

NIH Programs and Patient Perspective



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National Institutes of Health

The mission of the NIH is to uncover new knowledge that will lead to better health by:

- Conducting research in its own laboratories (intramural)
- Providing support for research conducted by scientists in universities, medical schools, hospitals, and other research institutions throughout the country and abroad (extramural)
- Training research investigators
- Fostering the communication of medical information

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NHLBI

NCCAM

CIT

NCI

FIC

CSR

NCMHD

CC

NEI

NHGRI

NIA

NLM

NIAAA

NIAID

NCRR

NIGMS

NIMH

NINDS

NINR

NIAMS

NIEHS

NIDA

NIDCR

NIDDK

NICHHD

NIBIB

NIDCD

Cushing's syndrome research at NIH

- Endocrinologists (Drs Stratakis and Nieman) with separate projects
- Surgeons: Endocrine, Neurosurgery, Thoracic
- Radiologists: Interventional (IPSS) and Investigational (PET)
- Oncologists: Rare tumor clinic
- Access to unusual resources: F-DOPA and DOTATATE PET, metabolic chambers/hood, frequent blood sampling
- Mission is to conduct research; staff is salaried (no patient numbers goals)
- No charge to patients (your taxpayer \$ at work)
- Patient must fit protocol inclusion criteria– may be an interventional or hypothesis-driven study or may be standard of care
- Not all patients are accepted to protocols

How to enter a Cushing's syndrome study at NIH

- Have your physician send a referral letter to an investigator with your history, physical examination and laboratory results.
- The investigator will evaluate whether you fit a protocol, and will often send to a colleague if you don't fit his/her studies.
- The investigator will let you know what the study involves so you can decide if you want to participate.

A Patient's story: My experience at NIH

Amy Dahm

CSRF Summit

October 5, 2019

My NIH Milestones

- Pre- Cushing's
- Diagnosis & Discovery
- Assessment
- Testing
- Treatment
- Post- Op Care: Welcome to AI!
- One Year Post- Op: Reality Check
- Waking Up "Sleeping Beauty"
- Recovery
- Impact

Pre-Cushing's

- Foreign Service Officer in U.S. State Department
- Wharton MBA
- Staffer to Secretary of State, Operations Center, Public Affairs



Diagnosis

- Oct 2012- Gynecologist called me back into his office for high cholesterol
- Every time I turned around, something was wrong
- Could not even open a jar of pickles, walk
- Went to dozens of specialists
- Sept 2013- Dr. Mark Sklar in Washington, DC, ran the full battery of tests, “The only thing left is Cushing’s”
 - Diagnosis: adrenal Cushing’s

NIH Discovery

- Problem: Where do you go when no one knows about your disease?
 - Rule of Thumb: Go someplace with a high number of Cushing's patients
- Before diagnosis, Dr. Sklar made vague threats about “referring me to NIH”

Second Opinion & Assessment

- Oct 2013- Second opinion at Mayo with Dr. Todd Nippoldt w/ Mom as Notetaker
- November 2013 – Met with Dr. Stratakis, Dr. Zilbermint, and Bobby for two hours



Testing

- January 2013 – 10 days' of testing and assessments at 5 NW at NIH in Bethesda, MD
 - Dropped off Sunday night
 - First time I felt like I could relax
 - Testing schedule from Bobby
 - What to Bring
 - Complementary Therapies
 - Wholistic Approach – Msgr Dominic, Speech Path
 - Rehab Assessment – shower chair, U clamp
 - Dr. Ge
 - List of Questions
- Diagnosis Refined: Adrenal Cyclical Cushing's
 - Hair Test
 - Diurnal Cortisol

Treatment

- January 15, 2014 - Checked back in for surgery
- Various Therapies on “the Inside” – Occupational & Vocational Therapy, Rehab, Dietician, etc
- Dr. Naris Nilubol and Fellow performed a unilateral adrenalectomy (ULA) and removed my diseased left adrenal gland and tumor
- CPAP saved my life
- One night in ICU

Post-Op Care: Welcome to AI!

- Woke up to medical bracelet lying neatly on side of bed “What’s This?”
- Post-Op Care: Welcome to AI!
 - Leg Massagers to prevent blood clots
 - Breathing Game to prevent pneumonia
 - Injecting the Orange
 - “Sick Day Rules” protocol
 - Emergency Injection Kit
 - No alcohol, caffeine

One Year Post-Op

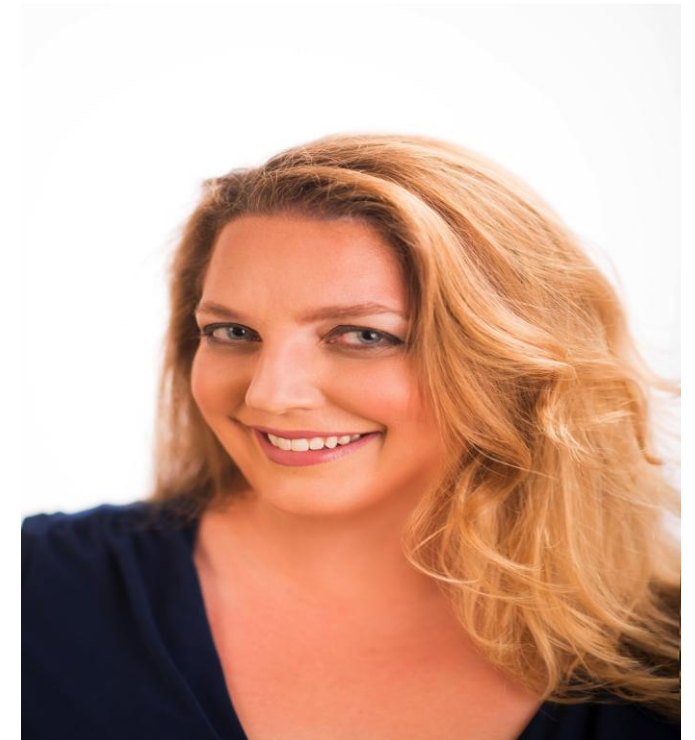
- One Year Check-Up With AI
- Dr. Stratakis told me I was going to have to “get more serious” about my recovery and dropping my hydrocortisone more quickly
 - Periodic stim tests – next stim test at 10 mg HC
- Went on medical leave from Hill Fellowship and State Department

Waking Up “Sleeping Beauty”

- January 2016 - After two years my right adrenal gland woke up!
Stimmed to 18
- Weaned off of daily steroids by May 2016
 - Dr. Stratakis warned me to follow the sick day protocols for at least a year afterwards while brain loops in

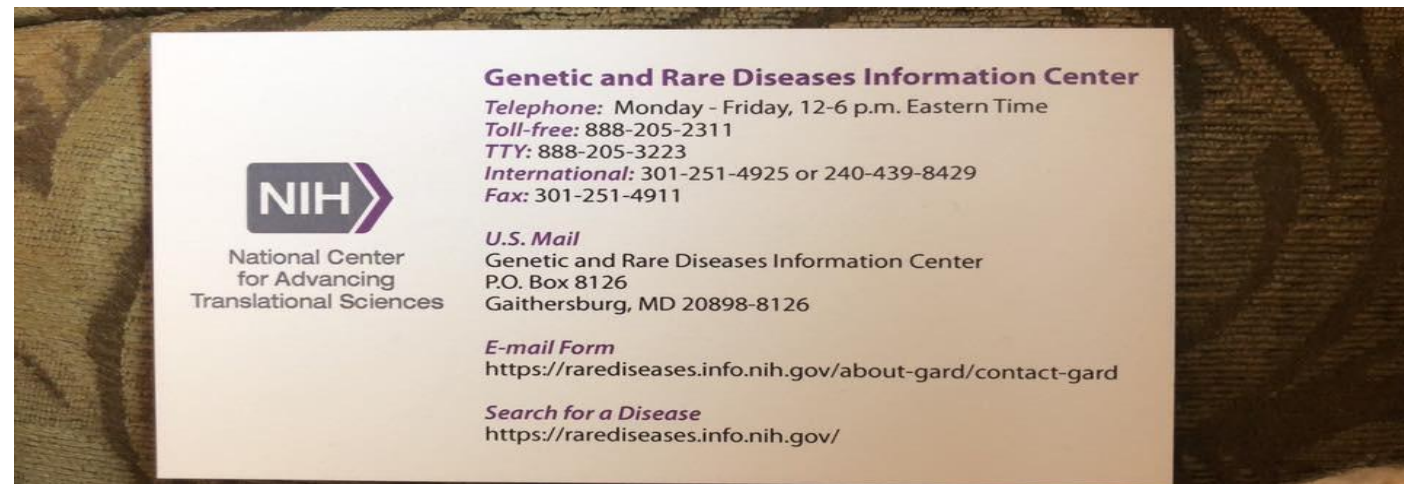
Recovery

- Medically retired from the State Department in 2017
 - Volunteer as patient advocate – write articles, appear on a podcast and radio, lobby Congress for additional funding
- Continue to Research and Help Others
- Pursue Avocations – travel, genealogy, writing, helping harassment victims



Additional NIH Resources

- NIH Library – anyone in the world can come and use it...for free
- NIH Rare Disease Hotline: 1-888-205-2311 or <https://rarediseases.info.nih.gov/about-gard/contact-gard>
- NIH Rare Tumor Center
- NIH Protocols



Impact

- Taught me Standards/ Best Practices for my illness
- Introduced me to CSRF
- Formed foundation of my advocacy
- Taught me importance of being “Captain of my own ship” and researching
- Brought me Back to Myself



The End

