Developing PRO Instruments
When One Does Not Exist

Session 4
1:30 PM – 2:30 PM

Moderator:
Kathryn O’Callaghan
Deputy Director, Division of All-Hazards Response, Science and Strategic Partnerships
Office of Strategic Partnerships and Technology Innovation
Center for Devices and Radiological Health
Q-PROM Development

Andrea Pusic, MD, MHS
Joseph Murray Professor of Surgery, Harvard Medical School
Director, Patient-Reported Outcomes, Value and Experience (PROVE) Center
Chief, Division of Plastic and Reconstructive Surgery Brigham and Women’s Hospital
Q-PROMs

- Condition-specific PROMs for surgical patients (adult and pediatric)
- Strong emphasis on patient input and rigorous qualitative methods (content validity)
- Rasch psychometric methods to ensure accurate, reliable, meaningful measurement

www.qportfolio.org
Q-PROM Team Leadership

Dr. Andrea Pusic MD MHS
Joseph Murray Professor of Surgery, Harvard Medical School
Director, Patient-Reported Outcomes, Value and Experience (PROVE) Center,
Chief, Division of Plastic and Reconstructive Surgery Brigham and Women’s Hospital

Dr. Anne Klassen, DPhil (Oxon)
Professor in Department of Pediatrics
McMaster University
Associate Member Department of Surgery and Department of Health Research Methods, Evidence & Impact McMaster University
Canadian Institutes of Health Sex and Gender Science Chair

Dr. Stefan Cano PhD
Founding Partner and Chief Scientific Officer, Modus Outcomes, London, UK
Patient-Reported Outcomes (PROs) in Medical Device Decision Making

Sung W. Yoon, MD FACS
Medical Officer
Center for Devices and Radiological Health
Office of Product Evaluation and Quality
Office of Surgical and Infection Control Devices (OHT4)
Division of Infection Control and Plastic Surgery Devices
Patient-Reported Outcomes (PRO)

• Patient perspective
• Development/validation of PRO instruments for regulatory use
• Measure effectiveness of a device in treating or diagnosing the condition
• Determine safety of device in symptom and functional impacts
• Evidence used in benefit-risk assessments
• Use in medical device labeling
Patient-Reported Outcomes (PRO)

BENEFIT

PRO

RISK

Medical Device Labeling
Patient-Reported Outcomes Following LASIK (PROWL) Instrument Development

Ron Hays, PhD
Professor, UCLA Department of Medicine, Department of Health Policy and Management
Input on Patient-Reported Outcomes Following LASIK (PROWL) Instrument

• Collaborators
  • Michelle E. Tarver, Gene Hilmantel, Malvina Eydelman (FDA), Frederick Ferris III (NEI), Elizabeth Hofmeister (Navel Medical Center, San Diego), Keri Hammel and Jeanine May (EMMES), Karen L. Spritzer and Steve Reise (UCLA)

• Feedback on draft items from:
  • Expert panel
    • Ophthalmologists, optometrists, psychometricians, clinical researchers
  • Patients who underwent LASIK

• Interviews with 18 patients to assess:
  • Content coverage, item redundancy, recall period, instructions, format
Regulatory Perspectives

Malvina Eydelman, MD
Director, Office of Ophthalmic, Anesthesia, Respiratory, ENT and Dental Devices
Center for Devices and Radiological Health
US Food & Drug Administration
LASIK Quality of Life Collaboration Project

- CDRH received complaints from patients regarding symptoms, such as dry eyes, glare, halos, starbursts and double vision significantly affecting patient’s quality of life following LASIK
- Collaboration with NEI and DOD
- Developed Patient-Reported Outcomes with LASIK (PROWL) – publicly available
  - Incorporates images and definitions to facilitate reporting of complex visual symptoms
  - Assesses visual symptoms both before and after LASIK to identify changes over time
  - Captures preoperative expectations as well post-operative satisfaction
  - Can be utilized to guide medical decisions and future research

AAO Task Force on Premium Intraocular Lenses (IOLs)

- Disruptive technologic advancements with potential objective and subjective visual compromises
- FDA/AAO 2014 Workshop – Developing Novel Endpoints for Premium IOLs
  - Develop PRO with definitions and images for symptom
- AAO Task Force for PRO development- Collaborative effort with AAO, FDA & Industry
  - Developed PRO with images and definitions
    - Field test at 20 sites (18 US; 2 OUS)
      - Preop completed (700 pts), >400 completed postop

2. American Academy of Ophthalmology
3. https://www.aao.org/premium-iols
Expediting Innovation of Minimally Invasive Glaucoma Surgical (MIGS) Devices

- 2014 FDA/AGS Workshop -Supporting Innovation for Safe and Effective MIGS
- 2015 - Leap Frog MIGS Final Guidance\(^1\)—does not incorporate patient voice
  - Bicoastal Centers of Excellence in Regulatory Science and Innovation (CERSI) Collaboration
    - Johns Hopkins CERSI - To determine the patient-preferences in glaucoma treatment with a focus on MIGS devices
    - UCSF-Stanford CERSI - To develop a survey to assess health-related quality of life in mild to moderate glaucoma patients
      - Collaborators: FDA, Stanford, American Glaucoma Society, American Academy of Ophthalmology, Verana Health
      - PRO Status - Development completed, Field testing began

**PRO for Temporomandibular Disorders**

- Developing PRO to assess patient symptoms
- Collaborating with TMJ Association (patients) and other government and academic centers

Patient Perspectives

Barbara Berney
Patient Representative & Advocate
Bad LASIK
a curse... and a blessing

LASIK changed my life in two ways –

a curse: irreparably damaged vision

a blessing: I am a voice for patients

This is my story, in a nutshell.

Barbara Berney, Artist
FDA Patient Representative
Patient Advocate
In 2001, I underwent LASIK... and lost my clear vision forever. Left with a plethora of complications of varying severity, I could find no medical information that could help me make sense of my “suboptimal” outcome. As an artist, this could have been catastrophic had I accepted that eventuality. I refused.

I soon discovered the Surgical Eyes (SE) patient bulletin board on line. Although I took comfort in knowing I was not alone, I realized that most contributors did not share my own personal response to their misfortune. I began working with unhappy patients, and in 2003, assumed leadership of SE. The following year, my executive director, Dr. David Hartzok, and I dissolved SE and founded the (former) nonprofit Vision Surgery Rehab Network, NFP (VSRN). *

Through my work with VSRN, the FDA found me following their April 2008 LASIK complications public meeting and recruited me to serve as a patient representative on their LASIK quality of life [PROWL] study. Thus began the blessing of advocating for patients in several health areas, and my first experience with PROM.

*With deep regret, VSRN closed its doors in November 2019.
INSPIRE Measures Development
Creating a PRO to Assist Patient Interaction with Emerging and Novel Technologies

Professor Katharine Barnard-Kelly PhD CPsychol AFBPsS
Dr. Courtney Lias
Why and How

Developer Perspective

- **Aim:** develop an instrument to rigorously assess psychosocial outcomes of Automated Insulin Dosing (AID) technology alongside clinical
- Informed by qualitative data, scientific literature, clinical trials, quantitative psychometric assessment
- Designed to meet needs of youth, parents, adults, and partners
- Addresses novel aspects of AID systems: hopes, expectations, anxieties, utility in context of lived experience

FDA Perspective

- **Aim:** Qualify PRO tools that diabetes device sponsors can use in the development and evaluation of diabetes technology
- Can help patients understand the potential value and limitations of available technology choices - Is this device right for them?
Impact and Future Steps

Developer Perspective

- Increases awareness of importance of PROs and their use in trials
- Ensures consistent, standardised assessment of PRO & AID systems
- Adapt for use as clinical tool to assess suitability and support onboarding
- Encourage others to follow MDDT process and become standard practice for psych measures

FDA Perspective

- Potential use of this new outcome tool in assessing safety and effectiveness of diabetes technology
- Demonstrate additional device benefits that can feed into the benefit/risk ratio during device review
- Enable patient-friendly labelling that highlights expectations for the technology in the context of lived experience
Q&A Discussion

To ask a question, either:

1. Use the live Q&A feature in the app
2. Click on the thought bubble icon in the webcast window