Patient-Generated Health Data
Foundations and Opportunities

Session 1
10:15 AM – 10:45 AM
DIGITAL HEALTH TECHNOLOGY:
PATIENT INSIGHTS AT THEIR LOCATION

BAKUL PATEL

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Overview

- Digital Health Landscape
- Future and Importance of Patient-Generated Health Data (PGHD)
- PGHD: Shaping Patient Care
- Digital Health Center of Excellence
- Significance of PGHD in FDA’s mission
The convergence of connectivity, data and computing power for healthcare and related uses across the life of an individual or a patient.

Digital Health

Moving health care from the Clinic to the Patient

Understanding patient's behavior and physiology “In the wild”

Focusing on prevention for early/smaller interventions

Healthy living | Prevention | Diagnosis | Treatment Recovery | Home care

Healthy living

Prevention

Diagnosis

Treatment Recovery

Home care

Leveraging computing power, sensors, connectivity, and software
Digital Health Technologies

Tailoring a Regulatory Framework

- Enhance patient access to high quality digital medical products
- Maintain a reasonable assurance of safety and effectiveness
- Enable manufacturers to rapidly improve software products with minor changes
- Least burdensome
Convergence of computing power, connectivity, sensors, and software used in healthcare.
# Digital Health Landscape

## Noninvasive

- **Treatment**
- **Monitoring**
- **Collection of PGHD**

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<th>Noninvasive Treatment Mechanisms</th>
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<tr>
<td><strong>Mobile Apps</strong></td>
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<td>• Screen interaction</td>
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<td>• Messaging</td>
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<td><strong>Immersive</strong></td>
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<td>• Artificial Reality (AR)</td>
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<td>• Virtual Reality (VR)</td>
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<td>• Gaming</td>
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<td><strong>Sensors</strong></td>
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<td><strong>Implantables</strong></td>
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<td>• Arrhythmia monitors</td>
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<td><strong>Ingestibles</strong></td>
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<td>• Capsule endoscopy</td>
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<td>• Drug adherence</td>
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<td><strong>Wearables</strong></td>
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<td>• Fitness watches</td>
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<td>• Activity trackers</td>
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Digital Health: Part of a Patient’s Lifestyle

PGHD allows us to understand patient behavior ... “in their environment”
Digital Health Technology: Shaping Patient Care

Digital tools can provide consumers with valuable health information.

Consumers who are better informed about health make better decisions.

What evidence and insights these DHTs provide during care delivery?

What standards should apply to digital health technologies?
**FDA’s Digital Health Center of Excellence**

*Empowering All to Advance Healthcare*

**Our goal:** Empower stakeholders to advance health care by fostering responsible and high-quality digital health innovation.

The Digital Health Center of Excellence aims to:

- **Connect and build partnerships** to accelerate digital health advancements.
- **Share knowledge** to increase awareness and understanding, drive synergy, and advance best practices.
- **Innovate regulatory approaches** to provide efficient and least burdensome oversight.
DHCoE Focus Areas

PGHD part of DHCoE Focus Areas

- Software as a Medical Device (SaMD)
- Artificial Intelligence/Machine Learning
- Wearables
- Patient Science
- Software in a Medical Device (SiMD)
- Digital Biomarkers
- Interoperability
- Medical Device Cybersecurity
- Virtual Reality/Augmented Reality
- Real-world Evidence and Advanced Clinical Studies
- Advanced Manufacturing
Digital therapeutic apps for
• Insomnia
• Substance Use Disorder
• Opioid Use Disorder
• ADHD
• Diabetes management

• As of September 2020, Apple, Samsung, Fitbit and Alivecor have OTC ECG apps that have been authorized by the FDA.
PGHD: Harnessing Real World Evidence

Shift toward Patient Generated RWE

- Key to understanding patients’ lifestyles and behaviors
- Can fill gaps in traditional clinical trial data
- Can be leveraged to better understand safety and clinical benefit
- Enables interventions that lead to improved outcomes
PGHD: Looking to the Future

• Gain understanding of the nature of various types of PGHD

• Assess appropriate context of use for various types of PGHD

• Explore and promote acceptance of potential for PGHD as valid scientific evidence
Further Questions or Feedback

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Opportunities for Using Real-World Evidence to Support Regulatory Decisions

Daniel Caños, PhD, MPH,
Director Office of Clinical Evidence and Analysis
Office of Product Evaluation and Quality
Center for Devices and Radiological Health
U.S. Food and Drug Administration
Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.
Valid Scientific Evidence – 21 CFR 860.7(c)(2)

“Valid scientific evidence is evidence from well-controlled investigations, partially controlled studies, studies and objective trials without matched controls, well-documented case histories conducted by qualified experts, and reports of significant human experience with a marketed device, from which it can fairly and responsibly be concluded by qualified experts that there is reasonable assurance of the safety and effectiveness of a device under its conditions of use.”
From FDA’s Real-World Evidence Guidance (2018):

**Real-World Data (RWD)** are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources (e.g., medical claims, electronic health records (EHRs), registries, digital health technologies)

**Real-World Evidence (RWE)** is clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD (involving various study designs, such as randomized or externally controlled trials as well as observational studies)

https://www.fda.gov/media/99447/download
Relevance

- Device Use
- Outcomes of Interest
- Study Population
- Relevant Variables
- Follow-up Information

https://www.fda.gov/media/99447/download
FDA RWE Guidance – Overview

Reliability

• Data Accrual
  ➢ How data are collected (e.g., operational manual, data element definitions, methods of aggregation, etc.)

• Data Assurance/Quality Control
  ➢ Quality control standards to ensure data and analyses are reliable and trustworthy (e.g., registry best practices)

https://www.fda.gov/media/99447/download
Regulatory Context for Real-World Evidence

- MDUFA IV Commitment and FDA Reauthorization Act for RWE framework and pilot projects – Starting 2016
- National Evaluation System for health Technology (NEST) as one of CDRH 2016-2017 Strategic Priorities – July 2017
- Guidance issued to clarify how RWE may be used to support regulatory decisions – August 2017
- Publication of RWE examples on FDA.gov – March 2021
Use of RWE in Regulatory Decision Making

• RWE has been used for regulatory purposes for years, consistent with the definition of valid scientific evidence for medical devices.

• On March 16, 2021, CDRH published 90 examples of RWE usage for regulatory purposes from FY 2012 to FY 2019, including a variety of:
  – Submission types (i.e., 510(k), De Novo, PMA, HDE)
  – Data sources (e.g., registries, medical records, claims, device generated data)
  – Purposes (e.g., new authorization, indication expansion, postmarket study)

https://www.fda.gov/media/146258/download
RWE in Submissions

- 3 examples of digital health devices, demonstrating validation of Software as Medical Device using RWD
- 11 examples using patient- or device-generated data
- 4 examples leveraging radiographic imaging from patient records to address endpoints
- 1 clinical trial embedded in a registry
NESTcc Research Network

Total Patient Population

157 million+

Data Sources

- Electronic Health Records
- Pharmacies
- Private Claims
- Public Claims
- Registries
- Patient-Generated Data
- Unique Device Identifiers
- Billing, supply chain, genomic data

NESTcc has established relationships with Network Collaborators to advance the evaluation and use of high-quality real-world data (RWD).
NESTcc Test Cases

- Test Cases explore the Network Collaborators’ ability to capture the data needed to support a range of studies and analyses across the Medical Device TPLC
- FDA is connecting with Test Case principal investigators to maximize the project relevance for regulatory decision making
- Lessons learned from Test Cases will inform the NEST future state

https://nestcc.org/test-cases/
NEST Patient-Generated Health Data Test Cases

Informing development of studies that collect and analyze PGHD:

(1) patient-reported data, such as responses to questionnaires, symptom and behavior tracking, and validated patient-reported outcomes (PROs)

(2) sensor data measuring a person’s daily activities, mental state, or physiological status, from wearables and remote sensors

(3) patient preference information (PPI) reporting patient valuations of benefit and risk related to relevant device types and specific illnesses and conditions
Summary

• FDA has been successfully leveraging RWE, including PGHD, for regulatory decision making as highlighted in the 90 examples.

• NEST Test Cases are providing an understanding of health outcomes that matter to patients whose condition is managed or treated with medical devices and technologies and providing insights on how these studies could inform regulatory decision-making.

• FDA is working with the NEST community to further integrate PGHD into RWE generation.
SOCIAL MEDIA FOR PATIENT GENERATED HEALTH DATA
ANNE HAMMER, HEALTH SCIENTIST
05.04.2021
Overview

- Social media use
- Social media data vs. traditional health data
- Benefits and challenges
- Value in regulatory decision making
- Key takeaways
Social Media

“...internet-based tools that facilitate the gathering of individuals and communities to communicate and share information, ideas, and experiences in real time.”

2 https://www.pewresearch.org/internet/fact-sheet/social-media/
Rise in Social Media Use

- **Twitter**: 500 million tweets sent daily
- **Facebook**: 28 billion users worldwide
  - 1.84 billion active users per day
  - Reaches 59% of social media users
- **Instagram**: 130 million users in the US
- **Global Statistics**:
  - 5.9% of users in 2019 were fake bots
  - Over 1 billion users worldwide

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What do Patients Share?

Health experiences or updates: 27%
Health symptoms and behavior: 18%
Reviews of treatments (devices, medications), doctors: 16%
Health-related videos or images: 16%

https://infographicsarchive.com/infographic-healthcare-industry-building-trust-through-social-media/
Potential Benefits of Social Media Data

- Data is readily available to researchers
- Unlike traditional studies, can offer insights into the performance of medical products in a short time frame
- Low burden for patients (travel, cost, time)
- May supplement traditional surveillance for adverse events which are less frequently reported in traditional systems
Social Media Data vs. Traditional Data

Differs from other types of Patient Generated Health Data

e.g., sensor-based technologies, patient reported outcomes, patient driven registries

Social media sites are designed for sharing information: not the collection of data intended for scientific research.
Social Media Data: Considerations

Standardization

- Different type and format than other established healthcare data sources (unstructured)
- Terminology may vary due to various factors, making analysis difficult
Social Media Data: Considerations

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### Generalizability
- Barriers to platform participation
- Lack of demographic data for most platforms
- Ability to self report
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**Data Duplication**
- Duplicative posts
- Participation in multiple platforms

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Integrity & Verifiability
- Data may not be authentic
- No validation of diagnosis or treatment
- Bot reporting
Research that incorporates patient experience data to be used in regulatory decision-making not only requires studies to be well-designed but also meet standards for data collection and analysis that meet Agency expectations for quality.¹
Moving Forward

Additional Research:

- The impact of social media in the regulatory framework
- Potential for social media data to be integrated with other data sources
- Under which conditions and device areas social listening can be used for insights on medical device performance
- Potential for mitigation of challenge areas (generalizability, verifiability, etc.)
- Leveraging machine learning approaches to robustly explore relevant data.
Key Takeaways

Social media data can provide insights and context around:

- Overall patient experience
- Patient perceptions regarding diagnosis and treatment
- Patient preferences
- Quality of life

It can also help to:

- Discover current topics of concern or interest
- Identify concepts or wording for Patient-Reported Outcome instruments and other survey tools
- Identify relevant patient populations appropriate for clinical trials

- **Social media data alone does not yet rise to the level of evidence required to evaluate safety and effectiveness**
Further Questions or Feedback

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