Alliance Discussion

featuring
Randall Rutta

July 27, 2022
Today’s Guest

Randall Rutta
Chief Executive Officer,
National Health Council
Patient Engagement in Drug Development: Vision to Impact

JULY 27, 2022
The Power of Patients
NHC Vision for Patient Engagement

Patient Centric

The NHC defines patient centered as any process, program, or decision focused on patients that play an active role as meaningfully engaged participants, and the central focus is on optimizing use of patient-provided information. Patient centered means doing things WITH – not FOR or TO – patients.1,2

Meaningful Patient Engagement

This refers to direct relationships and partnerships that are:

- bi-directional,
- reciprocal, and
- continuous.

Communications are open, honest, and clear.

Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent.1

A variety of “levels” of patient engagement exist and the appropriateness of engagement method selection is context specific. Patients should be key drivers of the effort instead of simply observers to efforts on their behalf. Considering patients as study subjects or just having casual discussions misses the mark entirely.

---

The Acceleration of Patient Engagement Demand & Progress
Working Towards Achieving a Lasting Impact

Declaration on Patient-Centered Healthcare


FDA & HIV Movement
Patients and Consumers Working Party

FDA Patients Representatives Making a Difference

PDUFA V: Benefit-Risk Framework Patient-Focused Drug Development (PFDD)

NIHR Centre for Engagement and Dissemination - Guidance on who to involve with research

NICE Details for Patient Involvement in HTA processes

March, 2020

March 2021

January 2021

EMA - CHMP Pilot Phase

NIHR Involve

Different experiences: A framework for conducting who might be involved in research

March 2021

ICH Reflection Paper

2020

2021

Linear progress until 2019 – 27 Years Timeline

Acceleration in the last 2 years

Source: National Health Council
PFDD Guidance

PDUFA VI includes a commitment from FDA to develop a series of four guidances:

• Guidance 1: Collecting Comprehensive and Representative Input
• Guidance 2: Methods to Identify What is important to Patients.
• Guidance 3: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments
• Guidance 4: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making
Figure 2: Roadmap to Patient-Focused Outcome Measurement in Clinical Trials

**Understanding the Disease or Condition**
- Patient/caregiver perspectives
- Natural history of the disease or condition
- Patient subpopulations
- Health care environment
- Other expert input (healthcare providers, payers, regulators)

**Conceptualizing Clinical Benefits and Risks**
- Identify concept(s) of interest (COI), i.e., how a patient feels, functions, or survives
- Define context of use (COU) for clinical trial

**Selecting/Developing the Outcome Measure**
- Select clinical outcome assessment (COA) type: PRO, ObsRO, ClinRO, or PerfO measure
- Search for existing COA measuring concept of interest in context of use

**Fit-for-Purpose COA**
- A. COI and COU clearly described
- B. Clear rationale
- C. Sufficient evidence to justify rationale
Patient-Centered Core Impact Sets (PC-CIS)
A Framework for Developing Disease-Specific Patient-Centered Core Impact Sets (PC-CIS)

Pool of Potentially Important Impacts
Examples of the wide range of things patients might report as important about the impact a disease or treatment has on their life.

- Symptoms (things only a patient can know)
- Signs (observed by clinicians or others)
- Biomarkers
- Function
- Resource Use
- Health System Experiences
- Treatment Experiences
- Overall Health-related Wellbeing/Quality of Life
- Mortality/Survival

Important Considerations:
Equity, Representativeness, SDOH, Health literacy & numeracy, Culture, Religion, Baseline characteristics, etc.

Prioritization Process
- Structured
- Transparent
- Multi-stakeholder

Stakeholder Engagement
Impacts that matters to other stakeholders

Environmental Scan
Impacts, outcomes, measures and endpoints studied or need to be studied

Most Important Impacts
reported by patients/carers/families

Pool of Important Impacts from all Stakeholders

Align Possible Uses
- Clinical Trials
- RWE/RWD Studies
- Product Development
- Audit
- Quality Measurement
- Value Assessment
- Value-Based Arrangements
- Clinical Decision Support
- Regulatory Decisions

Patient/Carer/Family Engagement
to get to the most important impacts

From Patients: Direct Impacts on Health/Health Outcomes
- From Other Stakeholders: Direct Impacts on Health/Health Outcomes
- From Patients: Other Meaningful Impacts
- From Other Stakeholders: Other Meaningful Impacts

RWD = Real-Word Data
RWE = Real-World Evidence
SDOH = Social Determinants of Health
Patient-Centered Core Impact Sets (PC-CIS)
Project Overview

Objective
➢ The NHC has undertaken the creation of a Blueprint for developing “patient-centered core impact sets,” or PC-CIS, to address inconsistencies between what is important to patients versus much of the information that is typically collected in research and care. A PC-CIS is a patient-derived and prioritized list of impacts a disease and/or its treatments have on a patient and their life as well as that of their family and caregivers.

Vision
➢ Create a smooth pathway for PC-CIS development by patient groups and their partners (e.g., patient-group consortia, medical-product companies, government entities, others) so the patient voice can be enhanced throughout a number of uses. e.g.:

- Clinical trials
- Real-world studies
- Regulatory decisions
- Outcomes research

- Value assessment and economic modeling
- Clinical-decision support, practice guidelines
- Quality-measure development, audits
Blueprint in Progress

- Draft Blueprint Sections and Develop Taxonomy
- Circulate Draft Blueprint for Community Review
- Hold PCORI-Funded Multi-Stakeholder Dialogue Meeting
- Finalize Blueprint and Support Pilot Studies
PFMD Purpose:

PFMD’s goal is to improve global health by co-designing the future of healthcare for patients WITH patients.

Its mission is to bring together initiatives and best practices that integrate the voice of the patient thereby speeding up the creation and implementation of an effective, globally standardized framework – that involves patients as partners – as well as the necessary tools, services and support to allow the adoption of the framework by various stakeholders.
PFMD Governance Structure & Diversity

**PFMD Governance Structure**

**New balanced Min–Max**

- **PFMD Board**
  - Min 30% Max 50% of PO’s
  - Min 30% Max 50% of industry members
  - Target 20% HTA’s, regulatory

- **Executive Director**

- **Executive Committee**

- **Strategic Advisory board**

**PFMD Member Organisations**

- Patient representatives
- Pharma companies (committed to adopt the new framework)
- Academics
- Patient Advocates

---

**PFMD Diversity**

1. **Diversity of Stakeholders groups:** HTA, Regulatory, academia, public health authority, and experts in Device, and Digital champions
2. **Geography, culture & race:** we aim to increase geographic & racial diversity through members and Board
3. **Gender:** we aim to sustain the current good gender balance
4. **Skills:** Commitment to increase connection with champions Digital, HTA, Device.
5. **Patient organization (or industry) size and scope:** Aspiration to build the membership with organizations with more specified or focused scope.

---

**Free membership for not-for-profit organizations**
Patient Engagement Management (PEM) Suite

A global hub for practical tools to plan, assess and execute any patient engagement initiative. PEM Suite is a living patient engagement solution - the PFMD membership tirelessly works on improving it, always making sure anyone has access to an up-to-date, integrated framework for patient engagement.
The PE Quality Guidance proposes **7 Quality Criteria**

- A PFMD co-creation effort that brought together various stakeholders
- Built on pre-existing patient engagement frameworks
- Published and available to all

**7 criteria for over 150 activities WITH patients in the lifecycle of medicine**, and any activity in medtech, digital health and health systems
Goal 1: Scaling PE in Drug Development

1A Shift from a linear drug development model focused on the product to a circular model organized with and around patients and health outcomes.

1B Streamline PE in drug development by sharing one coherent PE reference framework adaptable across stakeholders and the lifecycle of medicine.

1C Further develop the reference framework in terms of extending and deepening its relevance for patients and organizations.

1D Build on existing work to establish PE in health systems and devices.
Goal 2: Build the conditions and Enablers of PE

<table>
<thead>
<tr>
<th>Strategies</th>
<th>2A</th>
<th>2B</th>
<th>2C</th>
<th>2D</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Continue developing and deploying PE enablers to facilitate PE adoption</td>
<td>Scale the network and PE ecosystem to grow the sphere of influence, impact and shared practices</td>
<td>Build ecosystem capability, capacity and sustainability from existing and new solutions to foster a ready-made PE environment</td>
<td>Integrate Health Literacy in all activities to ensure clarity and shared understanding by all stakeholders starting WITH the patients</td>
</tr>
</tbody>
</table>
Goal 3: Build PE in Digital Health & Data

**Strategies**

3A Build a community of early adopters within digital health and data topic areas to inspire and help create a culture shift whereby PE becomes regular practice

3B Identify strategic areas for patient engagement in digital health and data and build on existing work to establish systematic PE good practices
Scaling PE in Wider and Deeper

PARADIGM Extending HTA early dialog toolkit to regulators
PARADIGM Community Advisory Boards
PEM Suite How-to guides

Common rationale for PE & PED research
Asia HTA & Regulatory dialogue
PARADIGM PE Performance Measurement
Patient Experience Data
Asia Capacity & Capability Building

Build PE in Digital Health and Data
PE potential and role of POs and patients in digital health & data
Clinical trial data global distribution network and CT finder

Build the conditions and enablers of PE
PARADIGM PE contracts toolkit for patients
PE Fair Market Value compensation

PEM Suite PE training
Extension to Devices
Book of Good Practices
PE Open Forum
PE Global Network & Management tool - SYNAPSE
Extend PEM suite based on identified needs
COVID19 360° Impact review

Paradigm Community Advisory Boards

Copyright © 2022 National Health Council. All rights reserved.
Thank you!

Randall L. Rutta
Chief Executive Officer
National Health Council
rrutta@nhcouncil.org
Join us next time!

Wednesday, August 3rd at 11:00am ET

Dr. Dan Bausch, MD, MPH&TM
President, American Society of Tropical Medicine and Hygiene;
Senior Director of Emerging Threats & Global Health Security, FIND