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DEVICES
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The Future of Global Evidence Generation – Utilizing Real World Data

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IMDRF International Medical
Device Regulators Forum



Overview

The Future of Global Evidence Generation

- Real World Data/Real World Evidence (RWD/RWE)
- Recent Regulatory Developments
- Greater Need for Global, Harmonized Practices
- Benefits/Challenges to Implementation



The Future of Global Evidence Generation

Purpose of clinical trials

- “The objective of a clinical investigation is to **assess the safety and performance/efficacy** of the device in question and evaluate whether the device is suitable for the purpose(s) and the population(s) for which it is intended (ISO 14155-1:2003).

When is a clinical trial necessary?

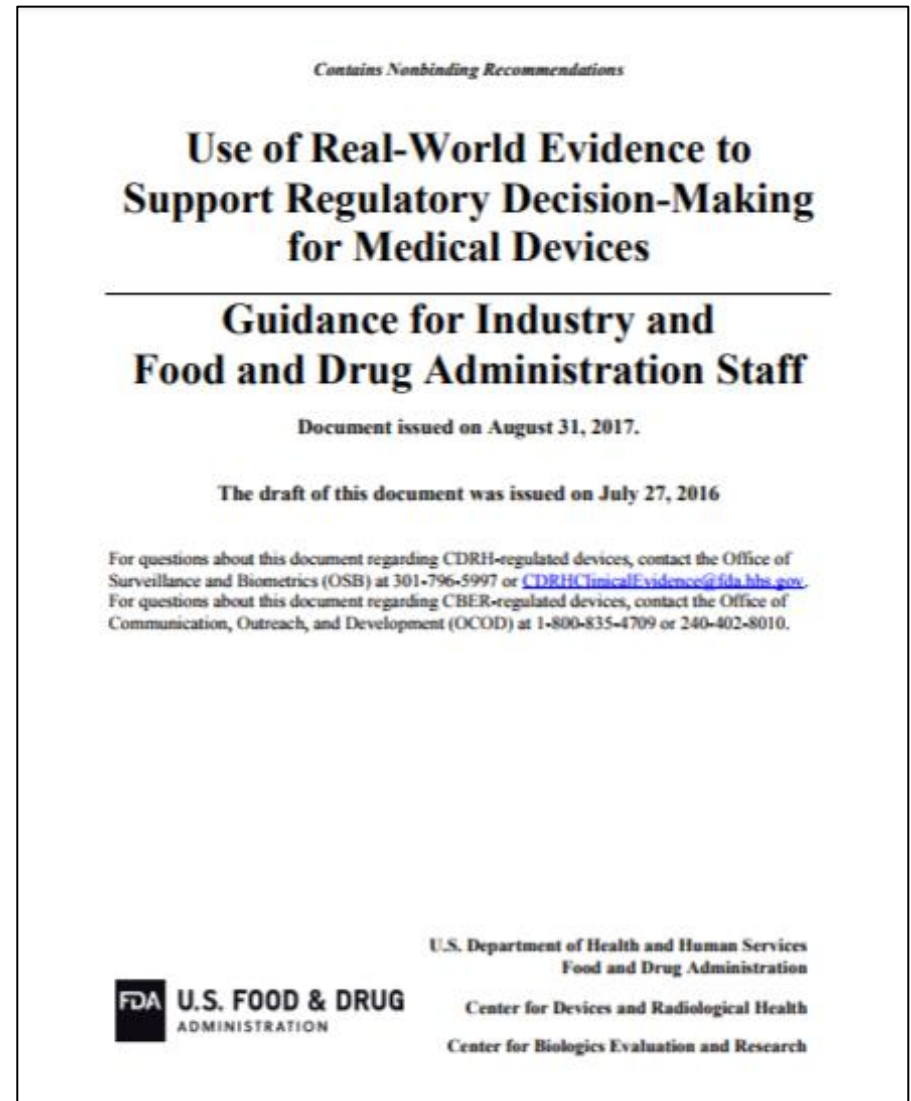
- Clinical investigations are necessary to provide the data **not available through other sources**. GHTF/SG5/N3:2010
- It is ethically important in deciding to conduct a clinical investigation that it **should generate new data and answer specific safety and/or performance questions** that remain unanswered by the current body of knowledge. GHTF/SG5/N3:2010

Recent Developments

US FDA Guidance:

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

- Issued on August 31, 2017



RWD/RWE

Real-World Data (RWD)

- FDA recognizes that a wealth of data covering medical device experience exists and is routinely collected in the course of treatment and management of patients (RWD)
- RWD, which is typically collected for non-regulatory purposes, may provide new insights into the performance of medical devices
- Under certain circumstances, RWD may be of sufficient quality to help inform or augment FDA's understanding of the benefit-risk profile of devices at various points in their lifecycle

Real-World Evidence (RWE)

- Clinical evidence regarding the usage, and potential benefits or risks, of a medical product derived from analysis of RWD.
- Under the right conditions, data derived from real world sources can be used to support regulatory decisions.

FDA Guidance document: "Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Device" (August 2017); <https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm513027.pdf>

Examples - RWE Used to Support Regulatory Decision Making:

- Pre Market:
 - Expanded Indications for Use
 - HDE to de Novo/PMA
 - General to Specific Claims
 - CE Mark (OUS approval) to US Approval
 - Control Group
- Post-Approval Device Surveillance as Condition of Approval
- Post Market
 - Postmarket Surveillance Studies
 - Registries
 - Passive Data Collection from Devices/Patients

CDRH 2016-2017 Strategic Priorities

U.S. Food and Drug Administration

2016-2017 Strategic Priorities

Center for Devices and Radiological Health

GOAL: INCREASE THE USE OF REAL-WORLD EVIDENCE TO SUPPORT REGULATORY DECISION MAKING

- By December 31, 2016, increase by 40 percent the number of premarket and postmarket regulatory decisions that leverage real-world evidence. (compared to FY2015 baseline)
- By December 31, 2017, increase by 100 percent the number of premarket and postmarket regulatory decisions that leverage real-world evidence. (compared to FY2015 baseline)

CDRH 2016-2017 Strategic Priorities

U.S. Food and Drug Administration

2016-2017

Strategic Priorities

Center for Devices and Radiological Health

GOAL: INCREASE USE AND TRANSPARENCY OF PATIENT INPUT AS EVIDENCE IN OUR DECISION MAKING

- By September 30, 2016, 50 percent of PMA, *de novo* and HDE decisions will include a public summary of available and relevant patient perspective data considered.
- By September 30, 2017, 100 percent of PMA, *de novo* and HDE decisions will include a public summary of available and relevant patient perspective data considered.
- By September 30, 2017, increase the number of patient perspective studies (e.g., evaluating patient reported outcomes or patient preferences) used in support of premarket and postmarket regulatory decisions. (compared to FY 2015 baseline)
- By September 30, 2017, increase the number of Expedited Access Pathway data development plans or regulatory submissions that consider patient perspectives. (Compared to FY 2015 baseline)

Harmonization of RWE practices

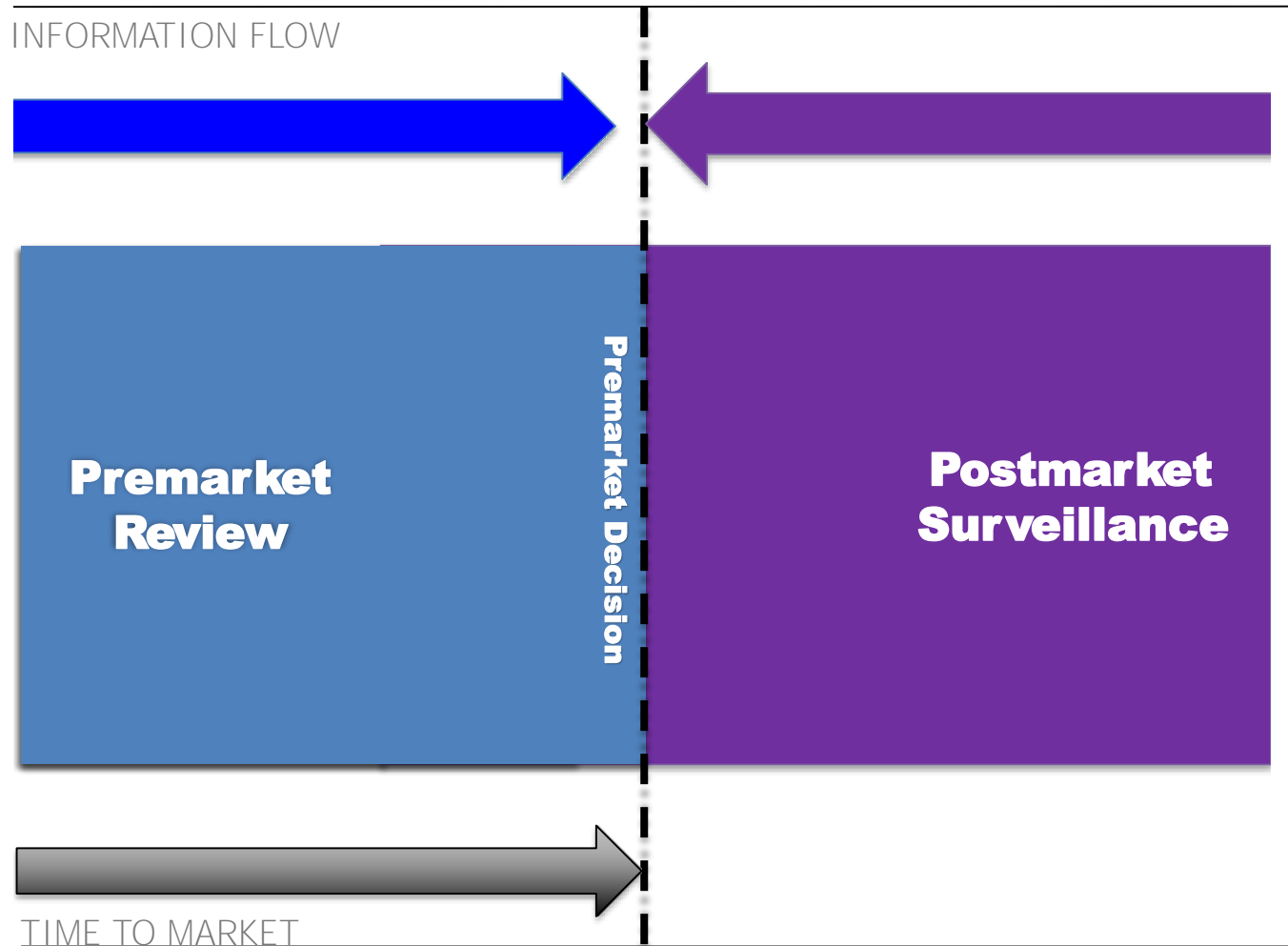
Greater need for global, harmonized practices.

- “We envision international collaboration in medical device evidence generation, synthesis and appraisal. Robust registries and collaborative registry consortia are key pillars of international enterprise.”
- “The international collaboration will harness the global strength of international experience with devices, and leverage individual country strength....”
- “Worldwide, regulators will initiate early engagement with their respective registries.”
- “The international collaboration will establish a forum and a set of priority questions related to devices in collaboration with registry leaders and other stakeholders.”

“IMDRF Principles of International System of Registries Linked to Other Data Sources and Tools,” IMDRF Patient Registries Working Group (30 September 2016)

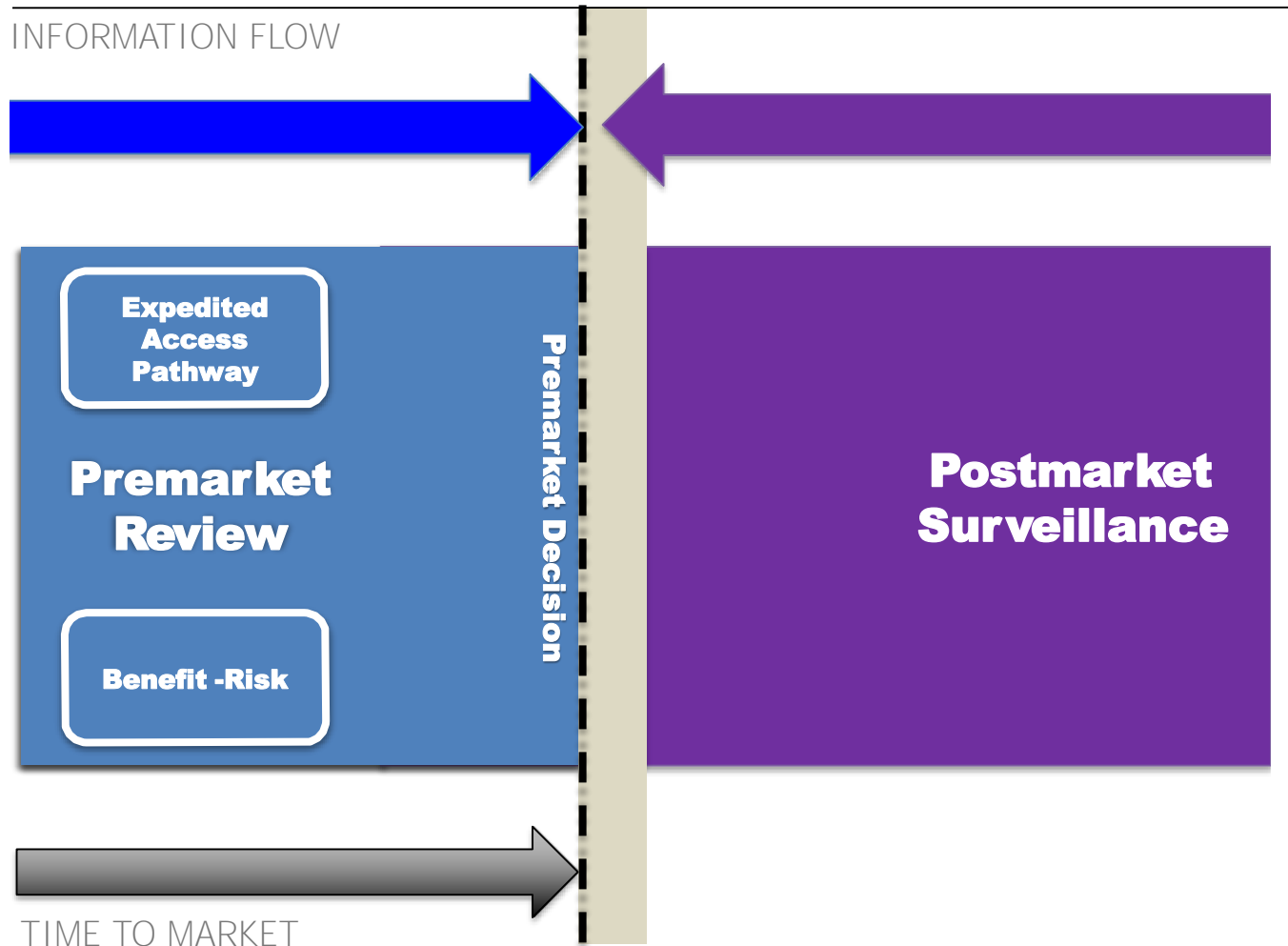
<http://www.imdrf.org/docs/imdrf/final/technical/imdrf-tech-160930-principles-system-registries.pdf>

Learning Medical Device Ecosystem



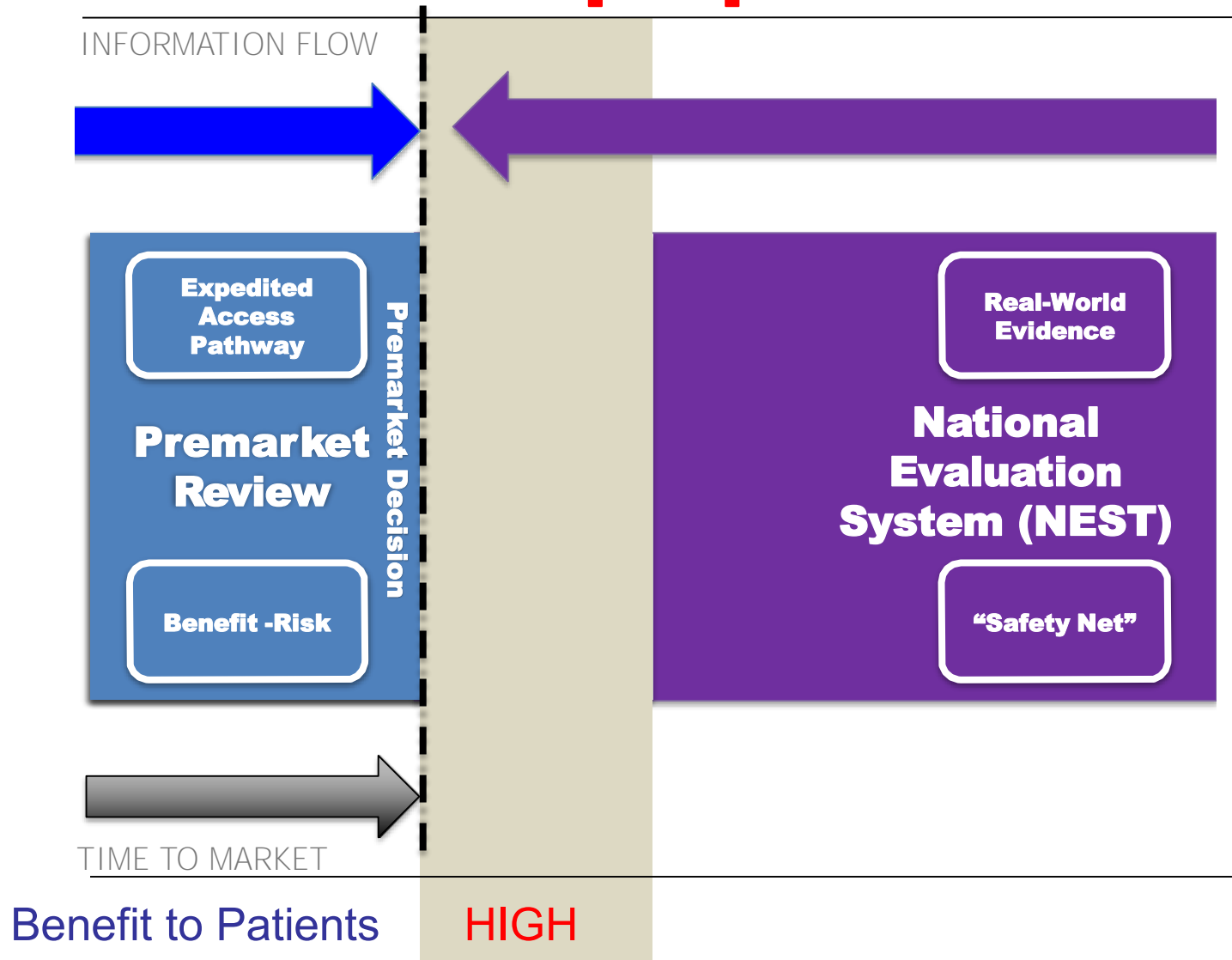
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Learning Medical Device Ecosystem



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Establishing NEST will enable the **pre-post market shift**



Benefits of a Harmonization Approach

- Global framework – ability to leverage global warehouse of data to promote innovation and protect patient safety.
- Global data used to enhance regulatory decision making with less potential risk to patients.
- To achieve benefits – must focus on consistency of methodology, standards, and consistent target to optimize data.

Challenges Related to Harmonization

- Complexity of establishing a global methodology
- in substance and administration
- Identifying consistent target(s) to optimize data
- Issues related to privacy of patient and data
- Implementing framework for
collaboration/methodologies/standards

Going Forward

Global regulators, academia, and industry form a global 'NEST'- type organization to:

- Identify and prioritize challenges to harmonization of regulations, standards, methodologies
- Define, prioritize and align on an an action plan that reflects global, regional and local differences in regulations, infrastructure, healthcare systems, healthcare practices
- Global protocols to provide benefit on global population and sub population levels (e.g. geographic – demographic- disease)

