understand what was happening. I would worry excessively about exams, and a teacher pointed out that what I was experiencing was anxiety. This realization was new to me, and it took some time to figure out how to address it. That’s when I decided to seek counseling. My hope was that by discussing these big and complicated feelings in a safe place, I could gain a better understanding of myself. It took some time, but I was able to develop coping strategies to manage my anxiety and identify my triggers early on. This gave me healthy ways to respond to them early without feeling too overwhelmed by them.”

I like this idea of having coping strategies for your anxiety - sounds really smart! Can you offer your definition of anxiety?

“I think it started for me with overthinking things. I couldn’t really let something go and it was difficult for me to move on from certain challenges. I didn’t feel confident in what I was feeling or experiencing. But this had a big impact on my body, and I realized I needed to share things more often. I would describe the feeling as hard to breathe. I couldn’t get a deep breath in, [and was] worrying a lot about things that I couldn’t do anything about. I was feeling fearful and anticipating stress even if it was a ways away.”

We often talk about anxiety like an alarm system. I find this language helpful, because an alarm indicates something needs attention, but we can evaluate how important the problem really is. Then we don’t feel trapped by the feeling.

“Yeah, it’s taken me a long while to trust my process with understanding this feeling. I’ve had to grow a lot in my intuition of problems and situations.”

What do you think people misunderstand about talk therapy?

“I do have some thoughts on this, especially regarding the idea that “talking to a therapist is not a sign of being crazy!” Initially, I questioned if my problems were significant enough to pursue therapy. However, I soon realized that the value of therapy lies in having a dedicated, neutral space to articulate feelings and be genuinely heard. This aspect has always been deeply appreciated. I grew up thinking that my feelings were dangerous because I could get sick from them or hospitalized if I couldn’t physically handle them and my blood pressure would get too high. I’m also reminded of a personal experience from my younger years. I used to try to hold my breath to avoid anxiety, only to pass out from lack of oxygen. It took me a long time to learn how to manage my anxiety without making myself ill.

Therapy offers something distinct from close friendships. While friendships are incredibly important, therapy provides unique perspectives and advocacy that may not always be present in those relationships. It can even strengthen significant personal connections by fostering self-awareness and clarity about one’s identity.”

I appreciate you bringing this up, as it’s something that resonates with me in other contexts as well, and I believe you articulated it very clearly. Therapy and friendships can be mutually supportive and are not substitutes for one another.

Are you open to sharing something more personal? What’s been an eye-opening realization about yourself recently?

“I’ve realized how much unaddressed emotions and the compartmentalization of my mental health have taken a physical toll on my body. As I started to pay more attention to myself, I began noticing things I hadn’t before, such as specific triggers and the importance of listening to my body and intuition. Previously, I wasn’t creating that space for myself, and everything would get pushed aside. However, these feelings never truly disappeared, leading me to become disproportionately upset about trivial matters when I was actually neglecting my body’s needs regarding more significant issues. This experience has highlighted the immense importance of self-awareness in my life.”

Thank you for our conversation. I continue to be inspired by this community, and you are a significant part of that inspiration.

“I wanted to express my sincere gratitude for the support I receive from the FD Foundation and for the opportunity to work with you. It truly means a lot to have someone who understands what FD looks like and offers non-judgmental support.”

“I am also deeply grateful to the Schwartzberg family in Canada. Their vision for this program and their sacrifices to keep it funded are inspiring. Life can often feel hectic, and knowing I have an advocate through this program has made some of the more overwhelming aspects feel much more manageable.”

I’m glad you mentioned the Schwartzberg family. Their generosity and vision for this program make me feel incredibly honored to participate. I hope this interview helps others better understand how they might benefit from therapy and feel less intimidated by the process.

COMEDY NIGHT IN TORONTO BENEFITS THE STEVIE SCHWARTZBERG MENTAL HEALTH COUNSELING PROGRAM

The Schwartzberg family hosted a Comedy Night event at Second City Toronto on October 26, raising CA\$60,000 to sustain the **Stevie Schwartzberg Mental Health Counseling Program**. The program, which offers virtual counseling through the Dysautonomia Center at NYU, was established in 2019 by the Schwartzbergs in memory of their son Stevie.

THANK YOU TO OUR SPONSORS			
LAUGH FOR FD OCTOBER 26 SECOND CITY, TORONTO			STEVIE SCHWARTZBERG MENTAL HEALTH COUNSELING PROGRAM
HEADLINER	STANDUP	OPENER	ONE-LINERS
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Jack, Daryl and Rowena Schwartzberg



Daryl Schwartzberg



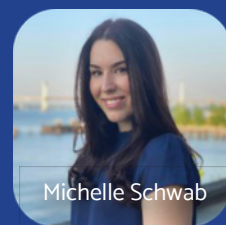
Second City cast



Florence and Nelson Asinowski (Montreal Chapter) with Lanie Etkind (FD Foundation)

KVELLING:

- We are beyond proud of **Faye Ginsburg**, who was recently elected to the **American Academy of Arts & Sciences**. The Academy, founded in 1780, is both an honorary society that recognizes and celebrates the excellence of its members and an independent research center convening leaders from across disciplines, professions, and perspectives to address significant challenges. Never one to rest on her laurels, Faye was also nominated by her peers and students for the **2025 Lifetime Achievement Award from the Society for Visual Anthropology**. Faye also received the CUNY Graduate Center President's Distinguished Alumni Medal – the institution's highest alumni honor.
- Mazel tov to **Elise** and **Robert Goldblat** on celebrating their 50th Anniversary. In lieu of gifts, the family requested donations to the FD Foundation in memory of their daughter, **Jamie**.
- Farewell, thanks and best wishes to **Mecky Kuijpers**, who left her role as Research Assistant at the NYU Dysautonomia Center to earn a PhD in Neuroscience at Dartmouth.
- Best of luck to **Michelle Schwab**, who facilitates the Foundation's HangOuts and Game Nights, who started a PhD program this fall studying counseling at Montclair State University.
- We are so excited for our good friends **Rick Guidotti** and **Positive Exposure**, who moved to a beautiful new gallery space at 83 Maiden Lane earlier this year. Rick invites everyone to stop by and visit his new space and view the latest installation. www.positiveexposure.org.
- Best wishes to **Fred Myers** on his retirement as a Professor of Anthropology at NYU. In his newfound "free time," Fred is continuing his lifelong research with Indigenous Pintupi people of central Australia, helping curate a show of their world-famous art, opening at the NYU's Grey Art Museum in January.
- Mazel tov to FD parent and Foundation VP **Paul Wexler** who, for the third year in a row, was awarded Honorable Mention for the Lifetime Achievement Award, presented by Invest in Others. Paul's nomination was evaluated based on leadership, dedication, contribution, inspiration, and impact. The FD Foundation will receive a \$2,000 donation from Invest in Others in recognition of Paul's charitable work!
- Happy (belated) Birthday to **Jason Gross** who celebrated his 50th on August 29th with a dinner party for about 30 friends and family at the Spruce House (where he shares a 2-bedroom apartment with his brother **Kevin**) in Hanover, NH!



- Congratulations to **Lee-Ann Lugg**, Program Coordinator at the NYU Dysautonomia Center, who earned an MPH in epidemiology this spring from NYU.
- Kudos to visiting physician **Margarita Grobocopatel Marra, MD** for her paper, “Advances in the treatment of familial dysautonomia: what does the future hold?” published in the July 2025 issue of Expert Review of Neurotherapeutics [see article page 4].
- Hooray for **Frannie Cohen**, who appeared live on the morning show “Rising” on WCCB-TV in Charlotte, NC on international coffee day to talk about her job as a barista at Bitty and Beau’s. (To view the clip, visit <https://tinyurl.com/fCohen>).



Lee-Ann Lugg



SHOPPING NIGHT FOR FD DRAWS A CROWD

On October 16, the **Goldberger family** hosted a shopping night at Alice + Olivia on Madison Ave. in NYC. Family and friends enjoyed a fun night of shopping, style and philanthropy, with a percentage of purchases made benefiting the FD Foundation. There was great turnout, and the event raised approximately \$20,000—very fashionable, indeed!



L to R: Kyle Winik, Carole Goldberger, Perry Goldberger, Jamie Fried



L to R: Pam Fried, Laurie Goldberger, Lisa Ganz, Robin Fiddle Posnack, Debbie Ganz



Dr. Felicia Axelrod and Perry Goldberger

FD DAY 2025 SHOWCASED OUTSTANDING PROGRESS IN FD RESEARCH AND CELEBRATED THE STRENGTHS OF OUR COMMUNITY



If you joined us for the Foundation’s 40th Annual FD Day on Sunday, June 8, you were surely blown away by the tremendous progress reported on the many research initiatives underway.

Drs. Horacio Kaufmann and **Alejandra Gonzalez-Duarte** and the clinical team at NYU Langone reported exciting results from Year One of the ASO Clinical Study.

We heard encouraging news on other therapeutics in the pipeline from **Dr. Anil Chekuri** (gene therapy), **Dr. Elisabetta Morini** (CRISPR gene editing), and **Dr. Susan Slaughaupt** (small splicing molecule). And **Adam Sachs** shared an update on **Tikun Therapeutics**.

Dr. Patricio Millar moderated the Q & A session with clinicians and researchers, and our Counseling Team (**Matthew Hertzberg**, **Michelle Schwab** and **Lily Armstrong**) reported on their supportive work with the FD community.

Congratulations to:

Distinguished Award Winners: **Chanoch Pearl** and **Frannie Cohen**

All who participated in the Art Competition, including **Mara Clawson** (1st place), **Justin Sachs** (2nd place), and **Peter Sonenshein** (3rd place).

FD Champion Award presented to: **Rick Guidotti**

If you missed this year’s program, or wish to re-watch (or share) any segment, please visit the Foundation’s website at www.famdys.org/fdday25.

Thank You to Our FD Day 2025 Sponsors			
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FD PARENT BARRIE RAPPAPORT IS A RARE ADVOCATE (AND YOU CAN BE ONE TOO!)

We all lead busy lives. work, family, maybe even try to eke out a little time for ourselves. but we also know that when something is a priority, we somehow make space for it in our lives. When **Barrie Rappaport**, parent of **Michelle**, learned that **Rare Across America**, sponsored by the **EveryLife Foundation for Rare Diseases**, was seeking advocates to meet with Congressional representatives in their local offices, she signed up. Through its **Rare Disease Legislative Advocates (RDLA) program**, the EveryLife Foundation supports advocacy of all rare disease patients and organizations, espousing the belief that every voice matters and that patients are the key to changing public policy. In her role as rare disease advocate, Barrie has had the opportunity to meet with the local staff of Senators Tammy Duckworth and Dick Durban and with staffers from the office of her local Congressional representative, Brad Schneider. Says Barrie, “I was even given the opportunity to speak during a meeting with Senator Duckworth’s office, which was ‘cool’.” RDLA has also connected Barrie with the Illinois Rare Diseases Commission, whose purpose is to “increase awareness of rare disorders that impact the lives of Illinois residents..” Barrie has found her involvement with RDLA to be very meaningful, and she encourages other parents, family members and friends to get involved in their respective communities.

To learn more, visit <https://everylifefoundation.org/rare-advocates>



Want to make a difference right now? Join us in our efforts to urge Congress to pass the Give Kids a Chance Act to reauthorize the Rare Pediatric Disease Priority Review Voucher Program through 2029. <https://rarediseases.org/driving-policy/take-action/#/260>



Nancy Mills, Dorit Kransdorf, Rachel Schlau (and caddy)



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SUNSHINE, SWINGS AND SUCCESS AT THE 2025 FD GOLF CLASSIC

You can count on the FD Golf Classic to offer an exciting day filled with golf, delicious food and drink, fun raffles, and tempting silent and live auctions. This year was no exception. Held on Monday, August 18, at Glen Oaks Club on Long Island, the 28th Annual FD Golf Classic, reached new heights, bringing in more than **\$350,000** to support the Familial Dysautonomia Foundation! We are grateful for the tireless efforts of our event chairs **Paul Wexler, Steve Fass, Steve Kietz** and **Rachel Schlau**, and for our lead sponsor, the **Kopelman Foundation**, as well as for all those who golfed, sponsored, donated and/or purchased a raffle ticket or auction prize. We hope to see you at our 29th annual event in 2026 – watch for a date to be announced soon!



NYU Clinical Team



Josh Kietz and Mandi Bergenfeld



Foundation Staff and Volunteers

A NEW WAY TO GIVE BACK

When FD board member **Brian Stillman**, proud dad of **Pyper** (and **Zoey**), relocated with his family from Arizona to Pacific Palisades last winter, he had no idea that three weeks later their house would burn down in the massive wildfires. After living temporarily with his wife Stephanie's parents on Coronado Island, Brian and his family ended up purchasing a new house in Los Angeles. Little did he know at the time that FD would benefit from the move. Brian and Stephanie soon learned that the real estate company they used, **Amalfi Estates**, has a long-standing policy of donating 10% of their net commissions to charity upon closing, so naturally Brian educated the broker, **Max Marguleas**, about FD, and the Foundation received a sizable check. Says Max, "Our goal is to be a philanthropic company that excels at selling real estate. Alongside our commitment to our clients stands our commitment to our community and we are thrilled to give a portion of our commissions to charity." The company has given back over 2 million dollars to charities over the years and Max hopes other companies will be inspired to give back to their own communities as well. Adds Max, "My father started our firm 30 years ago in Southern California. We wondered how we could make a difference and were one of the first real estate companies in our area doing this and now we are seeing others follow suit. Because I'm Jewish, when I learned more about FD after jumping on a zoom call, I was very inspired to include the Foundation as a beneficiary and I am proud to consider the Stillmans my new friends!"



Zoey Pyper, Stephanie and Brian Stillman with Max Marguleas (second from right)

TEAM FD RUNS THE 2025 TCS NYC MARATHON

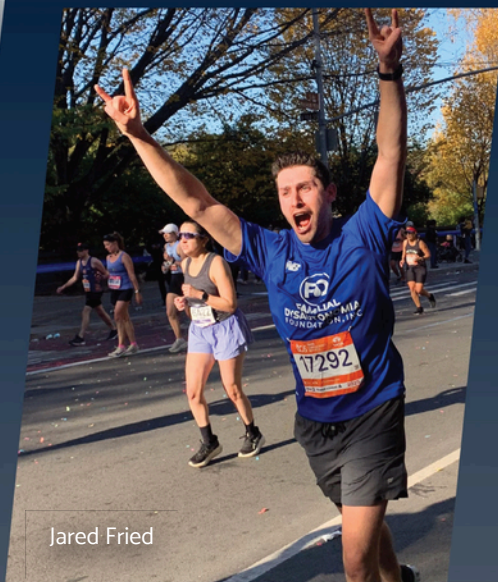


Eight members of **TEAMFD** ran the **TCS NYC Marathon** on Sunday, November 2nd. The Foundation's cheering section gathered at Mile 23 on 5th Avenue to encourage our runners, which included a mix of first-timers and veteran marathoners. Together Team FD raised close to \$73,000 for the FD Foundation.

Thank you TeamFD: **Adrian Hernandez-Liebo** (ran for **Josh Kietz**), **Benjamin Breitel** (ran for **Perry Goldberger**), **Dillon Shinder** (ran for **Jack Posnack**), **Jack Roseman** (ran for **Perry Goldberger**), **Jared Fried** (ran for **Perry Goldberger**), **Jeremy Cohen** (ran for the **Goldberger** and **Eisenson** families), **Jordan Figdor** (ran for **Sam Landau**), and **Matthew Hersey** (ran for **Sam Landau** and in memory of **Evan Kaplan**).



Jeremy Cohen



Jared Fried



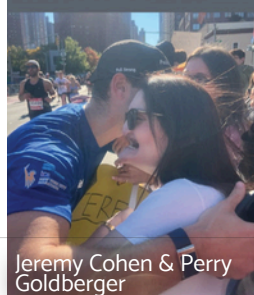
Dillon Shinder with his sister Rachel and friend.



Adrian Liebo



Team FD Cheering Section at Mile 23



Jeremy Cohen & Perry Goldberger



L to R: Bebe Landau, Jordan Figdor, Sam Landau, Matt Hersey

TEAM FD SPOTS AVAILABLE FOR NYC HALF MARATHON ON MARCH 15, 2026

For details or to reserve your spot, please email us at info@famdys.org



GRATITUDE TO:

- Please join us in thanking **Rowena, Jack and Daryl Schwartzberg** for organizing a Comedy Night fundraiser in Toronto on October 26 to benefit the Stevie Schwartzberg Mental Health Counseling Program. We are also grateful to **Florence and Nelson Asinowski** who manage the Montreal Chapter of Dysautonomia, for their crucial behind-the-scenes support of this event (See article page 6).
- We acknowledge and thank **The Slomo and Cindy Silvian Foundation** for their renewed grant of \$15,000 to support the Mental Health Counseling Program offered through the Dysautonomia Center at NYU Langone.
- Our heartfelt gratitude to FD parent **Mike Zucker** who, for the 6th year in a row, has organized a fall bike ride with his friends to raise funds and awareness for FD in honor of his daughter, **Sarah**. This year's ride, which took place over two days and covered over 100 miles, raised more than \$11,000.
- We are grateful to LA realtor **Max Marguleas** of **Amalfi Estates** for sending a donation representing 10% of his commission for selling a house to the **Stillman family** in Los Angeles (see article page 10).
- Many thanks to **Jason Joseph** for hosting a YouTube fundraiser for FD in memory of his brother, Justin (read more on page 13).



LONGTIME FD SUPPORTER JOINS Forever DEVOTED

Many thanks to **David Gelley**, the newest member of our Forever Devoted Society, for including the FD Foundation in his estate plans. Now 68 years old, David has been friends with FD board member **Paul Wexler** since the second grade. David is the FD Foundation's sole commercial printer. He prints all the Foundation's materials at cost including the annual commemorative Journal, the Forever Devoted brochure and this semi-annual Dyscourse newsletter, and he completely donates all of the printing for the golf outing each year.

According to David, who has lived in Las Vegas for the last 6 years, "I needed to update my will and decided to choose FD as a beneficiary of my estate since I'm divorced and don't have any children. Paul manages my money, and I wanted him to be aware that I chose FD since what he and Karen have done for the community is just amazing. I hope to inspire others to do the same!" Adds David, "It feels good to give back. If you can do it, why not? It's obviously a great cause!"

You, too, can invest in the future of the FD Foundation by including us in your estate plans.

Pledging 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.

JASON JOSEPH HOSTS YOUTUBE FUNDRAISER FOR FD

Meet **Jason Joseph**. Jason graduated from Rowan University with a degree in Sports Communication and Media. Having grown up outside of Philadelphia, Jason is a huge fan of all Philly sports teams. During a post-graduation studio internship at NFL films during the 2023-23 season, Jason launched his YouTube Channel called Play-By-Play with JJ (<https://www.youtube.com/@PBPWithJJ>), where he calls 76ers, Phillies and Eagles games.



Jason is also the brother of **Mitchell**, who had FD and passed away in 2020. On September 18 and 19, Jason hosted his first fundraising and awareness campaign for FD on his YouTube Channel. During two twelve-hour live streams, Jason remembered Mitchell with stories and memories and educated his audience (7,000+ followers!) about familial dysautonomia. Jason was proud to have raised more than \$3000 so far, with additional funds still anticipated to come through YouTube, far exceeding his expectations for his first fundraising effort.

Said Jason, “I’ve always wanted to incorporate charity work into my channel and find a way to connect it with my sports content. This cause really hits home for me because it’s personal: Mitchell played a significant role in my life. By sharing his story, I hope to honor his legacy and show my audience who I am and what I stand for.”

“Mitchell was the heart of our family. Honoring him during September felt absolutely right. He loved the Phillie Phanatic, baseball, and football, and he loved being the center of attention. If the spotlight wasn’t on him, he’d make sure it was—he had a knack for stealing the show!” Jason continued, “Throughout the two-day charity stream, we shared stories about Mitchell, screened the documentary I made about him in high school, and interviewed Lanie Etkind from the FD Foundation. This allowed me to provide meaningful content focused on Mitchell and the Foundation. I learned so much from this experience and am incredibly proud of how much we raised and the awareness we spread.”

Jason’s mom **Lisa Joseph** commented, “Jason knows other podcasters who have done various fundraisers, but he wanted to do something personal. He chose FD to honor his brother and spread awareness. His audience was so interested and supportive of him that it was a very successful endeavor, much more than Jason expected!”

Lisa added, “Jason has a couple close friends with FD who he sees and keeps in touch with who he knew this would benefit as well. They were part of this vision—they are sports enthusiasts on his channel. I credit Jason with taking his idea and running with it. It was a beautiful uplifting and meaningful way to align his interests with his audience in his realm. To be a sibling and make a difference has to be rewarding for him and of course a source of immeasurable pride to us, his parents.”

You can subscribe to Jason’s YouTube channel at <https://www.youtube.com/@pbpwithjj>.

If you have an idea for a fundraising and/or awareness campaign for FD, we’d love to help you launch it! Please contact the FD Foundation team at info@famdys.org or 212-279-1066.

TAX CONSIDERATIONS FOR YEAR-END GIVING

Recent tax law changes passed under the “One Big Beautiful Bill Act” (OBBBA) make 2025 a strategic year for philanthropy, with new rules taking effect in 2026 for both itemizing and non-itemizing taxpayers. Donors can plan now to maximize tax benefits, particularly with strategies like accelerating donations into 2025 or using a Donor-Advised Fund (DAF).

Visit <https://familialdysautonomia.org/TAX-WISEGIVING> to learn more.





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)

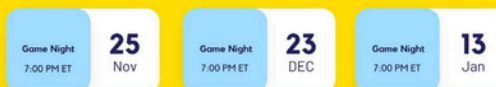
If you are interested in participating in a hangout group and would like to [register](#), please contact Michelle at info@famdys.org.

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 to receive a zoom invitation.



More 2026 Dates Coming Soon!

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:

Stefani Wexler Berdia

Jacquelyn Brace

Jessica Cook

Heather Marie Delanoy

Vivian Fisher

Mary A LaFountaine

Kay Stichauf

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

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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

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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.



DYS/COURSE 2025- 2

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2025



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