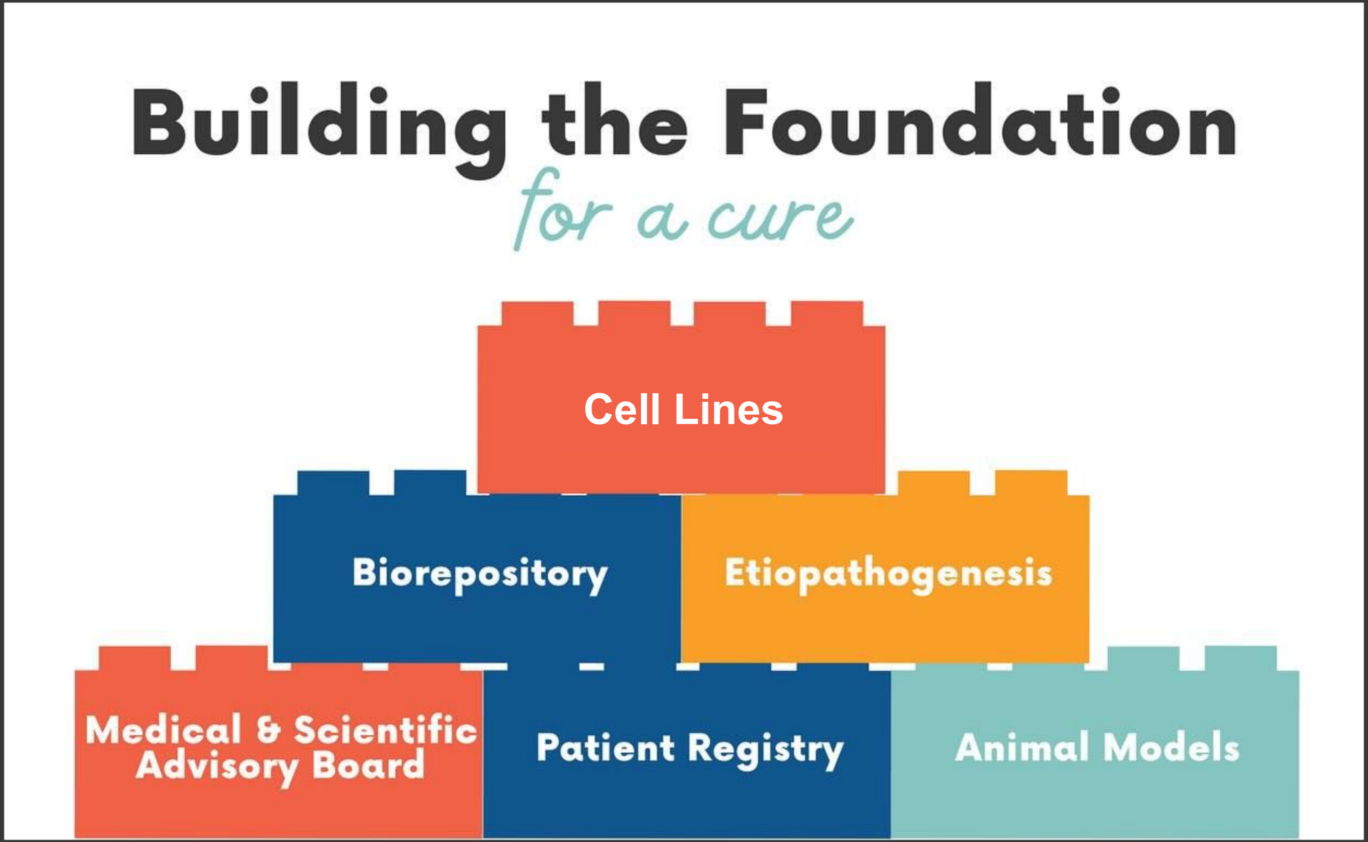
THE FUTURE OF

THE SPATA FOUNDATION


Building the Foundation
for a cure
Cell Lines
Biorepository
Etiopathogenesis
Medical & Scientific Advisory Board
Patient Registry
Animal Models

Current Projects & Research

Mouse Models | Jax Labs | SPATA5 & SPATA5L1

Drug Repurposing | Unravel Biosciences | SPATA5 & SPATA5L1

Tadpole models created & showing same symptoms. SPATA5: Toxicity Screening and First Efficacy Screening Done; Next is Final Efficacy Screen SPATA5L1: Toxicity Screening Done; Next is First Efficacy Screen

Mechanism Research | Tapas Mukherjee | SPATA5 & SPATA5L1

Natural History Study | Barbara Vona | SPATA5 & SPATA5L1

Current Researchers

Dr. Michael Kruer, MD & Team

Barrow Neurological Institute Phoenix Children's Hospital

Dr. Michael Buszczak, PhD & Team

Dept. of Molecular Biology UT Southwestern

Dr. Tapas Mukherjee, PhD

Dept. of Immunology University of Toronto

Dr. Barbara Vona, PhD & Team

Institute of Human Genetics Neuroscience

University Medical Center Göttingen

Dr. Roger Greenberg, MD, PhD, FRCP, FRCPath & Team

The Perelman School of Medicine at the University of Pennsylvania

Dr. Henry Houlden, MD, PhD & Team

Professor of Neurology and Neurogenetics at UCL (London)

Dr. Mark Hester, PhD

Institute for Genomic Medicine at Nationwide Children’s Hospital

Ruby Gupta

Neuroscience PhD Student | University of Tartu

Long-Term Goals

- **Treatment options for ALL those affected:**
    - **Drug Repurposing**
        - **Short-term more available treatment option**
        - **Treat symptoms NOW to improve quality of life**
    - **Gene Therapy**
        - **Long term “cure”**
    - **Other therapeutic options**
        - **ERT, ASO, mRNA**

DRUG THERAPY VS GENE THERAPY

DRUG THERAPY

Using pharmaceuticals to treat, prevent, and relieve symptoms associated with a disease.

COST

$90,000 for Discovery

May incur more costs if additional testing is needed

TIMEFRAME

5-6 Months for Discovery

If we find a drug already on the market & a clinical trial is necessary, Phase I & II of the trial would be bypassed and we'd only have to do Phase III which would most likely take a year or less.

Research has already shown drug therapy may be a very good treatment option

GENE THERAPY

Introducing a properly functioning copy of the gene (most likely just one time) in order to treat, cure or prevent disease.

COST

$2 Million +

Cost is very dependent on each phase of the process, and would be broken down by phase, but the overall cost of gene therapy development would be $2 Million or more.

TIMEFRAME

3-5 Years

Once the lab green lights the project, it would take at least 2 years to be clinical trial ready and an additional 2-3 years from then to potentially be available.

Gene therapy would target the neurological component of SPATA5 and SPATA5L1

Gene Therapy for SPATA5 and SPATA5L1

Is it feasible?

- Both SPATA5 and SPATA5L1 are within the size limits of AAV packaging, making them realistic and practical targets for gene therapy.

Is it coming?

- Currently in close discussion with three universities regarding potential collaboration for Gene Therapy

What stands in our way?

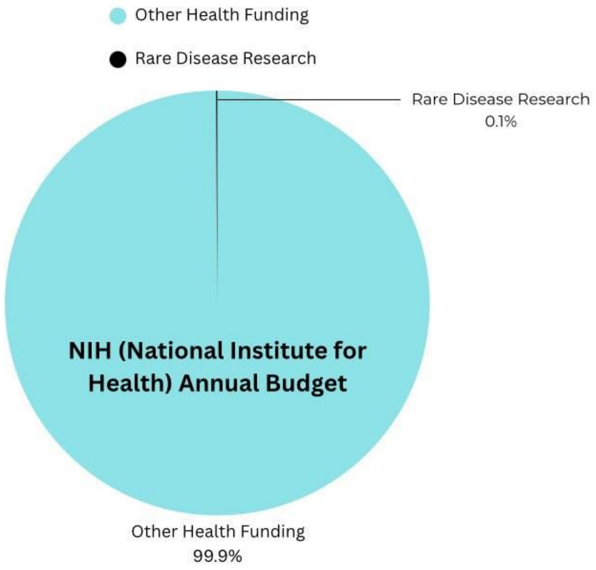
TIME

- **Time in General**
- **Unexpected setbacks**
- **Protocols**
- **Change of Direction**

MONEY

FREQUENTLY ASKED QUESTION

Why do Rare Disease Groups have to fundraise for research? Why won't the government fund it?



How can I help?

1. **Patience & Hope**
2. **Advocate**
    a. Stay informed about laws/initiatives that could benefit research and write your state representatives to encourage them to support these bills
3. **Be SOCIAL**
    a. Tag Te SPATA Foundation in relevant posts
4. **Fundraise**
    a. Share others fundraising efforts
    b. Host your own fundraiser
    c. Write to local fraternity organizations/orders (ie: Free Masons, Knights of Columbus, Elks)
    d. Sign up for RaiseRight (gift cards that give back)
    e. See if anyone you know works at a job that uses Benevity

Tell Your Story

- One of our biggest assets is our VOICE.
- Personal stories resonate with people and pull on the heart strings
- Share your story on social media. Reach out to news outlets.
- Let The Foundation use your photos/videos

You don’t have to put on a big production, you just have to tell your story.

2026 Goals

- Fund additional research initiatives
- Move into the next step of Drug Repurposing
- Begin creating tools for Gene Therapy (vector models, proof of concept)
- Database of all patients
  - Patient Count
  - Worldwide Patient Map

Family Reflection & Questions

This will be an open forum discussion for families to reflect on the day, ask questions, and seek advice from other families.

DINNER: 6:00 - 8:00PM