North American Spinal Cord Injury Consortium

Spinal Cord Outcomes SCOPE Partnership Endeavor

SCI Community Engagement in Research

SCOPE Monthly Meeting

December 13, 2022

Agenda

1. Introduction to NASCIC
2. Suite of Services
3. Project Collaboration - RWE
4. NASCIC Research Advocacy Course
5. Community Engagement & Collaborative Framework

Clinical Trial Shortcomings

500

A study by CTTI, $100,000 investment in community engagement by Clinical Trial sponsors & CROs could produce gains of more than 500x the initial investment.[1]

80

Nearly 80% of clinical trials fail to meet their enrollment timelines and up to 50% of research sites enroll one or no participants.[2]

50

Nearly half of all U.S. device trials do not meet their enrollment targets.[4]

62

"The cumulative impact of a patient engagement activity that avoids one protocol amendment and improves enrollment, adherence, and retention is an increase in net present value (NPV) of $62M."[3]

This underscores the importance of involving people with lived experience in the design of clinical studies as well as gaining their input and preferences throughout the total product lifecycle.

1) Bennett Levitan, Kenneth Getz, Eric L. Eisensten, Michelle Goldberg, Matthew Harker, Sharon Hesterlee, Bray Patrick-Lake, Jamie N. Roberts, Joseph DiMasi (2017). Assessing the Financial Value of patient Engagement: A Quantitative Approach from CTTI's patient Groups and Clinical Trials Process
2) Yale Center for Clinical Investigation (YCCI)
3) [2]Levitan B, Getz K, Eisenstein EL, Goldberg M, Harker M, Hesterlee S, Patrick-Lake B, Roberts JN, DiMasi J. Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project. Ther Innov Regul Sci. 2018 Mar;52(2):220-229. doi: 10.1177/2168479017716715. Epub 2017 Jul 17. PMID: 29714515; PMCID: PMC5933599.
4) Patient Engagement Advisory Committee Executive Summary for Patient Engagement in Medical Device Clinical Trials Meeting. U.S. Food and Drug Administration. October 2017. Accessed February 11, 2021. https://www.fda.gov/media/108162/download

Engagement Movement

About NASCIC

North American Spinal Cord Injury Consortium

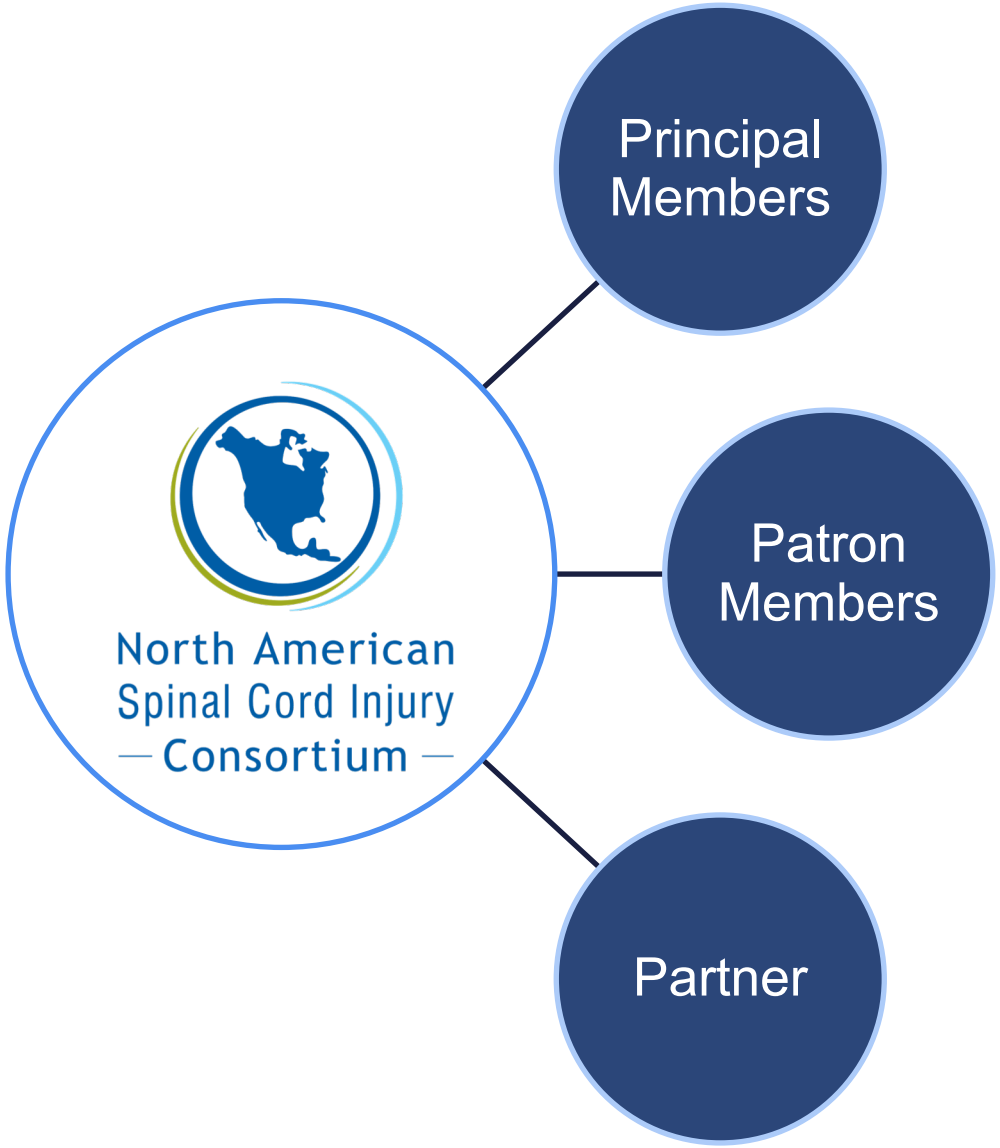
Mission – To bring about unified achievements in research, care, cure, and policy by supporting collaborative efforts across the spinal cord injury community.

To achieve this mission, NASCIC will **identify gaps**, **communicate resources**, and be a **conduit for collaboration** between the community of people living with SCI and the many stakeholders.

Timeline

2016
Call to action for the voice of lived experience
2017
Grassroots effort by the community
2018
Official launch of NASCIC
2019
Building infrastructure, Project Review, needs assessment
2020-2022
Engagement Projects & placement, Capacity Building, SCI Research Advocacy Course….

https://nasciconsortium.org/membership



NASCIC Membership Reach

2022

289 ~~27~~ Individual Members

118 Project Engagement Advocates

Over 50 Community Based Organizations and Model Centers

With a Reach over 700,000

Projects

- Comprehensive Inclusion of SCI in CDC Neurologic Registry Development
- Development of tool(s) to track participation and performance in Activity-Based Therapy after SCI/D
- Open Data Sharing in SCI Research - iCORD
- Information on Emerging Therapies

Cross Cutting Initiatives

FDA Partnership
Research Advocacy
SCITRIALS.ORG Clinical Trial Finder

Spinal Cord Injury TRIALS

FDA

Membership - Canada

New Brunswick
Ontario
Quebec
Saskatchewan
Alberta
British Columbia

Other Countries:
Curacao
Costa Rica

Membership - US States

Alabama
Arizona
Arkansas
California
Colorado
Connecticut
Georgia
Florida
Illinois
Indiana
Iowa
Kentucky
Louisiana
Maine
Massachusetts
Maryland
Missouri
Montana
New Hampshire
New Jersey
New Mexico
North Carolina
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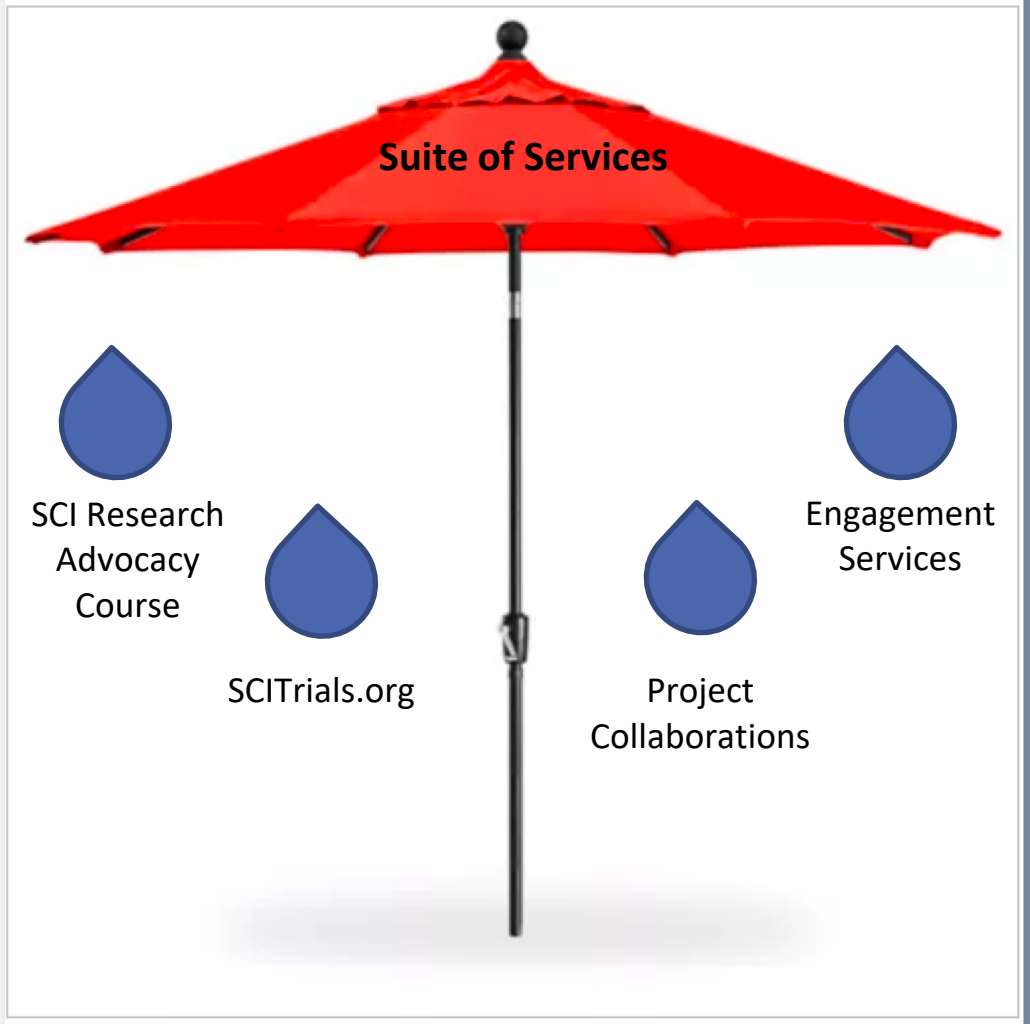
Oklahoma
Oregon
Pennsylvania
South Carolina
Tennessee
Texas
Utah
Virginia
Washington
Washington D.C.
Wisconsin

Community Engagement

With the support of the spinal cord injury (SCI) community, its members and the Craig H. Neilsen Foundation, NASCIC has the opportunity and responsibility to facilitate and implement community engagement across the SCI research process.

NASCIC offers a **suite of engagement services** designed to enhance profitability and reduce the in the development process by leveraging the expertise of individuals with lived experience at every stage.



Project Review Committee

- Consists of 10 active Members
- Monthly meetings
- Developed evaluation & tracking metrics of on-going projects
- Opportunities for new members each year
- Review external & internal proposals
- Interest in partnering for consumer engagement, letter of support, or research recruitment

Project Engagement Collaborations

Types of Engagements

- Consumer Advisory Boards
- Focus Group
- Community Surveys
- Advocacy Speaking
- Clinical Trial recruitment

Seeking input from people with SCI lived experience?
Contact NASCIC VP, Ian Burkhart
iburkhart@nasciconsortium.org

https://nasciconsortium.org/projects/application/

Engagement Projects

Full listing of all current projects is available https://nasciconsortium.org/projects/

Project
Open Data Sharing in SCI Research – iCORD
Upper Extremity Outcome Measures
Vagus Nerve Stimulation as an Immunomodulatory Therapy for Acute SCI
Survey on Info about Emerging Therapies
Guidelines for Activity-based Therapy after SCI/D
Expanding Knowledge and Information Delivery around Improving Upper Extremity Function after Cervical SCI
Consumer engagement with the development of a high bandwidth brain interface
Neuromodulation Working Group for Bladder & Bowel

Survey on Information about Emerging Therapies

Purpose:

This project was intended to assess how people living with SCI access timely and reliable information about newer therapeutic interventions and clinical trials.

Timeline:

- Recruitment *& Kick-off Meeting: Oct 2019
- Survey finalization: November - December 2019
- IRB approval: January 2020
- Distribution: January-March 2020
- Data Analysis and manuscript preparation: May-August 2020

Project Lead: Dr. Michael Stillman, MD

Lived Experience:

Three members with lived experience served on the Advisory Board. Duties included:

- review the survey,
- assist with distribution for survey responses,
- review and assist with the interpretation of the results and
- actively assist in the composition of the final manuscript (if volunteer to do so).

Publications & Distribution:

Pazzi C, Farrehi C, Capron M, Anderson K, Richardson B, Stillman M. An Assessment of Which Sociodemographic and Spinal Cord Injury-Specific Characteristics Influence Engagement With Experimental Therapies and Participation in Clinical Trials. Top Spinal Cord Inj Rehabil. 2021 Fall;27(4):28-39. doi: 10.46292/sci20-00070. Epub 2021 Nov 17. PMID: 34866886; PMCID: PMC8604506

Farrehi, C., Pazzi, C., Capron, M. *et al.* How individuals with spinal cord injury in the United States access and assess information about experimental therapies and clinical trials: results of a clinical survey. *Spinal Cord Ser Cases* 6, 103 (2020). https://doi.org/10.1038/s41394-020-00354-6

Lay language distribution through the NASCIC network

Upper Extremity Outcome Measures

Purpose:

The overall goal of this project is to establish item pools that evaluate movement in the context of function, create standardized administration and scoring procedures, and conduct a preliminary evaluation of reliability. This was for Phase II of a larger five phase study.

Timeline:

- Recruitment & Kick-off Meeting: February 2020
- Advisory Board Group Meetings: February-April 2020
- Individual Advisory Meetings (2/ea): April-May 2020
- Completion of Phase II for the Calibration Study: June 2020

Project Lead: Olivia Biller, OT, PhD candidate

Lived Experience:

Four members with lived experience served on the Advisory Board. Duties included:

- assess upper extremity proposed tasks prior to meetings,
- join the active discussion of 5 group meetings
- partake in at least 2 individual meeting/interviews

Publications & Distribution:

Poster at the International Spinal Cord Society 61st annual scientific meeting in Vancouver, Canada, September 2022

Anticipating a follow on proposal for the Phase III, IV & V

Lay language distribution through the NASCIC network

Neuromodulation Working Group for Bladder & Bowel

Purpose:

The role of the Advisory Team will be to help identify and define the bladder and bowel goals of individuals with SCI, and provide a critical perspective for creating a road map that balances individuals' needs, concerns, preferences, and goals with available and emerging neuromodulation approaches to address them..

Timeline:

- Recruitment *& Kick-off Meeting: Oct 2017
- Advisory Meetings: January-May 2018
- Distribution: June-August 2018
- Data Analysis and manuscript preparation: September-December 2018

Project Lead: Dr. Dennis Bourbeau, PhD

Lived Experience:

Six members with lived experience served on the Advisory Board. Duties included:

- actively help design and review the survey,
- assist with distribution for survey responses,
- review and assist with the interpretation of the results and
- actively assist in the composition of the final manuscript (if volunteer to do so).

Publications & Distribution:

Bourbeau, D., Bolon, A., Creasey, G. *et al.* Needs, priorities, and attitudes of individuals with spinal cord injury toward nerve stimulation devices for bladder and bowel function: a survey. *Spinal Cord* 58, 1216–1226 (2020). https://doi.org/10.1038/s41393-020-00545-w

Clinical Media coverage in UroToday: https://www.urotoday.com/beyond-the-abstracts/pelvic-health-reconstruction/neurogenic-bladder/125852-needs-priorities-and-attitudes-of-individuals-with-spinal-cord-injury-toward-nerve-stimulation-devices-for-bladder-and-bowel-function-a-survey-beyond-the-abstract.html

Presentation at NYC NANS & NER in 2020

Lay language distribution through the NASCIC network

Project Engagement Database

Types of Engagements
- Consumer Advisory Boards
- Focus Group
- Community Surveys
- Advocacy Speaking
- Clinical Trial recruitment

How to get involved?
- Benefit of membership
- Call for participants
- Enroll to get involved

https://nasciconsortium.org/projects/

Project Feedback

COURSE DEVELOPMENT

Project Goal

This project aims to help make SCI research more relevant and responsive to the needs of people living with SCI by greatly increasing the number of consumers who engage in the research process. To accomplish this outcome, the project will:

1. Engage individuals whose participation in research is hindered by lack of knowledge, such as the newly injured
2. Increase knowledge of the research process within the SCI community through resources and training
3. Organize and educate researchers and healthcare providers who are interested in consumer engagement across the spectrum of SCI research and care
4. Facilitate placement of individuals living with SCI as advisors on research projects

Objectives

BUILDING OF AN ONLINE SCI RESEARCH ADVOCACY COURSE

MATCHMAKING AND RECRUITMENT

LIVE INSTRUCTOR-LED PROGRAM

Online Course Modules

Introduction to research advocacy	Understanding the research process	Addressing historical challenges for SCI research	SCI biology, the injury
SCI biology, secondary complications & aging	Neuroprotection	Cell replacement	Regeneration
Neuroplasticity	Quality of Life research	SCI 101 for Researchers	Getting involved & using your skills

Working Group Members

Chair: Barry Munro, NASCIC Treasurer

Name	Background
Barbara Archer	Living with SCI – Pennsylvania, USA
Dr. William Bailey	Research Analyst – University of Kentucky College of Medicine
Dr. Linda Bambrick	Program Director, Division of Neuroscience in Extramural Programs, National Institute of Neurological Disorders and Stroke (NINDS)
Jake Beckstrom	Manager - Cure Advocacy Network, Unite 2 Fight Paralysis Living with SCI
Kim Beer	Director, Public Policy – Christopher & Dana Reeve Foundation
Dr. Stephanie Dubow	Medical Director, Neuroscience Medical Affairs, AbbVie Inc.
Claudia Garofalo	Living with SCI – Louisiana, USA
Dr. John Gensel	Associate Professor, Spinal Cord and Brain Injury Research Center, University of Kentucky
Edward Graver	Living with SCI – Michigan, USA
Lora Hornung	Parent of SCI consumer – Kentucky, USA
Tara Jeji	Assistant Program Director, DP Clinical Inc. Living with SCI
Jerrod Kerr	Living with SCI – Florida, USA
Dr. John (Kip) Kramer	Assistant Professor, School of Kinesiology, University of British Columbia
Dr. Ena Miller	Neurologist - San Pedro Sula, Honduras
Angele Parente	Living with SCI – Ontario, Canada
Dr. Kimberly Pfleeger	Scientific Director, Neuroscience Development, AbbVie Inc.
Dr. Gail Rosseau	Clinical Professor of Neurosurgery, George Washington University School of Medicine and Health Sciences
Molly Schneider	Living with SCI – Ohio, USA
Susan Schaeffer	President & CEO, The Patients' Academy for Research Advocacy
Dr. Andrew Stewart	Post-Doctoral Fellow, University of Kentucky College of Medicine
Jeff Welden	Living with SCI – New York, USA
David Worley	Living with SCI – Texas, USA

Course Production

Community Engagement Working Group

NASCIC formed a working group to advise and review the SCI Research Advocacy Course. The working group is comprised of 23 individuals who represent different areas of the SCI community in North America, including:

- Consumers
- Caregivers
- SCI Researchers
- Physicians
- Industry representatives

Course Consultant

Susan Schaeffer, President & CEO - The Patient's Academy for Research Advocacy

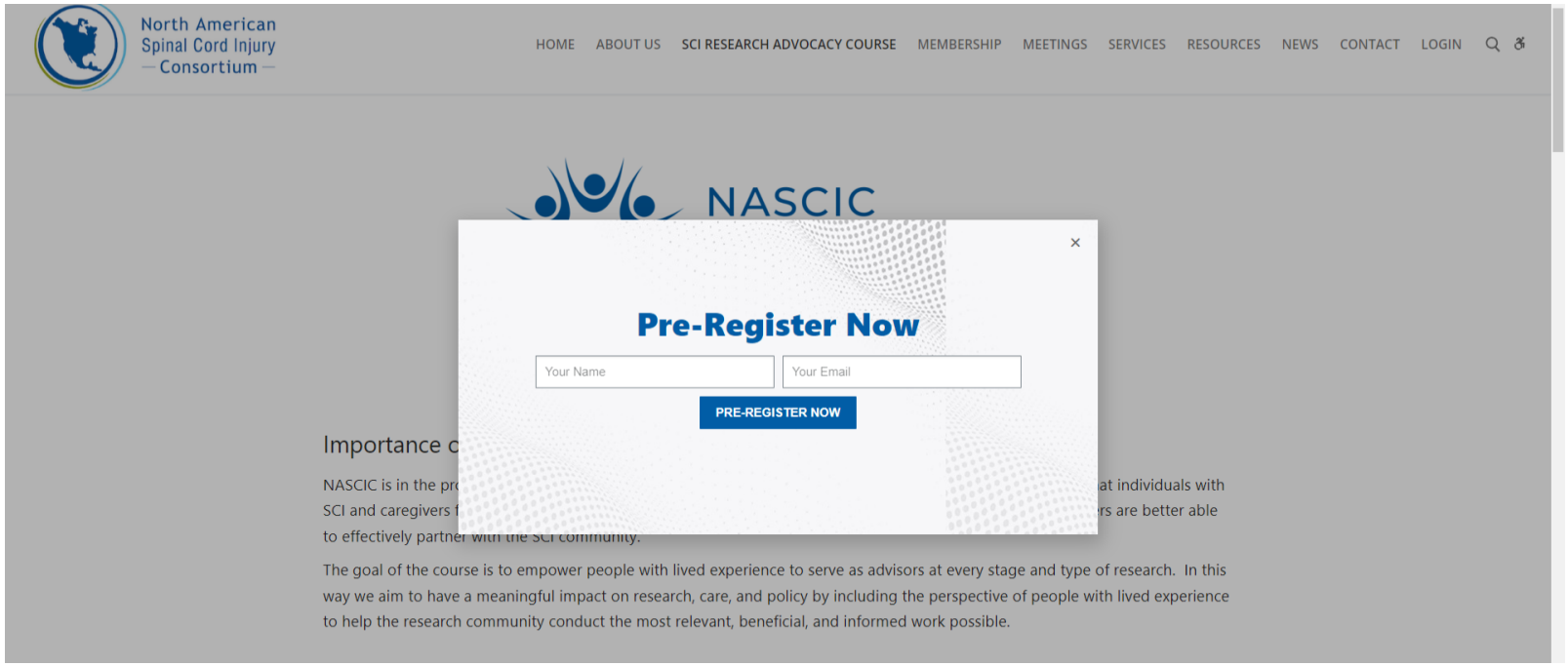
Susan has been contracted to work alongside NASCIC staff and the Community Engagement Working Group to develop the content of the SCI Research Advocacy Course.

PATIENTS' ACADEMY FOR RESEARCH ADVOCACY

Production Consultant

Incite Marketing Group has been contracted to assist with the production of the course module videos and a communications and marketing strategy once the course is available to the public.

IMG INCITE MARKETING GROUP



Pre-Register for the Course

https://nasciconsortium.org/nascic-sci-research-advocacy-course/

SCI COMMUNITY ENGAGMENT IN PFDD & PATIENT DEVICE PREFERENCES

North American
Spinal Cord Injury
— Consortium —

U.S. FOOD & DRUG ADMINISTRATION

CDER

Center for Drug Evaluation & Research

Office of Program & Strategic Analysis

https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/office-program-and-strategic-analysis-opsa

Est. 2012

CBER

Center for Biologics Evaluation & Research

Hybrid of CDER & CDRH on a programmatic level

CDRH

Center for Devices & Radiological Health

Office of Strategic Partnerships & Technology Innovation

https://www.fda.gov/about-fda/cdrh-offices/office-strategic-partnerships-and-technology-innovation

Est. 2013

FDA Guidance History

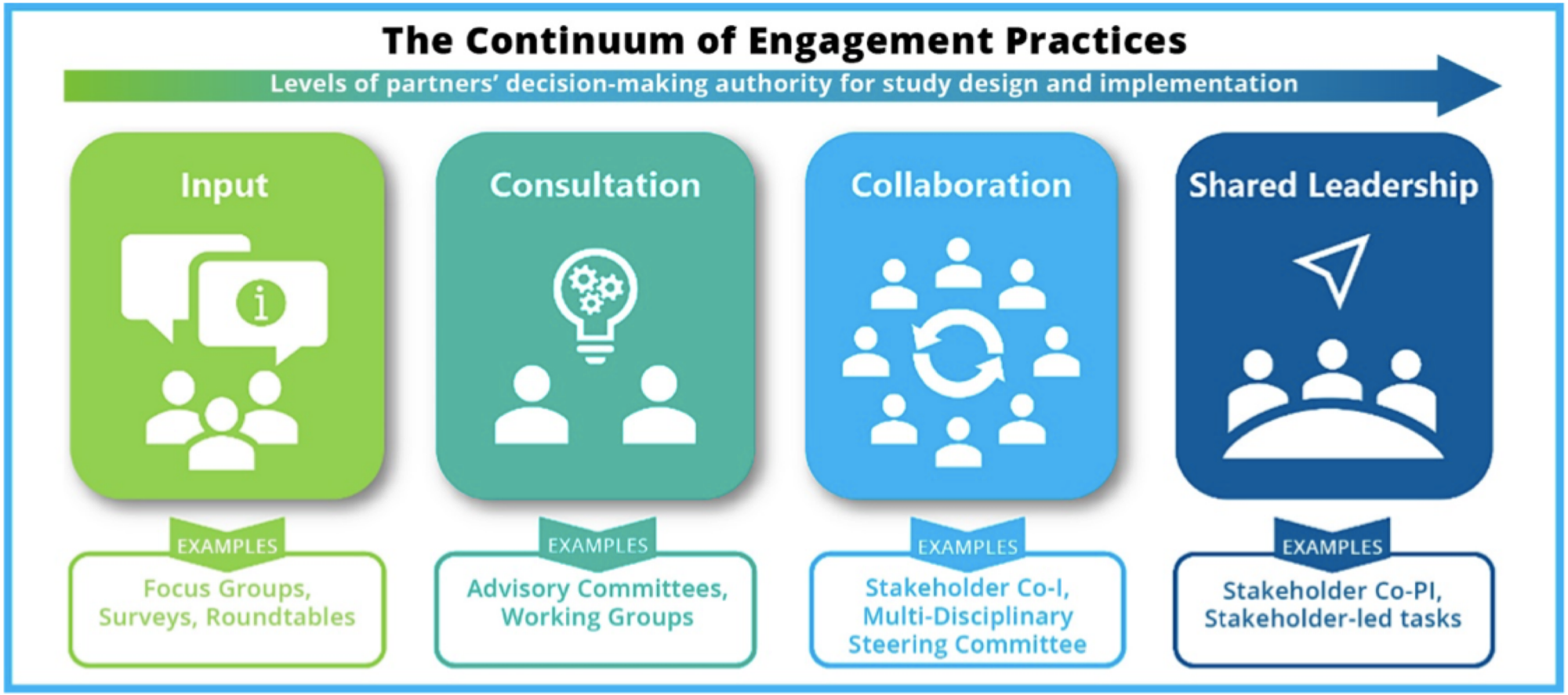
2022: Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-methods-identify-what-important-patients

Source: MDIC, Using Patient Preference Information in the Design of Clinical Trials Framework, April 2022

Engagement Continuum



Source: PCORI Budgeting for Engagement. Adapted from Hanley et al. (2004) Involving the public in NHS, public health and social care research. Carmen et al. (2013) Patient and family engagement: A framework for understanding the elements and developing interventions and policies. Health Affairs, 32(2), 223-231. Arnstein, S.R. (1969) A ladder of citizen participation. Journal of the American Planning Association, 35, 216-24

INTEGRATING COMMUNITY PREFERENCES

CORE set of factors in the consideration of community preference study development

S

Preference Sensitive Situations
multiple options that are decision maker dependent

P

Patients with Differing Perspectives Situations
experiences can be subjective or QoL is an important outcome measure

C

Benefit-Risk is Especially Challenging
assessments may be difficult to gather due to factors such as side effects, time lags, etc.

N

Regulatory Novelty
help to inform the many stakeholders including regulators, payers, or clinical areas.

Source: Medical Device Innovation Consortium Patient Centered Benefit-Risk Project Report: A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology. https://www.fda.gov/media/95591/download

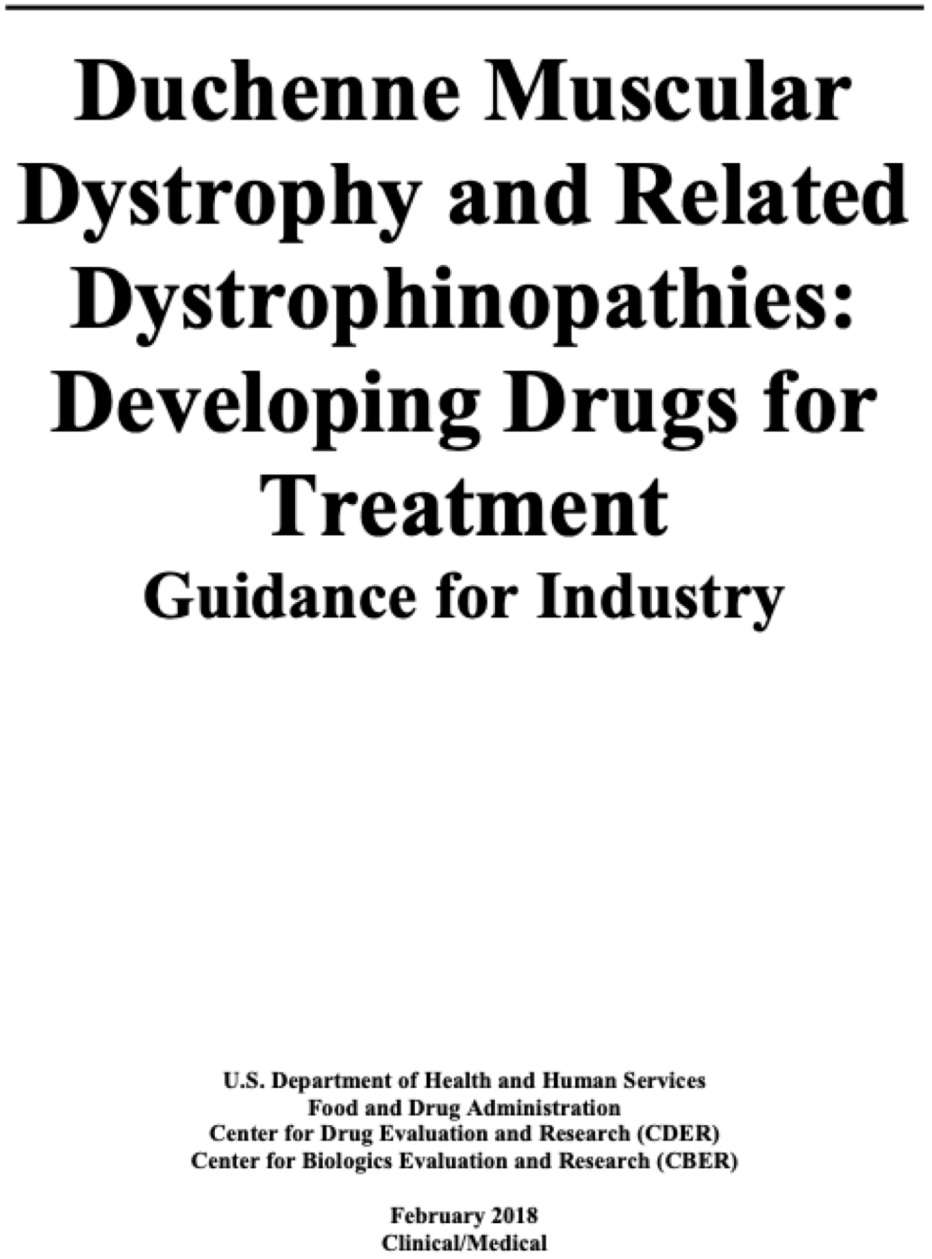
Framework Example

Parent Project Muscular Dystrophy

- Conducted 5 patient-focused benefit-risk studies
- Published 2 whitepapers on patient engagement
- Established a patient registry
- Created patient-reported outcomes that reflect the needs of the community
- Wrote draft guidance for Duchenne muscular dystrophy that the FDA later adopted.

https://www.parentprojectmd.org/fdas-patient-focused-drug-development-intiative-what-have-we-learned/

End Result



Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment

Guidance for Industry

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

February 2018
Clinical/Medical

Framework Example

Parent Project Muscular Dystrophy

PPMD Preference Studies 2013 - 2021

Quantifying the patient voice

2013

2021

5 studies completed, 1 ongoing, 1 early stage

1. Caregiver preferences for emerging treatments
2. Patient vs Caregiver preferences
   - Symptom priorities for treatments
   - Meaningful benefit in pulmonary outcomes
3. Research as an event – testing discreet choice (DCE)
4. Preferences for emerging Gene Therapies
5. Global study of treatment preferences and advocacy needs
6. Steroid patient experience study (ongoing)
7. Second preference study on Gene Therapies (DUK, est start May 2021)

Research Collaborators

- Dr. John Bridges (The Ohio State)
- Nonie Crossnohere (JHSPH)
- Dr. Holly Peay (RTI International)

Methods

- Best-Worst case 1 and 2
- Discreet Choice
- Threshold technique
- Likert scales
- Qualitative – surveys, interviews, focus groups

Funding and Partnership

- Pfizer
- Everylife Foundation
- Solid Biosciences
- Santhera Pharmaceuticals
- Mallinckrodt
- PTC Therapeutics

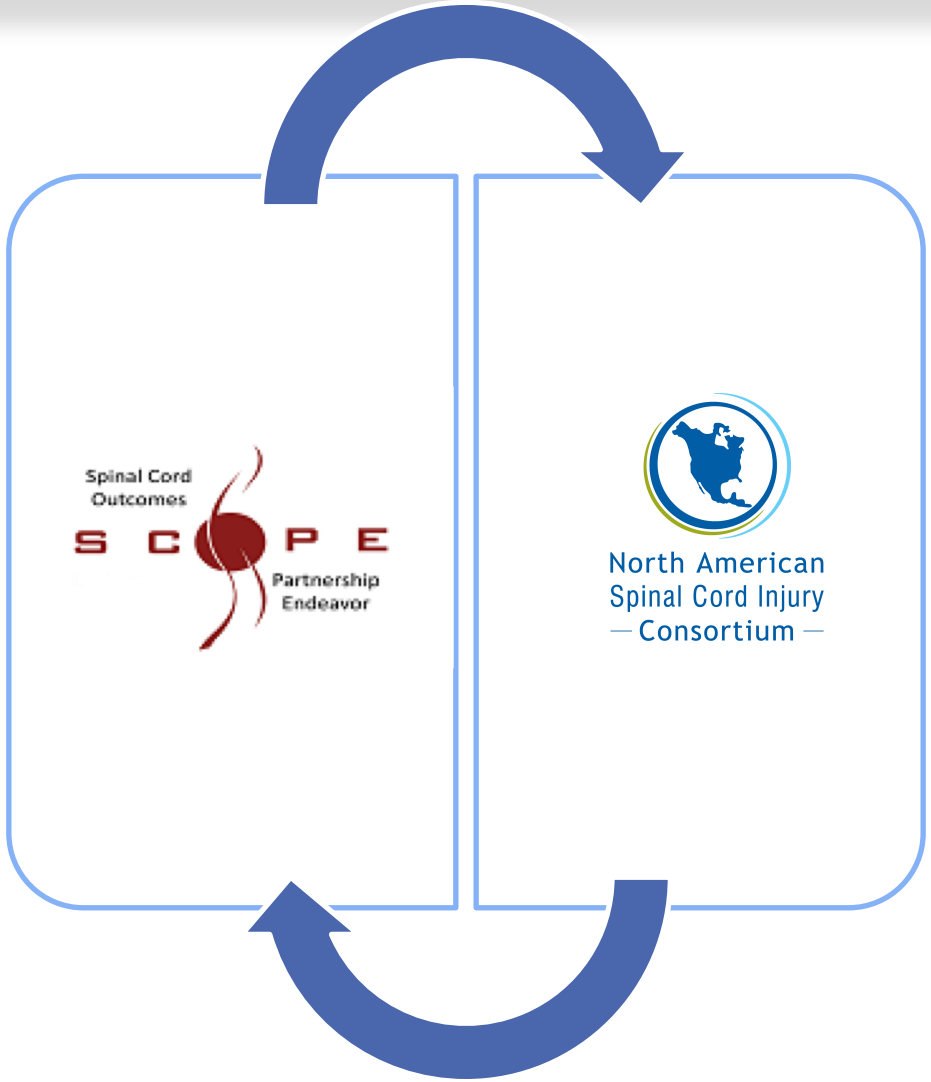
Reference: Hollin IL, Caroline Young, Hanson C, Bridges JFP, Peay H. Developing a Patient-Centered Benefit-Risk Survey: A Community-Engaged Process. Value Health. 2016 Sep-Oct;19(6):751-757. doi: 10.1016/j.jval.2016.02.014. PMID: 27712702.

Success Factors

Based on interview with Ryan Fischer, Chief Strategy Officer of PPMD and program lead

- Academic Collaborators who are patient preference experts/**social scientists** - this is key. It should not be disease specific researchers, i.e. MD or SCI researchers with an inherent bias.
- They approached preference studies first with hypotheticals with both lived experience and caregivers. They also studied worries/concerns specific to a treatment scenario or symptoms paired with experience. i.e. respiratory health/cough assist.
- Every study has a stakeholder advisory board: clinician, lived experience, caregiver, industry, researcher, project lead
- Mitigate response rate reporting assumptions. They approached this by sending a wide recruitment, and people respond to a link to receive the survey. This appeased publishers and the FDA. FDA also wanted to see opt out options when comparing treatment scenarios.
- Focus on methods with your collaborators.
- Assembled a **working group consisting of MD community representatives** (both LE & caregivers), research collaborators, funding and partners, consultant, plus a representative from FDA's Office of Strategic Programs, CDER and a member of Faster Cures leadership and policy team.

PFDD-PPI Project Collaboration



Thank You to our Supporters!

CRAIG·H NEILSEN FOUNDATION

Paralyzed Veterans of America

ONWARD
EMPOWERING MOVEMENT

PATIENTS' ACADEMY FOR RESEARCH ADVOCACY

abbvie

Spinal Cord & Brain Injury Research Center

Blackrock Neurotech

NIDILRR
National Institute on Disability, Independent Living, and Rehabilitation Research

KESSLER FOUNDATION

ANEUVO
pathways to new horizons

SYNAPSE
Biomedical Inc.

North American Spinal Cord Injury Consortium

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Happy Holidays

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https://www.instagram.com/nasciconsortium

https://www.linkedin.com/company/north-american-spinal-cord-injury-consortium