Goal and Objective was to raise awareness and gain support (endorsement and financial) for the execution of a PFDD meeting

Agenda

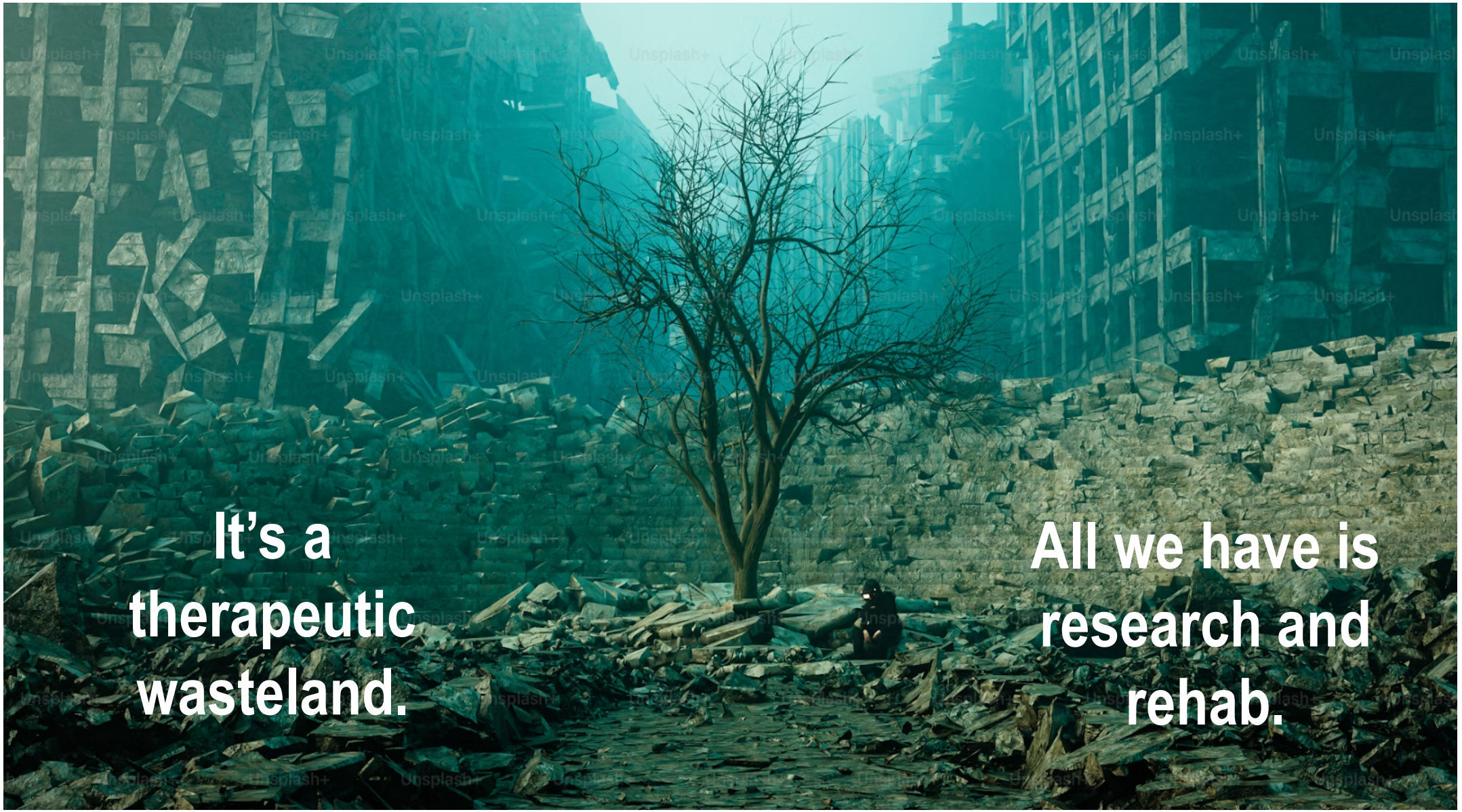
- The Opportunity and Scene Setting- Brian Culley
- Patient Focused Drug Development, An Overview- Jonathan Stokes
- Challenges and Opportunities in SCI Research: A Physicians Perspective- Steve Kirshblum
- A Roadmap to Addressing Unmet Needs in SCI Care Through PFDD- Linda Jones and Barry Munro
- Where does this leave us: A perspective from the Christopher and Dana Reeve Foundation- Marco Baptista
- What Next- Brian Culley

Key Takeaways

- Well received, fully endorsed, web-based feedback ongoing, financials not discussed at meeting
- Require ~$500K to properly develop, deliver and execute a PFDD meeting (project management, meeting delivery, PLEX survey, marketing)
- Need to maintain the momentum with the goal of raising the funds by end of 2024
- Need to define the threshold that triggers a ‘Go’ decision for a PFDD meeting
- Assessing small investment now that can help drive funding to support PFDD meeting- speculate to accumulate

Immediate Next Steps

- Small investment to support and drive funding of PFDD, speculate to accumulate mentality
- Communications ‘pack’: pitch deck, one pager, emotive call to action video(s), donation website- generate FoMo mindset
- Proactive and targeted outreach to potential contributors
- Proposal received today


It’s a therapeutic wasteland.
All we have is research and rehab.

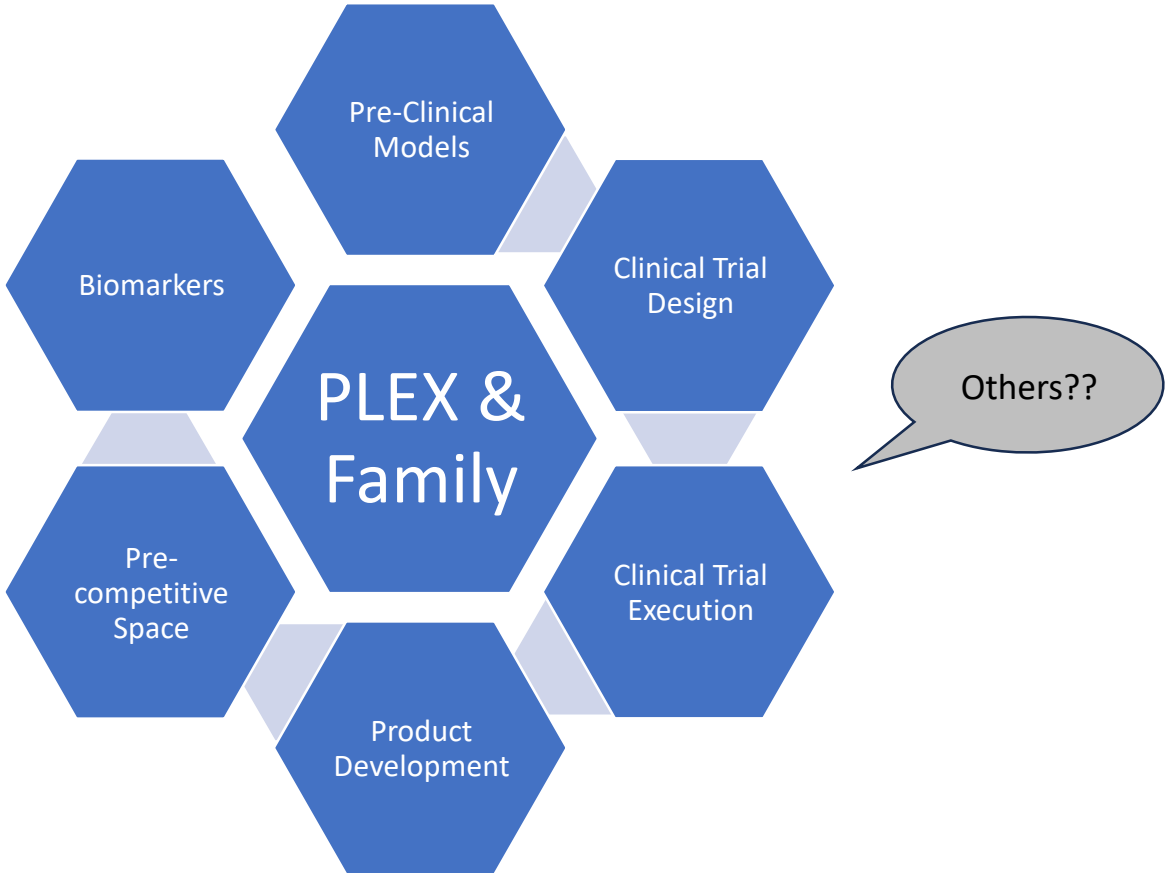
Opportunity: Tapping into the SCI Investor Forum

DISCUSSION

Harness the Value of Attendees

ACTION

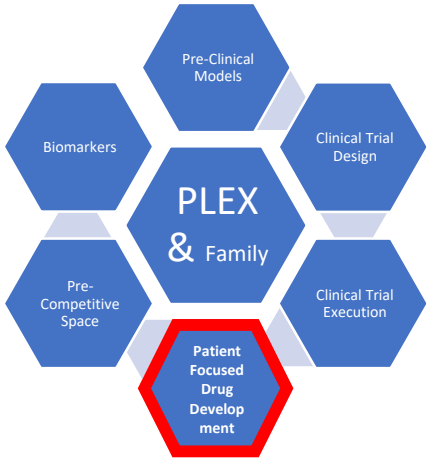
Actively address opportunities



SCIIS as Convener & Incubator…..

…Attendees as Catalysts and Drivers of Solutions

PFDD as a (big) Pilot/Test Case



Patient Focused Drug-Development

PFDD Objectives

Seek and gain regulatory and stakeholder alignment on scales, measures and endpoints (focus on primary) to clinically assess treatments aimed at providing meaningful and relevant improvements for persons living with SCI

What to Measure	How to Measure	Endorsement	Development	Continued Investment	Make a Difference
Persons with Lived Experience	Clinical & Research	Regulators & Payers	Industry & Academia	SCI Community	Persons with Lived Experience

Session 2

"Patient-Focused Drug Development: An Overview"

Jonathan Stokes, MBA

Senior Director, Patient-Centered Outcomes Research Value & Evidence

PCOR

Be Purposeful. Elevate Patients. Drive Impact.

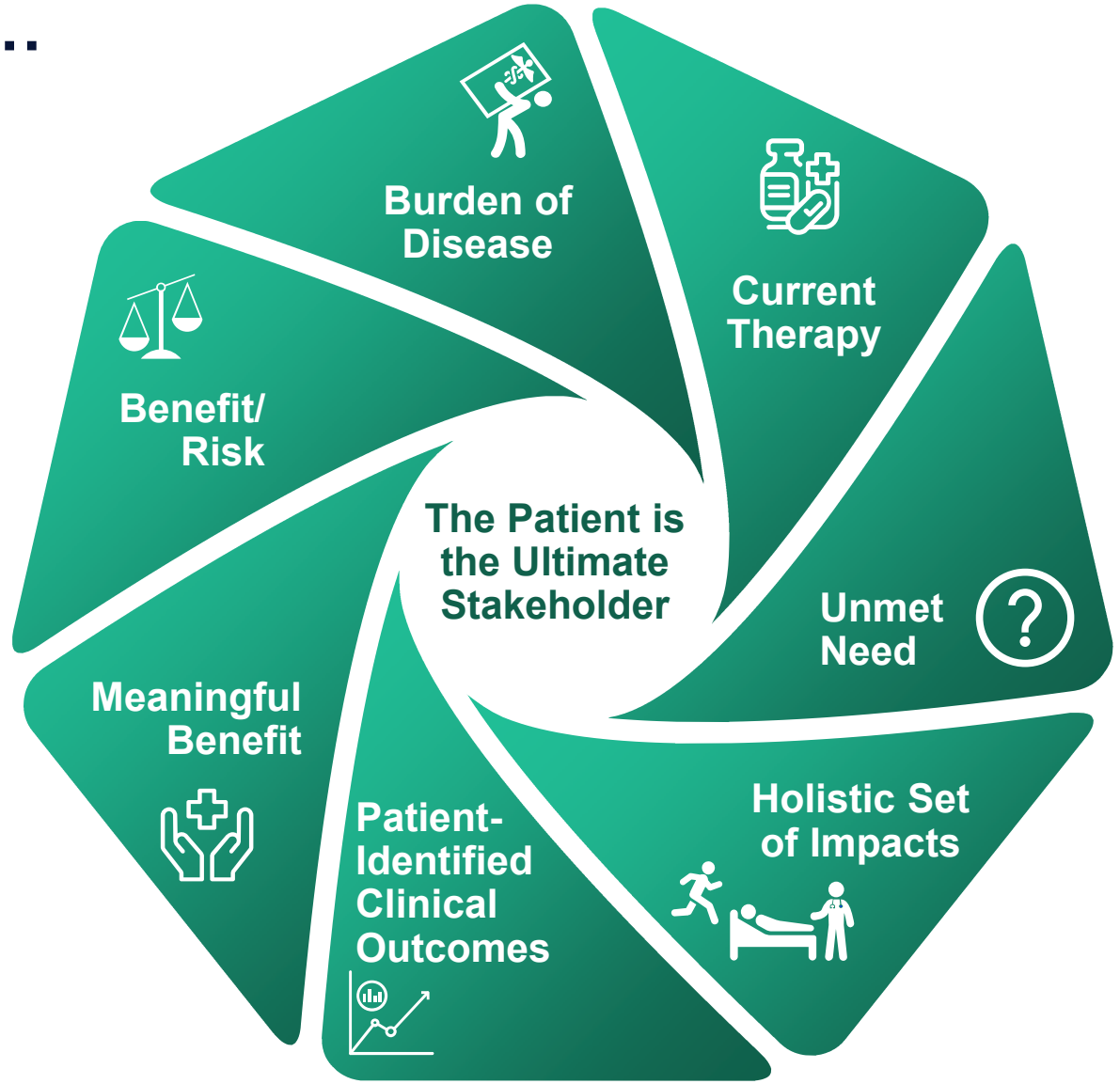
Patient-focused drug development (PFDD) is a systematic approach to help ensure that **patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated** into drug development and evaluation.

Source: https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Value of Capturing the Patient Voice…

Inclusion of the patient voice in clinical trials of new therapies and generation of patient experience data (PED) is critical:

- As part of the Patient Focused Drug Development (PFDD) initiative, regulators are frequently requiring evidence of treatment efficacy from the patient's perspective
- Patients, providers, and payers are increasingly seeking patient-relevant endpoints and evidence when making medical care and reimbursement decisions
- By generating such evidence and characterizing the patient voice within regulatory (e.g., NDA/BLA) submissions, we can demonstrate the true benefit of products to our ultimate stakeholders



Source: Evidera, The Evidence Forum, A Perspective on the 21st Century Cures Act: Patient-Focused Drug Development, Nov 2017

BLA, Biologics License Application; NDA, New Drug Application

Patient Experience Data (PED)*

Includes data that are collected by any persons and are intended to provide information about patients' experiences with a disease or condition.

Can be interpreted as information that captures patients' experiences, perspectives, needs, and priorities related to (but not limited to):

1. The symptoms of their condition and its natural history;
2. The impact of the condition on their functioning and quality of life;
3. Their experience with treatments;
4. Input on which outcomes are important to them;
5. Patient preferences for outcomes and treatments; and
6. The relative importance of any issue as defined by patients.

* Defined in Title III, section 3001 of the 21st Century Cures Act, as amended by section 605 of the FDA Reauthorization Act of 2017 (FDARA)1

FDA's PFDD Guidance Series Provides Best Practices & Guiding Principles for Development of PED

Guidance 1

Identifying research questions and developing a sampling strategy to collect representative patient input; operationalizing data collection, management and analysis

Who is the Target Patient Population?

Final Guidance: June 2020

Guidance 2

Methods to elicit detailed, unbiased, and comprehensive input from patients, patient groups, and caregivers

What Concepts Matter Most?

Final Guidance: February 2022

Guidance 3

Using patient input to develop or identify appropriate clinical outcome assessments (COAs) for use in clinical trials

What is the Appropriate Assessment?

Draft Guidance: June 2022

Guidance 4

Developing COA-related clinical trial endpoints based upon patient input; interpreting those endpoints

What is the Appropriate Endpoint Definition?
Is the Observed Treatment Effect Meaningful?

Draft Guidance: April 2023

https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical

Clinical Outcome Assessments (COAs) Can Be Used to Generate PED

- A COA is any assessment that may be influenced by human choices, judgment, or motivation and may support either direct or indirect evidence of treatment benefit
- Unlike biomarkers that rely completely on an automated process or algorithm, COAs depend on the implementation, interpretation, and reporting from a patient, a clinician, or an observer

The 4 Types of COAs

PRO

Report that comes directly from the patient based on the patient's perception of a disease and its treatment

ClinRO

Report that comes from a trained healthcare professional using clinical judgement

ObsRO

Report that comes from someone other than the patient or health professional (e.g., parent or caregiver) who can observe the patient in everyday life

PerfO

Measurement based on standardized task(s) actively undertaken by a patient according to a set of instructions

Sensor-based functional outcomes emerging area of opportunity for holistic understanding of patient perspective

https://www.fda.gov/drugs/development-approval-process-drugs/drug-development-tool-ddt-qualification-programs
ClinRO, Clinician-reported Outcome; ObsRO, Observer-reported Outcome; PerfO, Performance Outcome; PRO, Patient-reported Outcome

QULIPTA: Assessing Treatment Benefit on Functional Improvement in Episodic & Chronic Migraine

Concept
("Thing" measured)

Social and Work-related Activities

Performance of Daily Activities

Physical Impairment

Patient Informed

Instrument
(Tool to measure concepts)

Migraine Specific Quality of Life Questionnaire (MSQ v2.1)

Activity Impairment in Migraine – Diary (AIM-D)

Patient Informed

Endpoint
(Precisely defined variable based on instrument)

Key Secondary Efficacy Endpoint:

- Change from baseline in MSQ v2.1 Role Function-Restrictive domain score at Week 12
- Change from baseline in mean monthly Performance of Daily Activities domain score of the AIM-D across the 12-week treatment period
- Change from baseline in mean monthly Physical Impairment domain score of the AIM-D across the 12-week treatment period

Communication
(Documentation of treatment benefit)

Qulipta® Label (2021)

in mean MMD (3-month average), the change from baseline in mean monthly Activity Impairment in Migraine-Diary (AIM-D) Performance of Daily Activities (PDA) domain scores, the change from baseline in mean monthly AIM-D Physical Impairment (PI) domain scores, across the 12-week treatment period, and the change from baseline at Week 12 for Migraine Specific Quality of Life Questionnaire version 2.1 (MSQ v2.1) Role Function-Restrictive (RFR) domain scores.

The AIM-D evaluates difficulty with performance of daily activities (PDA domain) and physical impairment (PI domain) due to migraine, with scores ranging from 0 to 100. Higher scores indicate greater impact of migraine, and reductions from baseline indicate improvement. The MSQ v2.1 Role Function-Restrictive (RFR) domain score assesses how often migraine impacts function related to daily social and work-related activities over the past 4 weeks, with scores ranging from 0 to 100. Higher scores indicate lesser impact of migraine on daily activities, and increases from baseline indicate improvement.

Data to Inform Decision-making

Concept elicitation → Item generation → Cognitive debriefing → Psychometric validation

PFDD and PED: Key US Legislation

Rationale: Obtain patients insights in a more systematic way to inform understanding of therapeutic context for their benefit-risk assessments

PDUFA V: 2012	• Established FDA's PFDD initiative – PFDD meetings to inform benefit-risk assessments; "Voice of the Patient" reports • Enhanced Patient-Centered Outcomes Assessment staff
The 21st Century Cures Act 2016	• FDA required to issue a statement on patient experience data (PED) submitted with NDA or BLA • FDA will develop a guidance series on the collection, use and submission of patient experience data
PDUFA VI: August 2017	Further enhances PFDD through: • Enhanced staff capacity • FDA-led public workshops for recommendations on methods and approaches
PDUFA VII 2023-2027	• Internal FDA training on PED methods; external outreach for trainings/information • Seek public input on methods for PED in B-R decisions and labeling • Standard Core COAs and Endpoints • Draft guidance on use and submission of patient preference information

PFDD Meetings

- In 2012, FDA established PFDD meetings designed to elicit patients' perspectives on:
  (1) the most significant symptoms of their condition and the impact of the condition on daily life; and,
  (2) their current approaches to treatment.
- Meetings can be granted as FDA-led or externally-led
- Stakeholders include:
  - Patients and caregivers
  - FDA
  - Patient advocates
  - Researchers
  - Medical product/drug developers
  - Healthcare providers
- Output: Voice of the Patient report. Additional publicly available materials include transcripts, webcast recordings, presentation slides

Example indications

- Alopecia Areata
- Alpha-1 Antitrypsin
- Autism
- Breast Cancer
- Chagas Disease
- Chronic Fatigue Syndrome/Myalgic Encephalomyelitis
- Chronic Pain
- Female Sexual Dysfunction
- Fibromyalgia
- Functional Gastrointestinal Disorders
- Hemophilia A, B, and Other Heritable Bleeding Disorders
- Hereditary Angioedema
- Human Immunodeficiency Virus (HIV)
- Huntington's disease
- Idiopathic Pulmonary Fibrosis
- Inborn Errors of Metabolism
- Lung Cancer
- Narcolepsy
- Neuropathic Pain Associated with Peripheral Neuropathy
- Non-tuberculous Mycobacterial Lung Infections
- Opioid Use Disorder
- Patients Who Have Received an Organ Transplant
- Parkinson's Disease
- Psoriasis
- Pulmonary Arterial Hypertension
- Sarcopenia
- Sickle Cell Disease
- Stimulant Use Disorder
- Systemic Sclerosis
- Vitiligo

Note: An increasing number meetings are externally-led using FDA meetings as a model

https://www.fda.gov/industry/prescription-drug-user-fee-amendments/fda-led-patient-focused-drug-development-pfdd-public-meetings

Externally-Led PFDD Meetings

FDA acknowledged that there are more disease areas than can be addressed within the FDA-led PFDD meeting model. As of 2015, **FDA has encouraged patient organizations to identify and organize externally-led PFDD meetings**.

FDA suggests PFDD meetings that target disease areas with the following characteristics

- An identified need for patient input.
- A disease area that is chronic, symptomatic, or affects functioning and activities of daily living.
- A disease area for which aspects of the disease are not formally captured in clinical trials.
- A disease area for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives.
- Disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly).

FDA suggests a townhall-style meeting with two panels

1. The first focuses on **the symptoms and daily impacts of the condition**
2. The second focuses on the **current treatment approaches and what participants would look for in an ideal treatment**. May include considerations for participating in clinical trials and benefit-risk tradeoffs patients may perceive as acceptable.

https://www.fda.gov/industry/prescription-drug-user-fee-amendments/externally-led-patient-focused-drug-development-meetings
https://www.fda.gov/media/160223/download?attachment

Process for Externally-led PFDD Meetings

- Identify and organize appropriate stakeholders to lead the initiative
- Develop a letter of intent (LOI) and submit to FDA. FDA suggests no more than three pages, including the importance of the meeting in the context of the disease area, proposed agenda, and other meeting considerations
- LOI should be submitted approximately 1 year before the anticipated meeting date
- FDA has noted that they “generally do… not encourage the use of event planners, consultants, scientific writers, or other external resources” though operationally, this is difficult without sufficient support
- Suggest building into planned patient organization meetings, for efficiency
- FDA reviews, may provide input/feedback and grants/does not grant meeting
- FDA works with organizers leading up to plan meeting and subsequently attends
- Meetings sponsors conduct meeting and draft subsequent Voice of the Patient document, for FDA review and publishing

https://www.fda.gov/media/152029/download

Benefits of PFDD Meetings

FDA hears you:
Provides regulators with a deeper understanding of a disease/condition. Informs benefit/risk assessments for reviewing new products/treatments and understanding of how individuals are managing with existing treatments and what would constitute beneficial outcomes

Cross-stakeholder learnings:
Informs areas of unmet need in the patient populations, as well as the need to develop assessments to evaluate treatment outcomes for potential therapies. Generates momentum and stimulates discovery.

Connectivity:
Brings together stakeholders across the drug development ecosystem to form relationships and promotes further engagement

Pull-through:

Voice of the Patient report is invaluable documentation of the concepts that characterize a disease/condition

- Publicly available evidentiary basis for anyone to use
- For drug developers, serves to substantiate the evaluation of the concepts that are important and relevant in clinical trials
- Codified/ endorsed by FDA

Examples of Key Learning Moments

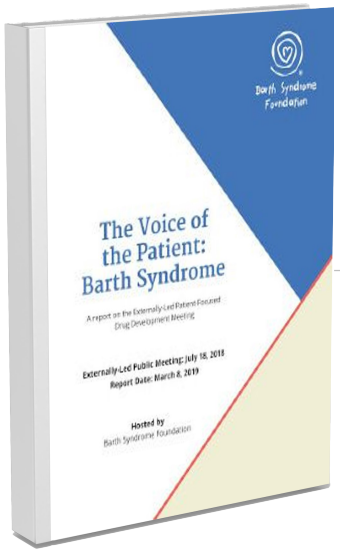
Barth Syndrome Foundation hosted an externally-led PFDD meeting in 2018

Fatigue was identified as one of the most debilitating and bothersome symptoms to affected individuals

Subsequently, fatigue was identified as a key measurement target and included in a symptom-based COA measure (BTHS-SA) in pivotal Ph3 clinical trials

Topic 1 - Effects of Barth Syndrome

1) Which of the following symptoms has the most significant impact on you (the person for whom you are responding)?	# of responses (from 91 respondents to this question)	% of respondents who chose this as one of their responses
Heart failure	34	37%
Arrhythmia	12	13%
Neutropenia, infections	49	54%
Fatigue	**76**	**84%**
Muscle weakness/exercise intolerance	78	86
Eating problems/digestive problems/ nutritional issues/nausea	53	58%
Pain (headaches, stomach aches, etc.)	28	31%
Sleeping difficulties	14	15%
Speech problems	10	11%
Mood disorder/depression/anxiety	14	15%
Learning disability/attention problems/other cognitive issues	22	24%
Short stature	26	29%
Healing	10	11%
Other	4	4%
Total	**430**	



B. Extreme fatigue

> ***The only word in the English language that comes even close to describing how I feel is 'depleted.' I get so utterly exhausted that I have to really concentrate just to lift my arm.***

At least four out of five polling participants identified the *"chronic fatigue"* associated with BTHS as being one of their most significant symptoms-and it was extensively discussed. Many participants described this *"all-encompassing fatigue"* as affecting virtually every aspect of their lives: *"On a bad day, I have no energy to chew meat,"* said one young man. More than one parent even observed this fatigue in their

https://www.barthsyndrome.org/advocacy/pfdd/voiceofthepatient.html

Gwaltney C, Stokes J, Aiudi A, Mazar I, Ollis S, Love E, Shields AL (2021). Development and content validity of the Barth Syndrome Symptom Assessment (BTHS SA) for adolescents and adults. *Orphanet Journal of Rare Disease*, Issue 16, Article 264

Examples of Key Learning Moments

Cystic fibrosis has classically been characterized as by its pulmonary presentation

A PFDD meeting was held in 2018 and Voice of the Patient report subsequently published

Gastrointestinal issues emerged as a primary concerns/issues for patients/caregivers, a concept under-researched in clinical trials

Which CF-related symptoms do you/your loved one cope with on a regular basis?

Disease Symptoms In Order of Response	Percent	Rank
Gastrointestinal Issues	**74%**	**1**
Pulmonary Exacerbations//Infections	72%	2
Excessive Cough	65%	3
Sinus Disease	59%	4
Mental Health Issues	58%	5
Fatigue	58%	5
Shortness of Breath	57%	6
Tight Chest	38%	7
CF-Related Diabetes	33%	8
Chronic Pain	32%	9
Liver Disease	7%	10
103 Responses		

- Respiratory complications were the primary challenge and source of concern for meeting participants, including lung infections, lung bleeds and lung collapse, yet it was consistently stressed that mental health issues and gastrointestinal complications also significantly impact CF patients' quality of life. Other common symptoms include CF-related diabetes, sinus polyps, liver disease, osteoporosis, and reproductive health challenges.
- The CF community has a strong desire for new therapies that reduced lung infections which lead to permanent lung function loss, pneumothorax, hemoptysis, hospitalizations, IV antibiotics, and lost work/ school time. Individuals seek therapies that improve their ability to breathe. New drugs to address gastrointestinal complications must be developed. In light of the current treatment burden, new therapies that do not require additional time would be highly valued.

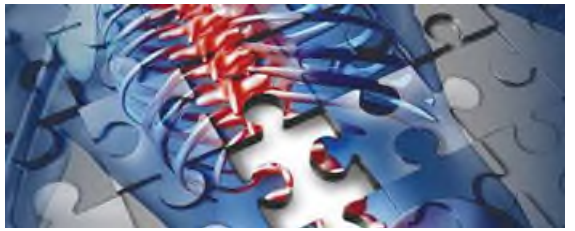
https://cfri.org/wp-content/uploads/2019/03/EL-PFDD.CFRI_.VoiceofCFPatientReport.pdf

Integrating the Patient Voice through PFDD Meetings: Key Takeaways

There is a growing emphasis on patient-centricity in healthcare and PFDD ensures patients are a critical stakeholders in the process

PFDD meetings are an effective mechanism to communicate a disease/condition experience to FDA to help them inform benefit/risk for evaluation of treatments

Voice of the Patient reports serve as an invaluable, publicly available evidentiary source documenting the outcomes that matter to patients

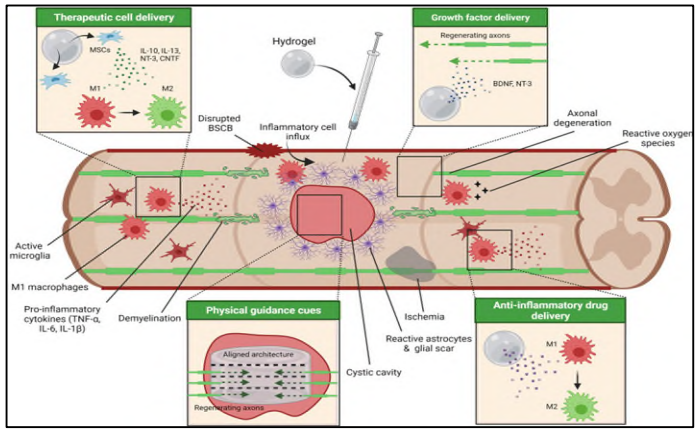


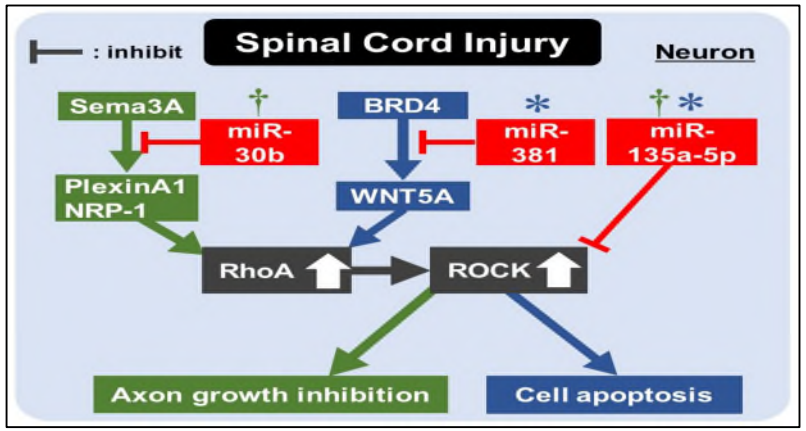
“Challenges and Opportunities in SCI Research: A Physicians Perspective…..”

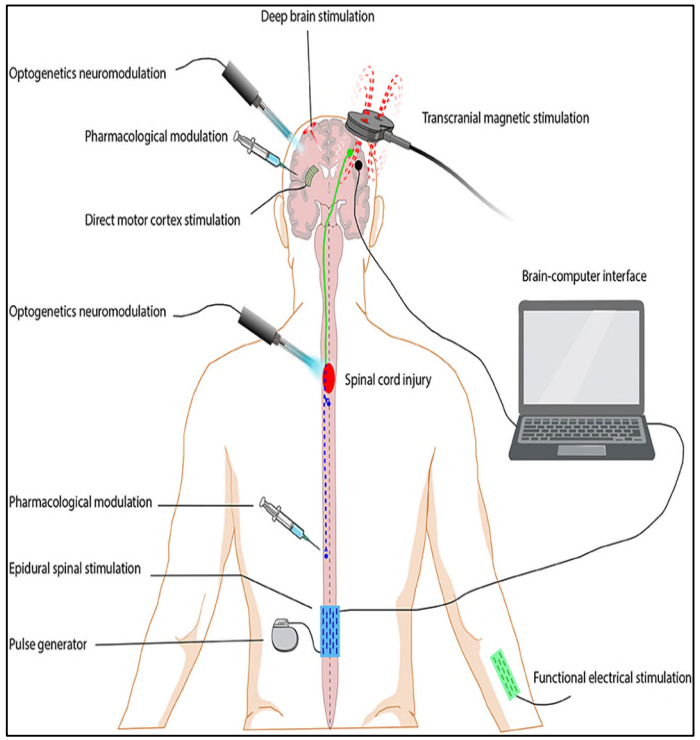
Steven Kirshblum MD

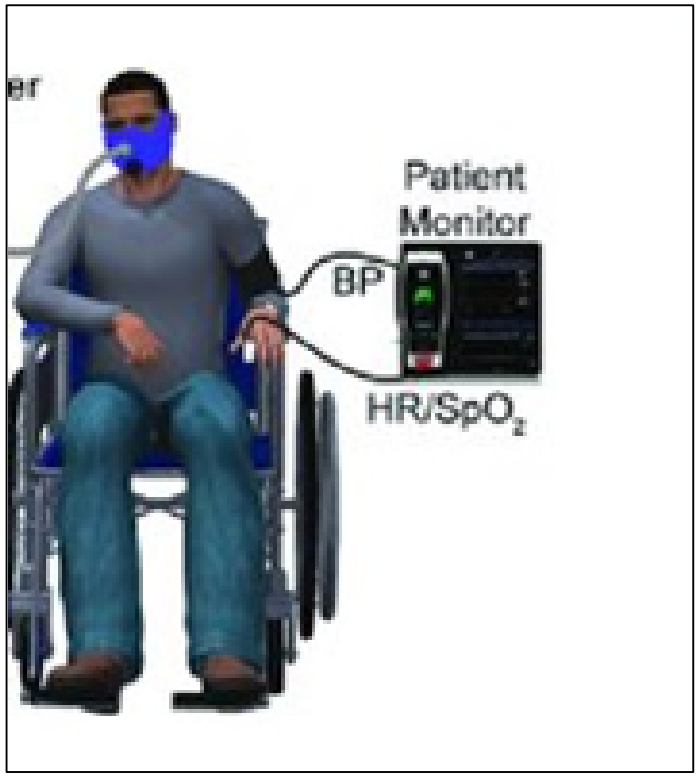
- VIDEO (REMOVED)

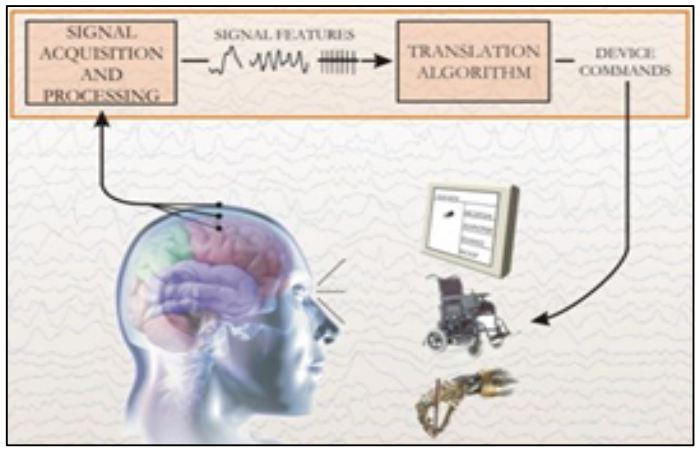
High Level of Innovation in SCI Research……









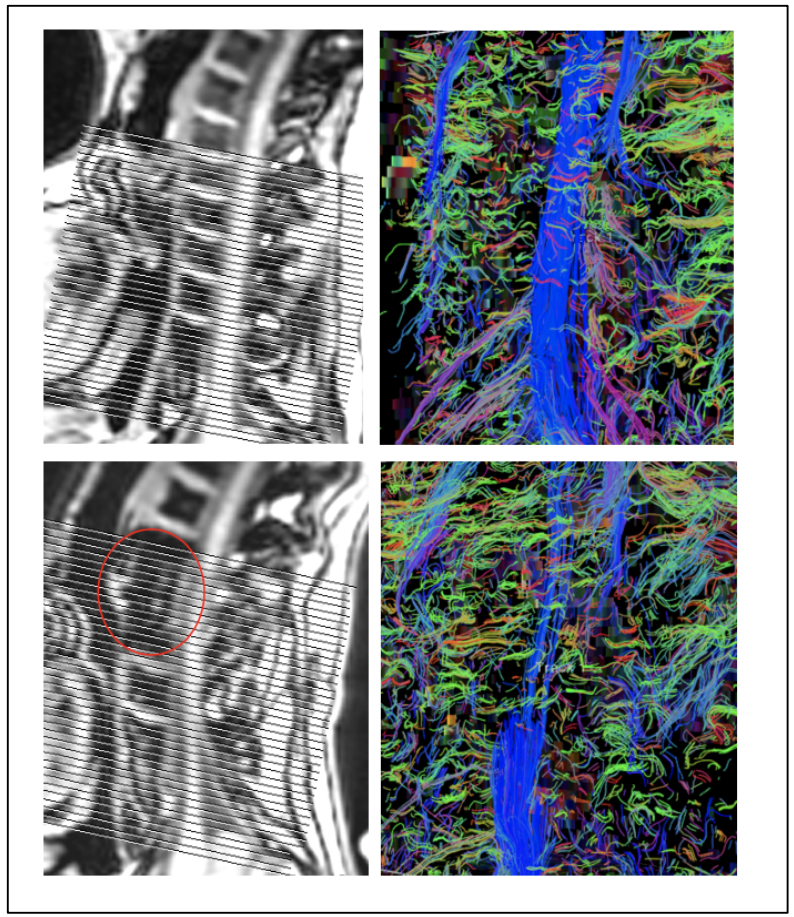


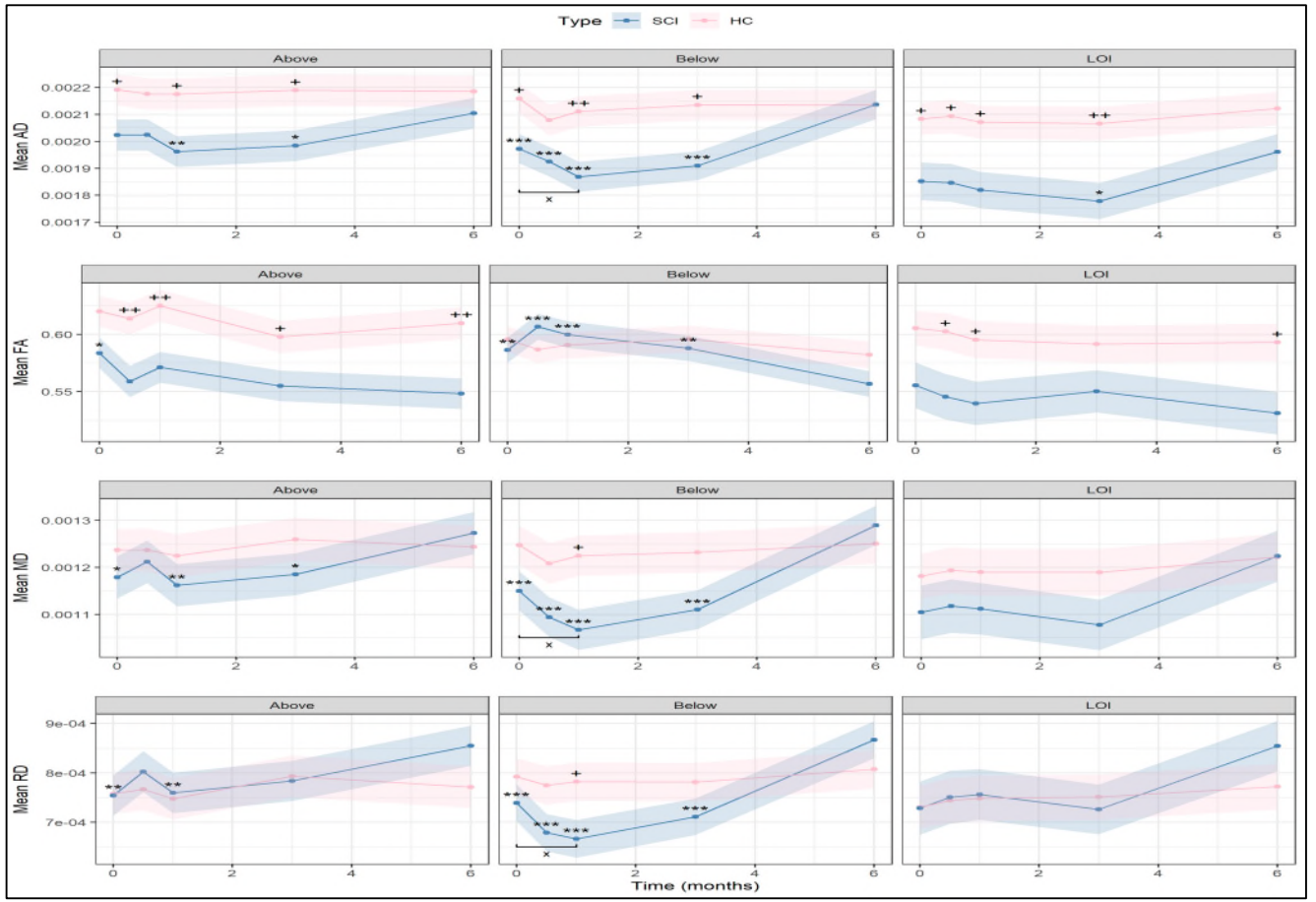
…and we continue to learn how to do things better

A Diffusion Tensor Imaging Study on Assessing the Recovery of Spinal Cord After Injury

Bing Yao*[1,4], Hannah Ovadia[1], Gail Forrest[2], Steven Kirshblum[3,4]

[1]Rocco Ortenzio Neuroimaging Center, [2]Center for Spinal Stimulation Research, Kessler Foundation, West Orange, NJ, United States
[3]Kessler Institute for Rehabilitation, West Orange, NJ, United States
[4]Department of Physical Medicine & Rehabilitation, Rutgers University, Newark, NJ, United States





Recent Trial Experience

Significant opportunity exists to involve PLEX (earlier), to help mitigate challenges and explore opportunities……

Physician & Researchers

- Eligibility
- Safety

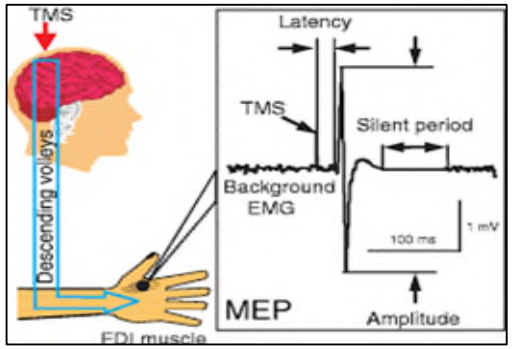
PLEX

Main Challenges:

1. **Enhance relevance of research**
2. **Improve study design**
3. **Develop patient-centered outcomes**
4. **Enhance patient buy-in and engagement**

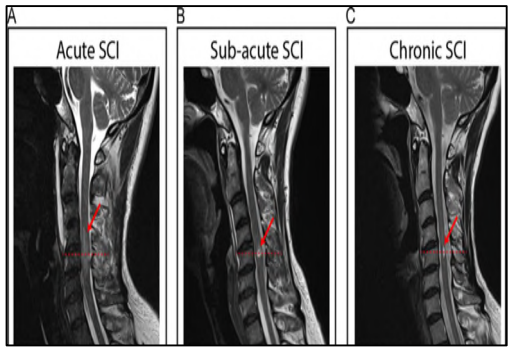
Important to recognize that physician and researchers perspectives may be very different to PLEX

Neurophysiology

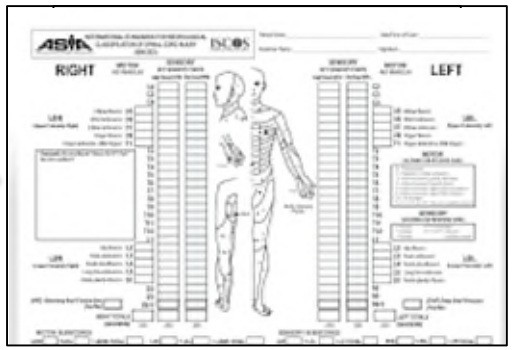


Spasticity Scores

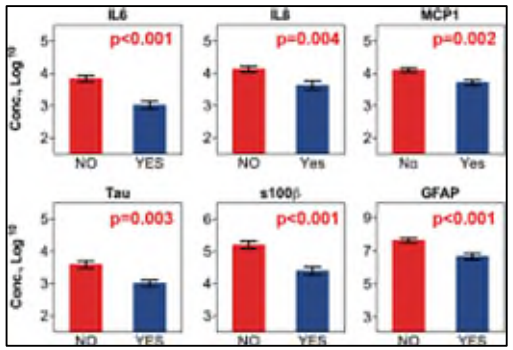
Imaging



Outcome Measures



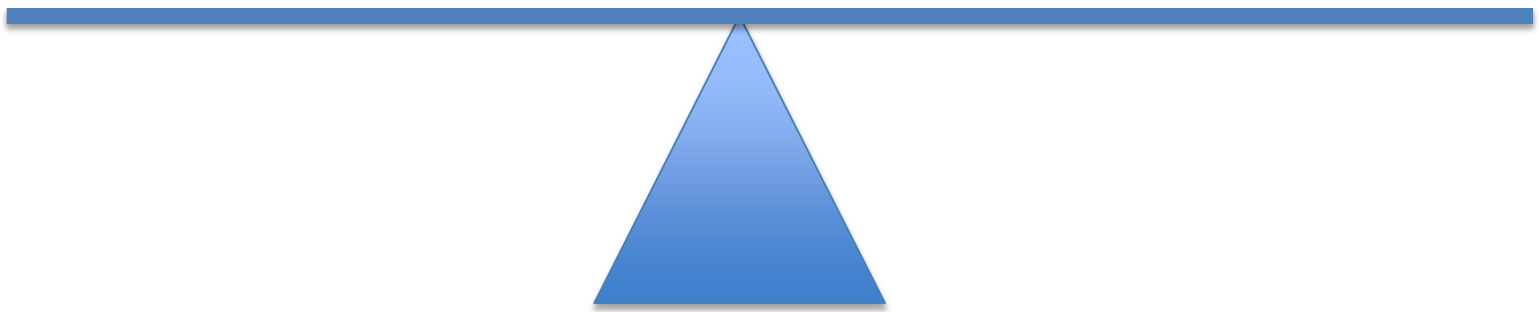
Biomarkers



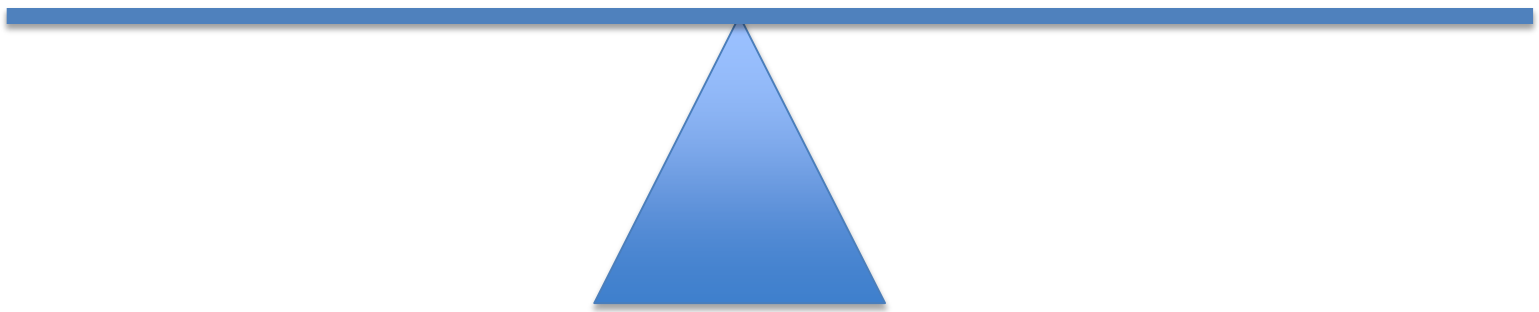
Statistical Significance

Statistical Significance and
p-value < 0.05
What does it mean?

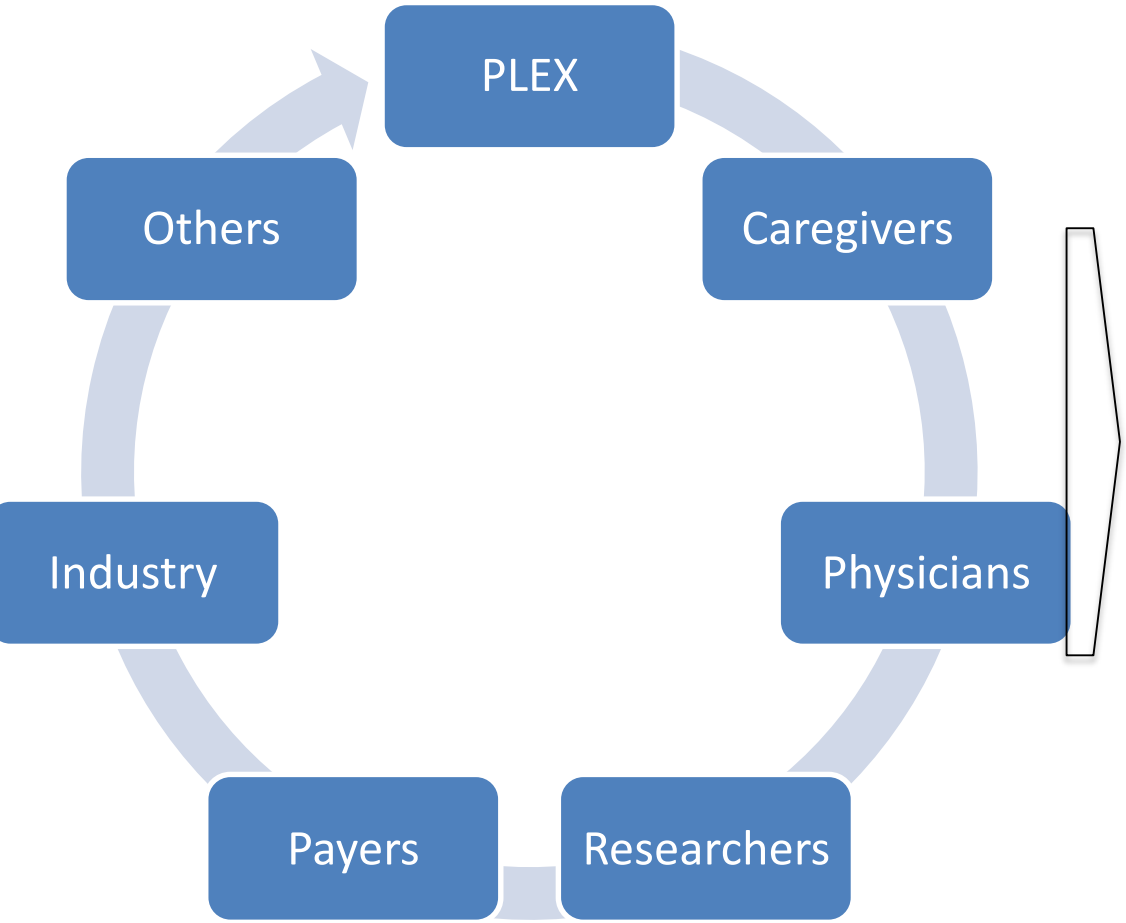
Model of Research



Model of Research

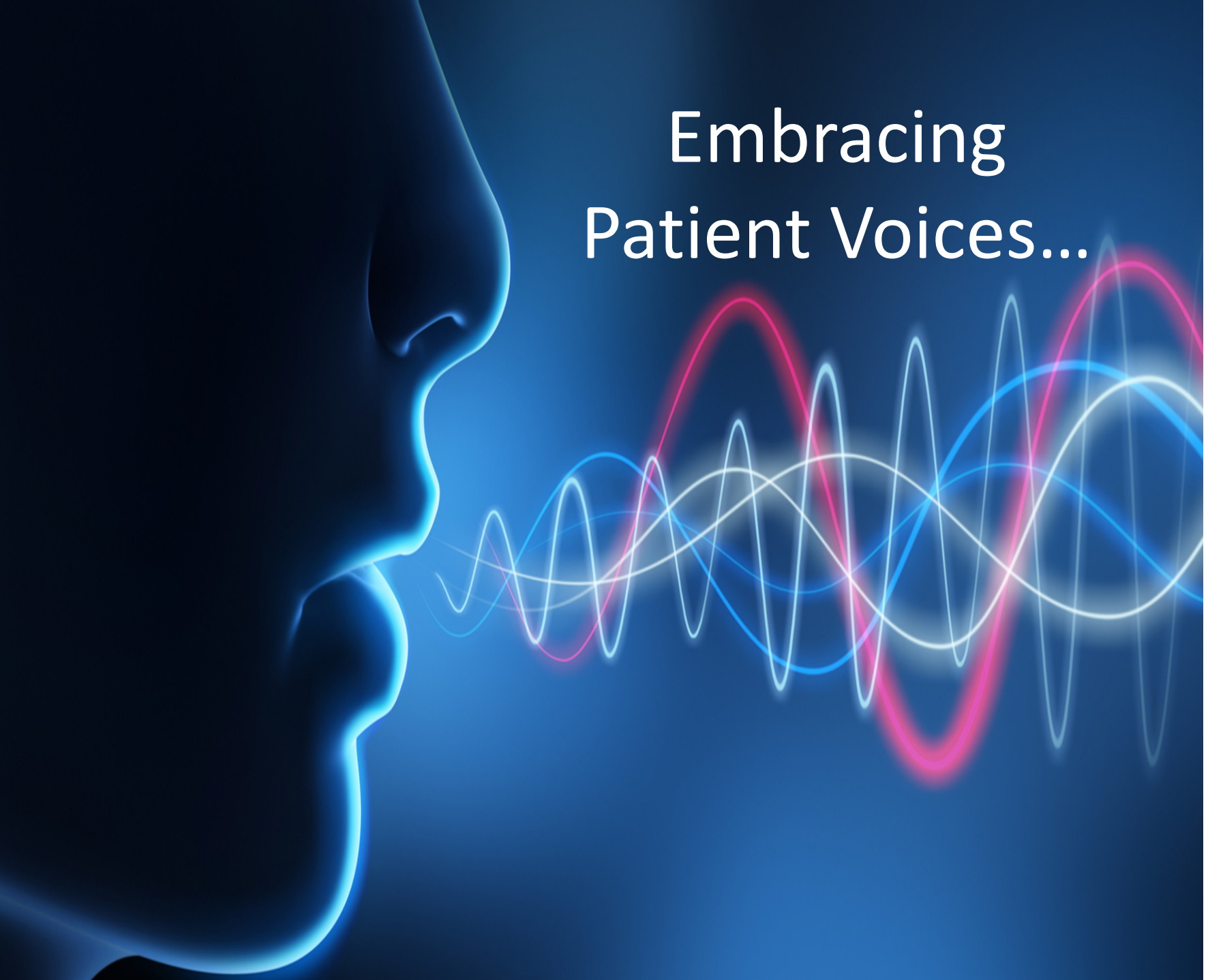


To solve challenges and realize the opportunities in SCI, stakeholder must come together and collaborate



1. **Enhance relevance of research**
   - addressing real-world priority issues
   - better communication of these objectives
2. **Improve study design**
   - more feasible and acceptable (risks vs benefits)
   - identify potential barriers to participation/adherence
   - accommodate the diverse needs and not overly burdensome
3. **Develop patient-centered outcomes**
   - meaningful outcomes
4. **Enhance patient buy-in and engagement**
   - identify and mitigate barriers
   - assure appropriate resources and accommodation


If you want to go fast - go alone.
If you want to go far - go together



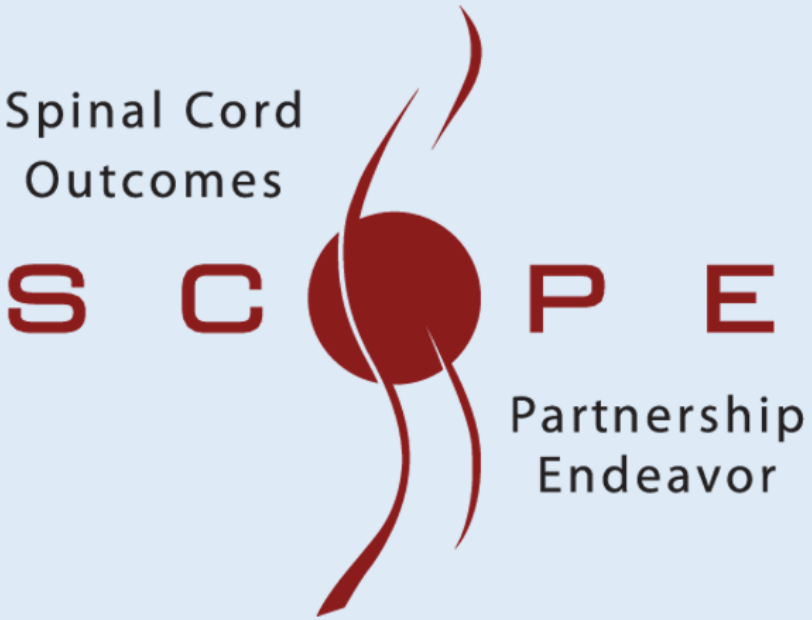
"A Roadmap to Addressing Unmet Needs in SCI Care Through PFDD"

White paper created by:

SCOPE
Spinal Cord Outcomes Partnership Endeavor

North American Spinal Cord Injury Consortium

Linda Jones
&
Barry Munro



ASIA

AMERICAN SPINAL INJURY ASSOCIATION

SCOPE Mission and Leadership

An **academic-industry-agency partnership** established in 2007 to:

- Enhance the development of clinical trial and clinical practice protocols.
- Validate therapeutic interventions for spinal cord injury, leading to the adoption of improved best practices.

Jane Hsieh, MSc
Co-Chair

Wings for Life

Linda Jones, PT, PhD
Co-Chair

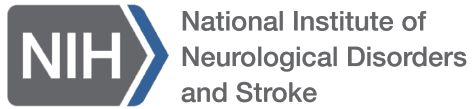
Thomas Jefferson University

Christopher & Dana Reeve Foundation
TODAY'S CARE. TOMORROW'S CURE.

SCOPE Partners

SCOPE members: https://scope-sci.org/scope-planning-committee/

SCOPE Members



Recent Accomplishments

Data Safety Monitoring Boards: Overview of Structure and Role in Spinal Cord Injury Studies

Blight A, **Guest J**, Hamer J, Hsieh J, **Jones L**, Magnuson D, **Pfleeger K**

Lessons Learned and Recommendations from the SCOPE 2023 Spinal Cord Injury Clinical Trials Update

Kondiles BR, Rana S, Weiner D, Blesch A, St. John J, Haag-Molkenteller C, Freund P, **Guest J**, **Mikol D,** Harkema S, Trumbower R, **Fehlings M**, Weidner N, Hogge GS, Field-Fote EC, **Baptista MA**, Curt A, Hsieh J, & **Jones L**

From Molecules to Movement: Scope, Utility, and Application of Biomarkers for SCI Clinical Trials and the Development of SCI Precision Medicine

North American Spinal Cord Injury Consortium

PFDD PRESENTATION

June 26, 2024

The History of SCI Research Engagement:

2016:
Praxis
Conference

2018:
- NASCIC Formed
- IKT Guiding Principles

2019:
- NIH Meeting (A Decade of Disruption)

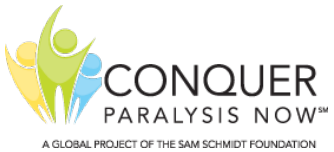
2023:
- NASCIC SCI Research Advocacy Course

North American Spinal Cord Injury Consortium

About NASCIC

- Formed in 2018 from grassroots advocacy efforts across the community living with SCI – wanting to be more active and united in our efforts, initially related to research.
- Membership includes **60** community-led organizations, **336** individuals with lived experience of SCI, and **116** partner organizations and individuals in the SCI field
- 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.

Principal Members



Partner Members

SCOPE
Spinal Cord Outcomes Partnership Endeavor

North American Spinal Cord Injury Consortium

PFDD: Our Objective

To provide guidance for the Spinal Cord Injury (SCI) community in informing the development of an effective and well-represented SCI Patient Focused Drug/Device Development approach

Our Methodology

Kim Anderson

Metro Health

Marco Baptista

Christopher & Dana Reeve Foundation
Today's Care. Tomorrow's Cure.

Megan Moynahan

IFR
Institute for Functional Restoration

Brian Culley

LINEAGE CELL THERAPEUTICS

MJ Mulcahey

Thomas Jefferson University

Steve Kirshblum

KESSLER FOUNDATION
Changing the lives of people with disabilities

Michael Lauw

LINEAGE CELL THERAPEUTICS

James Valentine

Hyman, Phelps & McNamara PC

Our Key Questions

PFDD Outcomes	Moving the PFDD Process Forward
1. How can PFDD **improve outcomes** for people living with SCI? 2. How can we **narrow the scope** of an initial SCI PFDD, given the heterogeneity of the SCI patient population?	3. What are the **special considerations** for organizing a PFDD meeting that allows for representative SCI patient participation? 4. What are the **organizational and financial considerations** in planning an externally-led PFDD meeting?

Lessons Learned

1) Expert interview summary - opportunity for stakeholders to incorporate PwSCI input into **regulatory, research, and medical product developer** decision-making.

2) One PFDD meeting meant to represent the entire SCI community would be too broad. By commissioning a representative patient preferences survey(s) and preparing a **summary of the state of the science**, our community can align on an initial meeting scope addressing problems that developers can realistically tackle.

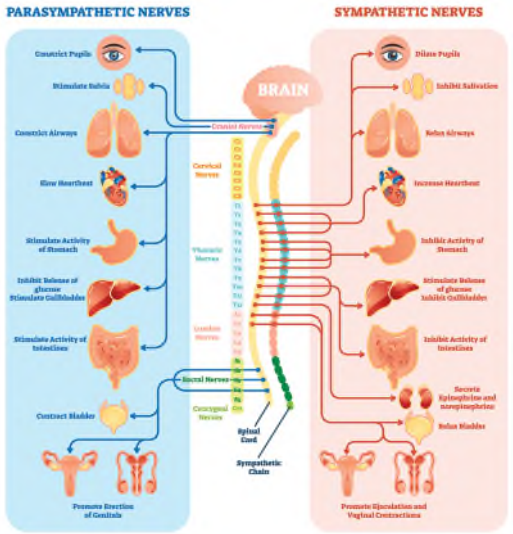
3) PFDD meeting organizers should integrate efforts to ensure **diverse and accurate representation** of the SCI patient population in every step of their planning efforts.

4) Organizing a virtual SCI PFDD meeting is more affordable than an in-person meeting. A **virtual meeting** also allows for broader participation, alleviating barriers to participation such as physical limitations and travel.

Key Question #1: How can PFDD improve outcomes for people living with SCI?

PFDD can help improve outcomes by identifying, informing, promoting…

Priorities

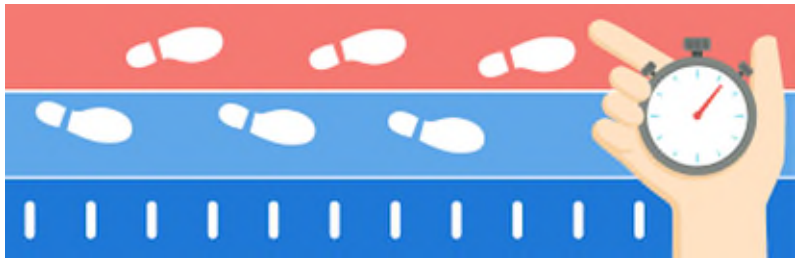


Targeting Recovery: Priorities of the Spinal Cord-Injured Population

KIM D. ANDERSON

Interventions and Clinical Trials

Outcome Measures and Endpoints



FDA

Clinical Outcome Assessment (COA) Qualification Program

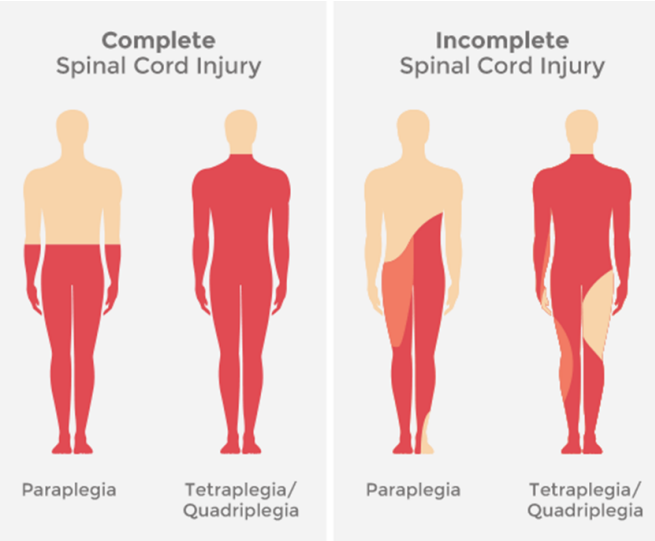
Key Question #2: How can we narrow the scope of an initial SCI PFDD, given the heterogeneity of the SCI patient population?

Key Question #2

One PFDD meeting meant to represent the entire SCI community would be too broad. SCI is not one-size-fits-all

Differing experiences & preferences

Location and Severity of Injury



Chronicity of Injury

Our recommendation for deciding on the scope of the first SCI PFDD meeting

1) Commission a representative patient preference survey, or surveys, to inform the scope of an initial SCI PFDD meeting.

2) Prepare a summary of the state of the science to support evaluating the survey results in the context of ongoing developments.

3) Informed by the results of the patient preference survey(s), align on an initial PFDD meeting scope addressing problems that developers can realistically tackle given the current state of the science.

Key Question #3: What are the special considerations for organizing a PFDD meeting that allows for representative SCI patient participation?

Key Question #3: Special Considerations

When planning a PFDD meeting for SCI patient populations, organizers may face unique barriers distinctive from other communities where patients do not experience the same severity of physical disability.

Key Question #3

Practical Adaptions and Considerations

1. Transportation to an in-person meeting is more burdensome, and more expensive, for people living with SCI than for non-disabled individuals who participated in other disease-specific PFDD meetings.

2. There must be physical accommodations at an in-person meeting to ensure accessibility.

3. There must be acknowledgment and consideration in the meeting planning of participants' differing levels of bowel function, bladder function, degree of pain, spasticity, and upper and lower extremity function.

4. Organizers should consider unpaid caregiver costs.

Takeaway: Organizing a remote SCI meeting could address some of the potential issues outlined above. In recent years, since the COVID-19 pandemic, most EL-PFDD meetings have been held remotely.

Key Question #3

Ensuring Diverse and Accurate Representation of the SCI Patient Population

1. Organizers should consider chronicity and severity of injury in their planning and take steps to recruit individuals with diverse risk-benefit profiles, including people who are risk-averse, risk-tolerant, risk-seeking, etc.

2. Attendees must be racially, ethnically, and socioeconomically diverse to accurately represent the patient population. Organizers should be deliberate in their planning to recruit a diverse, representative group of participants.

3. If patient preference surveys are used to inform the scope of a meeting, organizers should ensure diverse and accurate representation.

Takeaway: PFDD meeting organizers should consider and address the need to ensure diverse and accurate representation of the SCI patient population throughout every step of their planning efforts.

Key Question #4: What are the organizational and financial considerations in planning an externally-led PFDD meeting?

Key Question #4

There are five key stakeholders in a well-represented SCI PFDD approach

PWLE and Caregivers
Only people living with SCI and their caregivers can present and share their opinions during PFDD meetings.
The meetings aim to hear from people who have direct lived experience with the condition, not researchers, advocates without lived experience, medical product developers, or others.

Patient Advocacy Organizations (PAO)
PAOs are responsible for organizing EL-PFDD meetings. Typically, the process is led by one PAO but with the support of other similar organizations. The leading PAO (or PAOs if it is a coalition) is the decision-maker in planning and organizing the PFDD meeting.

Medical Product Developers
PAOs are responsible for organizing EL-PFDD meetings. Typically, the process is led by one PAO but with the support of other similar organizations. The leading PAO (or PAOs if it is a coalition) is the decision-maker in planning and organizing the PFDD meeting.

Practitioners/Researchers
PAOs are responsible for organizing EL-PFDD meetings. Typically, the process is led by one PAO but with the support of other similar organizations. The leading PAO (or PAOs if it is a coalition) is the decision-maker in planning and organizing the PFDD meeting.

The FDA
PAOs are responsible for organizing EL-PFDD meetings. Typically, the process is led by one PAO but with the support of other similar organizations. The leading PAO (or PAOs if it is a coalition) is the decision-maker in planning and organizing the PFDD meeting.

Financial Considerations

	Virtual	In-Person
Major Expenses	• Planning and Execution • Production Support • Medical Writer for VoP Report	• Venue • Travel • Hotels • Food • Planning and Execution • Production Support • Medical Writer for VoP Report
Estimated Total Cost	$85,000	$150,000 - $160,000

Next steps for the SCI community

1. Major SCI stakeholders align and endorse a PAO, or coalition of organizations, to lead the SCI PFDD efforts.
2. Commission a representative patient preference survey, or surveys, to inform the scope of an initial SCI PFDD meeting.
3. Prepare a summary of the state of the science to support evaluating the survey results in the context of ongoing developments.
4. Informed by the results of the patient preference survey(s), align on an initial PFDD meeting scope addressing problems that developers can realistically tackle given the current state of the science.

Homework and Dinner Topics – Survey & Input

1. Name and affiliation (not required)
2. Is this a good use of SCIIS time and resources?
3. How might you/your org. benefit from a PFDD meeting?
4. Are the appropriate stakeholders included?
5. Could your organization contribute to funding?
6. Do you have ideas or suggestions for funding or other resources?
7. What has the working group not included in the pre-planning discussion?
8. Would you like to be contacted about the next PFDD meeting?
    a. Not interested at this time
    b. Kept informed on an ongoing basis
    c. Like to be actively involved
    d. Other (free text)

Big ‘ol QR Code

"What happened on Day 1 and Homework Assessment…."

Brian Culley

“Where does this leave us: a perspective from the Christopher and Dana Reeve Foundation”

Marco Baptista

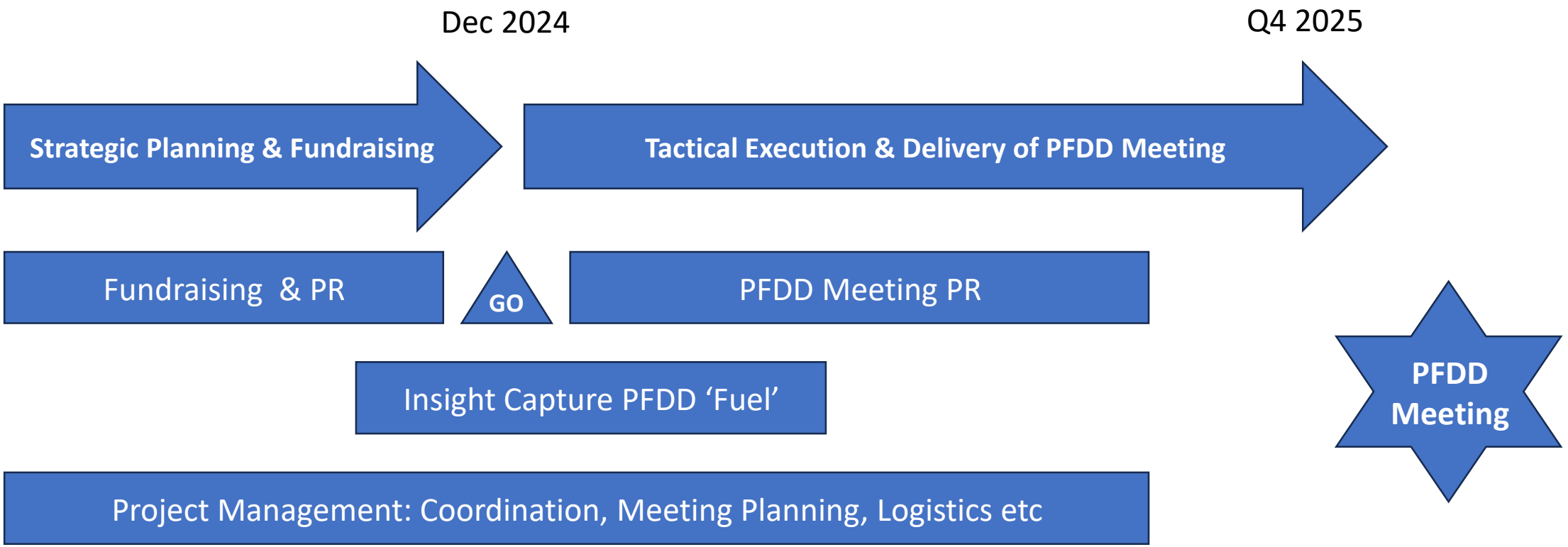
My Key Take-Aways and Perspectives……..

- COMMUNITY COLLABORATION
- MUST HAVE NOT NICE TO HAVE
- SETS the NORTH STAR
- CATALYZES the COMMUNITY
- RAISES AWARENESS
- DRIVES INVESTMENT

"Discussion into Action"

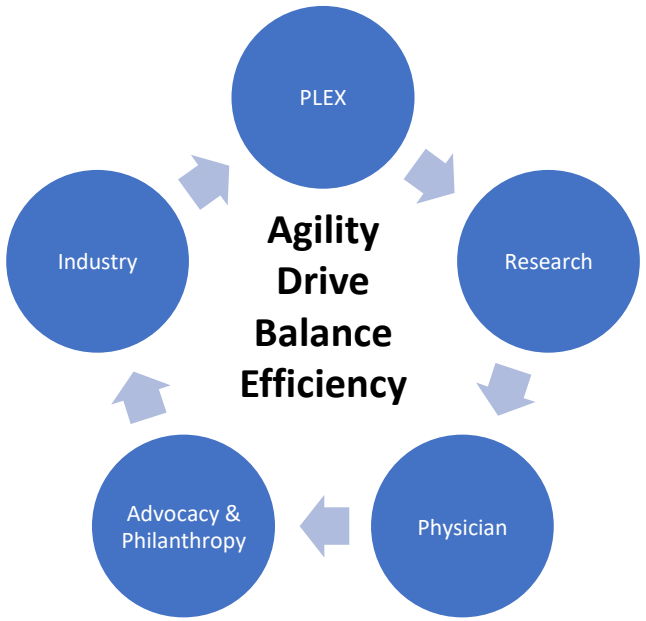
Brian Culley

The Plan: Key Building Blocks, Timing & Funding



The Plan: Getting the Job Done

Multi-Stakeholder Core Team + Project Management (Vendor Support) + Funding



Key Activity	Higher End
Project Management & Coordination (Vendor)	$150K
Meeting Delivery & Execution	~$150K
PLEX Survey, Report & Publish	~$150K
Marketing, PR & Communication	~$50K
TOTAL	**~$500K**

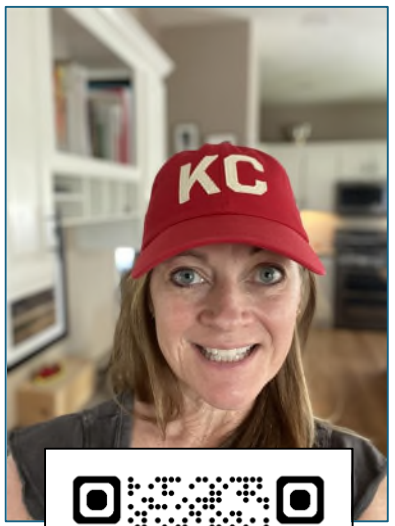
Deliver High Quality
PFDD Meeting
On time, on message, on budget

WE
NEED
YOU


THANK YOU
for your support!

Tell Your Friends', Tell Your Friends'

We are running the Chicago Marathon to raise funds for the Christopher and Dana Reeve Foundation to support those living with paralysis and help provide funds for groundbreaking research.



Ellie

https://give.reeve.org/gonnabeatmydad

David

https://give.reeve.org/iminfrontofu

Kim

https://give.reeve.org/fundraiser/5522078