



www.phoenixnestbiotech.com

Phoenix Nest INC

Finding treatments for families.

ASGCT
May 10th, 2021
Patient Advocates'
Role in Advancing Gene
Therapy

When a pharma executive tells you:

“Your disease is so rare I wouldn’t touch it with a ten-foot pole.”

There are three things you can do:

Sink, **Swim** or Tread-Water.

If you decide to swim, you’ll need:

- Shark repellent- Thick skin
- A Kickboard- Funding
- Life jacket- **Partners**



Session 2 –Understanding the research process: Being equipped for success

‘Building a successful partnership’

Partner: any number of individuals with interests and investments in a business or enterprise, among whom expenses, profits, and losses are **shared**.

Partners:

- NIH Partner
- Business Partners
- Advocacy Partners
- KOL’s
- Sympathetic Partners
- Clinical Partners
- Research & Manufacturing
- FDA

Top 3 Partnerships:

- #1 Life Partner
- #2 Patient Partners
- #3 Academic Partners



Life Partner or significant other as partners

For better, or worse, for richer, or poorer, in sickness and in health, to love and to cherish, until death do us part.

Who can you depend on to help?

- Financial strain
- Business travel
- Child-care
- Stress
- Depression
- Commiserate



Patient Families as Partners

H.A.N.D.S. – a consortium of like-minded parents

We're like The Avengers- as a TEAM we are:

More **Powerful** in Numbers.

Heroes, no child is left behind.

Co-workers, we **share** the workload.

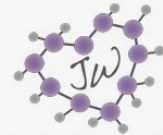
Global, everywhere at once.

Smarter, more skill sets

Not alone

Helping Advance
Neurodegenerative
Disease Science

JLK
Sanfilippo Research
Foundation



Jonah's Just Begun



Le combat
de Haïtem
— contre —
Sanfilippo

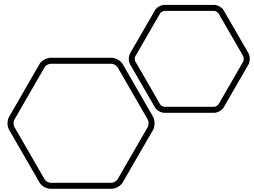


Sanfilippo
Brasil



Sanfilippo Sud





Academics as Partners

- Experts in your disease
- Generate treatment ideas
- Publications
- Grant applications

Academic researchers come with Strings- INSTITUTIONS

- Contracts
- I.P. considerations
- Royalty negotiations
- Delays

Manchester University, UK



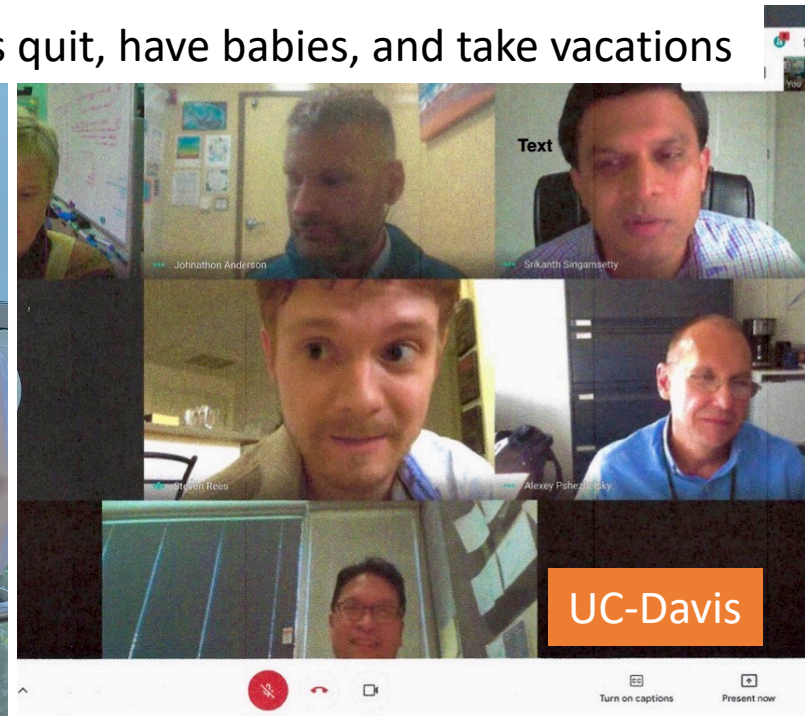
CHU Sainte Justine, Canada



Reminder, Post-docs quit, have babies, and take vacations



Lundquist Institute



UC-Davis

NIH as a Partner

Highly Recommended

- Funding Source (my kickboard)
- Knowledge and Advice
- In house resources

*1R41HD092110-01 Award Amount: \$225,000.00

Structure-based optimization of a novel pharmacological chaperone for MPS

*1R41NS092221-01 Award Amount: \$224,469

Engineering stem cells to make mucopolysaccharidosis IIIB

*1R42NS089061-01 Award Amount: \$223,102

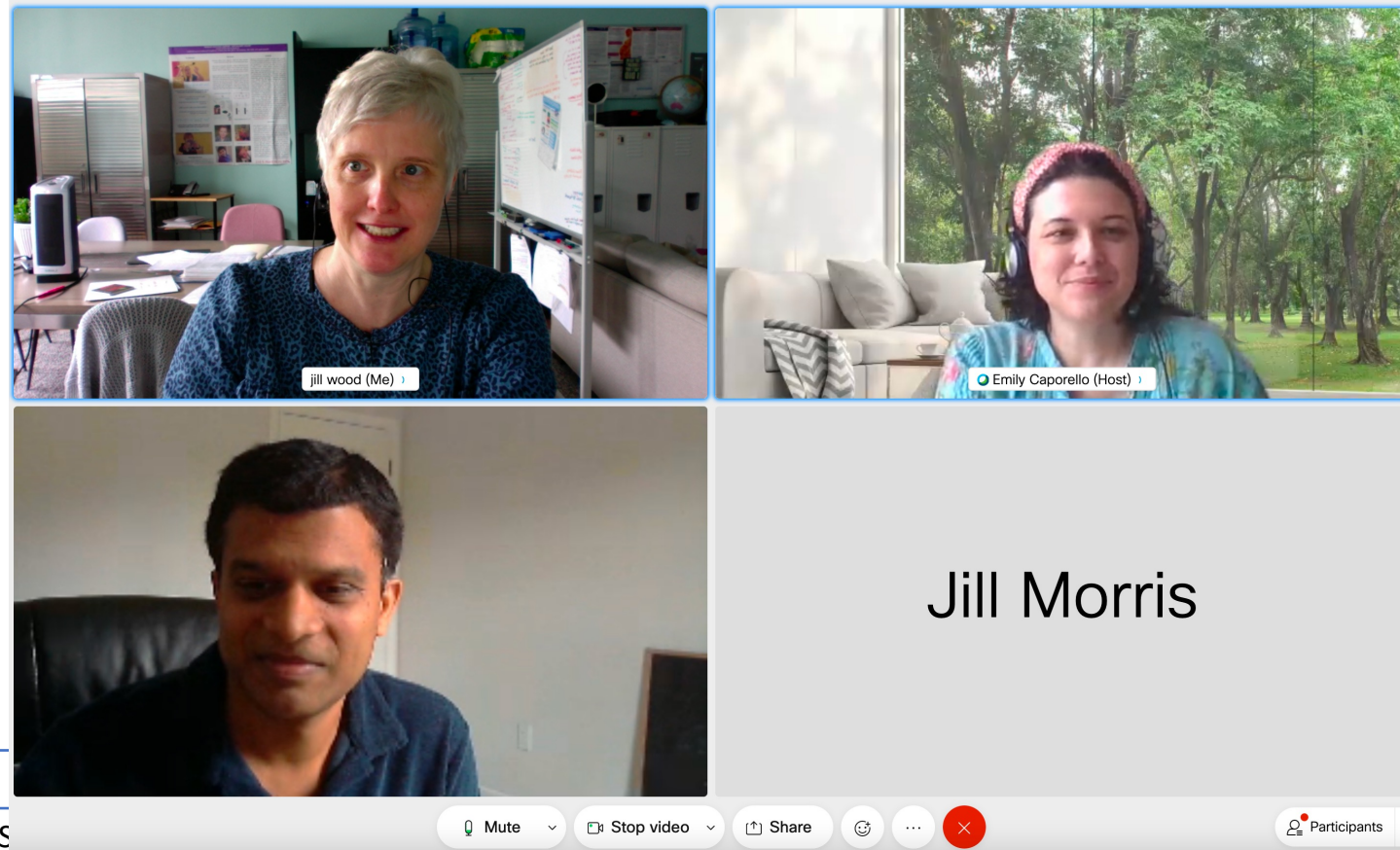
Development and in vitro validation of therapy for mucopolysaccharidosis IIID

*2R42NS089061-02 & 03 Award Amount: \$1,495,008

Development and validation of therapy for mucopolysaccharidosis IIID

*2U44NS089061-04 & 05 Award Amount: \$5,612,276

Development and validation of therapy for mucopolysaccharidosis IIID



Monthly calls with NIH Grant Managers.

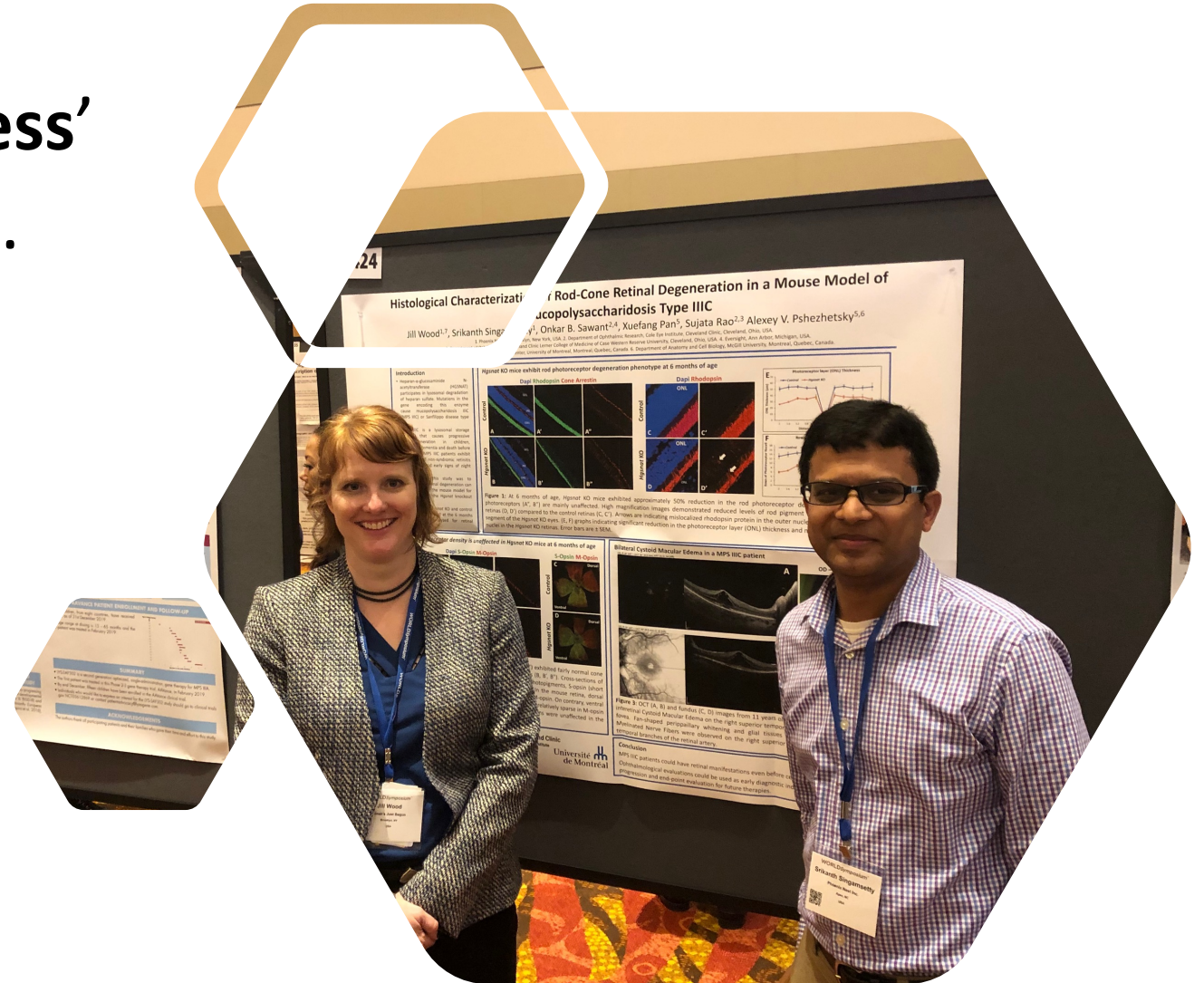
Business Partnerships

'It isn't personal it's just business'

This saying does not apply here.

When forming a biotech-
out of desperation:

- Own your company
- Hire an administrator
- Outsource



Advocacy Partners

Aligned Goals & Shared Resources

Collaborating:

- Newborn screening
- Patient Registry
- Funding research
- Connecting with families
- Rare disease legislation and public policy

H.A.N.D.S.

The National MPS Society

The EveryLife Foundation

The Cure Sanfilippo Foundation



Key Opinion Leaders as Partners

Who are your KOL's?

- Pharma Executives
- Researchers
- Clinicians
- Seasoned Patient Advocates

What can they do for you?

- Offer advice
- Sounding board
- Make introductions
- Point you in the right direction
- Advocate for you
- Give you credibility



Sympathetic Partners

Anybody that will give their services for free or at a great discount.


- Foundations founded by CEO's
- Members of Congress
- Accountants & Lawyers
- Consultants: CMC Experts, Regulatory, Clinical
- Medical & Grant writers

Jude Samulski, AskBio
The Columbus Foundation



Emil Kakkis, Ultragenyx
The EveryLife Foundation





Research and Manufacturing Companies as Partners (CRO and CMO) Keep your researchers as consultants!

A company for every need:

- Regulatory help
- Animal studies
- Assay development
- Plasmid & Vector production
- Statisticians
- Medical writing

Benefits of CMO/CRO over Academia:

- Regular updates
- No IP or royalty negotiations
- Timely
- Cost effective
- Professional

Shout out to **Jameson & Company**: An outsourced accounting for government grants and contracts

Clinical Partners

If your goal is an interventional trial

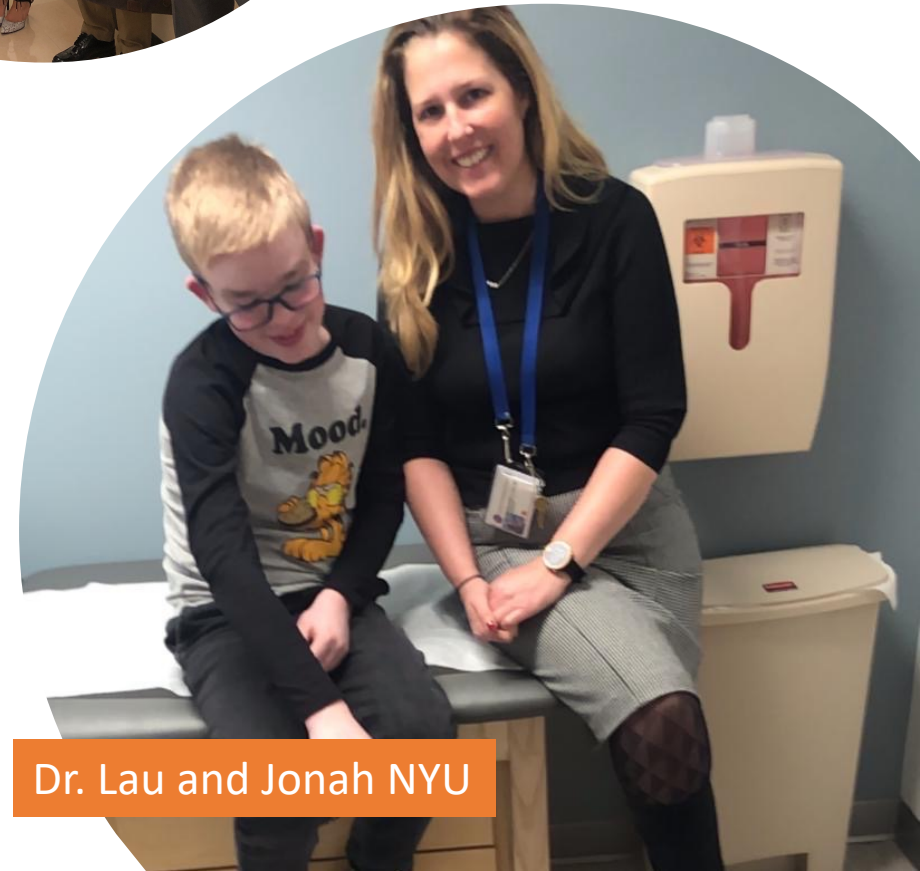
Qualities to look for:

- They WANT to do it
- Reliable
- Progressive IRB
- Resources and Expertise

Clinical team at UTSouthwestern, TX



Dr. Lau and Jonah NYU



The FDA as a Partner

Phoenix Nest Pre-IND meetings

- CDER April 2019 Type B meeting
- CBER July 2019 Type B meeting

Accessibility?

- Response Time
- Response.

Attention?

- Slowly Progressing
- No approved biomarker
- Lack of Natural History
- Tiny patient population

Assurance?

- Guidance's
- Goal posts

Attainable?

- Realistic goals
- Right endpoints

Mucopolysaccharidosis Type III (Sanfilippo Syndrome): Developing Drugs for Treatment Guidance for Industry

Additional copies are available from:

*Office of Communications, Division of Drug Information
Center for Drug Evaluation and Research
Food and Drug Administration
10001 New Hampshire Ave., Hillandale Bldg., 4th Floor
Silver Spring, MD 20993-0002*

*Phone: 855-543-3784 or 301-796-3400; Fax: 301-431-6353; Email: druginfo@fda.hhs.gov
<https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs>*

and/or

Contains Nonbinding Recommendations

**ressive, Low-Prevalence Rare Diseases With
sition That Result From Single Enzyme Deficiency
Providing Evidence of Effectiveness
for Replacement or Corrective Therapies
Guidance for Industry¹**

Thank you and Happy belated Mother's Day!



Contact me at: jwood@phoenixnestbiotech.com