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

05.04.2021

www.fda.gov/digitalhealth

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

Digital Health

The convergence of connectivity, data and computing power for healthcare and related uses across the life of an individual or a patient.

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

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

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

Digital Health Technology

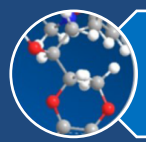
Healthy Living | Prevention | Diagnosis | Treatment | Recovery | Home care | Management

Convergence of computing power, connectivity, sensors, and software used in healthcare.

Used as a medical product

Incorporated into a medical product (include a pharmacologic product)

Used to develop a medical product



Used to study a medical product

Used as a companion or adjunct to a medical product, including diagnostics and therapeutics.

Digital Health Landscape

Noninvasive

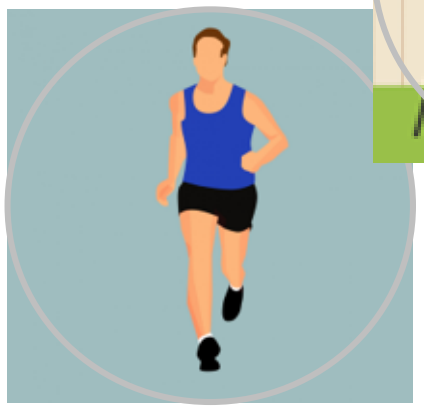
• *Treatment* • *Monitoring* • *Collection of PGHD*

Noninvasive Treatment Mechanisms		
Mobile Apps • Screen interaction • Messaging	Immersive • Artificial Reality (AR) • Virtual Reality (VR) • Gaming	• Sound • Motion / Haptic feedback • Avatar-based interaction

Sensors		
Implantables • Arrhythmia monitors	Ingestibles • Capsule endoscopy • Drug adherence	Wearables • Fitness watches • Activity trackers

Digital Health: Part of a Patient's Lifestyle

PGHD allows us to understand patient behavior …
"in their environment"

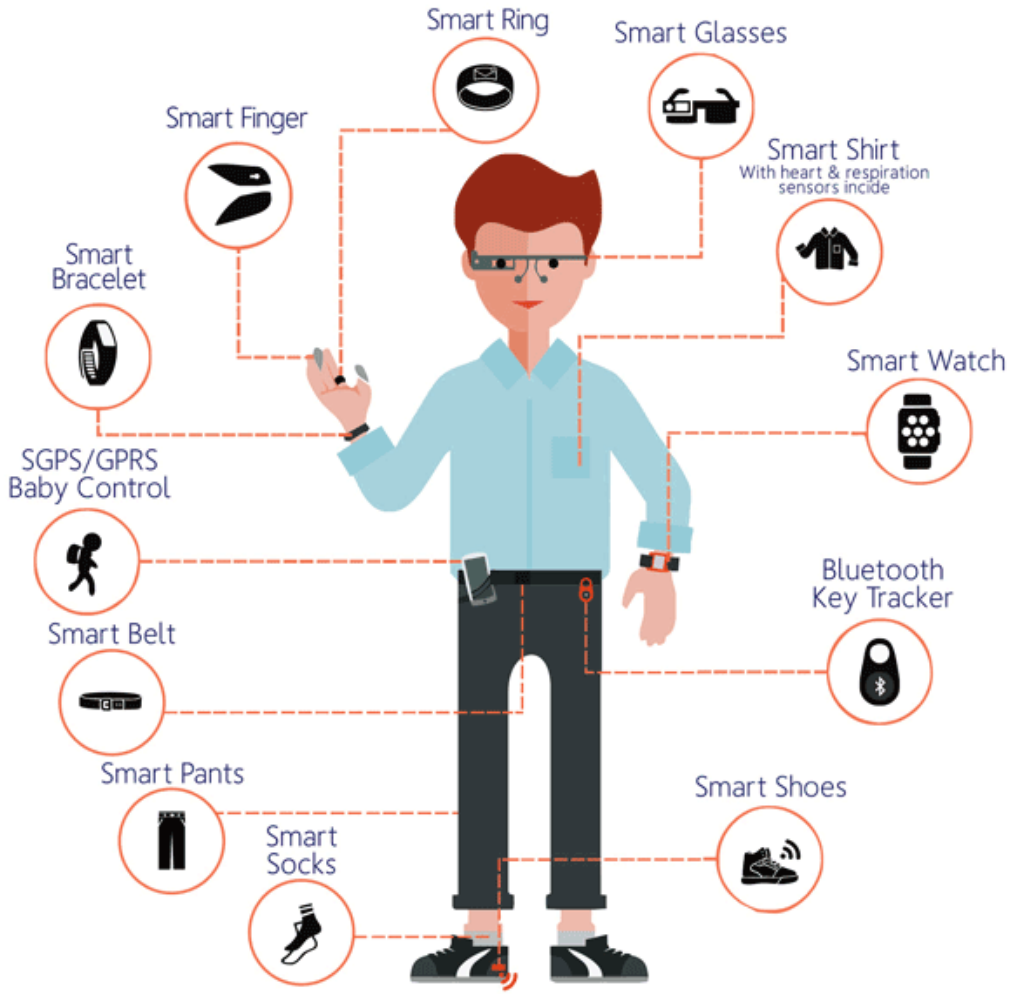


Digital Health Technology:

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 digital health technologies ?

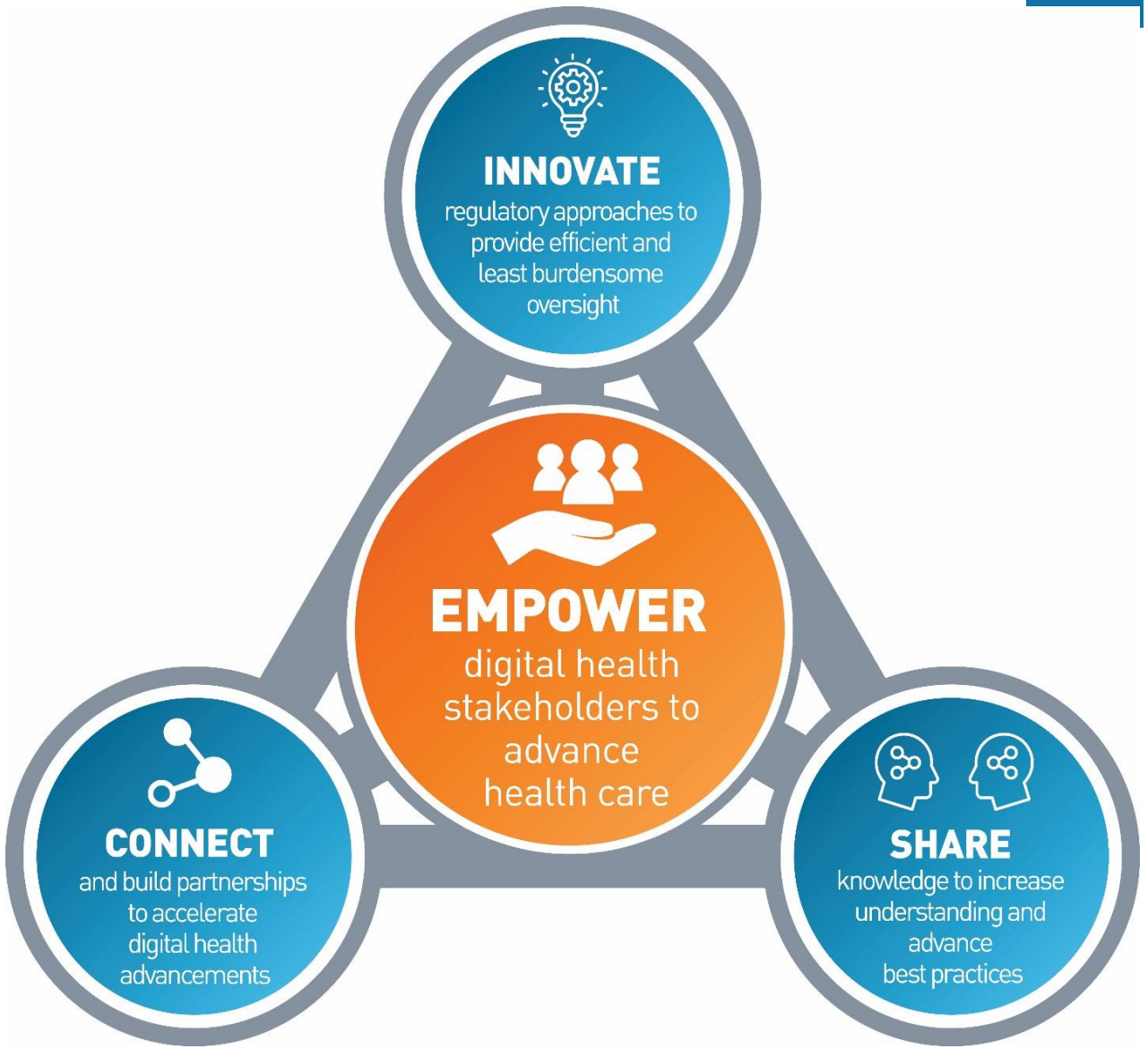
FDA's Digital Health Center of Excellence

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

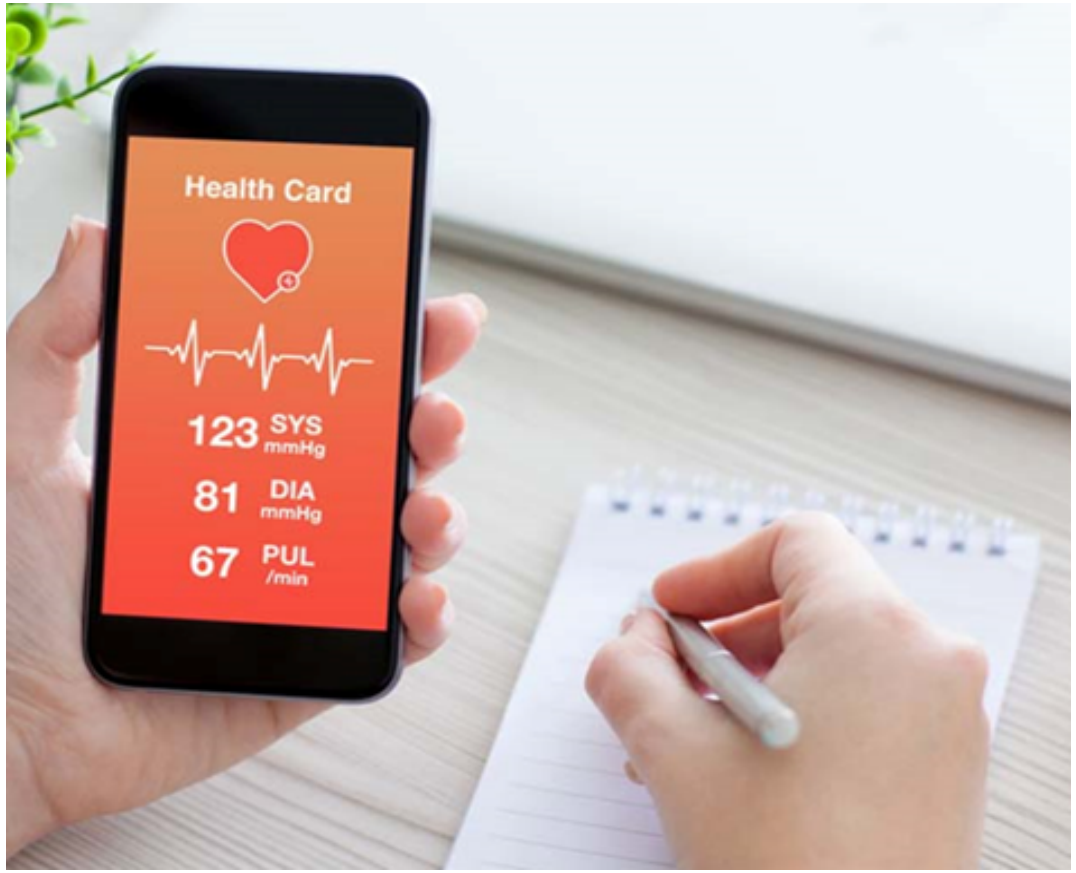
PGHD part of DHCoE Focus Areas

- 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

A First for FDA's Regulatory Purposes

FDA's Digital Health Center of Excellence

Empowering digital health stakeholders to advance healthcare



INNOVATE

Notable Authorizations

Digital therapeutic apps for

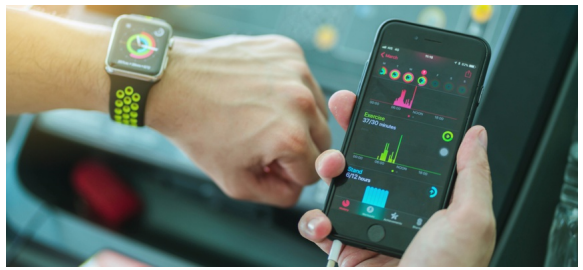
- Insomnia
- Substance Use Disorder
- Opioid Use Disorder
- ADHD
- Diabetes management

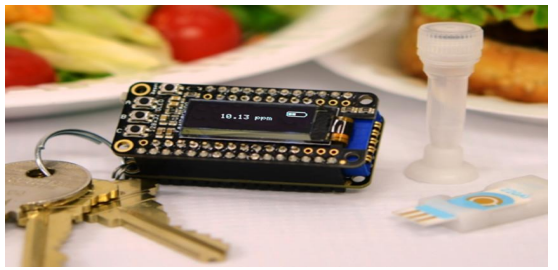
- *As of September 2020, Apple, Samsung, Fitbit and Alivecor have OTC ECG apps that have been authorized by the FDA.*

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

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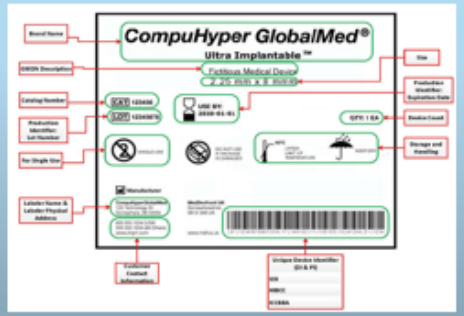
Director Office of Clinical Evidence and Analysis
Office of Product Evaluation and Quality
Center for Devices and Radiological Health
U.S. Food and Drug Administration

Real-World Data

Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources

Diagnostic Laboratory and Imaging

Device/Patient Registries



Device-Generated Data

Patient-Generated Data

Electronic Health Records

Medical Billing Claims

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."

‘Real-World’ Definitions

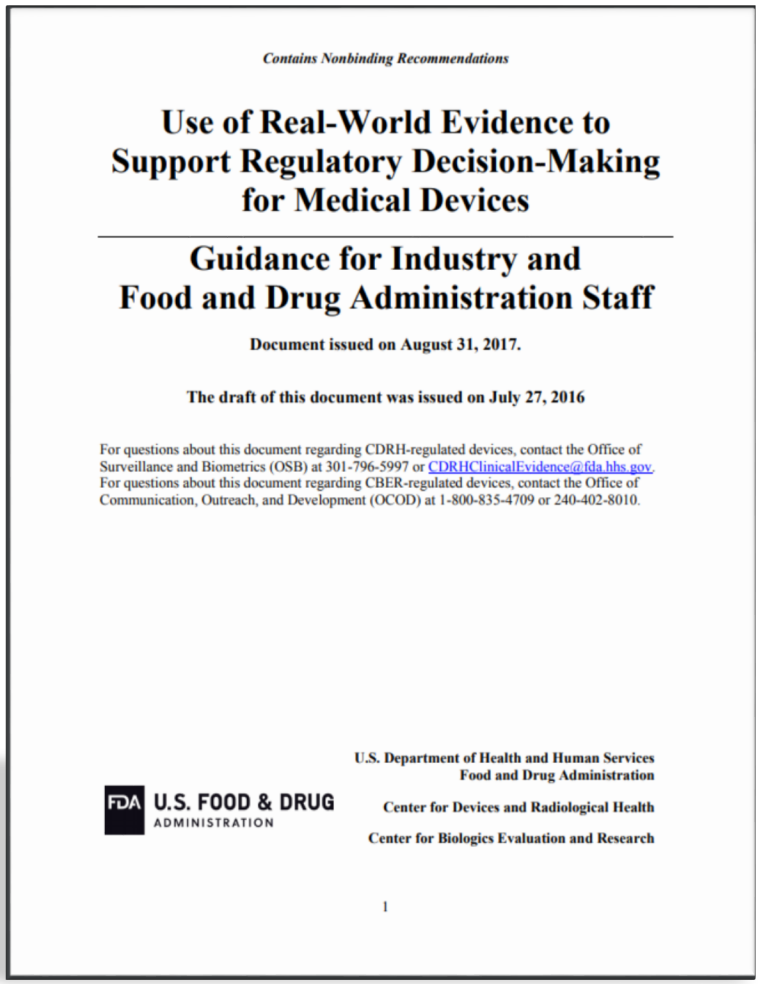
From FDA’s Real-World Evidence Guidance (2018):

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

FDA RWE Guidance – Overview



Contains Nonbinding Recommendations

Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices

Guidance for Industry and Food and Drug Administration Staff

Document issued on August 31, 2017.

The draft of this document was issued on July 27, 2016

For questions about this document regarding CDRH-regulated devices, contact the Office of Surveillance and Biometrics (OSB) at 301-796-5997 or CDRHClinicalEvidence@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.

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Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

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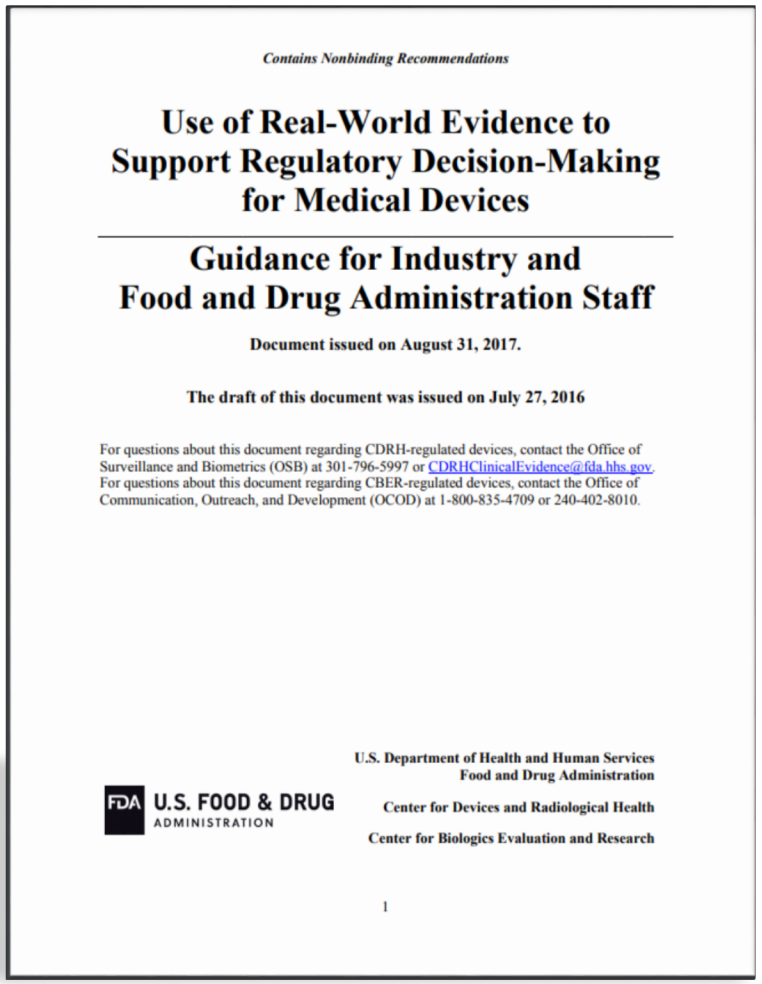
Relevance

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

FDA RWE Guidance – Overview



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Reliability

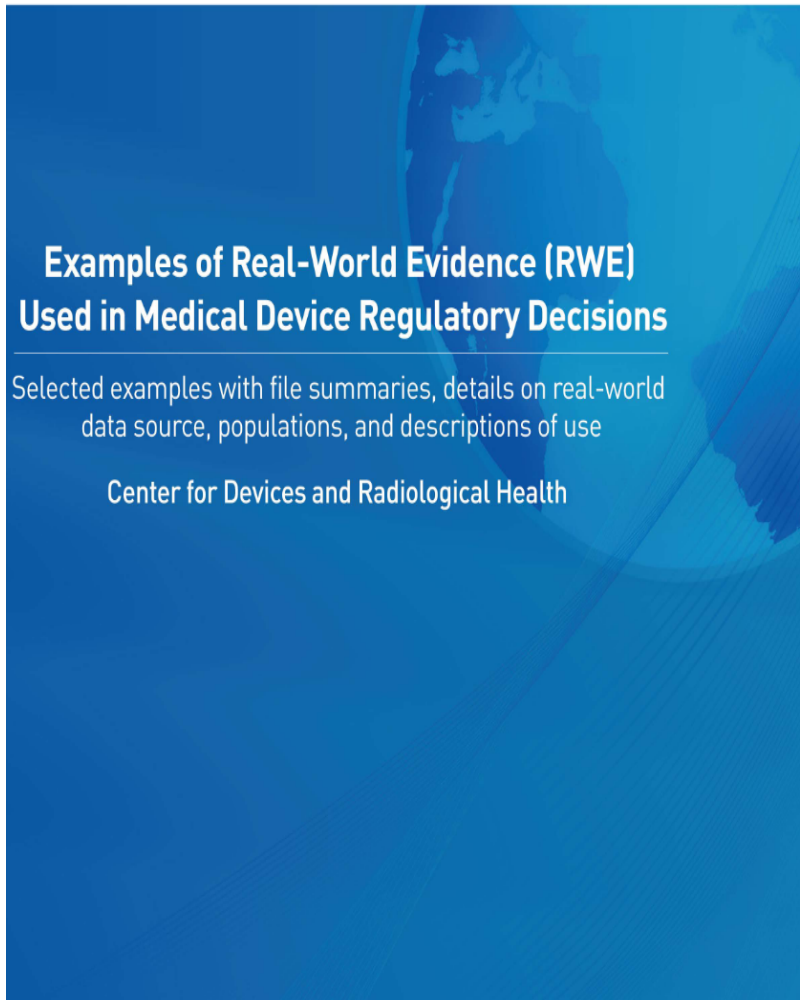
- Data Accrual
  - How data are collected (e.g., operational manual, data element definitions, methods of aggregation, etc.)
- Data Assurance/Quality Control
  - Quality control standards to ensure data and analyses are reliable and trustworthy (e.g., registry best practices)

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)**



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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

157 million+

Total Patient Population

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

Data Sources

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

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/

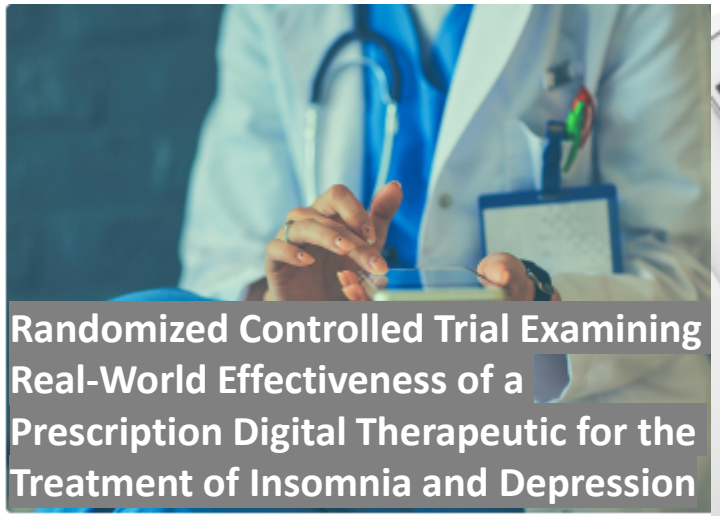
NEST Patient-Generated Health Data Test Cases

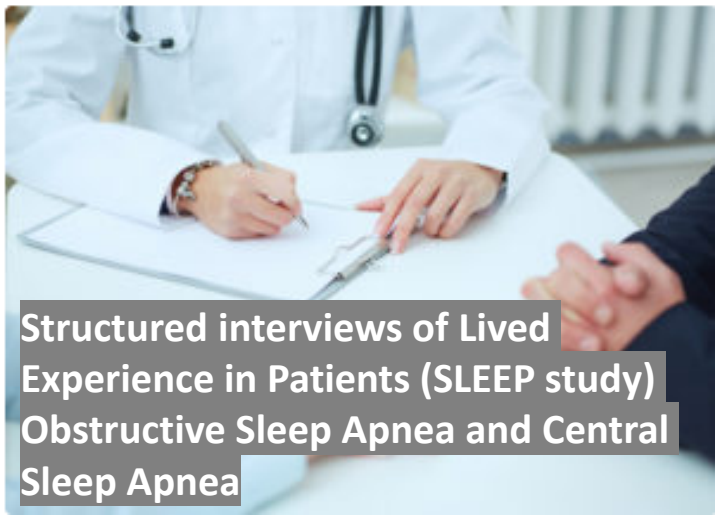
Informing development of studies that collect and analyze PGHD:

(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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SOCIAL MEDIA FOR PATIENT GENERATED HEALTH DATA

ANNE HAMMER, HEALTH SCIENTIST

05.04.2021

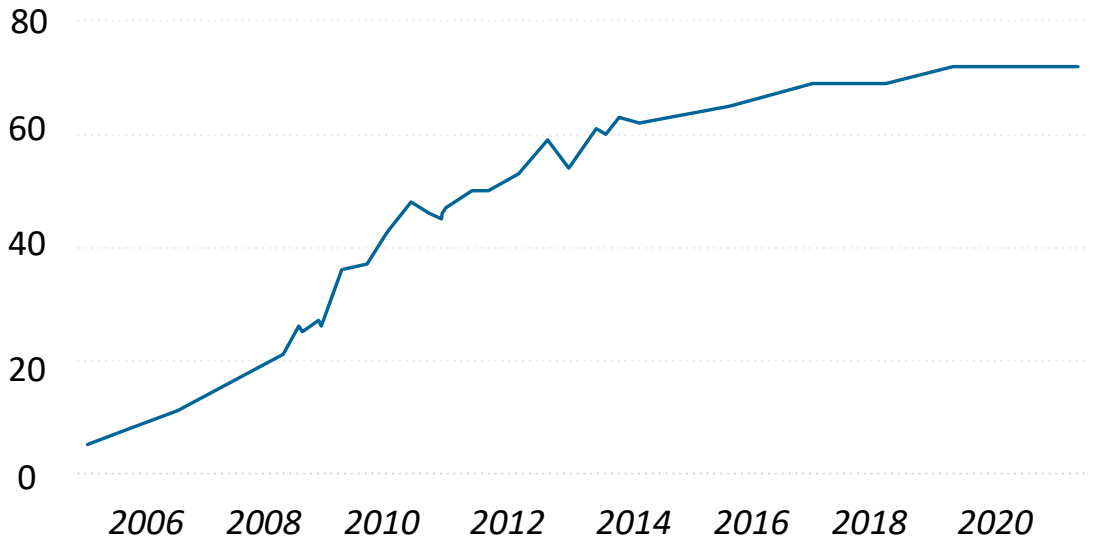
Overview

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

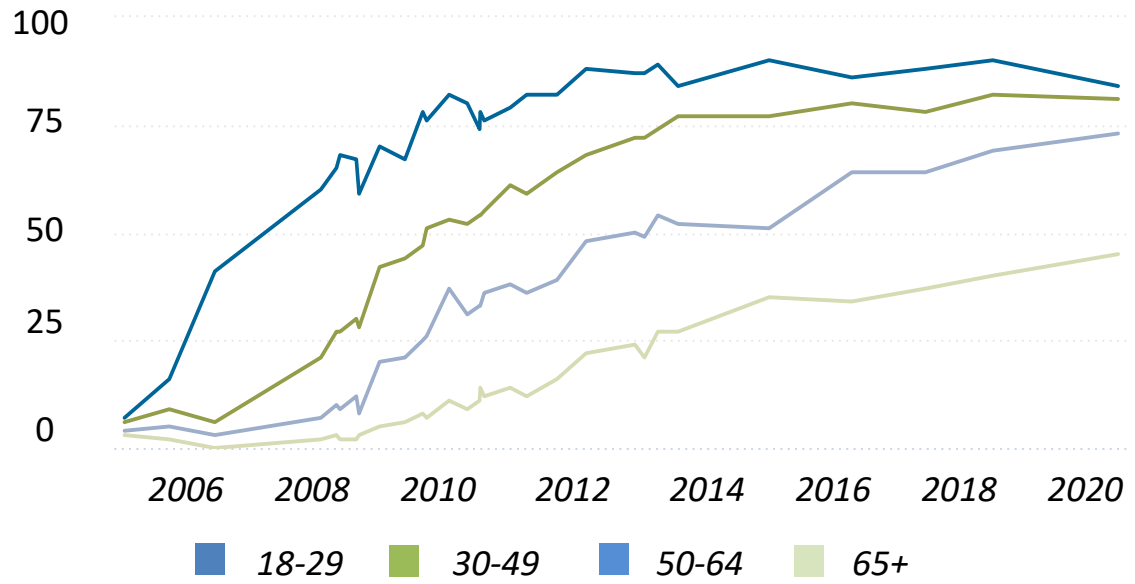
Social Media

"…internet-based tools that facilitate the gathering of individuals and communities to communicate and share information, ideas, and experiences in real time."[1]

% of U.S. adults who say they use at least one social media site [2]



% of U.S. adults who say they use at least one social media site, by age [2]



1 https://www.ema.europa.eu/en/documents/report/social-media-m-health-data-subgroup-report_en.pdf
2 https://www.pewresearch.org/internet/fact-sheet/social-media/

Rise in Social Media Use

TWITTER

500 MILLION TWEETS SENT DAILY

300 million active users worldwide

192 million daily active users

FACEBOOK

USERS SHARE ONE MILLION LINKS EVERY TWENTY MINUTES

28 billion users

1.84 billion active users per day

reaches 59% of social media users

INSTAGRAM

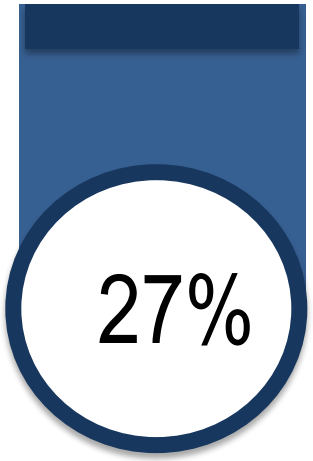
130 million users in the US

1 billion users worldwide

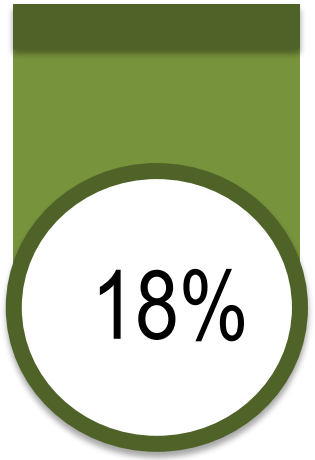
9.5% OF USERS IN 2019 WERE FAKE BOTS

What do Patients Share?

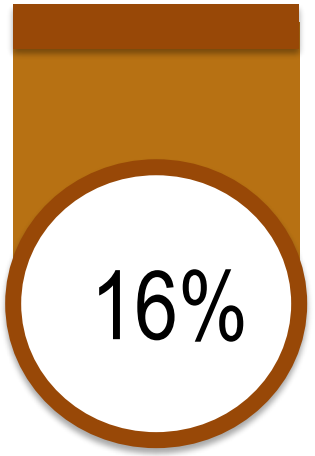
Health experiences or updates



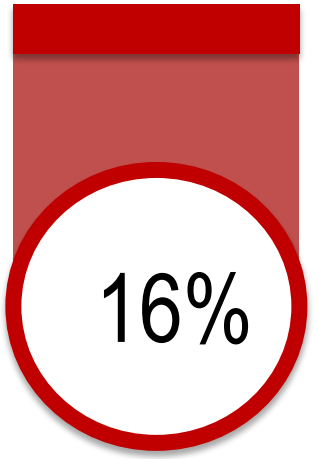
Health symptoms and behavior



Reviews of treatments (devices, medications), doctors

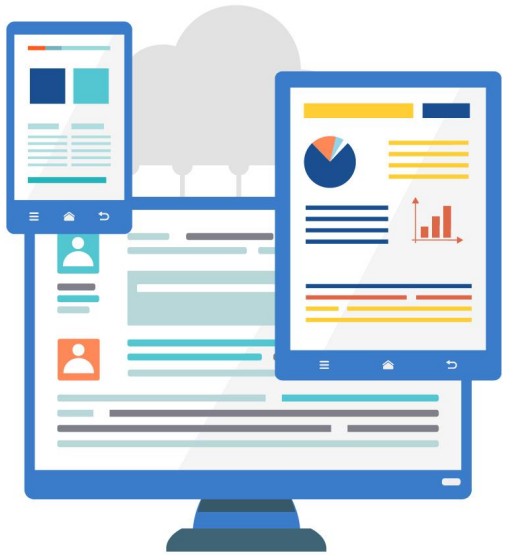


Health-related videos or images



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

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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

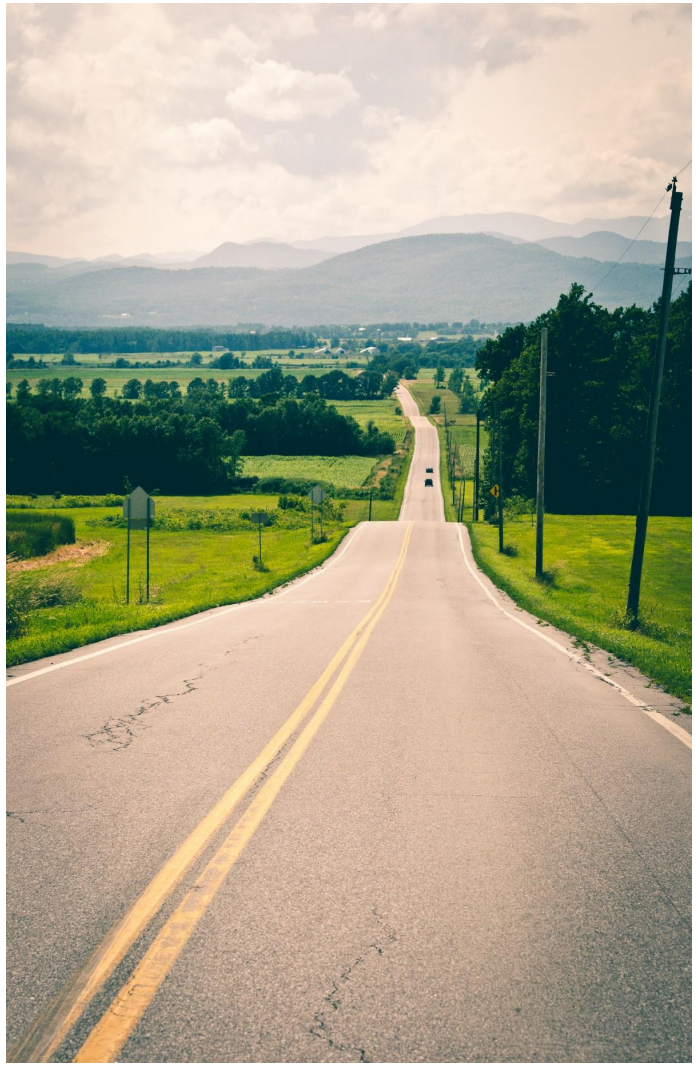
- Data may not be authentic
- No validation of diagnosis or treatment
- Bot reporting

Social Media: Value in Regulatory Decision-Making

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]

Patient-Focused Drug Development: Collecting Comprehensive and Representative Input: Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

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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Digital Health Center of Excellence

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