Public workshop: Incorporating patient preferences into medical device clinical trials

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May 19, 2018

Welcome

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Sculpting the fog…

A near impossible task…

Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and *De Novo* Requests, and Inclusion in Decision Summaries and Device Labeling

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

Document issued on August 24, 2016.
This document will be in effect as of October 23, 2016.

The draft of this document was issued on May 18, 2015.

For questions about this document regarding CDRH-regulated devices, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anindita Saha at 301-796-2537 (Anindita.Saha@fda.hhs.gov)..

For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

But we did it!

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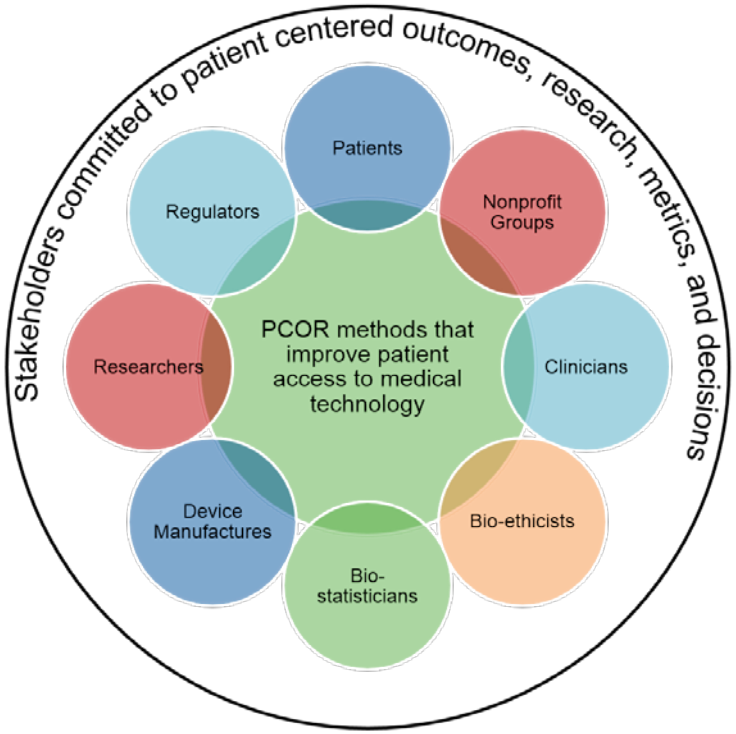
How did we do it?

An intriguing question

What if patients' **urgency for new therapeutic options** and **tolerance of uncertainty** were taken into account when designing and sizing clinical trials?

A New Collaboration to Move Clinical Trials from Generic p-value of 0.05 to Therapy-Specific Patient-Values

A new approach to designing and interpreting clinical trials



http://mdic.org/pcor

Developing and testing a method to incorporate Patient Perspectives on Benefit/Risk as an explicit means to set significance levels in clinical trial design

Investigative Partners: FDA, RTI, MIT, MDIC

Patient Partner: The Michael J. Fox Foundation for Parkinson's Research

Platform for real world patient data: Fox Insight – an online research study to gather the world's largest collection of data about life with Parkinson's.

Specific Aims

1. Identify the outcomes important to patients, family members, and caregivers

2. Design and conduct a patient preference assessment study

3. Design methods for clinical trials approval based on explicit patient input

4. Assess medical device stakeholder acceptance of clinical trial designs based on patient preference

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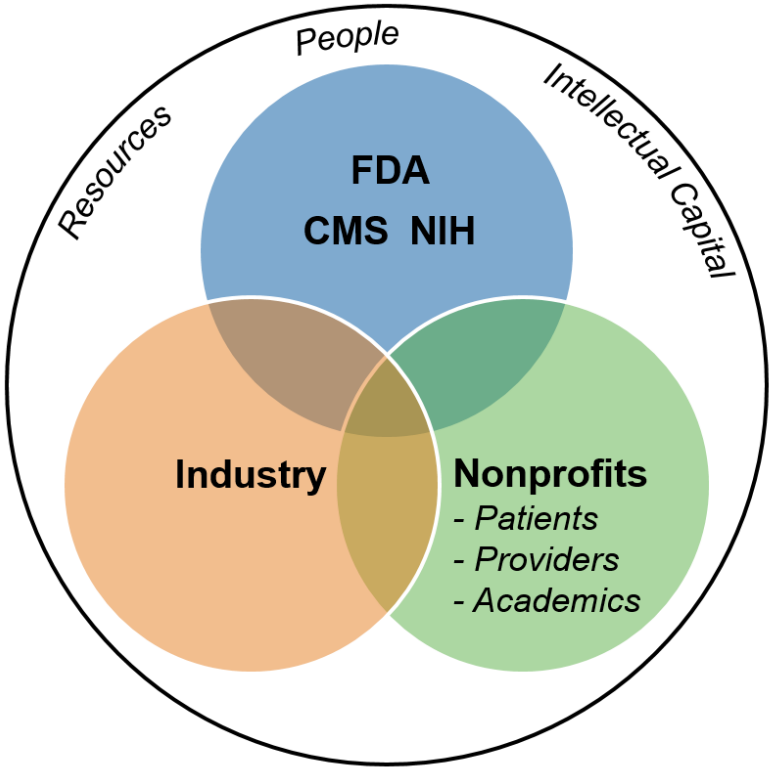
Stakeholder engagement

- Not enough just to create a really cool new widget (or medical device)
- Will the people buy it?

Patients, clinicians, statisticians, regulators, community

- Transformative research requires transformative engagement

Why MDIC?



MDIC is a 501(c)(3) non-profit organization and is the first-ever public-private partnership created with the sole objective of advancing regulatory science of medical devices for patient benefit

Why this matters to MDIC members (and more importantly, patients)

- Meeting patient needs
- CDRH commitment to advancing use of patient preference
- Efficiency and effectiveness in clinical trials

http://www.milkenreview.org/articles/p-values-vs-patient-values

Agenda for the day

- Keynote speakers
- Unpacking the results
- What does this mean for the future of clinical trials?

Thank you!

Patient Scientists from the Michael J. Fox Foundation for Parkinson's Research

- Anne Cohn Donnelly, co-chair
- Margaret Sheehan, co-chair
- Ken Cater
- Christopher Chadbourne
- Quentin Dastugue
- Cynthia Gray
- Nicole Jarvis

Thank you!

Investigator Team

- Andrew Lo, MIT
- Shomesh Chaudhury, MIT
- Brett Hauber, RTI Health Solutions
- Brennan Mange, RTI Health Solutions
- Murray Sheldon, CDRH
- Annie Saha, CDRH
- Heather Benz, CDRH
- Brittany Caldwell, CDRH
- Kyle Myers, CDRH
- Martin Ho, CDRH
- Katrina Gwinn, CDRH
- Mo Zhou, CDRH
- John Ruiz, CDRH
- Lauren McLaughlin, MJFF
- Dawn Bardot, MDIC
- Stephanie Christopher, MDIC

Thank you!

Stakeholders and Supporters

- Karen Anderson, Georgetown University
- Donald Berry, University of Texas
- Marc Boutin, National Health Council
- Sohini Chowdhury, Michael J Fox Foundation for Parkinson's Research
- Paul Ford, Cleveland Clinic
- William Heetderks, FDA
- Ross Jaffe, Versant Ventures
- Pamela Goldberg, MDIC
- Bill Murray, MDIC
- Dave Obringer, RTI Health Solutions
- Peter Saltonstall, National Organization of Rare Disorders
- Randall Schiestl, Boston Scientific
- Murray Sheldon, FDA
- Jeff Shuren, FDA

Thank you!

MDIC Science of Patient Input Steering Committee

- Kobby Dankwah, Abbott
- Scott Goates, Abbott
- Melissa Schooley, Abiomed
- Tara Federici, AdvaMed
- Suzanne Schrandt, Arthritis Foundation
- Dawn Stenstrom, Boston Scientific
- Katie O'Callaghan, CDRH
- Mimi Nguyen, CDRH
- Annie Saha, CDRH
- Sean Tunis, Center for Medical Technology Policy
- Dean Bruhn-Ding, CVRx
- Kelly Close, DiaTribe/ Close Concerns
- Emily Fitts, DiaTribe/ Close Concerns
- Barry Liden, Edwards Lifesciences
- Bryan Luce, Evidera
- Sandi Statz, Exact Sciences
- Cyndi Grossman, FasterCures
- Tanisa Carino, FasterCures
- Jessica Foley, Focused Ultrasound Foundation
- Matt McCarty, ICON
- Kara Haas, Johnson & Johnson
- Eric Relkin, LivaNova
- Stephanie Christopher, MDIC
- Mike Otlewski, MED Institute, Inc.
- Diana Salditt, Medtronic
- Marc Boutin, National Health Council
- Peter Saltonstall, NORD
- Heather Howell, NSF Health Sciences
- Claudia Grossmann, PCORI
- Lesley Maloney, Roche
- Brett Hauber, RTI Health Solutions
- Ross Jaffe, Versant Ventures/ National Venture Capital Association