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De-Risking Next-Gen Science:

Cross-Disease Platform Approaches

A FasterCures TRAIN Webinar

April 29, 2026

FasterCures Programs

Mission: To build a biomedical innovation system that is effective, efficient, and driven by a clear vision: patient needs above all else

R&D Environment

- ENRICH-CT (Enabling Networks of Research Infrastructure for Community Health Through Clinical Trials)
- Representation in Clinical Trials
- Future of Biomedical Innovation

Policy

- CMS/FDA Alignment: Accelerating Treatments to Patients
- Building Patient Engagement Capabilities at CMS
- Prevention-First Health

Patient Engagement

- TRAIN (The Research Acceleration and Innovation Network)
- LeadersLink
- Patient Engagement in Medtech Development
- Vital Voices: Patient Engagement with CMS

Innovation

- Future of Cancer Care in the US
- Cell, Gene, and RNA Therapies
- Emerging Technologies
- Data and AI

International

- Project Prevent
- Global Cancer Care
- Anti-Microbial Resistance
- Early Warning System

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TRAIN: The Research Acceleration & Innovation Network

The objectives of TRAIN are to:

- **To encourage a more strategic and entrepreneurial approach to funding medical research**
- **To create peer sharing and networking opportunities to amplify innovative approaches and ideas**
- **To provide platforms for patient foundations to leverage their perspectives to elevate and influence issues that impact patient-centered research and biomedical R&D policies, activities, and practices**

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TRAIN Communities of Practice

- FasterCures launched Communities of Practice (CoPs) for members of TRAIN and the Rare As One Mentorship Program as collaborative opportunities for peer patient organizations to share promising practices, strategies, and ideas
- Community topics include the 4 domain areas represented in the Research Partnership Maturity Model
  - **Expertise**
  - **Patients**
  - **Money**
  - **Partnerships (June 17th)**
- Meetings take place every other month and topics rotate between the domain areas

To join, complete the following intake form:

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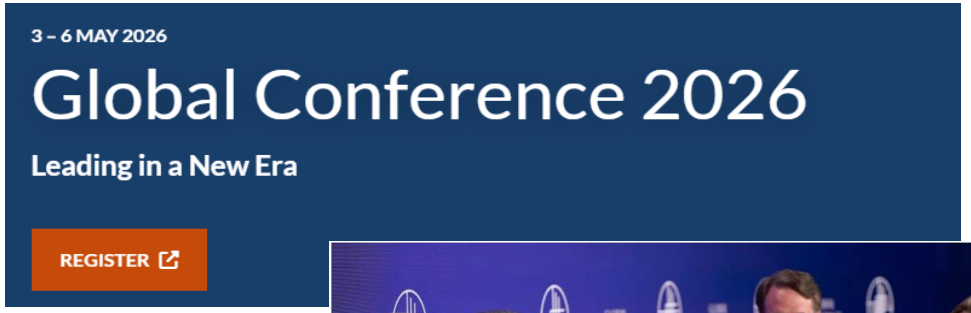
Milken Institute Convenings

Milken Institute Global Conference

May 3-6, 2026
The Beverly Hilton & Waldorf Astoria Beverly Hills
Los Angeles, CA
LiveStream: Livestream | Milken Institute



Milken Institute Future of Health Summit

November 11-13, 2026
The Salamander Hotel
Washington, DC

Save the date!

Today's Webinar

Background: The 2026 TRAIN webinar series is designed to help patient organizations understand and engage in upstream strategies that meaningfully de-risk and accelerate the therapeutic pipeline.

Today's Objective: Highlight cross-disease platform approaches (shared research infrastructure, platform trial design, and collaborative funding models) that enable patient organizations to:

- Reduce scientific and financial risk
- Maximize the impact of limited patient populations, data, and resources
- Accelerate translation of next-generation science into treatments for patients

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Panelists

Jennifer Farmer

CEO

Friedrich's Ataxia Research Alliance (FARA)

PJ Brooks

Acting Director, Division of Rare Diseases Research Innovation (DRDRI)

NCATS, NIH

Gwen Nichols

Chief Medical Officer

Blood Cancer United

MODERATOR

Raymond Puerini

Director

FasterCures, Milken Institute

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Jennifer Farmer

CEO

Friedrich's Ataxia Research Alliance (FARA)

Shared Infrastructure and Regulatory-Relevant Data

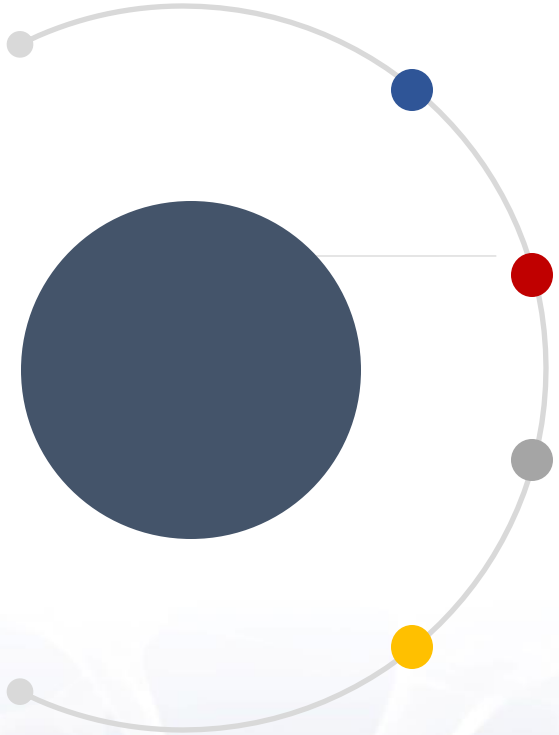
De-Risking Next-Gen Science

April 2026

Jen Farmer, CEO, Friedreich's Ataxia Research Alliance (FARA)

FARA - Internal Use

Disclosures



The Friedreich's Ataxia Research Alliance (FARA) is an independent 501(c)(3) non-profit organization dedicated to the pursuit of scientific research leading to treatments and a cure for Friedreich's ataxia.

FARA does not endorse, recommend, or promote any specific treatment programs, products, or companies.

Information shared during this presentation is for educational purposes only.

FARA receives sponsorship and grant support from the following companies: Alexion, Amgen, Biogen, Biologics by McKesson, Design, Goldenrod, Larimar, Lexeo, Neurocrine, PTC, Solid Biosciences, and Voyager.

FARA holds common stock shares in a company, Bioelectron LLC, a company that is eligible to receive milestone payments contingent upon regulatory approval of vatiquinone and again upon commercialization.

Mapping the relentlessly progressive FA journey: Loss of ambulation, coordination, cardiomyopathy

Early Childhood

- Thinning of spinal cord
- Clinical symptoms may be present, but mild

Childhood

- Stumbling and falling
- Scoliosis
- Diagnosed with FA

Adolescence

- Full-time wheelchair user
- Dysarthria, dysphagia
- Impaired auditory processing
- Loss of visual acuity
- Cardiomyopathy
- Type 1.5 diabetes

Adulthood

- Loss of upper limb function
- Significant vision and hearing impairment
- Dilated cardiomyopathy
- Advanced congestive heart failure

FARA - Internal Use

THE FA GCC
GLOBAL CLINICAL CONSORTIUM

UNIFAI NATURAL HISTORY STUDY

GLOBAL ALIGNMENT

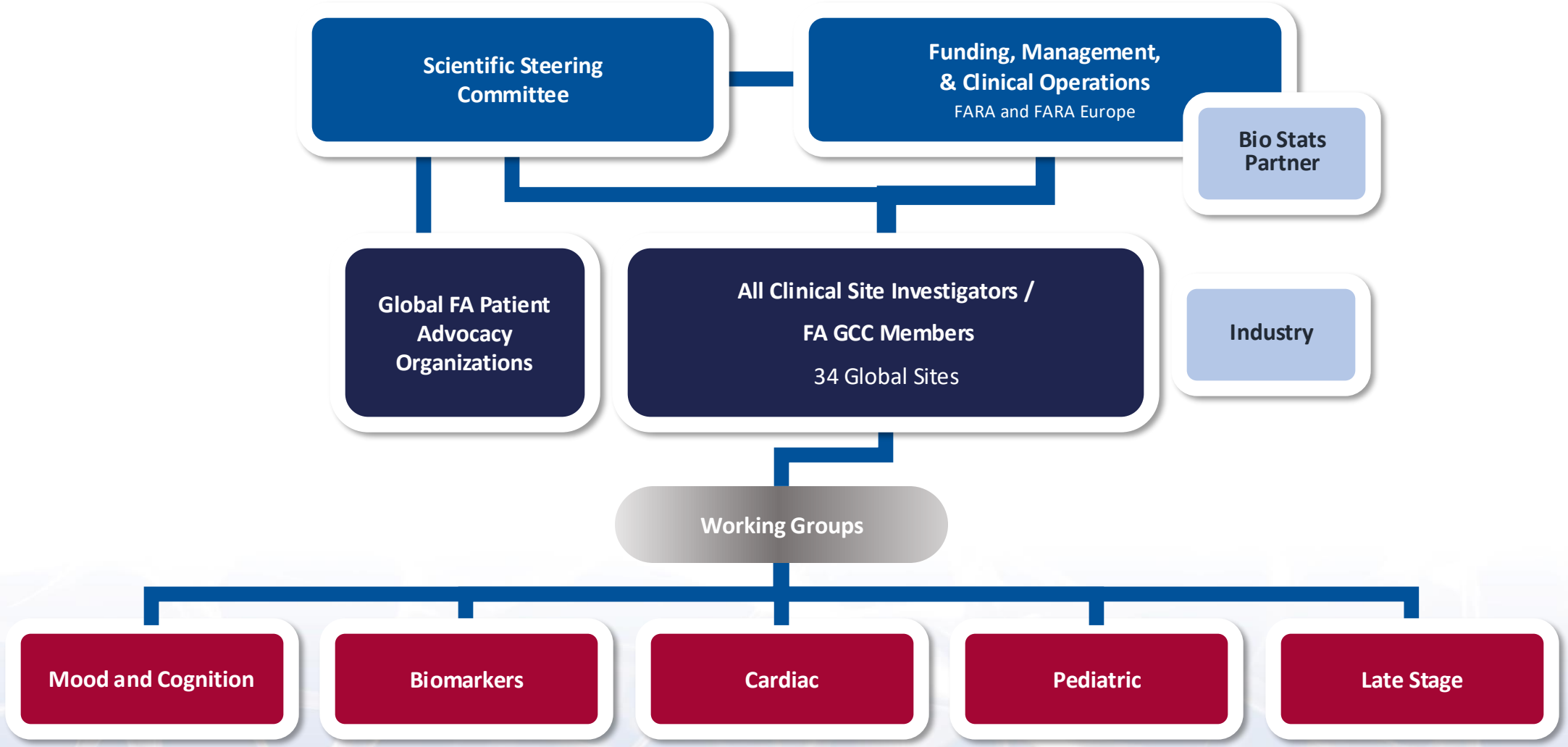
POWERFUL PARTNERSHIPS

SHARING DATA & RESOURCES

RESEARCH INFRASTRUCTURE

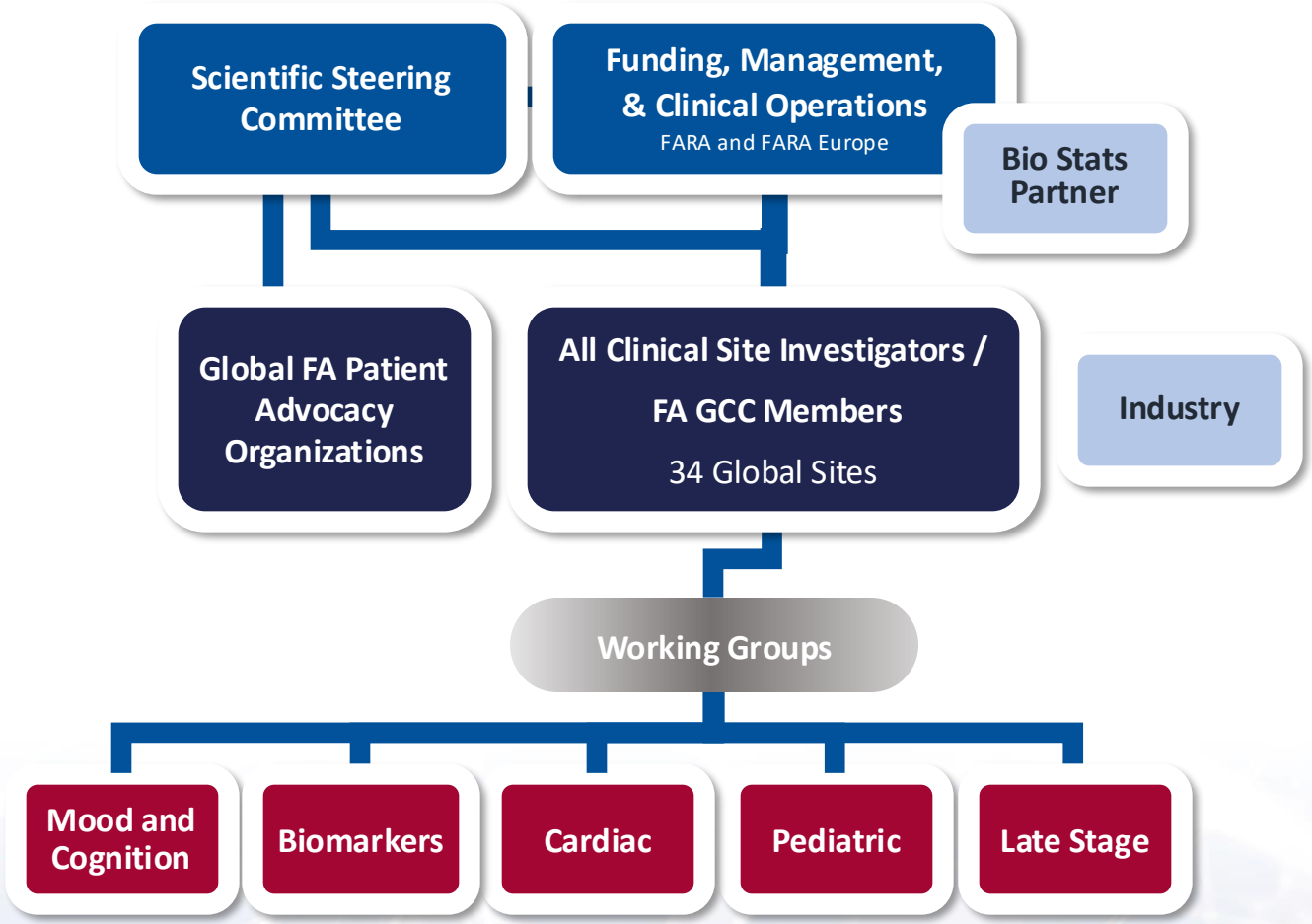
FARA Friedreich's Ataxia Research Alliance

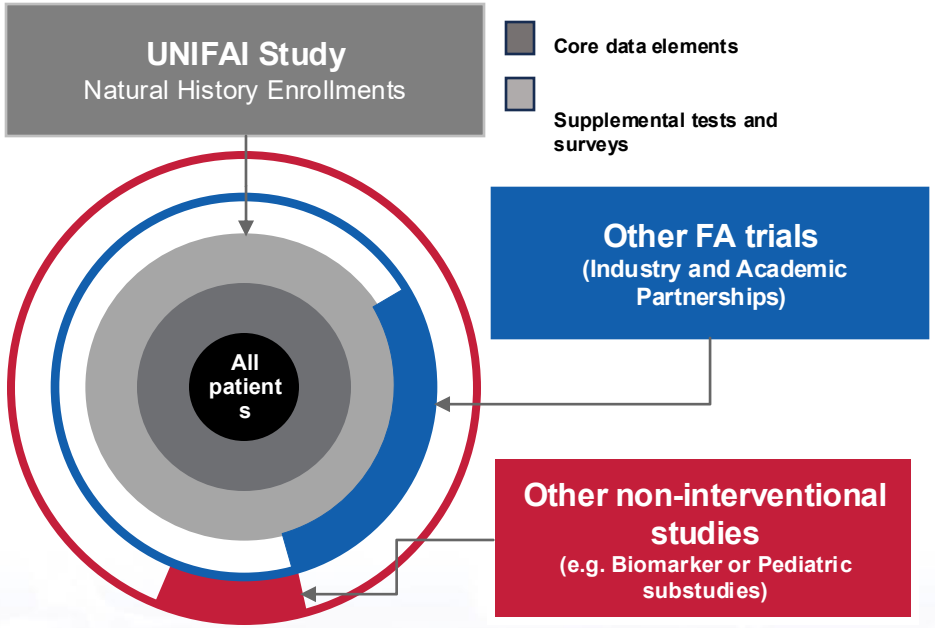
FA Global Clinical Consortium



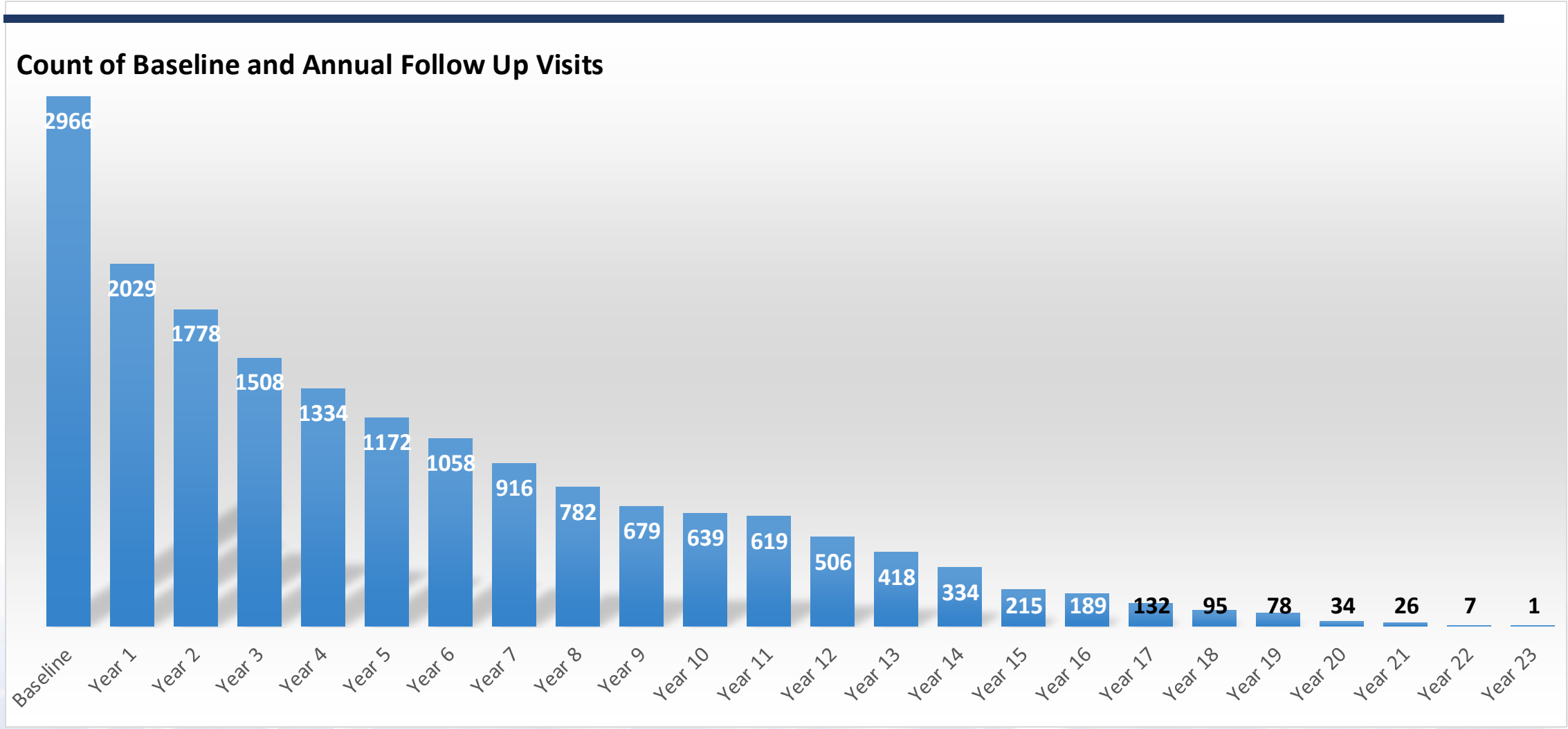
FARA Friedreich's Ataxia Research Alliance

FA Global Clinical Consortium





FA Natural History Study is a longitudinal study with both contemporary enrollment and high retention rates



FARA Friedreich's Ataxia Research Alliance

How FARA's Natural History study began:

COAs for FA

MDA Muscular Dystrophy Association & FARA Friedreich's Ataxia Research Alliance

2003: Co-funded grant

- *Original to FA*
  FARS, ADL, staging scale
- *Borrowed measures from MS*
  9HPT, T25FW, PATA, Low Contrast Letter Acuity, MSQLI

Natural History Visits Begin

2006 : FARA commits to funding the natural history study and network

Goal 1

Systemic Data Collection

Understand natural history of FA through systemic collection of clinical data

Goal 2

Disease Measures

Develop outcome measures and inform clinical trial design

Goal 3

Clinical Network

Create a network of clinical research centers in FA that will provide quantitative clinical data on patients.

FARA Friedreich's Ataxia Research Alliance

Quality of the Data

NIH NINDS

Common data elements

Peer-reviewed publications share longitudinal data with the FA research community

Multicenter Study > Diabetes Res Clin Pract. 2022 Apr;186:109828.
doi: 10.1016/j.diabres.2022.109828. Epub 2022 Mar 14.

Friedreich's Ataxia related Diabetes: Epider and management practices

Jaclyn Tamaroff [1], Anna DeDi
Karla Leavens [4], Christian Ru

> J Neuroophthalmol. 2020 Jun;40(2):213-217. doi: 10.1097/WNO.0000000000000878.

Correlation of Visual Quality of Life With Clinical and Visual Status in Friedreich Ataxia

Parisa Afsharian [1], Rachel Nolan-Kenney, Abigail E Lynch, Laura J Balcer, David R Lynch

Observational Study > Ann Clin Transl Neurol. 2021 Ju
doi: 10.1002/acn3.51352. Epub 2021 May 5.

Scoliosis in Friedreich's ataxia: characterization in a large heter

Christian Rummey [1], John M Flynn [2],
George Wilmot [5], Sub H Subramony [6]

> Ann Clin Transl Neurol. 2020 Sep;7(9):1708-1712. doi: 10.1002/acn3.51118. Epub 2020 Aug 11.

Test-retest reliability of the F rating scale

> Neurol Genet. 2019 Oct 29;5(6):371. doi: 10.1212/NXG.0000000000000371.
eCollection 2019 Dec.

Psychometric properties of the Friedreich A Rating Scale

Christian Rummey [1], Louise A Corben [1], Martin B Delatycki [1], S H Subramony [1],
Khalaf Bushara [1], Christopher M Gomez [1], Joseph Chad Hoyle [1], Grace Yoon [1], Be
Katherine D Mathews [1], George Wilmot [1], Theresa Zesiewicz [1], Susan Perlman [1],
Jennifer M Farmer [1], David R Lynch [1]

> EClinicalMedicine. 2020 Jan 8;18:100213. doi: 10.1016/j.eclinm.2019.1
eCollection 2020 Jan.

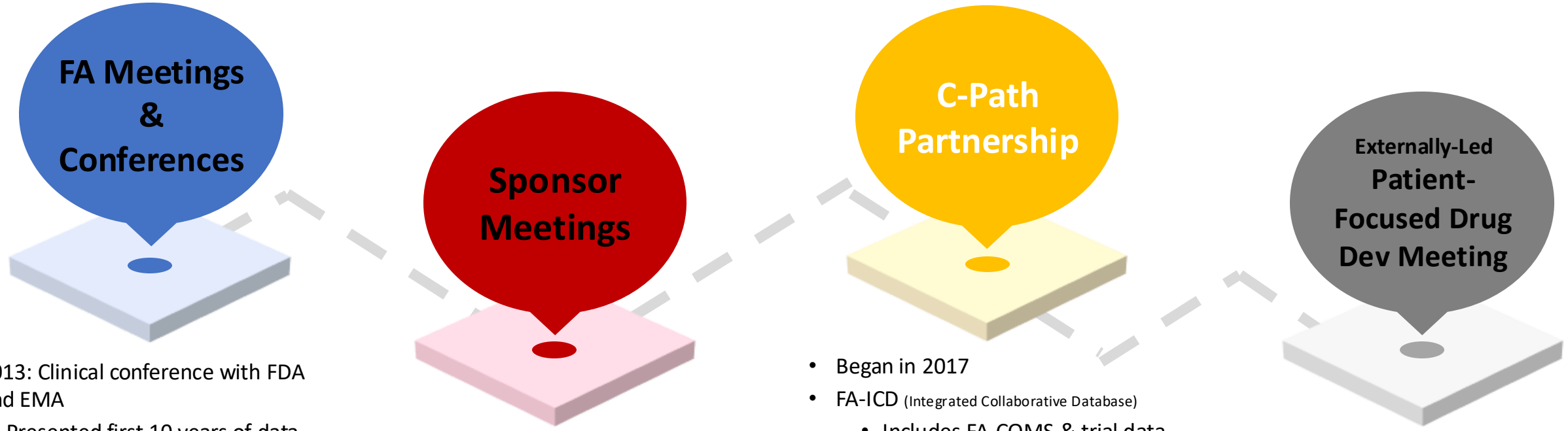
Predictors of loss of ambulation in Frie

Christian Rummey [1], Jennifer M Farmer [2], David R Lynch [3]

>30 articles published based on the study's data

FARA Friedreich's Ataxia Research Alliance

Regulatory Engagement



2013: Clinical conference with FDA and EMA

- Presented first 10 years of data
- Informal feedback
- Beginning of evolution from FARS to mFARS

- Confidential meetings
- FARA attends as a guest of our industry partner
- Opportunity to discuss how natural history data informs trial design and endpoints

- Began in 2017
- FA-ICD (Integrated Collaborative Database)
  - Includes FA-COMS & trial data
  - Available to industry sponsors, FDA, and RDCA-DAP
  - Data in CDISC STDM standard

- Helped FDA and industry partners understand the lived experience of FA
- Communicated outcomes that are valued by the community

The Path to the First Approval for Friedreich's Ataxia

2009
Nrf2 target first reported by research group in France

2013
California research group links Nrf2 to pathophysiology in FA Mice

FARA seeks out Reata: a company with a drug targeting the pathway

2015
First clinical trial launched.

Trial conducted at FARA's Natural History study sites, by Natural History study investigators.

2017
Second clinical trial launched.

Natural history data informed trial design & endpt selection.

2020
Multiple meetings with FDA

2021
FARA coordinates a petition to Reata and the FDA
74,000+ signatures collected!

2021
FARA Natural History study provides confirmatory evidence through a propensity-matched study.

2022
Reata initiates rolling submission of NDA for omaveloxolone

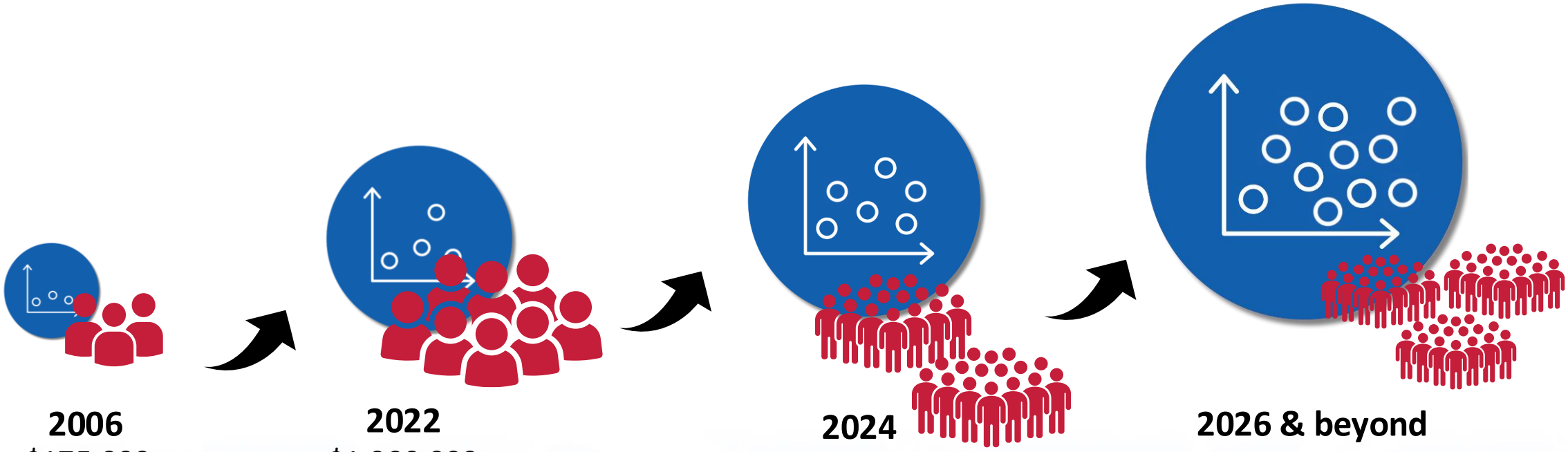
2023
FDA approves first-ever treatment for Friedreich's ataxia: SKYCLARYS™

2024
EMA approves SKYCLARYS™

Ongoing
Post-approval market studies conducted at FARA's Natural History sites

FARA Friedreich's Ataxia Research Alliance

A regulatory-relevant natural history study requires significant and sustained investment



2006
$175,000
150 subjects
6 sites

2022
$1,000,000
>1,000 subjects
14 sites

2024
$2,000,000
>3,000 subjects
33 sites

2026 & beyond
Expanding to new geographies, supporting substudies, expanding the scope of nat hx data collection

Objectives for the Natural History study have expanded over time

Expanding Objectives:

Enable clinical trials with COAs. Use clinical observations to focus basic research.

Provide confirmatory evidence for regulatory filings

Collect real-world evidence and de-risk drug development

Lessons Learned

Data ownership and stewardship is a big responsibility

Rigor drives data quality and reliability

Running the natural history study with good clinical practice – as close to a clinical trial as possible – is essential for future use in regulatory decision-making

Academic researchers feel ownership over data.

Time delay the submissions to C-PATH

Sharing data on C-PATH creates greater returns

External researchers expand the scope and bandwidth of how the data is used

Comparisons to other C-PATH disease datasets extends learnings

Advice for building a regulatory-relevant dataset: Consistency and Comparability

Duration

Study initiated in 2003 and still ongoing today with strong participant retention

Investigators

Same sites/investigators are conducting both the history study and clinical trials

Assessments

The natural history study uses the same assessments as clinical trials

Contemporary Data

Collection of the natural history dataset is contemporary to clinical trials

Thank You!

Acknowledgements:

FARA Natural History Study Site Investigators

Friedreich's Ataxia community

for participating in our natural history study, clinical trials, and for raising funds to enable these efforts

FARA Friedreich's Ataxia Research Alliance

FARA - Internal Use

PJ Brooks

Acting Director, Division of Rare Diseases Research Innovation (DRDRI)

NCATS, NIH

NCATS

COLLABORATE. INNOVATE. ACCELERATE.

“Many diseases at a time” approaches to rare disease therapy development

Philip John (P.J.) Brooks, PhD

Acting Director,

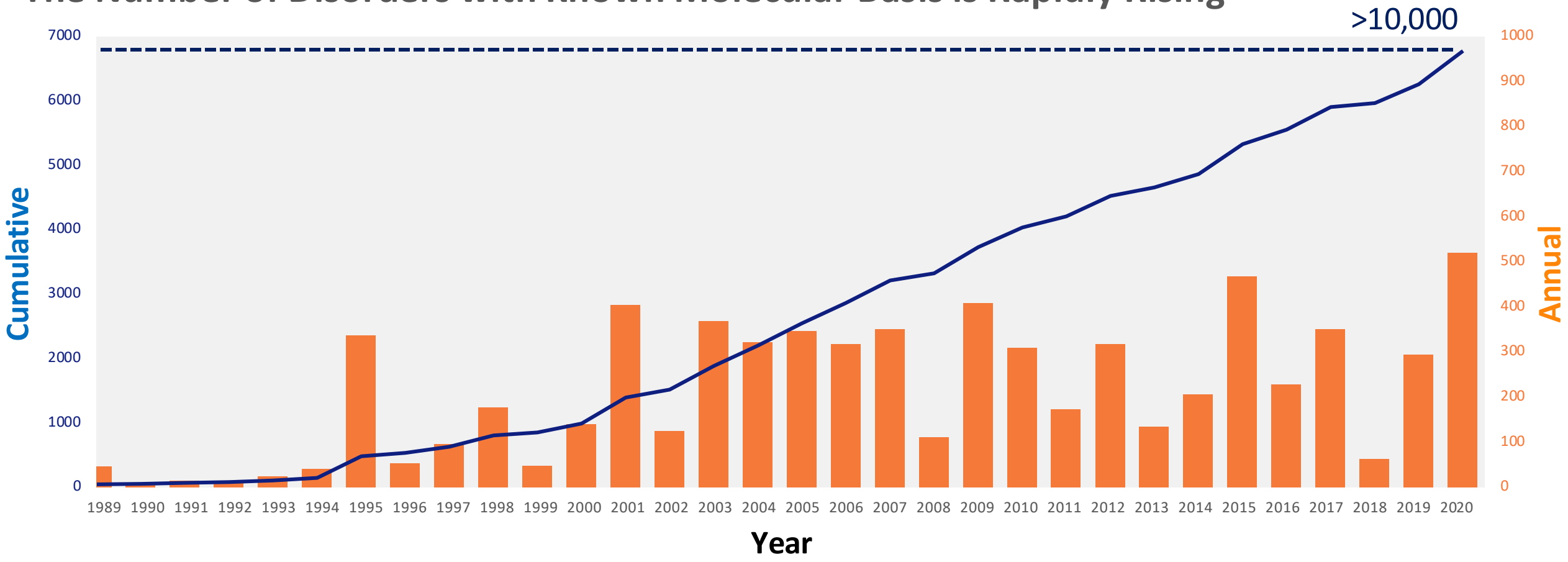
Division of Rare Diseases Research Innovation,

NCATS

National Center for Advancing Translational Sciences

FasterCures 4 29 26

The Number of Disorders with Known Molecular Basis is Rapidly Rising



But the number of diseases with approved therapies is lagging far behind (≈600)

Adapted from Online Mendelian Inheritance in Man (OMIM),
https://www.omim.org/statistics/geneMap

NIH National Center for Advancing Translational Sciences

Thousands of Rare Diseases, far fewer etiologies

- Limited number of mutation types
  - Nonsense mutations → premature stop codon
  - Missense mutations → abnormal protein folding
  - Abnormal RNA splicing
  - Dominant (gain of function) mutations
    - Signalopathies
      - RAS-opathies
      - MTOR-opathies
      - Interferon-opathies
      - Blank-opathies

National Center for Advancing Translational Sciences

Basket Clinical Trials of Drugs Targeting Shared Molecular Etiologies in Multiple Rare Diseases (UG3/UH3 Clinical Trial Required)

https://grants.nih.gov/grants/guide/rfa-files/RFA-TR-20-031.html

Emerging therapeutic candidates for rare maternally inherited mitochondrial diseases with shared etiologies

5UG3TR003897-02	CHIARAMELLO, ANNE ELIANE GROPMAN, ANDREA LYNNE	GEORGE WASHINGTON UNIVERSITY

Safety and Efficacy of Itacitinib in treatment of JAK/STAT pathway disorders with activating mutations

5UG3TR003908-02	FORBES, LISA TORGERSON, TROY R	BAYLOR COLLEGE OF MEDICINE

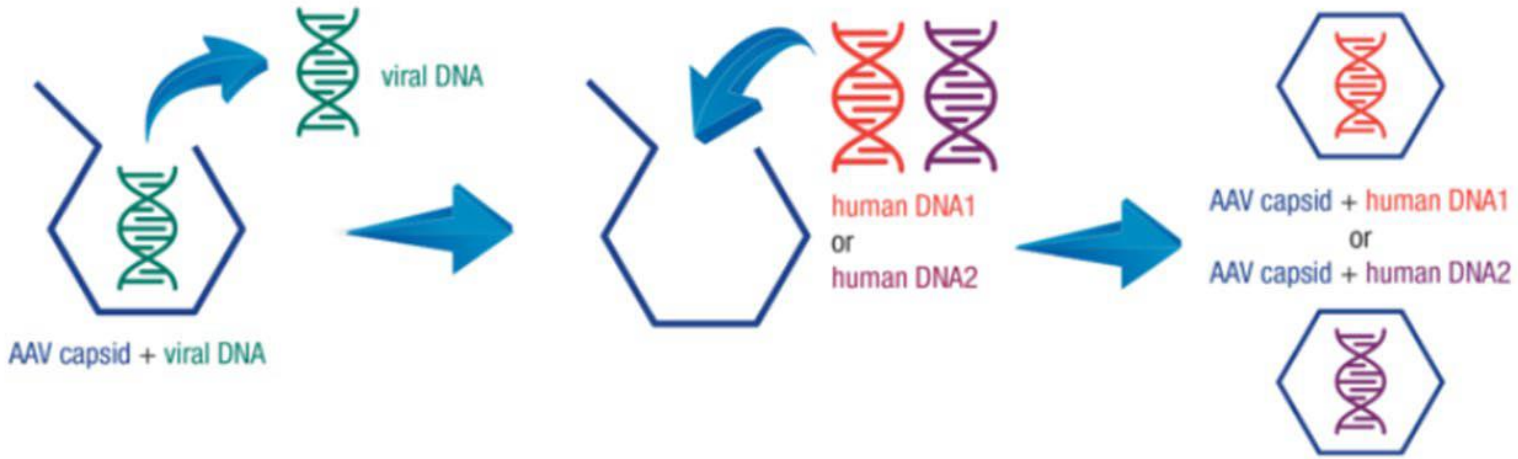
IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Shared Molecular Etiologies (SaME) Underlying Multiple Rare Diseases

MEMBERS

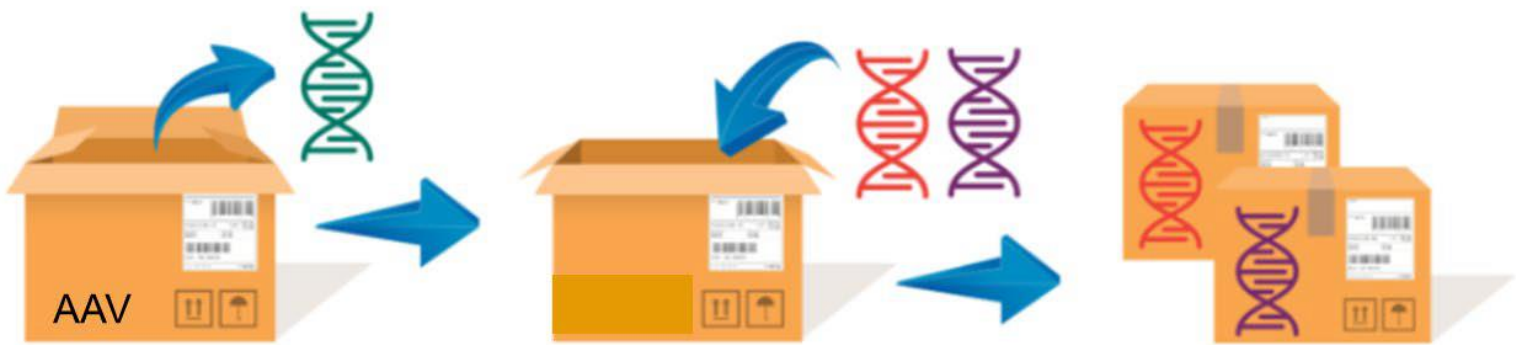
- P.J. Brooks, NIH, NCATS, USA (Chair)
- Marc Dooms, University Hospital Leuven, Belgium (Co-Chair)
- Kate Baker, University of Cambridge, UK
- Daniel O'Connor, MHRA, UK
- Christina Waters, Rare Science, USA
- David Pearce, Sanford Research, USA
- Jason Wan, NIH, NIDCR, USA
- Simone Louisse, ePAG, ERN-Heart, Europe
- Kristina Larsson, EMA, The Netherlands
- Luis Castaño Gonzales, Cruces University Hospital, Spain
- Antony Hall, Healx, UK
- Macarena Garrido Estepa, Instituto de Salud Carlos III, Spain
- Ana Crespo, Sanofi, Italy
- Rima Nabbout, Université Paris Descartes, France
- Mihalis Panagiotidis, Cyprus Institute of Neurology and Genetics, Cyprus
- Maurizio Taglialatela, University of Naples, Italy
- Joanna Trubicka, Children's Memorial Health Institute, Poland
- Damjan Osredkar, Medical University of Lubijana, Slovenia
- Katie Donahue, FDA, CDER, USA
- Ralf-Dieter Hilgers, Aachen University, Germany
- Galliano Zanello, IRDiRC / EJPRD

Zanello G, Garrido-Estepa M, ….. Brooks PJ. *EMBO Mol Med*. 2023 Jul 10;15(7):e17159. doi: 10.15252/emmm.202217159.



Tx for disease 1

Tx for disease 2



Muscle
Liver
Brain

Same “ delivery box” to treat multiple muscle, liver, brain diseases

Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC)

THE TWO CRITICAL PATHWAYS OF BGTC RESEARCH

BGTC BY THE NUMBERS

13 Industry Partners

12 Government Agencies, NIH Institutes and cross-institute programs

10 Non-Profits

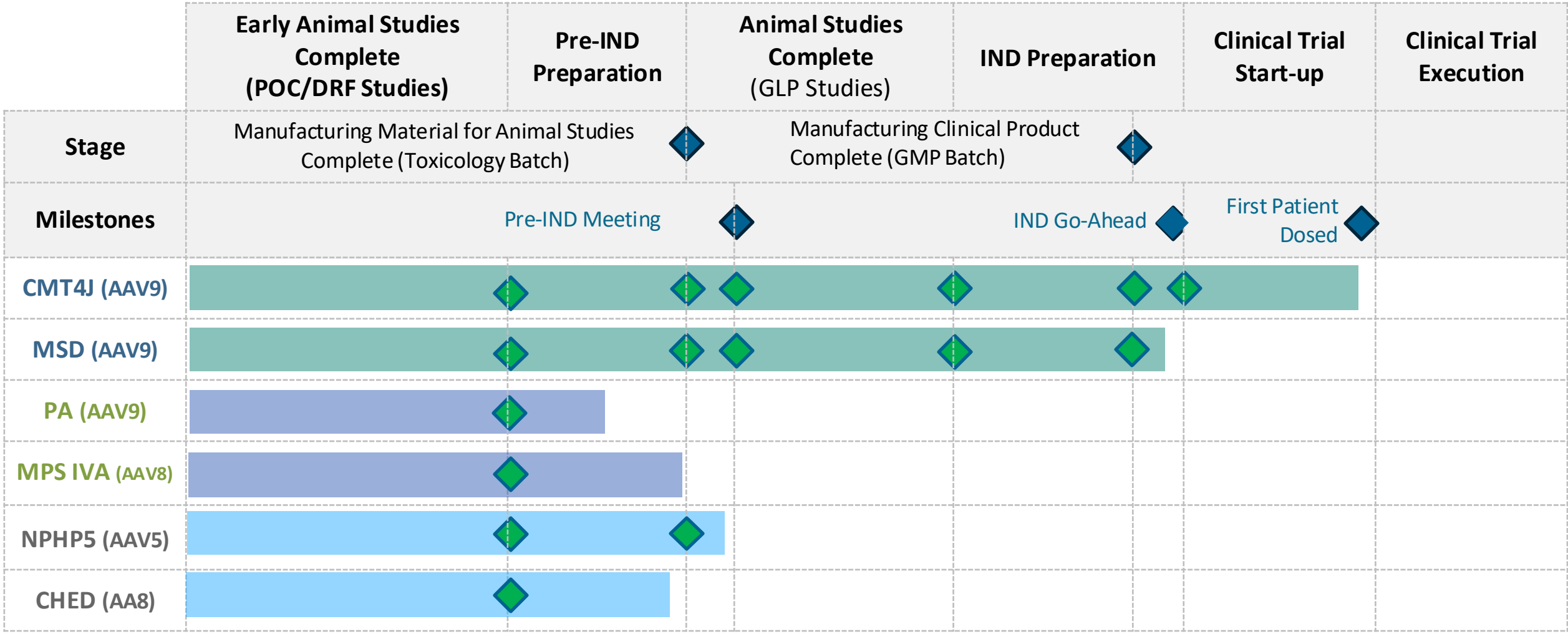
$80.5 million project budget

FNIH

https://fnih.org/our-programs/AMP/BGTC

Learn more and subscribe for email updates

BGTC teams advancing to pre-clinical and clinical milestones

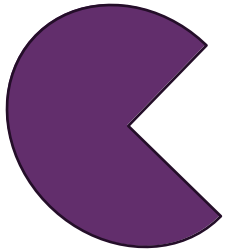


POC=Proof-of-Concept, DRF=Dose Range Finding, GLP=Good Laboratory Practice, IND=Investigational New Drug

Milestone **Organ System:** Neurological Systemic Ocular

AAV gene therapy

Gene editing as a modular therapeutic



(or mRNA encoding)

delivery system

guide RNA (if necessary)

National Center for Advancing Translational Sciences

Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (UG3/UH3, Clinical Trial Required) RFA-RM-22-016		
PI Name	**Institution Name**	**Title**
JIANG, YONG-HUI (contact) BERRY-KRAVIS, ELIZABETH MARA ZHOU, JIANGBING	YALE UNIVERSITY	A non-viral CRISPR-mediated genome editing delivery platform as a potential therapy for neurogenetic diseases

IND-enabling Studies for Platform Clinical Trials of Genome Editors in Multiple Diseases (U01 Clinical Trial Not Allowed) RFA-RM-24-001		
PI Name	**Institution Name**	**Title**
AHRENS-NICKLAS, REBECCA CLARE (contact) MUSUNURU, KIRAN	CHILDREN'S HOSP OF PHILADELPHIA	Personalized prime editing as a platform for hepatic inborn errors of metabolism
CHEN, ZHENG-YI	MASSACHUSETTS EYE AND EAR INFIRMARY	AAV-mediated editing to treat human autosomal dominant hearing loss DFNA41 and DFNA2

Provide support for applications that propose a novel genome editing clinical trial that includes at least two different diseases, using the same genome editor, route of administration, and delivery system.

https://www.npr.org/sections/shots-health-news/2025/05/15/nx-s1-5389620/gene-editing-treatment-crispr-inherited

https://www.nejm.org/doi/full/10.1056/NEJMoa2504747

The NEW ENGLAND JOURNAL of MEDICINE

CURRENT ISSUE SPECIALTIES TOPICS

ORIGINAL ARTICLE | BRIEF REPORT

Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease

Authors: Kiran Musunuru, M.D., Ph.D., Sarah A. Grandinette, B.S., Xiao Wang, Ph.D., Taylor R. Hudson, M.S., Kevin Briseno, B.S., Anne Marie Berry, M.S., Julia L. Hacker, M.S., +37, and Rebecca C. Ahrens-Nicklas, M.D., Ph.D. Author Info & Affiliations

Published May 15, 2025 | DOI: 10.1056/NEJMoa2504747 | Copyright © 2025

Rare Disease Day at NIH, 2026

National Center for Advancing Translational Sciences

How to create personalized gene editing platforms: Next steps toward interventional genetics

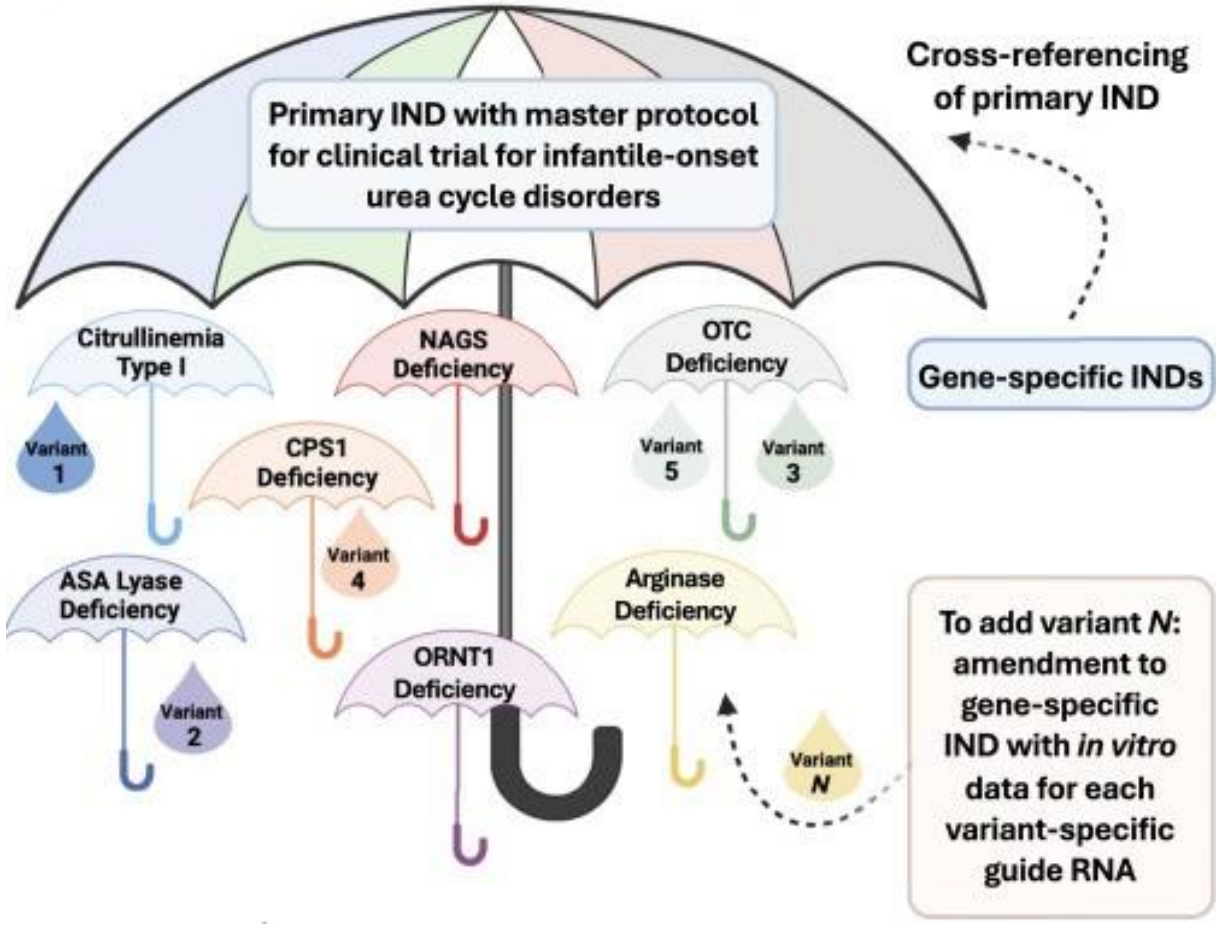
Rebecca C. Ahrens-Nicklas[1,2,3,*] and Kiran Musunuru[1,2,3,4,5,*]

https://pubmed.ncbi.nlm.nih.gov/41175865

ONE toxicology biodistribution study

in ONE animal species for

ALL 7 diseases



Urea Cycle Disorders Consortium

National Center for Advancing Translational Sciences

The American Journal of Human Genetics (2025) 112, 2826–2829

Many

~~One~~ rare disease**s** at a time

https://media.sciencephoto.com/image/z9430051/800wm/Z9430051-Herd_of_zebra.jpg

National Center for Advancing Translational Sciences

Team DRDRI

Philip Brooks

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Alice Chen

Jeanine D'Armiento

Eric Sid

Joanne Lumsden

Mary Baez

Meera Shah

Linda Ho

National Center for Advancing Translational Sciences

Gwen Nichols, MD

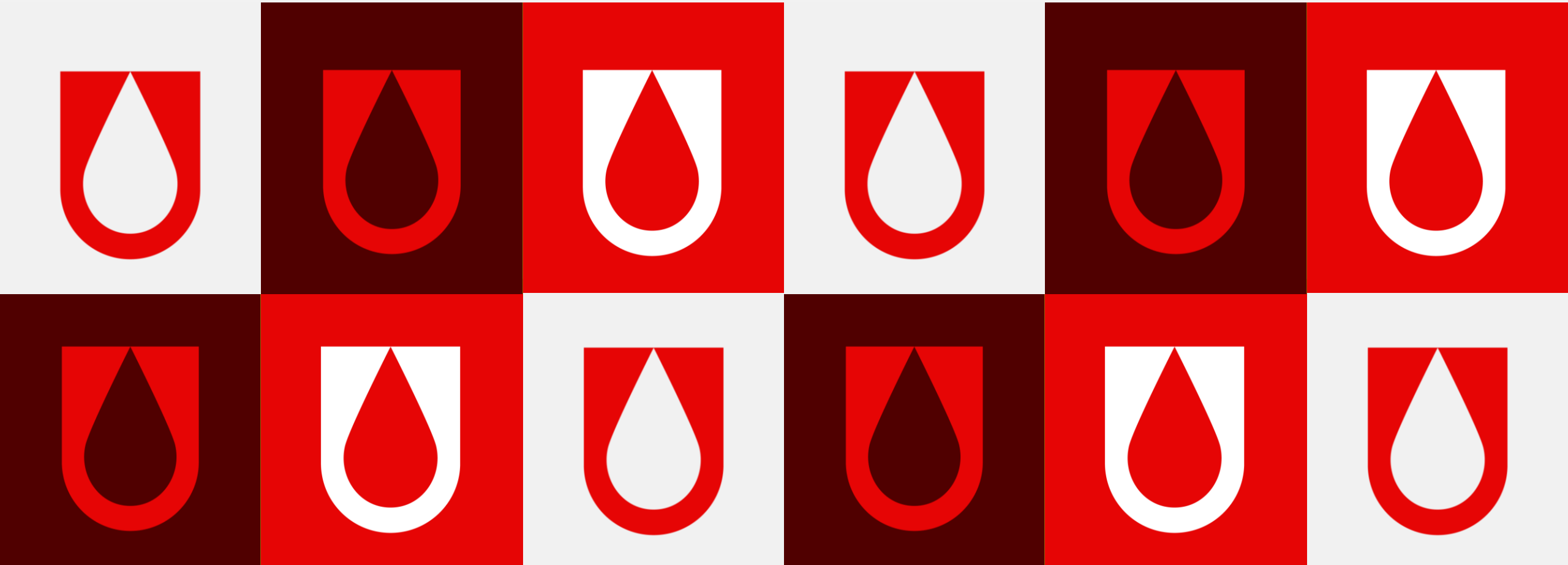
Chief Medical Officer

Blood Cancer United

FasterCures: Cross-disease platform approaches – PedAL Master Trial

April 2026

Gwen L Nichols, MD Chief Medical Officer
Blood Cancer United

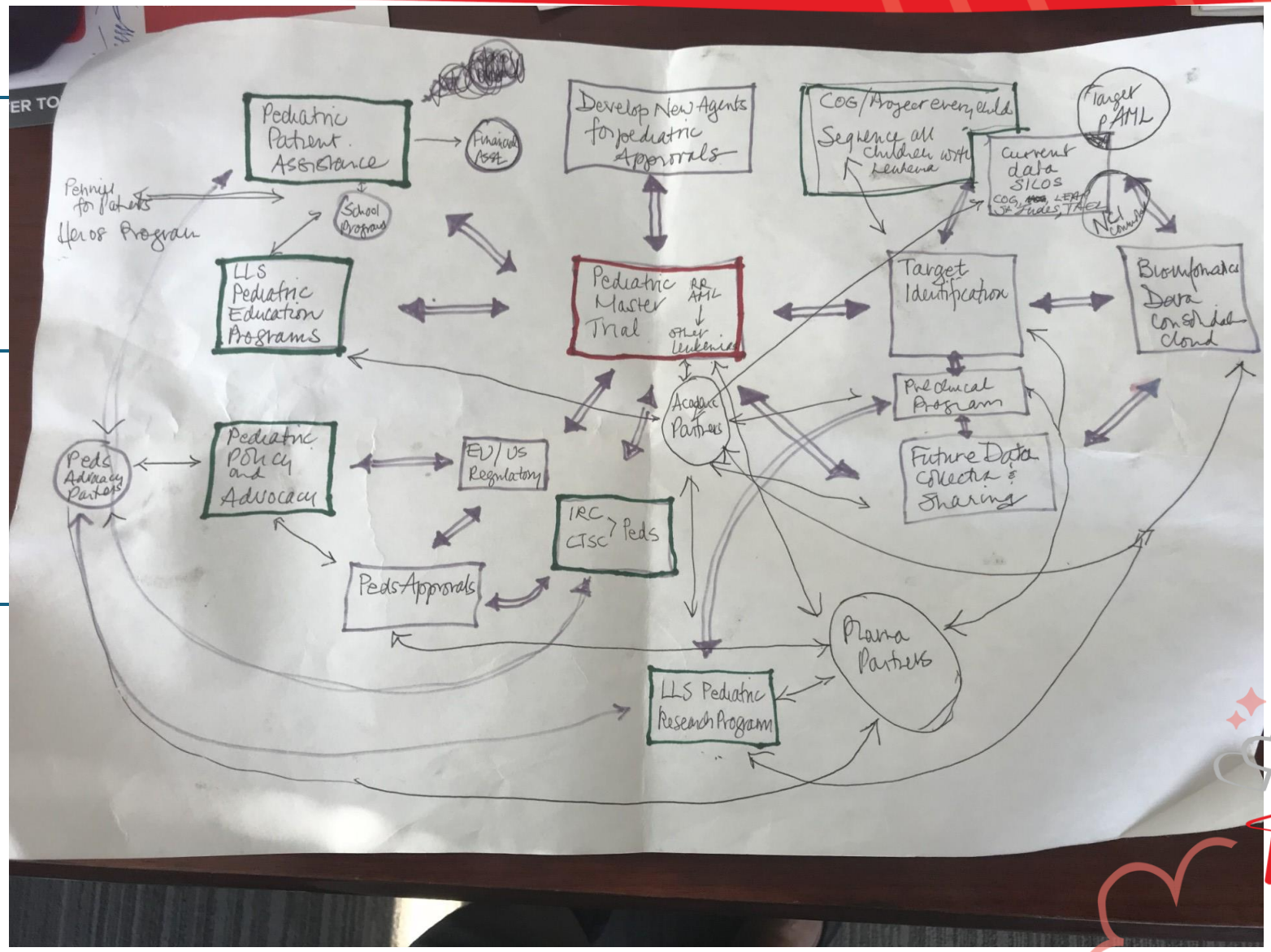


An Origin Story

There is a poor business case for pediatric cancer drug development

At Blood Cancer United I had the opportunity to try and change that paradigm

I imagined a platform for both clinical and basic research as well as education and support to address the need



The Challenge We Faced

- Pediatric leukemia is rare and there are many biologic subsets
- The biology of pediatric leukemia is distinct from leukemia in adults, yet new drugs are mostly developed in adults
- A comprehensive, global effort for trials is necessary to be successful
- Despite regulatory incentives, only seven drugs were approved for pediatric cancers (US) in the past 10 years, while more than 100 new drugs were approved for adults
- Essential data exists within individual international consortia and pediatric hospitals; data sharing has been a challenge (different endpoints, different standards of care)

Blood Cancer United®
Dare to Dream Project
Transforming treatment and care for kids

The Problem

Most new pediatric oncology drug approvals in the past 10 years have been based on small, single-arm phase 1/2 or phase 2 trials that have occurred years after the adult approval.

Drug	Approval Date	Indication	Trial Phase	Sample Size
Revumenib	Nov 2024	Pediatric R/R KMT2A AML	1/2	23
Bosutinib	Sept 2023	Pediatric CML	1/2	49
Azacitidine	May 2022	Pediatric JMML and Advanced MDS	2	18
Atezolizumab	Dec 2022	Alveolar soft part sarcoma	2	52
Tisagenlecleucel	Aug 2017	Pediatric B-ALL	2	63

DARE TO DREAM PROJECT

A solution: PedAL

- **SCREEN** – Via the PedAL screening trial, screen all children with relapsed leukemia for precision-medicine trial eligibility based on clinical data, leukemia cell surface markers and next-generation genomic sequencing
- **DISCOVERY –** sample acquisition for correlative biology, target discovery, and biobanking
- **ACCESS** – Partner with pharma, NCI and FDA/EMA, and European study groups to support drug development platform that achieves regulatory objectives to ensure that children have early access to effective therapies
- **DATA** – Create an internationally agreed data dictionary. Improve data collection to inform changes in primary outcome measures and toxicity definitions that are essential for approval of new therapies for children with leukemia
- **Blood Cancer United is the trial sponsor and works collaboratively with academia and pharma partners to collect regulatory quality data**

Blood Cancer United®
Dare to Dream Project
Transforming treatment and care for kids

PedAL Progress

screening
trial

182
sites

611
patients

4
countries

venetoclax
subtrial

88
sites

102
patients

20
countries

ziftomenib
subtrial

20
sites

24
patients

3rd trial in contract

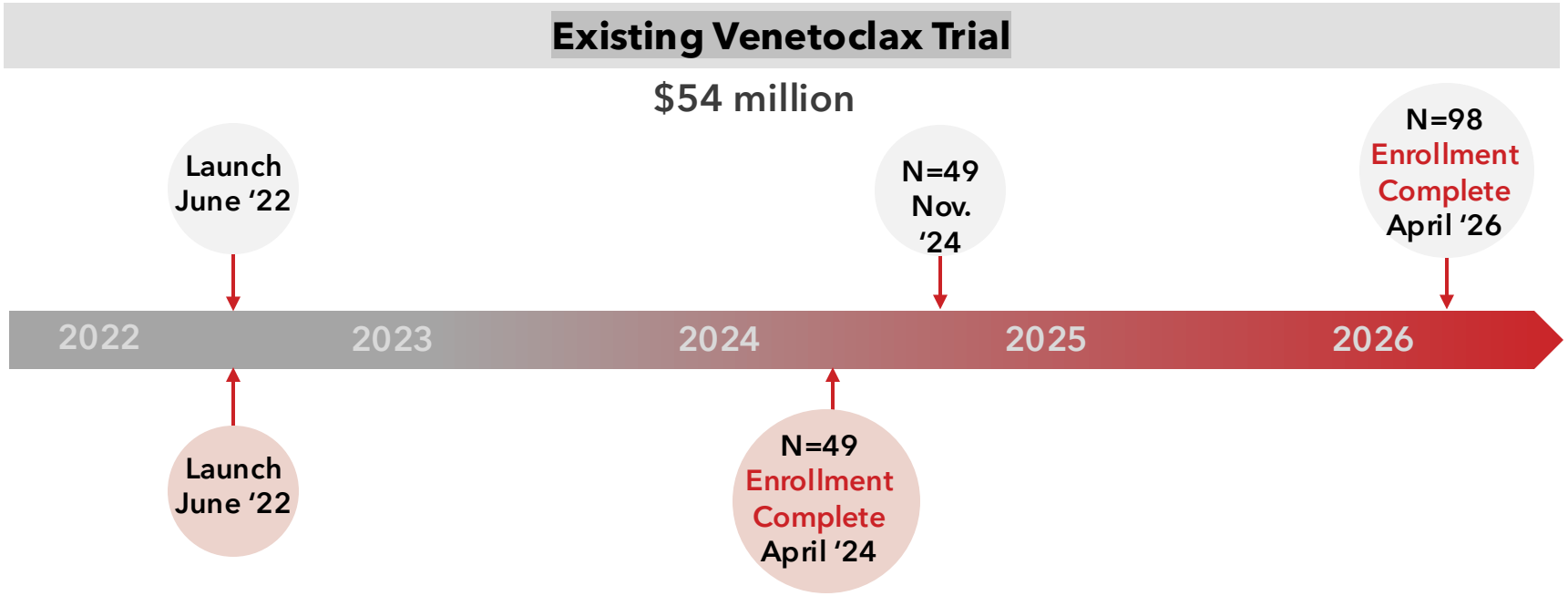
7
countries

Blood Cancer United
Dare to Dream Project
Transforming treatment and care for kids

abbvie

KURA ONCOLOGY

A Tale of Two Trials



- All 49 enrolled patients receive experimental therapy
- 49 matched controls extracted from RWD

Trial could enroll in less than half the time and no patient receives SOC

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Where we go next: Real World Evidence Project

- We've made substantial progress with PedAL, but we are not satisfied.
- It's not fast or efficient enough: time and cost for trials remains high.
- Large randomized trials are not feasible in targeted subsets of patients
- Having evidence for regulators of target efficacy and outcomes with standard of care could decrease the time and cost of trials in rare diseases

Blood Cancer United is partnering with Tempus AI to create a RWD repository in pediatric AML using parental/patient consent.

RWE can be generated from this database for proposed trials. This will be freely available for investigators and available at cost to pharma.

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The FDA is supportive of using RWD

FDA is committed to realizing the full potential of fit-for-purpose RWD to generate RWE to advance the development of therapeutic products and strengthen regulatory oversight of medical products across their lifecycle

The 21st Century Cures Act of 2016 was designed to accelerate medical product development and bring new innovations and advances faster and more efficiently to the patients who need them. FDA released guidance on using RWE in clinical trials in response

Under the Cures Act, FDA's RWE Program must evaluate the potential use of RWD to generate RWE of product effectiveness to help support approval of new indications for drugs approved under FD&C Act Section 505(c) or to help to support or satisfy post-approval study requirements

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

There is precedent for using RWD

Eflornithine

Pediatric neuroblastoma (Dec 2023)

First time FDA relied on a single externally controlled trial to support an approval in oncology

Single-arm trial (n=90) compared with external control group derived from a COG-sponsored trial (n=270)

https://pubmed.ncbi.nlm.nih.gov/37883734/

Avelumab

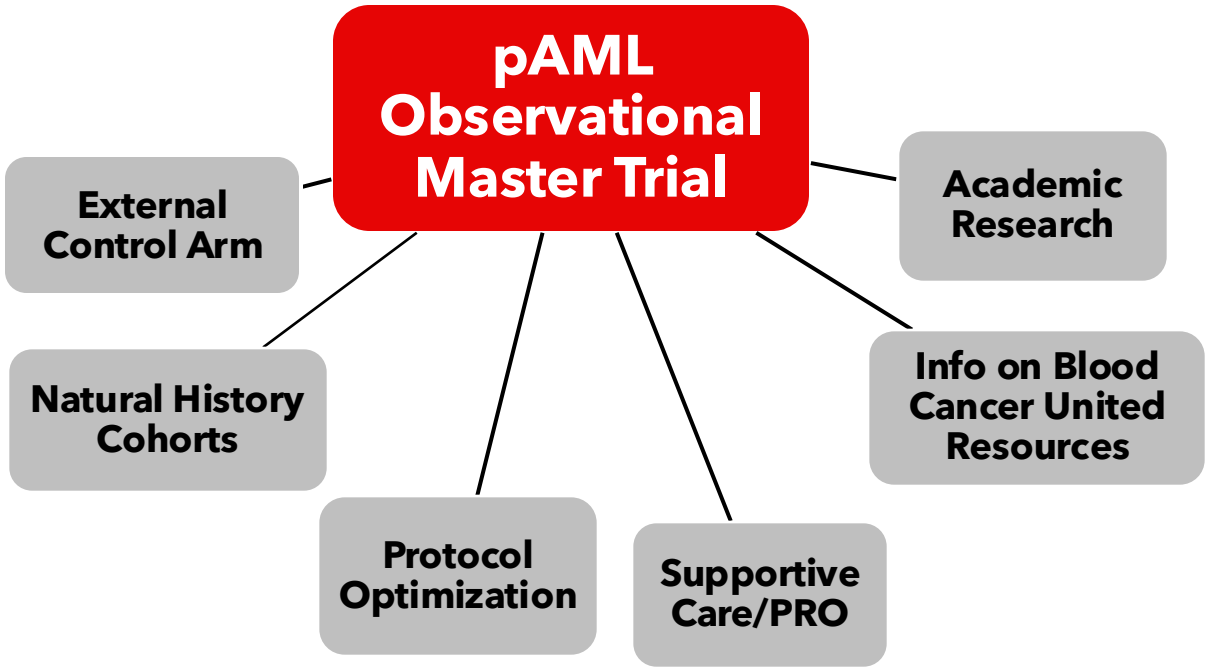
Adolescent & adult metastatic Merkel cell carcinoma (March 2017)

A single arm trial (n=88) generated from retrospective chart review of SOC-treated patients describing the natural history of metastatic Merkel cell carcinoma

Submitted alongside data from single-arm interventional trial to support regulatory approval

These were single use efforts and manually created. We are proposing a "reusable" resource to be shared by investigators and pharmaceutical partners

pAML Observational Trial



pAML Observational Master Trial

- Patients/parents consent electronically confirming access EMR data from all sources
- Parents have access to consolidated records and can analyze data using the with links to Blood Cancer United and other resources for support
- Data is de-identified for research purposes, and regularly updated
- Data is organized according to agreed data dictionary and modified for research purposes

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Conclusions

- While progress in the PedAL trial has been gratifying, the development of new agents in pediatrics remains expensive and lengthy
- We are initiating a registry of RWD for pAML in order to speed development for trials of more targeted agents for subsets of this already rare disease
- Parents and patients will become “citizen scientists” and have access to consolidated medical records from multiple sources
- We look forward to discussing further with the FDA and making certain we collect the elements which will provide the appropriate patient level data for regulatory purposes
- The registry will be a free resource for research, and available to pharmaceutical partners as needed to determine therapeutic targets or for regulatory purposes
- We believe this model is replicable for other rare diseases and look forward to sharing our learnings

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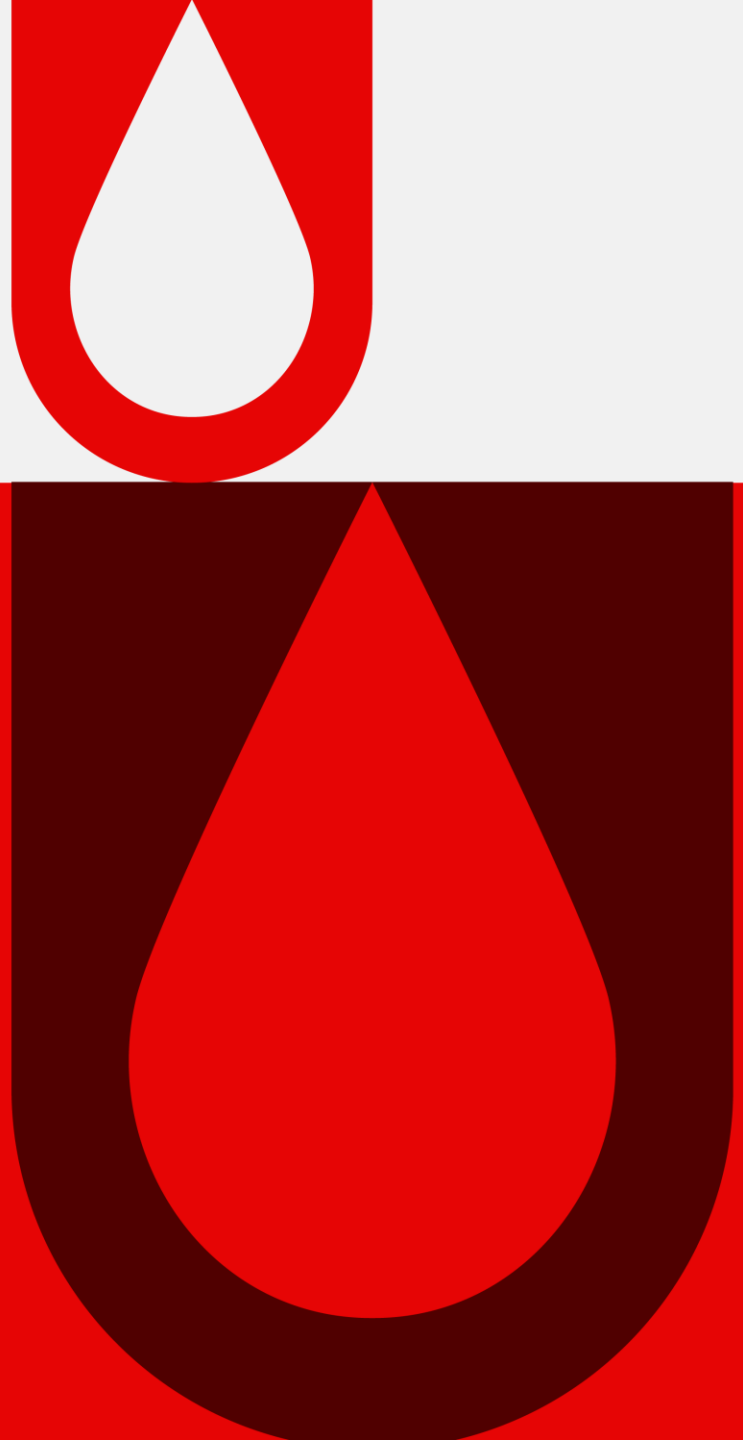

Thank you

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Thank You!

For additional questions or to learn more about TRAIN, please contact TRAIN@milkeninstitute.org