

Welcome!



 **YAYA**
FOUNDATION

Organization & Team



Started in 2018, the Foundation is the only organization representing patients and families affected by 4H (POLR3-Related) Leukodystrophy.



Ron Garber
Co-Founder, Board Pres



Christina Butterworth
Executive Director



Valerie Greger, PhD
Research Director



Shannon Healey
Advancement Advisor



4H Leukodystrophy Session Agenda

Time	Presentation	Speaker(s)
9:00 – 9:10 am CT	Introduction: The Yaya Foundation's Vision	Ron Garber
9:10 – 11:00 am CT	Research Focus of the Yaya Foundation: Data Collection Natural History Animal Models Gene Therapy Approach and Status	Christina Butterworth Dr. Nicole Wolf Dr. Ian Willis Dr. Jun Xie & Valerie Greger
11:00 – 11:30 am CT	Outlook: Novel Therapies, Future Approaches for 4H	Dr. Mack Michell-Robinson
11:30 – 11:40 am CT	Break	
11:40 – 12:00 pm CT	Yaya Foundation Research: Vision, Priorities, and Roadmap	Valerie Greger
12:00 – 12:25 pm CT	Yaya Foundation Family Programs, Support, and Input	Christina Butterworth & Shannon Healey
12:25 – 12:30 pm CT	Closing	Christina Butterworth

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*Fighting for
People Affected by
4H Leukodystrophy*

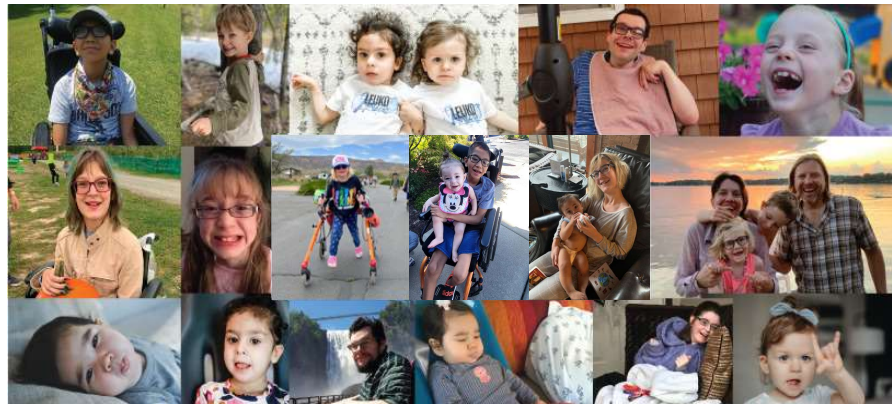
4H Leukodystrophy Data Collection Program

What is a Data Collection?

Effort to collect data, individual impacts, and experiences of 4H Leukodystrophy to expand and improve research.

The goal is to generate the most comprehensive 4H Leukodystrophy Data Collection Program, accelerating research and the development of new therapies.

Aim to provide de-identified disease data to researchers working on various research projects for 4H Leukodystrophy and other rare diseases.



Why Is Data Collection Important?

Make your voice heard!

- Informs researchers how 4H Leukodystrophy changes over time
- Aids clinicians in counseling individuals and families what to expect
- Informs understanding of basic disease mechanism
- Allows evaluation of the effect of treatments
- Enables the use of data as a placebo (instead of actual patients) in a clinical trial
- Aids in identifying possible candidates for clinical trials
- Reduce the time it takes to study new medicines
- Speed up the time to get treatments to patients
- Key building block to engaging with industry



Data Collection Launch

- Partnered with nonprofit organization, Rare-X, to build the Data Collection for 4H Leukodystrophy
- Launched Summer 2021; additional surveys, functionality released January 2022
- Make it easy for individuals and families to upload medical and other disease impact information
- Secure, standardized, robust, and patient-driven/-controlled platform that enables collaboration across diseases

4H Leukodystrophy

HOMEGETTING STARTEDFAQ

RAREX

4H Leukodystrophy - Data Collection Program



4H Leukodystrophy patients, families, and communities are excited to participate in data collection to expand and improve medical research. By coming to this site, you can begin the first step in making your patient information available to researchers. By generating the most comprehensive 4H Leukodystrophy Data Collection Program, we can accelerate research and the development of new drugs, devices, or other therapies. Only you hold the key to unlock future discoveries.

Start Your Journey

GET STARTED

By clicking Get Started, you are agreeing to the [RARE-X Terms of Use and Privacy Policy](#).

Already Enrolled?

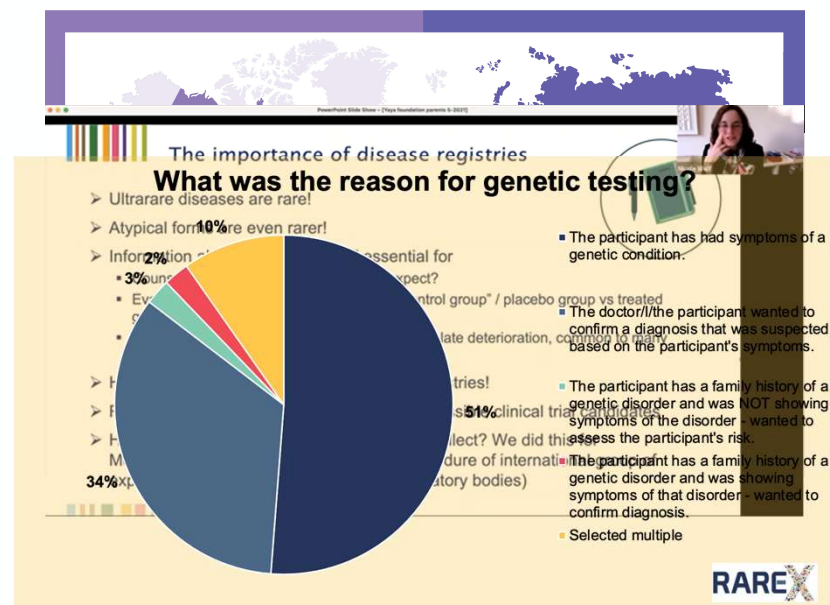
LOGIN

By clicking Login you are leaving the RARE-X site and entering the Data Collection Platform supported by Matrix, a third-party service provider. By clicking Login, you are agreeing to the [Matrix Terms of Use and Privacy Policy](#).



4H Data Collection Progress

- In 10 months, more than 60 individuals from 14 countries are participating in Data Collection!
- Hosted #4HLFamilies call with Dr Wolf, hosted three Data Collection Info Sessions, invite individuals to participate
- Preliminary, aggregated data shows early insights and provides some context
- Participation in Data Collection has caught the attention of research and provider community
- Desire to map patient-reported outcomes to provider-reported outcomes
- Existing de-identified data used in recent grant submission!



Rare-X Data Collection

- Across Rare-X Data Collection platform, 27 rare disease groups are currently participating, including atleast one other leukodystrophy
- Nearly 750 individuals have contributed to the Data Collection platform
- Next deep dive focus is on the brain!

Next Domain Priorities

- Neurodevelopmental
- Neurodegeneration
- Neuromuscular
- Sleep
- Seizures / Epilepsy
- Diagnostic Odyssey
- Medical Management
- Clinical Trial Readiness
- Lab Report Upload*
- Genetic Testing Report Upload*
- ClinGen L3**
 - Seizures
 - Hearing
 - Eyes & Vision
 - Development
 - Headache

Domain Expansion & Depth

- Renal
- Vision
- Cardiology / Cardiovascular
- Hearing / Hearing Loss
- Dermatology
- Respiratory
- Gastrointestinal
- Pain
- Endocrinology
- Medication usage
- Mental Health
- Musculoskeletal
- Diet and Nutrition
- Mitochondrial
- Autoimmune
- Genetic Data Abstraction & Curation
- Surgery
- Transplant
- Medical Equipment
- Diagnostic testing
- Treatment/Effectiveness
- Disease-specific validated instruments
- Electronic Health Record (EHR) linkages
- Remote Monitoring linkages

We Need You!

Help us reach the goal of 100 participants by the end of 2022!



If you're already
participating,
THANK YOU!



If you haven't updated
your password since
February, log into DCP to
authenticate.



If you haven't
enrolled, visit
[https://4hleukodystrophy.
rare-x.org/](https://4hleukodystrophy.rare-x.org/)
to participate!





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Yaya Foundation Research: Vision, Priorities, and Roadmap

Our Research Mission

Accelerate and support research that will help to:

- Better understand 4H Leukodystrophy
- Develop therapies that will help patients with 4H Leukodystrophy
- Find a cure for 4H Leukodystrophy

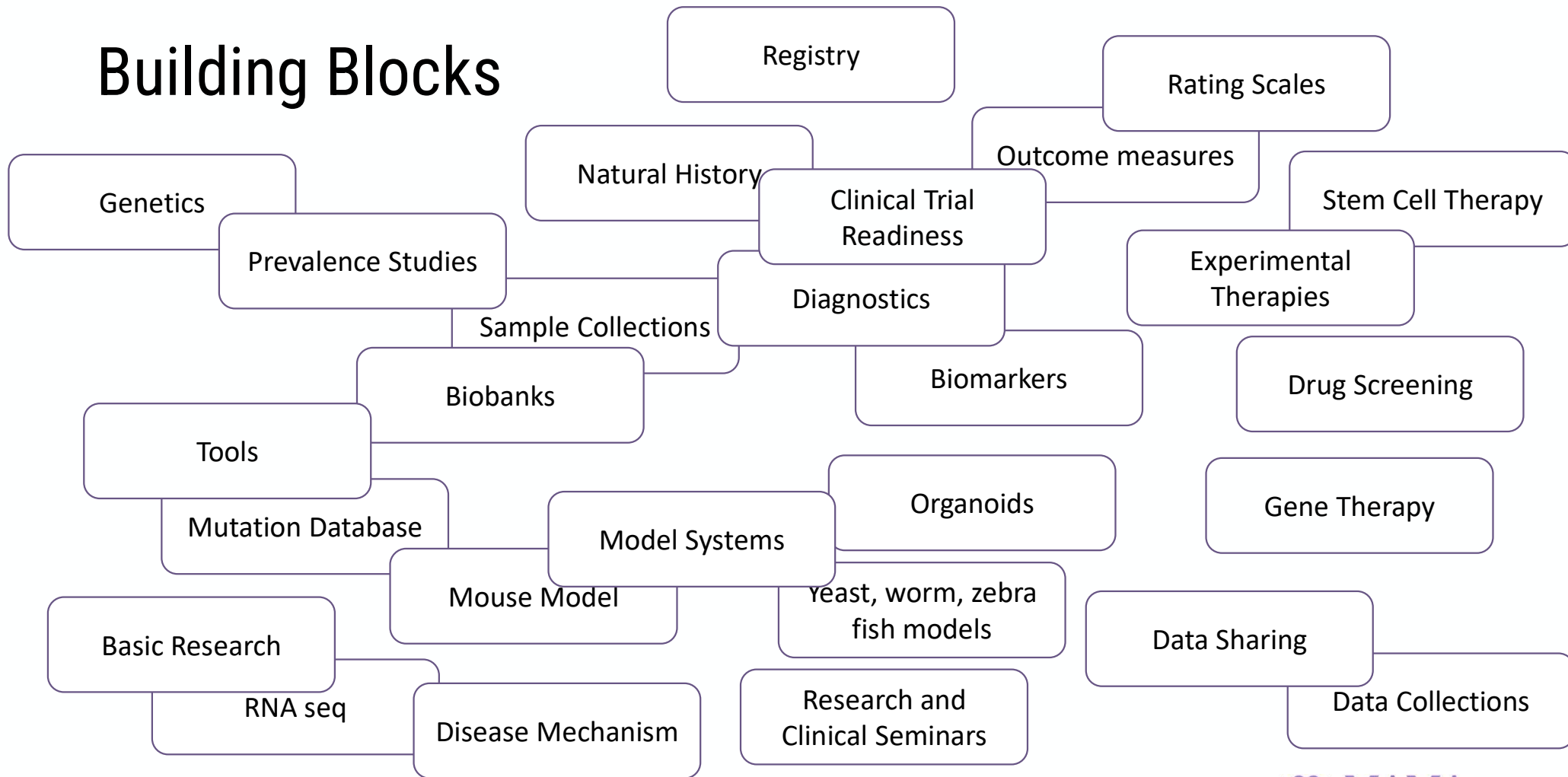


Our Research Approach

- Develop a collaborative network of scientist and clinicians dedicated to 4H Leukodystrophy research and connect it with our families
- Raise money to support relevant research through grant awards

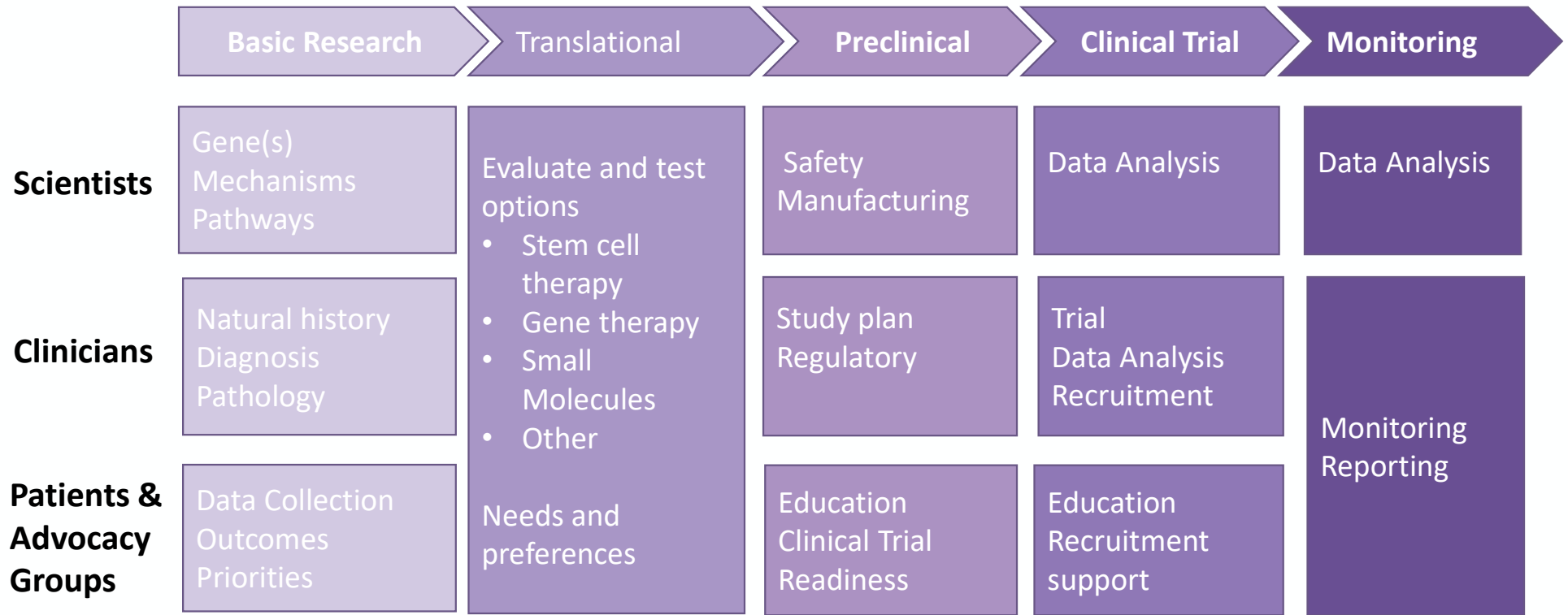


Building Blocks



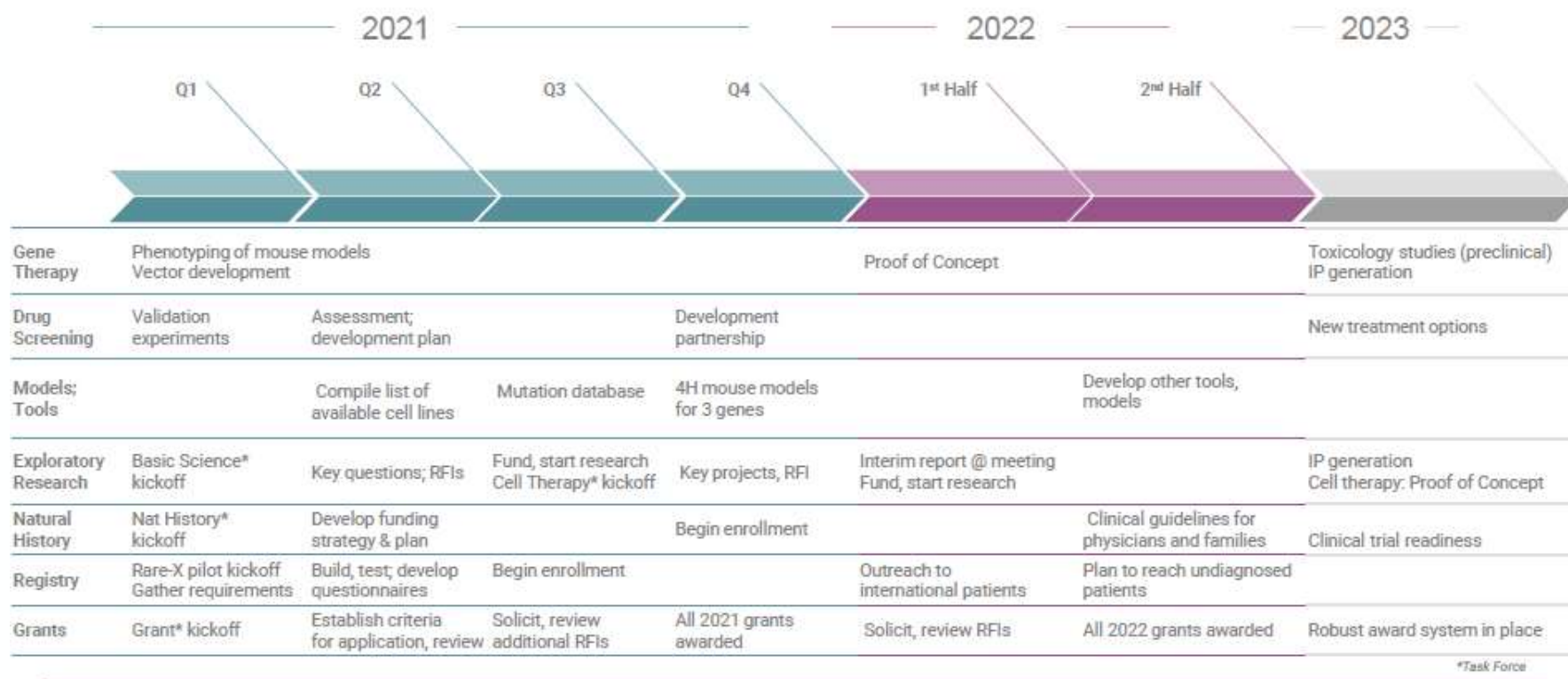
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From Research to Treatments and Cures



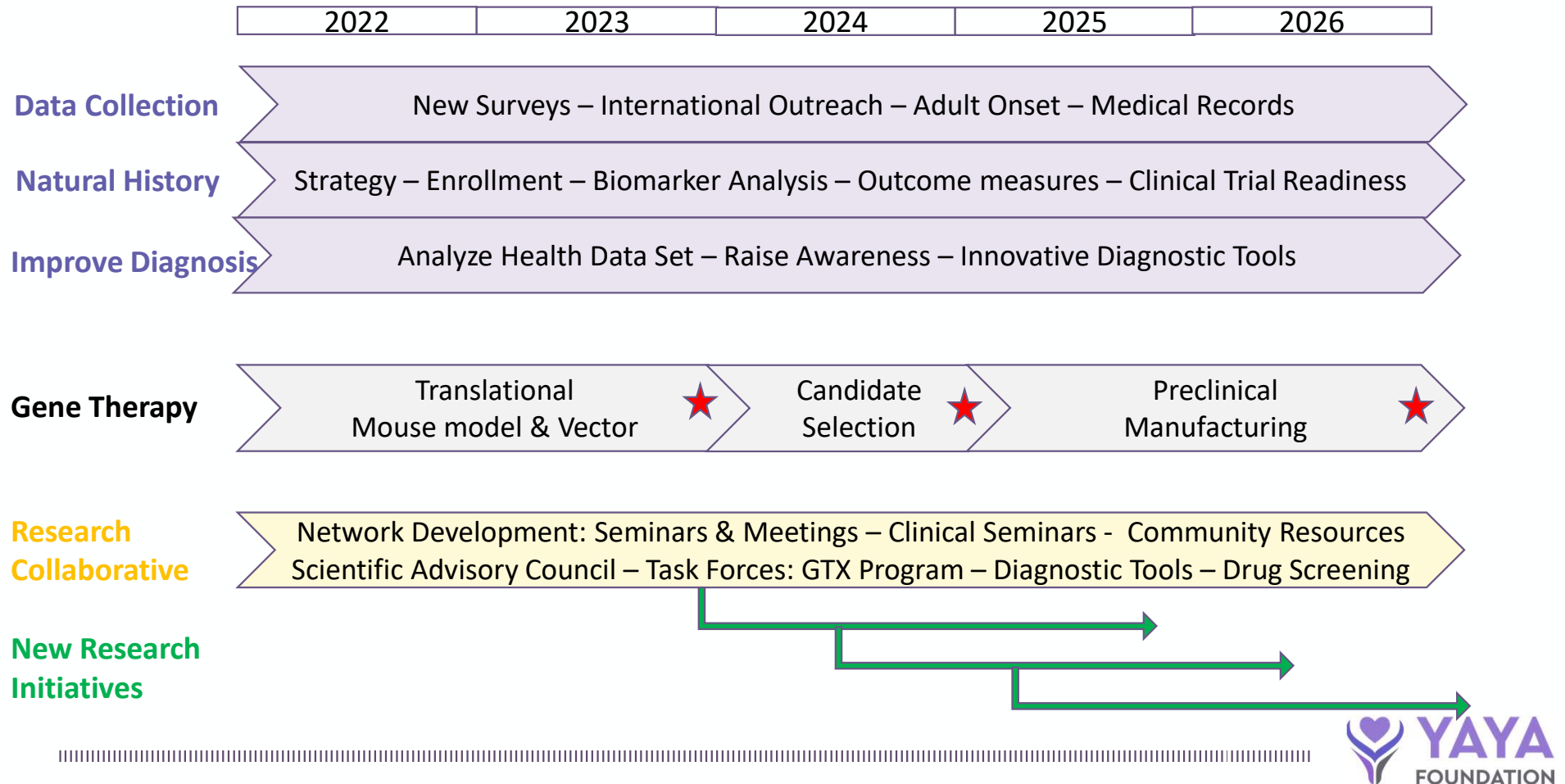
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Roadmap: First draft

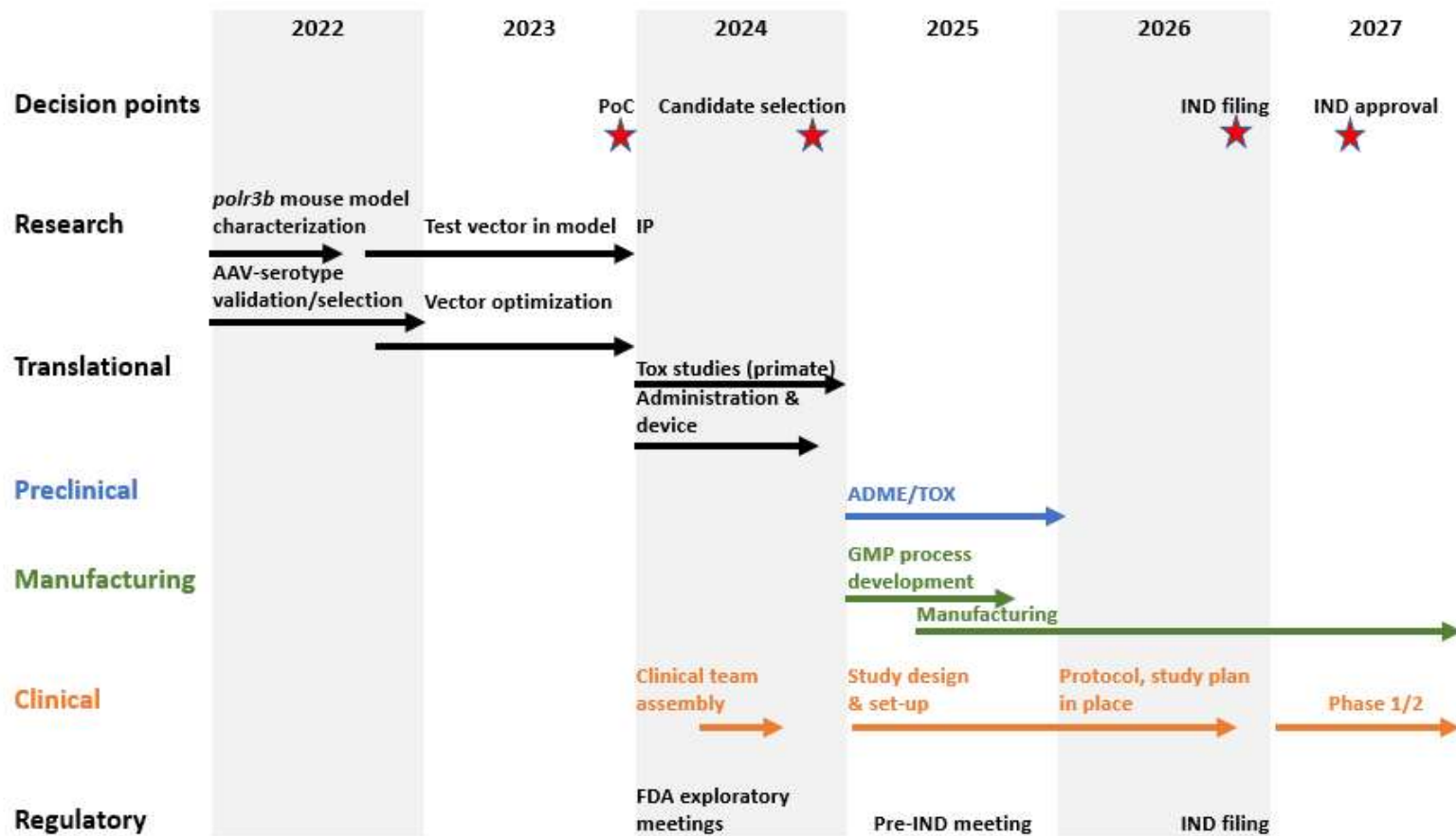


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Roadmap: where we are now



Gene Therapy Program





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Yaya Foundation Family Programs, Support, and Input

2022-25 Priorities

The mission of the Yaya Foundation is to fight for people and families affected by 4H Leukodystrophy by accelerating discovery of therapies and providing educational and emotional support to affected families.

Research Agenda

- Data Collection Program
- Diagnostic Odyssey
- Natural History Study
- Gene Therapy Grant
- Grant Program
- Research Roadmap
- Collaborative Research Network
- Industry Partnership

Individual & Family Support

- Conferences
- Family Calls
- Connect to Care
- Research Readiness
- Expanding Clinician Awareness
- Extending Foundation Reach

Development Strategy

- Identify, Apply For Grants
- Build Major Donor Program
- Support Peer to Peer Fundraising



Family Programs & Support



Family Calls



Connection to Care



Conferences



Fundraising

1

Grants

- Increase grant submissions
- Raise awareness for 4H through outreach
- Connect with like-minded organizations

2

Corporate Sponsorships

- Find partners across different industries that share our goals
- Identify new corporate partners for support

3

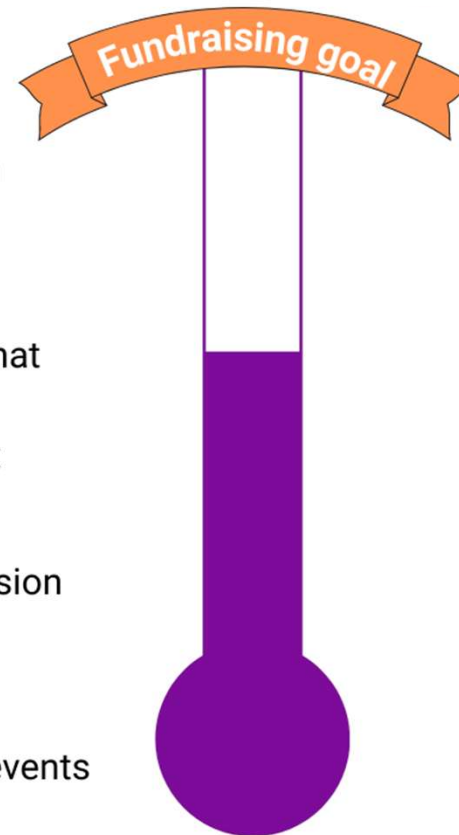
Major Gifts

- Meet with individuals interested in our mission
- Identify/solicit new major gift donors

4

Third-Party Events

- Support families and others as they host events to benefit Yaya Foundation



Needs Assessment



Needs Assessment Goals

Temperature read

Identify trends

Inform development
work

<https://forms.gle/rBLVNiAltS6ckKwB8>

Thank you!

