Natural History Studies to Support Regenerative Medicine: A How-To Webinar

Thursday, October 27, 2022, 11:00 a.m.–1:00 p.m. ET

Office of Tissues and Advanced Therapies (OTAT)
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration (FDA)

Webinar Agenda

11:00 a.m.	Welcome Session
11:05 a.m.	Natural History Studies: Introduction
11:40 a.m.	Panel Presentations: Natural History Studies — Perspectives from Researchers, Participants, and Caregivers
12:25 p.m.	Natural History Studies Q&A
12:55 p.m.	Closing Remarks

Virtual Meeting Considerations

- The webinar will be recorded and available online after the event.
- Closed captioning is available in Zoom.
- Use the Q&A box to submit questions throughout the event.
- Use the chat box to share general comments and report technical difficulties.

RegenMedEd Series

- OTAT's event series about regenerative medicine
- Goals of the RegenMedEd Series:

Discuss foundational information about regenerative medicine therapies, including gene therapy and cell therapy

Explore opportunities to engage with FDA and advance regenerative medicine research and drug development

Hear from FDA, patients, advocates, researchers, and other important stakeholders about their experiences

Natural History Studies: Introduction

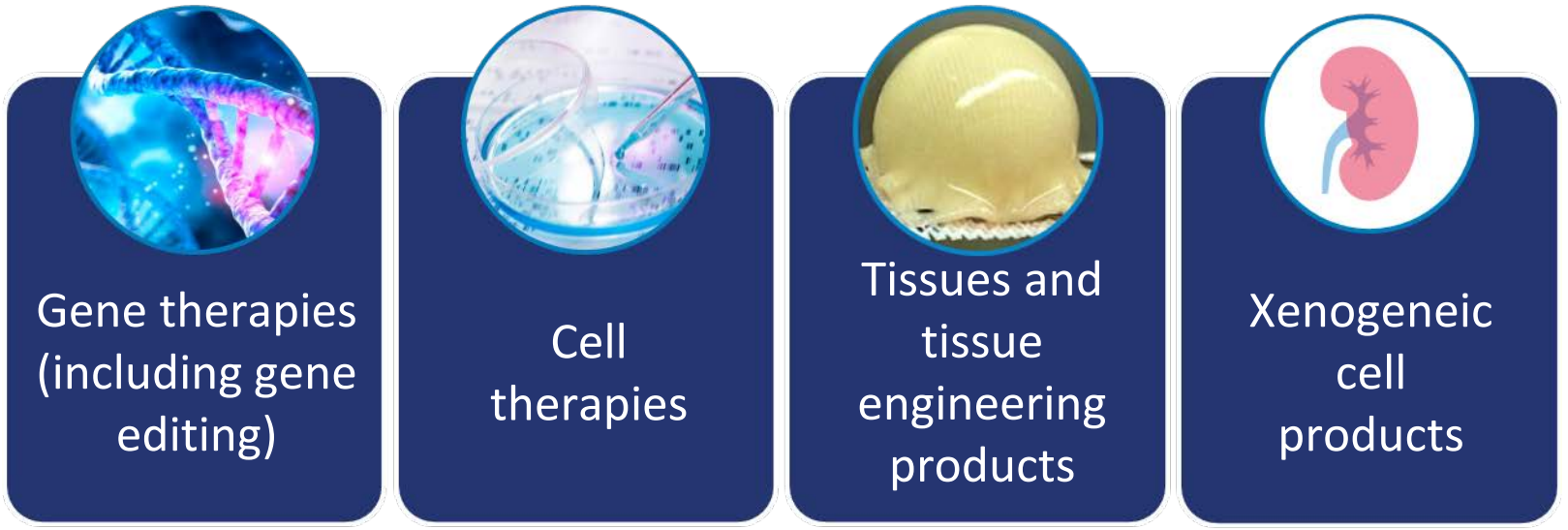
Speaker:
Wilson W. Bryan, MD
Director
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

Our Mission

The Office of Tissues and Advanced Therapies (OTAT) promotes the public health through collaborative, science-based regulation of medical products. This includes facilitating drug development and ensuring safety of individuals. OTAT's regulatory decisions are data-driven, impartial, and compassionate.

Regenerative Medicine Therapies

- Regenerative medicine involves using stem cells, engineered biomaterials, gene editing, and other technologies to repair or replace damaged cells, tissues, or organs.
- Types of regenerative medicine therapies (RMTs):



Rare Diseases and Regenerative Medicine

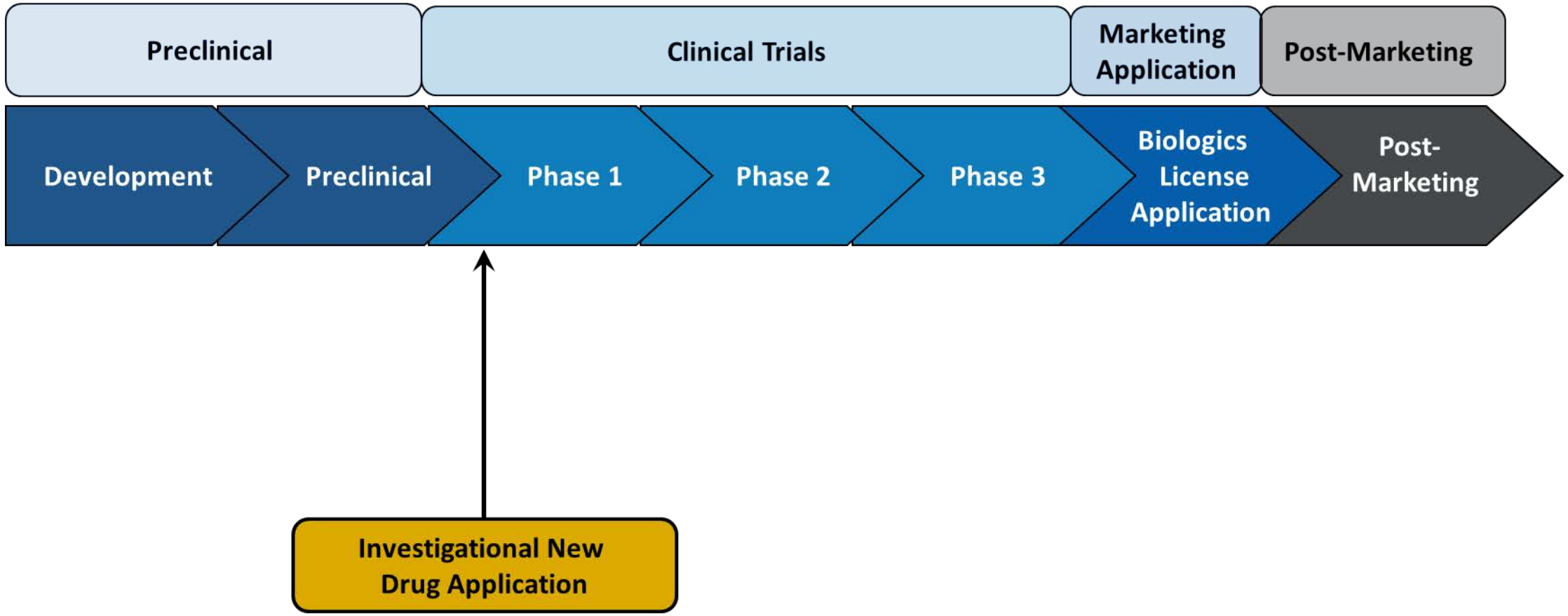
OTAT has over 2,000 investigational new drug applications (INDs) for cell and gene therapy products.

80% of rare diseases are caused by a single-gene defect.

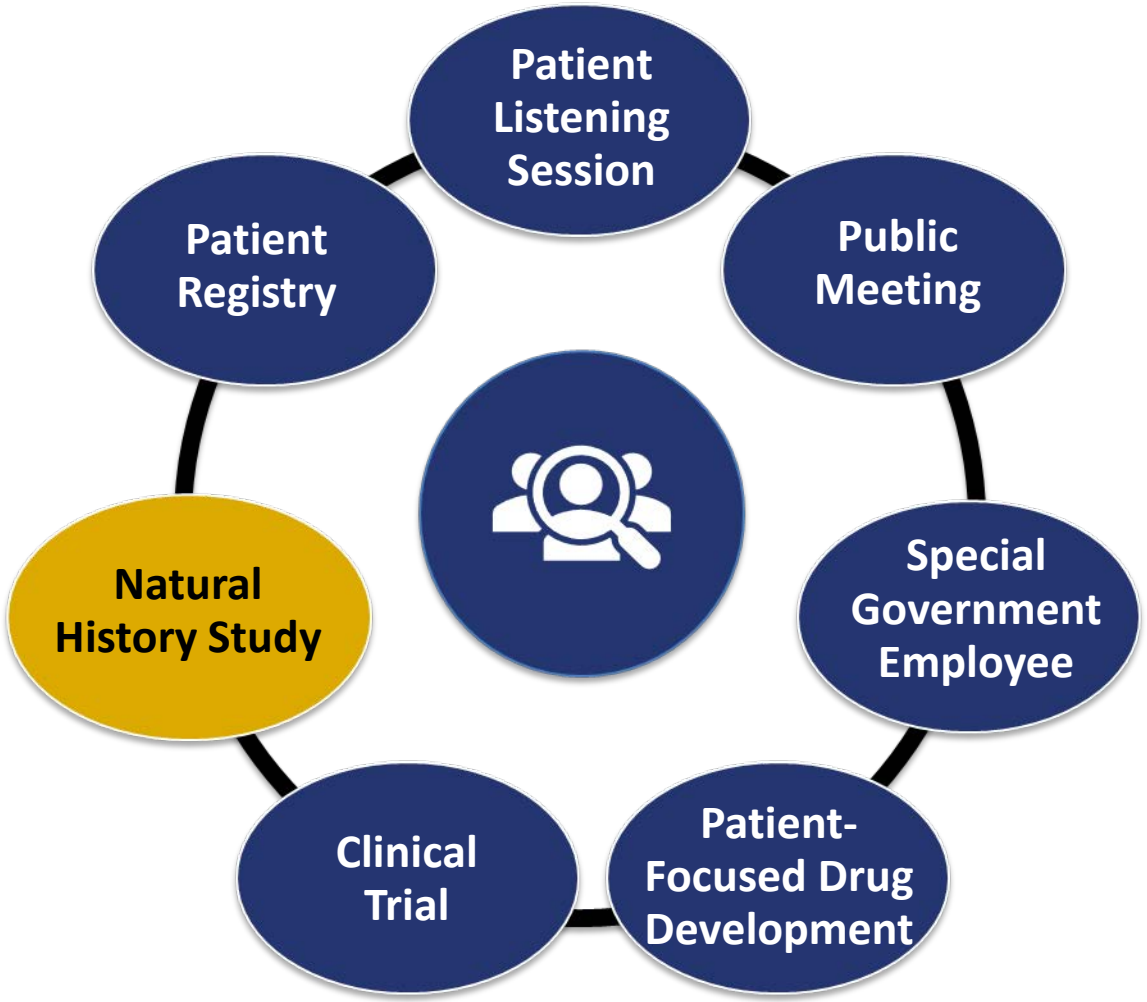
FDA has approved four gene therapies for single-gene disorders.

Most rare diseases are not well understood.
Patient participation in clinical research is critical.

Drug Development Overview



How Patients Can Advance Research & Development



Natural History Studies: *Defined*

- A study that follows a group of people over time who have, or are at risk of developing, a specific disorder.

- A natural history study collects data in order to understand the disorder. Information may include:
  - Age
  - Diagnosis
  - Symptoms
  - Effect on quality of life
  - Test results
  - And more

Natural History Studies: *Purpose*

- Identify demographic, genetic, environmental, and other variables that correlate with disease development and outcomes.
- Better characterize the disease and the patient population.
- Clarify the disease impact on the lives of patients and their families.
- Collect patient-reported outcomes and other clinical outcomes that are specific to the disease.
- Inform the clinical drug development process.

Natural History Studies: *Types*

Retrospective versus prospective natural history studies: Both rely on data collected from patient visits.

Retrospective: **Past**	• Often a first step in describing progression • Data are collected from existing medical records
Prospective: **Ongoing**	• Establish definitions and data to be collected ahead of time • Data are collected from ongoing patient visits

Natural History Studies: *Types (continued)*

Cross-sectional versus longitudinal natural history studies: Both rely on data collected from a cohort.*

Cross-sectional	Data collected over a specified, limited time period
Longitudinal	Data collected at various time points over a long period

**Can be either retrospective or prospective*

Natural History Studies: *Protocols*

FDA recommends that natural history study protocols specify, in detail:

Who should be included in the study (inclusion and exclusion criteria)

What information is to be collected

When the data will be collected (if prospective)

How data are to be collected and analyzed

Natural History Studies: *Drug Development*

- The FDA does not require that natural history studies be conducted for drug development programs.

- To be applied in a drug development program:
  - The FDA recommends an early evaluation of the depth and quality of existing natural history knowledge.
  - Natural history studies are most useful if data are available prior to initiating clinical trials.

- Data should be collected for a sufficient duration to capture clinically meaningful outcomes and determine variability in the course of the disease.

Natural History Studies: *Benefits to Disease Research in Clinical Development*

These studies can inform and de-risk drug development for rare and/or heterogeneous diseases:

1. Refines the target patient population
2. Identifies and develops clinical outcomes
3. Identifies and develops biomarkers
4. Informs design of future clinical trials
5. Serves as external control (in limited circumstances)

Natural History Studies: *Purpose in Rare Disease Research*

- The progression of rare diseases is often poorly described.
- Natural history data can be critical for drug development.
- When knowledge about the disease is insufficient to guide clinical development, a natural history study may help in designing an efficient drug development program.

Steps to Get Started

For researchers and rare disease groups looking to start a natural history study, FDA encourages the following:

Do a literature review

Talk to experts in the disease area

Perform a retrospective natural history study

Recommended design: prospective and longitudinal

Contact Information

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FDA Headquarters

Panel Presentations: Natural History Studies — Perspectives From Researchers, Participants, and Caregivers

Moderator:
Katherine Needleman, MS, PhD, RAC
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Meet Our Panelists

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FDA CBER Webinar

Natural History Studies to Support Regenerative Medicine: A How-To Webinar

October 27, 2022

The How and Why of Natural History Studies for Rare Diseases

Richard S. Finkel, MD

Center for Experimental Neurotherapeutics

St. Jude Children's Research Hospital

Disclosures – Richard Finkel

- Research Support: AveXis, Biogen, Capricor, Catabasis, Cytokinetics, NIH, Ionis, Italfarmaco, MDA, PTC Therapeutics, ReveraGen, Roche, Sarepta, Santhera, Scholar Rock, Summit
- Personal Compensation for advisory board participation: AveXis/Novartis, Biogen, Catabasis, Neurogene, Genentech/Roche, Sarepta, Summit
- Unpaid participation: EveryLife Foundation, n-Lorem Foundation, MDA, Cure SMA, SMA Europe, Florida and N Carolina State Dept of Health (newborn screening)
- Editorial fees from Elsevier
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Objectives of This Presentation

1. Why are natural history studies important?
2. How we conducted natural history studies – challenges and lessons learned
3. How the data were used and obstacles

Why Natural History Studies Are Important

To understand the disease and its progression:

1. *Identify the main clinically important features to the patient*
   - How the person survives, functions, or feels
   - These determine the outcome measures in a clinical trial
2. *Understand trajectories of change* for these topics as the disease evolves
   - By age, sex, and stage of disease
3. *Impact of standard-of-care*
4. *Consider fit-for-purpose use in clinical trials*
   - "Power Calculation" – how many participants are needed
   - Duration of the study
   - Clinically meaningful change in the main outcome measures

My Participation in Natural History Studies: Different aims, funding support, challenges

- **Duchenne muscular dystrophy**
  - NIH-sponsored “UDP”: genetic/clinical associations
  - DoD-sponsored CINRG network: outcome measures and natural history
  - Industry sponsored DMDNH: run-in to gene therapy study
- **Spinal Muscular Atrophy**
  - SMA Foundation-sponsored PNCR Network for SMA: comprehensive
  - Industry-sponsored RESTORE registry: targeting gene therapy patients
- **Charcot-Marie-Tooth Inherited Neuropathies**
  - NIH-sponsored (RDCRN) Inherited Neuropathies Consortium
    - gene discovery, natural history, biomarkers
- **Pompe Disease (metabolic disorder)**
  - Industry-sponsored

How to Run an Effective Natural History Study

Example of Spinal Muscular Atrophy (SMA)

- PNCR study group developed (Boston, NY, Philadelphia – now 6 sites)
- Sponsored by SMA Foundation with a long-term vision of clinical trial readiness
- Natural history study devised in 2004, remains active and evolving
- Formal protocol, IRB-approved, Patient/Parent consent
- Structure the study similar to a clinical trial with structured visits
  - Evaluations at 0, 2, 4, 6, 9, 12, 18, 24, 30, 36 months
  - Trained clinical evaluators, with annual retraining
  - Examine a lot of testing items – exploratory at first
  - Then decide what works and what doesn't
  - Develop and test biomarkers (blood, MRI, nerve stimulation testing)

The Observational Protocol For SMA

The observational study of the progression of SMA
Key Name: FinkelR_10-XXXXXX
XXXX-XXX
July 21, 2010

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Observational study of spinal muscular atrophy type I and implications for clinical trials

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ABSTRACT

Objectives: Prospective cohort study to characterize the clinical features and course of spinal muscular atrophy type I (SMA-I).

Methods: Patients were enrolled at 3 study sites and followed for up to 36 months with serial clinical, motor function, laboratory, and electrophysiologic outcome assessments. Intervention was determined by published standard of care guidelines. Palliative care options were offered.

Results: Thirty-four of 54 eligible subjects with SMA-I (63%) enrolled and 50% of these completed at least 12 months of follow-up. The median age at reaching the combined endpoint of death or requiring at least 16 hours/day of ventilation support was 13.5 months (interquartile range 8.1–22.0 months). Requirement for nutritional support preceded that for ventilation support. The distribution of age at reaching the combined endpoint was similar for subjects with SMA-I who had symptom onset before 3 months and after 3 months of age ($p = 0.58$). Having 2 *SMN2* copies was associated with greater morbidity and mortality than having 3 copies. Baseline electrophysiologic measures indicated substantial motor neuron loss. By comparison, subjects with SMA-II who lost sitting ability (n = 10) had higher motor function, motor unit number estimate and compound motor action potential, longer survival, and later age when feeding or ventilation support was required. The mean rate of decline in The Children's Hospital of Philadelphia Infant Test for Neuromuscular Disorders motor function scale was 1.27 points/year (95% confidence interval 0.21–2.33, $p = 0.02$).

Conclusions: Infants with SMA-I can be effectively enrolled and retained in a 12-month natural history study until a majority reach the combined endpoint. These outcome data can be used for clinical trial design.

Neurology® 2014;83:810–817

Figure 1 Time-to-event curves for SMA-I

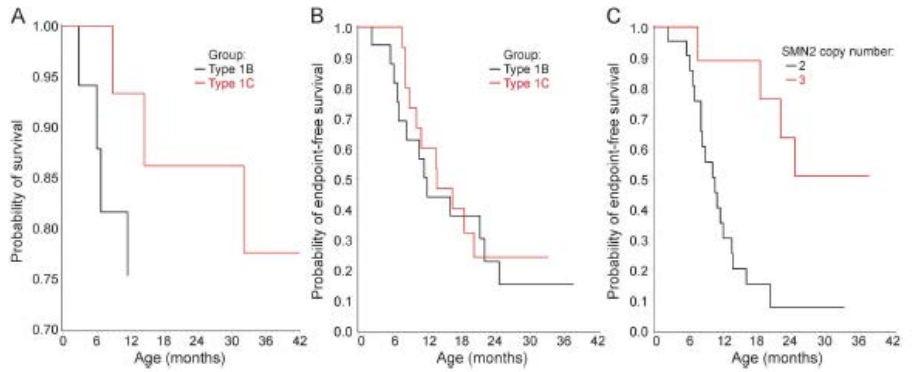
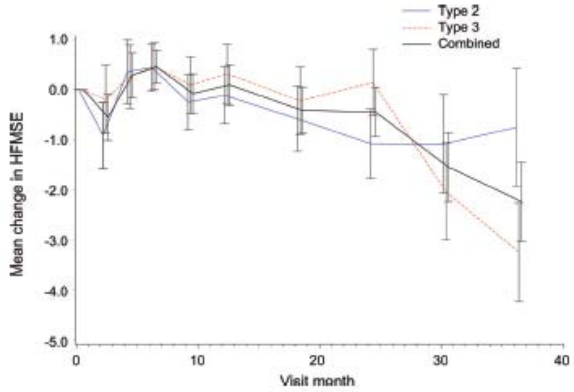


Figure 2 Mean change in Expanded Hammersmith Functional Motor Scale (HFMSE) score over time estimated using a repeated measures analysis of covariance model with time treated as a categorical variable



Prospective cohort study of spinal muscular atrophy types 2 and 3

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ABSTRACT

Objective To characterize the natural history of spinal muscular atrophy type 2 and type 3 (SMA 2/3) beyond 1 year and to report data on clinical and biological outcomes for use in trial planning.

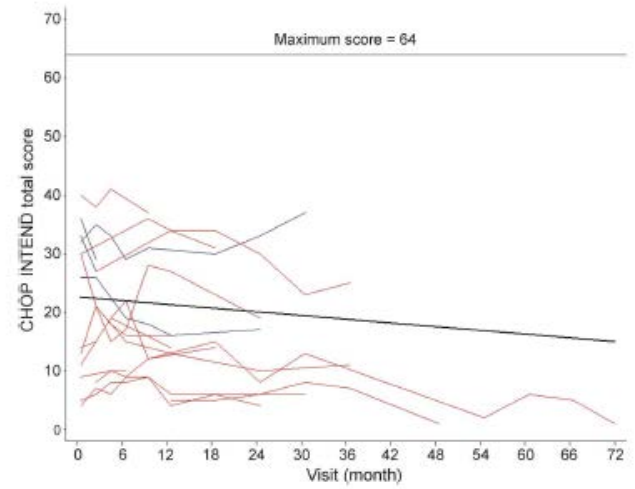
Methods: We conducted a prospective observational cohort study of 79 children and young adults with SMA 2/3 who participated in evaluations for up to 48 months. Clinically, we evaluated motor and pulmonary function, quality of life, and muscle strength. We also measured *SMN2* copy number, hematologic and biochemical profiles, muscle mass by dual x-ray absorptiometry (DXA), and the compound motor action potential (CMAP) in a hand muscle. Data were analyzed for associations between clinical and biological/laboratory characteristics cross-sectionally, and for change over time in outcomes using all available data.

Results: In cross-sectional analyses, certain biological measures (specifically, CMAP, DXA fat-free mass index, and *SMN2* copy number) and muscle strength measures were associated with motor function. Motor and pulmonary function declined over time, particularly at time points beyond 12 months of follow-up.

Conclusion: The intermediate and mild phenotypes of SMA show slow functional declines when observation periods exceed 1 year. Whole body muscle mass, hand muscle compound motor action potentials, and muscle strength are associated with clinical measures of motor function. The data from this study will be useful for clinical trial planning and suggest that CMAP and DXA warrant further evaluation as potential biomarkers.

Neurology® 2012;79:1889–1897

Figure 2 CHOP INTEND motor function in SMA-I: Longitudinal data



Finding cures. Saving children.

Challenges We Faced

- Parents and patients – why bother?
- Getting doctors motivated to participate – funding is crucial
- Some testing not covered by insurance – need more funding
- Ensuring good compliance over years – travel support, flexibility in scheduling
- Sustainability of the registry – major issue
- Tissue bank – funding, patient acceptance
- Ownership of data, samples – variable
- Governance of the network – minor

How Was the Data Used and Shared? SMA Example

- Get the word out
  - Presentations (many) at medical conferences
  - Publications (many)
- Share the dataset with collaborative agreements
  - International informal collaboration (“iSMAC”) – 1200 patients with SMA
  - Pharmaceutical companies
- Share patient samples with other academic researchers and pharma

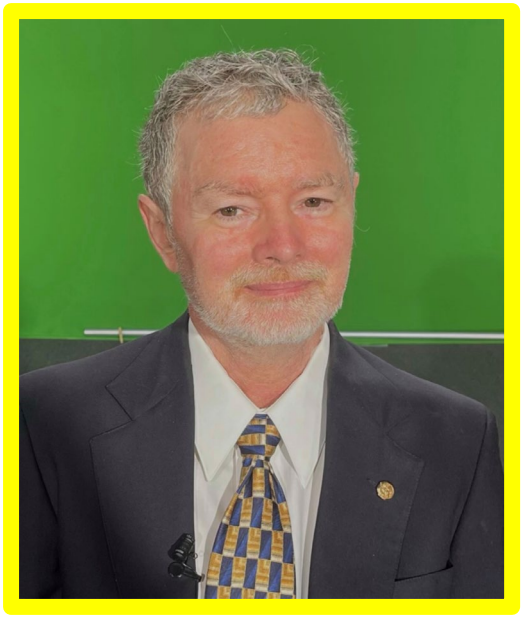
St. Jude Children's Research Hospital

Thank You.

Questions?

Meet Our Panelists

Richard Finkel, MD
Director, Center for
Experimental Neurotherapeutics
St. Jude Children's Research Hospital



Bradley Williams, PhD
Director of Research & Diagnostic Innovation,
Jain Foundation

Erin Ward, MEd
President & Co-Founder
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Natural History Studies: Lessons From Limb Girdle Muscular Dystrophies

Bradley Williams

Director of Research, Jain Foundation

Dysferlinopathy Clinical Outcome Study participant

Components of a Natural History Study

What does your disease area need?

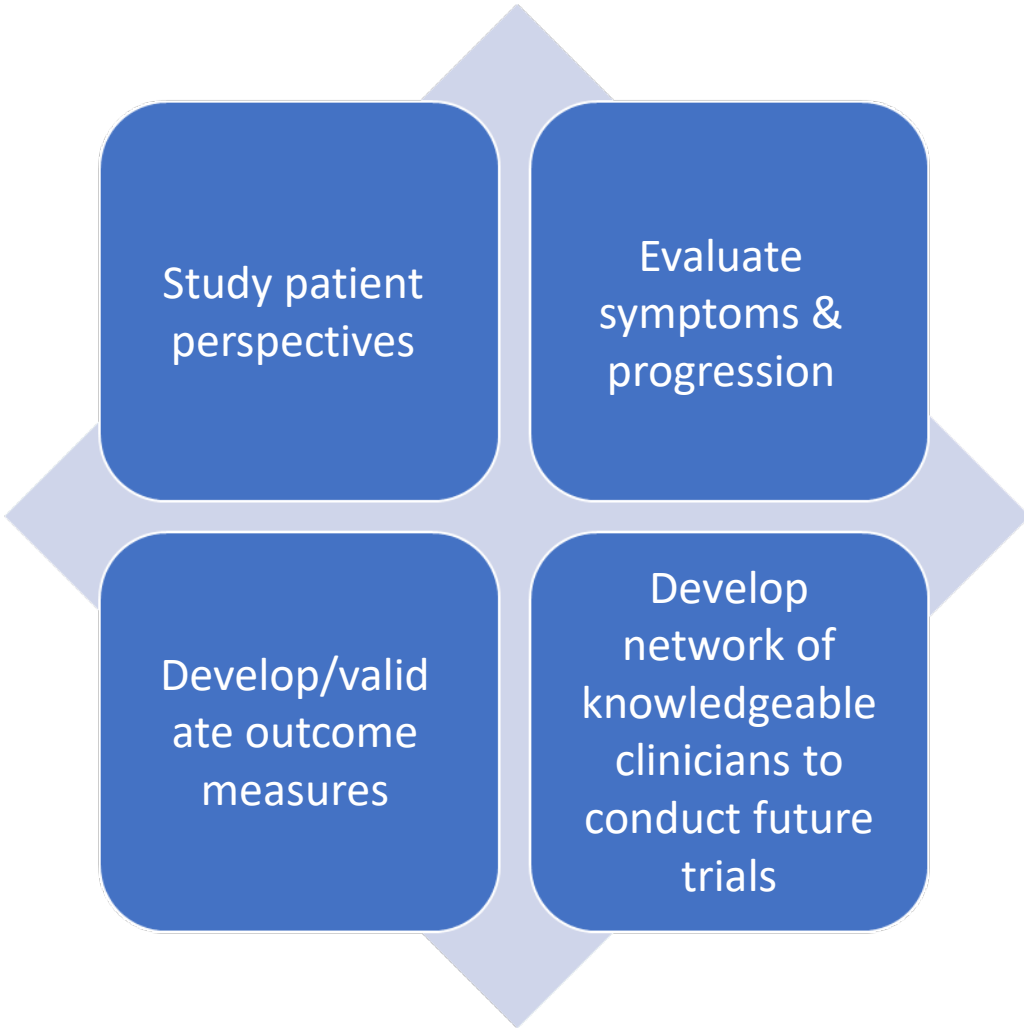
- What are barriers to clinical trial readiness?
- What is/isn't known about symptoms and progression?
- Are outcome measures available? Do they need to be developed/validated?
- What is important to patients/family members?
- What would be important for a meaningful treatment to provide?

Setting goals for study

What do you intend the study to accomplish?

Study goals should address at least some needs for clinical trial readiness

It may not be practical to address every clinical trial readiness need



Deciding on study parameters

WHAT
TO TEST?

WHO TO
TEST?

WHERE TO
TEST?

LENGTH
OF STUDY?

NUMBER OF
PARTICIPANTS?

ONE OR
MULTIPLE SITES?

Setting a timeline

- Setup can take up to 2 years
- Time needed to run study depends on disease area
- Make sure to factor in time to analyze data and publish findings

NATURAL HISTORY STUDIES TAKE A LONG TIME!!!

DON'T WAIT. START NOW!

Recruiting

- Recruiting is a major challenge in a rare disease natural history study
- Disease registries are extremely helpful in recruiting!
- Make sure all participants truly have the disease being studied
- Cover subpopulations
  - Different stages of progression
  - Different genetics influencing phenotype
  - Diverse ethnicities

Consenting

Make sure consent covers all the possibilities

Think of everything that the data might be used for in the future…

- Designing a clinical trial
- A historical control for a clinical trial
- Correlating genetic data or samples with phenotype

…And who might be using it.

- Other clinicians/researchers
- Drug developers
- Regulatory agencies

The participant experience

Some feedback from COS

Top reasons for participating:

- Help advance science
- Help fellow patients
- Have access to knowledgeable clinicians

"Felt exhausted after visits due to traveling"

"Sad to end visits because it's great to talk to people who know about the disease"

"Can be emotional having to think about my condition when I normally try to just get on with life"

Recommendations

- Consider impact of travel and assessments
- Incorporate remote assessments?
- Determination of patient experience
  - Impact on function/quality of life
  - Include patient-reported outcome measures
  - Patient attitudes on what treatment benefit would be meaningful
- Important to give participants feedback on what's being learned

Quality control

If a multicenter study, make sure data are being collected/scored consistently between sites

Periodic visits/training for study sites and new personnel

Check (and “scrub”) data—
data entry errors do happen!

Data collection & analysis

Important to have a plan for data analysis—biostatistician needs to be part of team from the very beginning

Unstructured data are hard to analyze and may be less useful

Plan on and budget for data analysis continuing for a significant time after study visits have ended

Ultimate goal isn't just to collect data, it's to analyze it and make it accessible to the scientific/medical community

Biomaterials and Archived data

- What kind of samples to collect?
- Who will be able to access data/samples, and under what circumstances?
- Where will the data and samples be stored? Plan for long-term custodianship (e.g., data repository, biobank)
- Ensure compliance with HIPAA, GDPR, etc.

Thanks to…

University of Newcastle

- Heather Hilsden COS Project Manager
- Meredith James COS Lead Physiotherapist
- Jordi Diaz-Manera, MD, PhD COS Site PI
- Volker Straub, MD, PhD COS Lead PI

Nicolas Johnson, MD, Virginia Commonwealth University

Head of GRASP-LGMD Consortium

Laura Rufibach, PhD, Jain Foundation

JF COS Advisor/Liaison

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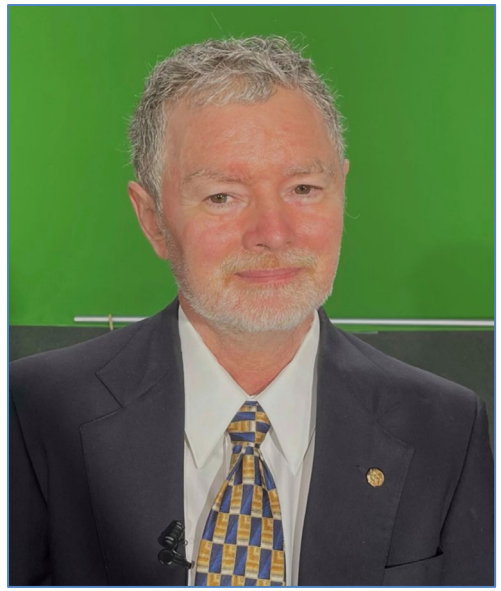

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Natural History Studies to Support Regenerative Medicine: A How-To Webinar Caregiver and Patient Advocacy Leader Perspective

Erin Ward, *MEd, Rare Parent & Caregiver*

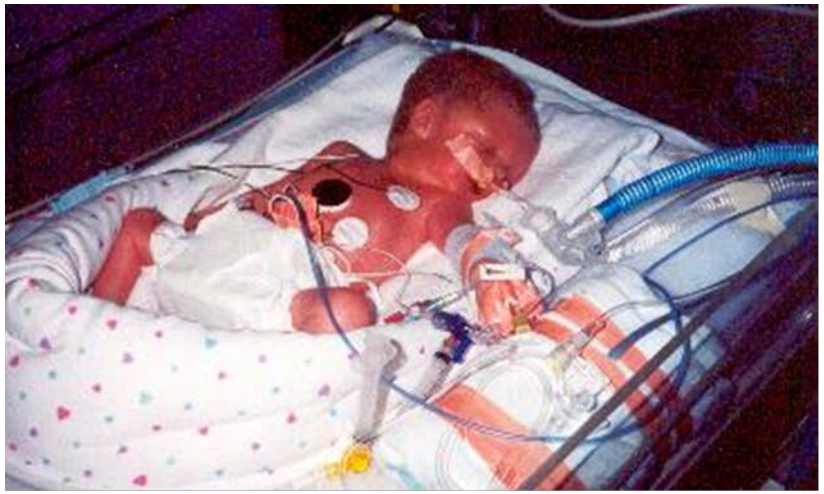
Co-Founder & President, MTM-CNM Family Connection

Natural History Studies: Caregiver & Advocacy Leader Perspective
Talk Aims & Aspirations

- Natural History Studies: The Caregiver Experience
- Cumulative Advocacy: Understanding Your NHS landscape
- Co-designing Natural History Studies
- Collaborating with Multiple Stakeholders to optimize NHS

"Let's talk!"

Will Ward
2001-2021



A Little Will ...Goes a Long Way

MTM-CNM Family Connection

Connecting Families with MTM/CNM to Research, Resources, and Relationships

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MTM-CNM Family Conferences

Gathering Your Community: National Conferences:

- Patient-Professional Collaborative Conference Model
- Community infrastructure brings stakeholders together
- Bidirectional learning opportunities
- Partnered in developing & facilitating natural history studies
- Accelerated preclinical research on site at conferences
- Early engagement with pharma companies introduced to the community at conferences
- Discussion forums on community priorities and meaningful treatment outcomes for patients & families
- Advocacy for patient engagement & partnership throughout drug & therapy development

Patient Engagement With the FDA:

- Patient Listening Session (2020)
- Patient-Focused Drug Development Meeting (2021)

Why Focus on NHS Development?

- Better understand the disease across a disease community
- Help to guide development of standards of care
- Discover new qualities of a disease not previously understood
- Optimize and de-risk clinical trials
- Essential for care of today AND the development of potential treatments of tomorrow

The Challenges of Collecting NHS

- Population may be small and difficult to access
- Lack of current academic researchers or industry interest
- Participation fatigue when there are competing efforts to collect the same data
- Smaller disease communities can often feel limited, with options or pressure to compromise goals to not lose interest
- Perhaps no tangible benefit for those participating in terms of a direct treatment
- Lack of financial support for long-term sustainability

Getting Started: NHS Tips for Patients, Caregivers, & Advocacy Leaders

- Understanding the landscape: cumulative advocacy
- Taking inventory of what has already been done
- Connecting with all stakeholders
- Look to other rare diseases that can serve as models
- Continually ask:
  - How can you partner together & accelerate development?
  - How can you ensure patient engagement at every step?

Cumulative Advocacy:

"The evolution of rare disease development that increases through the selfless giving of individuals and families that enter into the community's relay race. It is the culmination of participation, growth, and building on each other's progress that can set rare disease communities up for the greatest likelihood of success. If everyone does what they can, when they can, even if it may be unlikely that they themselves or their loved one may benefit from a treatment, we have the greatest chance of reaching the finish line."

The ultramarathon of gene therapy development of rare diseases: How can we cross the finish line together? Ward, E. Clinical Therapeutics Vol. 44 No. 8 2022

Taking Inventory of Community NHS

- What data exists?
  - Registries
  - Natural history studies:
    - Disease-community based
    - Academic-researcher driven; institution sponsored
    - Industry sponsored; product based
  - Preclinical observational studies
  - Retrospective clinical chart reviews
  - Community surveys: Quality of life, impact of disease studies
  - Real-world evidence

Community Considerations for Developing an NHS

- Who “owns” the data?
- Where does all the data live?
- Who has access, and how can it be optimized?
- How portable or compatible is the data?
- Is the information being collected “pre-competitive”?
- Is the data developing an understanding of the disease that is important in care for patients now? Outside the context of a product?
- Will the NHS stand the test of time? Allow for growth?

Connecting With All Stakeholders: Through Community Conferences

- Partnered with academic researchers and industry sponsors to collect natural history data and preclinical trial data on site at conferences
- Opportunity for co-designing NHS with voice of patient and caregiver included
- Accelerated the pace of data collection, saved resources, & increased participation
- Navigated barriers together and created broader opportunities following the conference (i.e., home site visits)

Rare Disease NHS vs. Product-Based NHS

- What will allow for the greatest understanding of the disease?
- Is the product-based NHS sponsor willing to partner with the disease community in developing, conducting, analyzing, and sharing NHS collected?
- Is the sponsor willing to share de-identified data, especially with regard to data that has a direct impact on better understanding the disease outside the context of a product? As soon as it is collected? Prior to publishing?
- Is there a plan in place to ensure data is not lost if a sponsored-program ends?
- Are sponsors willing to share data with disease community consortiums?

Operationalizing Patient Engagement in NHS Development: Tips for Academic and Industry Partners

- Engage early & often with the community, throughout process
- Support & complement what is already happening
- Align expectations
- Co-design natural history studies with patients
- Create opportunities to hear the natural history of the patient experience
- Include patients in the analysis and publications of NHS data
- Integrate collection of meaningful real-world evidence (RWE)
- Remember that patients ultimately “own” their data

Natural History Studies: Commitment to the Process

- Collection of natural history must be an iterative process that evolves as the understanding of the disease evolves
- Commit to sharing, learning, improving, & recalibrating when needed
- Consistently ask how the community can optimize the usability, adaptability, compatibility, and portability of the NHS data
- How can patients be empowered now with their own data?

Challenges & Lessons Learned

- Consider that natural history studies will ultimately grow the ability of your rare disease community to learn as much as possible, as soon as possible, for as many as possible, and for as long as possible in direct collaboration with rare disease patient communities.
- Never underestimate the power of the patient and caregiver voice and insist on the inclusion of patient and caregivers in the development of, monitoring of, use of, and publication of future natural history studies.

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Thank you & connect with us:

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- Email: info@mtm-cnm.org
- Facebook: MTM-CNM Family Connection
- Twitter: @mtmcnmfamily






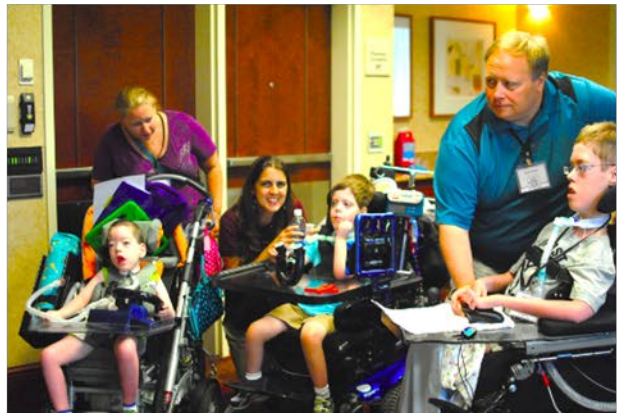

Photo credits: Levi Gershkowitz

living in the Light

Panel Discussion

Please type your questions in the Q&A box at the bottom of your screen.

Stay Connected!

Helpful Resources:

- Visit CBER's website
- Sign up for our newsletter, "What's New @ CBER"
- Follow us on Twitter: @FDACBER
- Learn more about natural history studies:
  - Rare Diseases: Natural History Studies for Drug Development
  - FDA Natural History Study Grants
  - FDA Natural History Study FAQs
  - FDA Funding Opportunities for Rare Disease Research

Thank you!

- Webinar materials will be available in the coming weeks on FDA.gov.
- Stay tuned for future OTAT events, including:
- Patient-Focused Drug Development Listening Meeting – Tuesday, November 15
- OTAT Town Hall: Cell Therapy Chemistry, Manufacturing, and Controls – Wednesday, December 7
- Webinar: Information for Practitioners – FDA's Regulatory Oversight of Regenerative Medicine
- Products – Thursday, November 17

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