Real-World Evidence: A CDRH Perspective

Karen Ulisney, MSN, CRNP
Policy Analyst, Clinical Trials Program
Office of Device Evaluation
CDRH, FDA

Case for Quality Forum July 20, 2017

Patients are at the Heart of What We Do

CDRH Vision: Patients in the U.S. have access to high-quality, safe, and effective medical devices of public health importance first in the world

We face a critical public health challenge

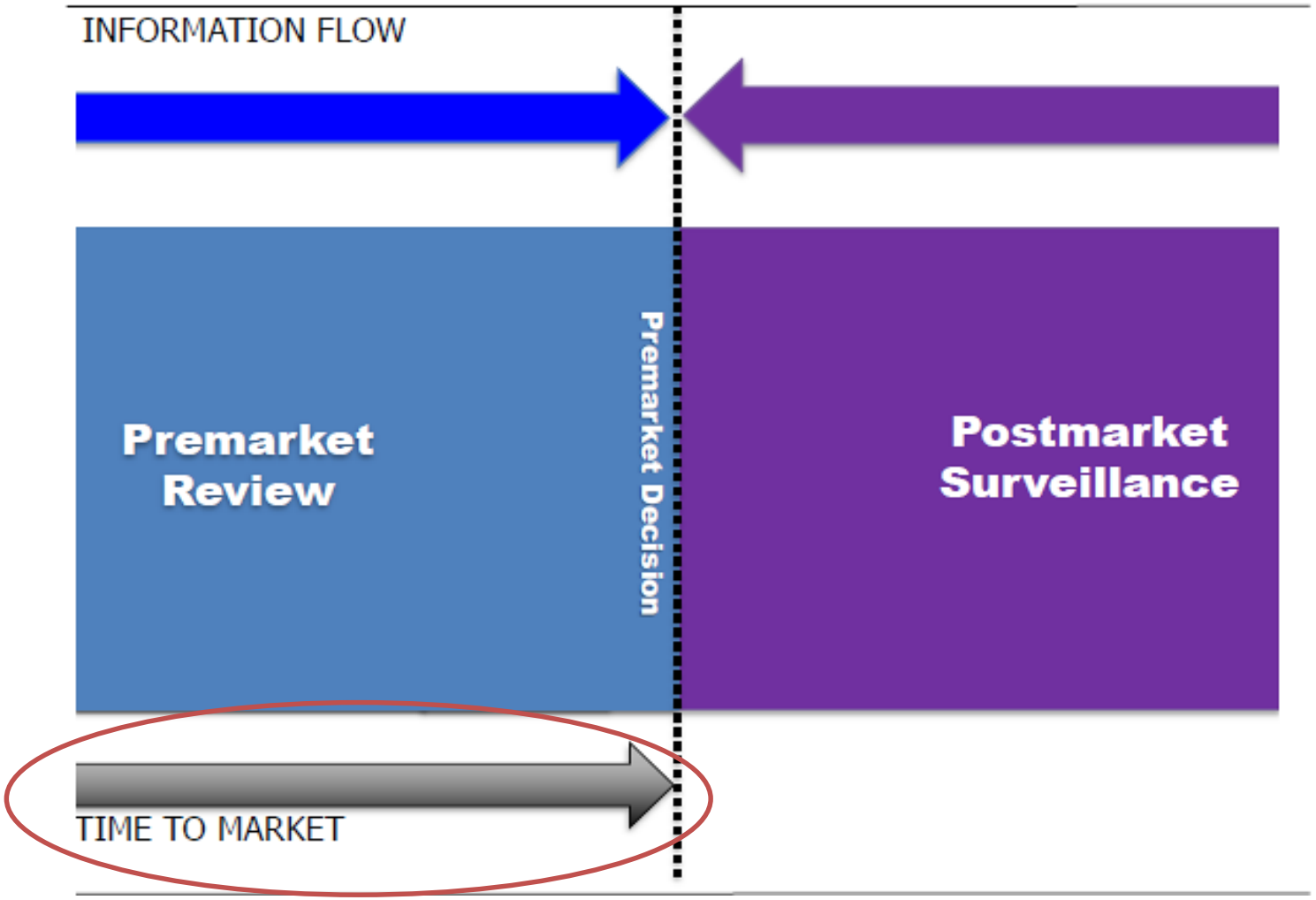
The U.S. regulatory standard for market approval protects patients by setting a high public health bar but imposes costs that make the U.S. marketplace less attractive for innovators thereby delaying patient access to important technologies

The solution is to reduce the time and cost of the total product life cycle…

device development, assessment, review, manufacturing, monitoring, and reimbursement – without compromising the reasonable assurance of safety and effectiveness standard

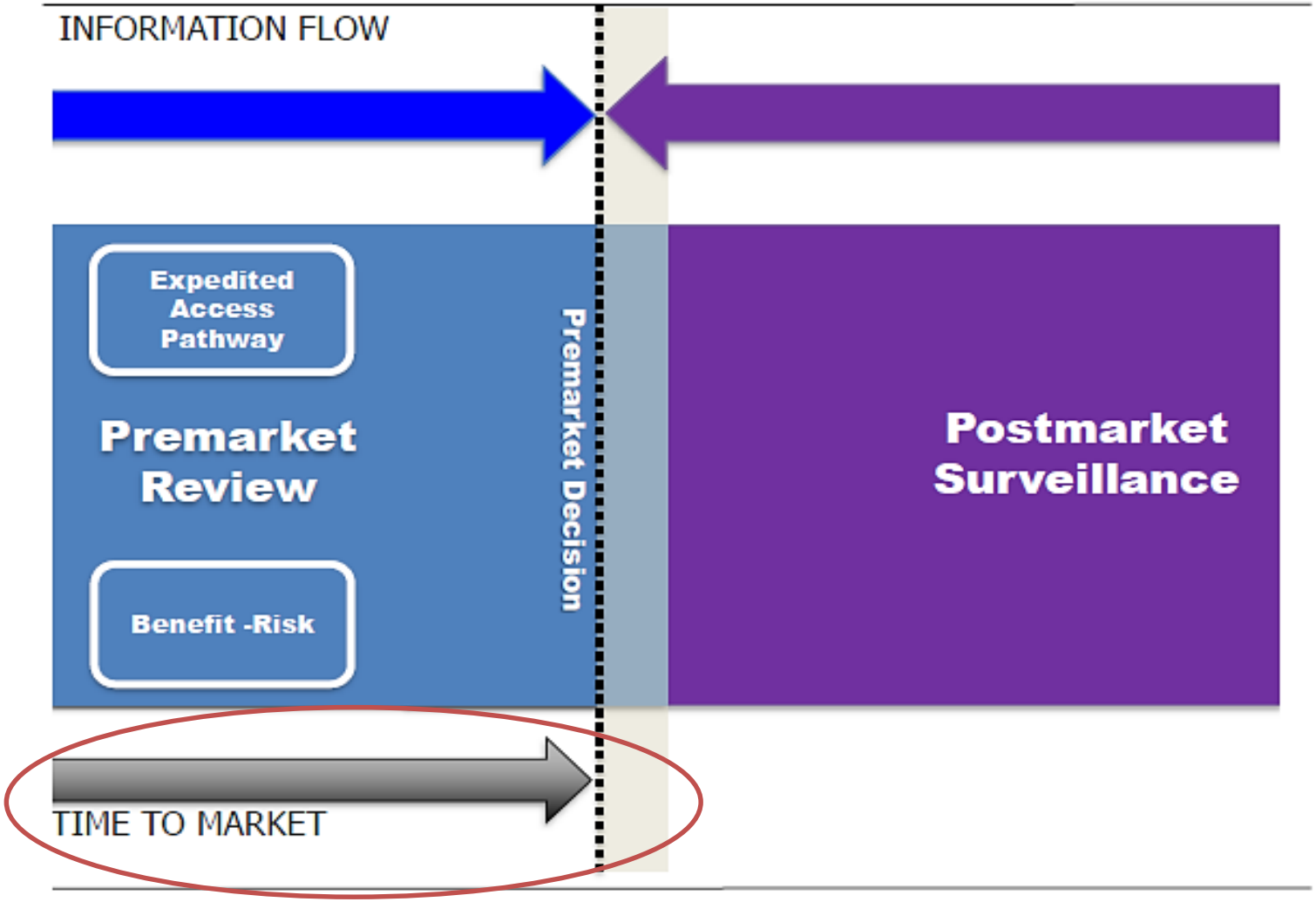
Learning Medical Device Ecosystem

Total Product Life Cycle (TPLC) Framework



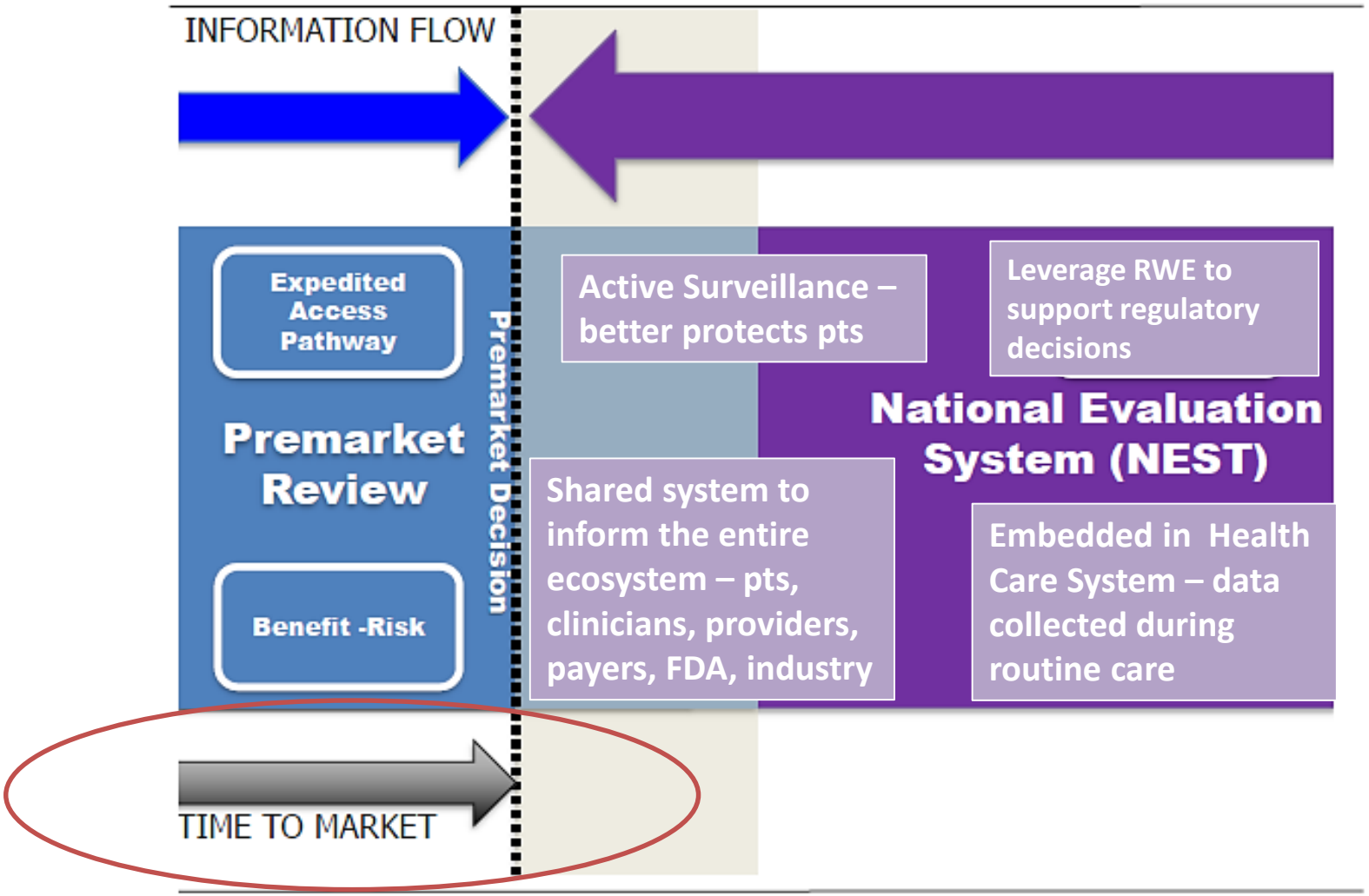
Learning Medical Device Ecosystem

Total Product Life Cycle (TPLC) Framework



Learning Medical Device Ecosystem

Total Product Life Cycle (TPLC) Framework



What are the Opportunities- Clinical Evidence Along the TPLC

Flexibility

- "Can't always get what you want…."
- But if we are flexible, we can "get what we need"

Innovation

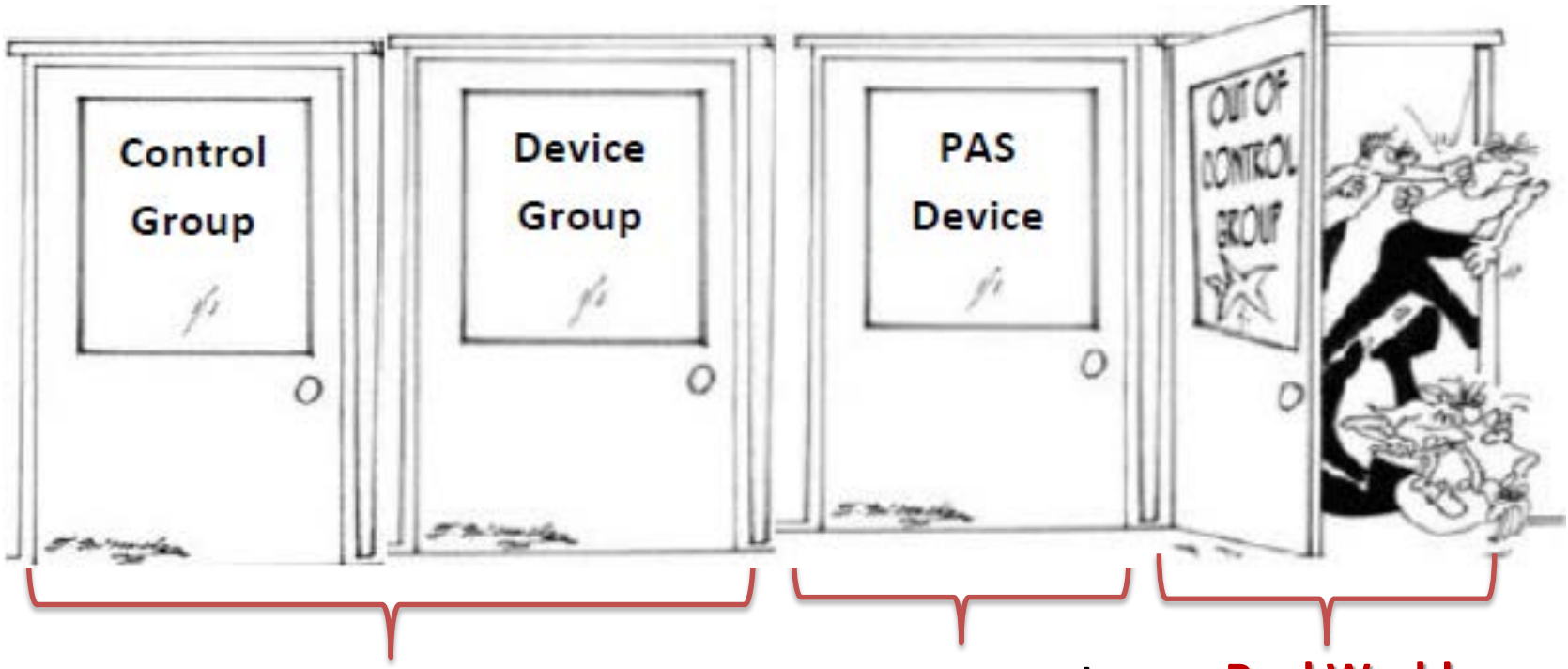
- Real World Evidence – significant human experience with a marketed device
- Clinical research incorporated into clinical practice and workflow

Collaborations

- NEST
- Industry groups
- Patient and clinician groups

Innovation – Evidence Gathering

RWD Sources
EHR's, Claims, Registries, Administrative Data, Patients, Mobile Devices



Trial Data

Post Approval Study Data

Real World Experience Data

Courtesy of Dr. Laschinger

Good Decisions Require High Quality RWE

'Fit for Purpose'

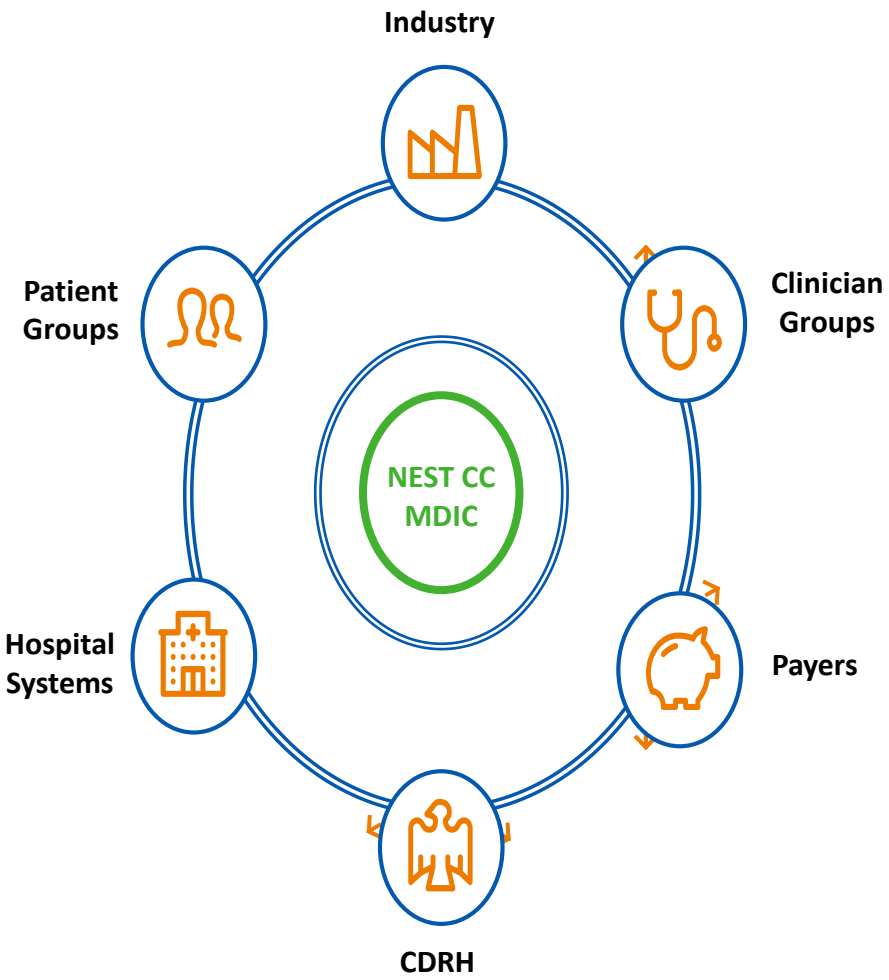
Factors that improve data quality - completeness, consistency , accurate, and contain all critical data elements needed to evaluate a medical device and its claims

Relevant & Reliable

Benefit

Risk

Collaborations – CDRH Priorities to Support RWE NEST



- Award grant to MDIC to support NEST development and implementation (3 M in 2016)
- MDUFA IV User Fee Agreement– Pilot projects funded to determine the usability of RWE for Expanded IFU, New clearances/approvals, improved device malfunction reporting
- Issue Draft Guidance to clarify how RWE may be used to support regulatory decisions (issued 7/27/16)
- Increase access to and use of RWE to support regulatory decisions
- Work with the Medical Device Ecosystem, e.g.; federal partners, health care system, manufacturers, payers and patients to build NEST

The Value Proposition for NEST

Patients/ Clinicians

- More timely access to safer, more effective devices
- Better information about the use of a given device in practice

Hospitals, Health Systems

- Improved quality
- Reliable assurances of safety
- Possibly, reduced reporting requirements

Payers

- Access to high-quality evidence on device performance in clinical practice

Medical Device Industry

- High-quality evidence at lower cost, in less time, to support premarket approval/clearance, payer coverage
- Meet or reduce the need for postmarket study and adverse event reporting requirements
- Potential for premarket-postmarket shift owing to strong assurances that postmarket RWE would be generated
- May obviate the need for FDA premarket review of some device modifications because more timely and informative routine data collection

Some Regulatory Uses for RWE

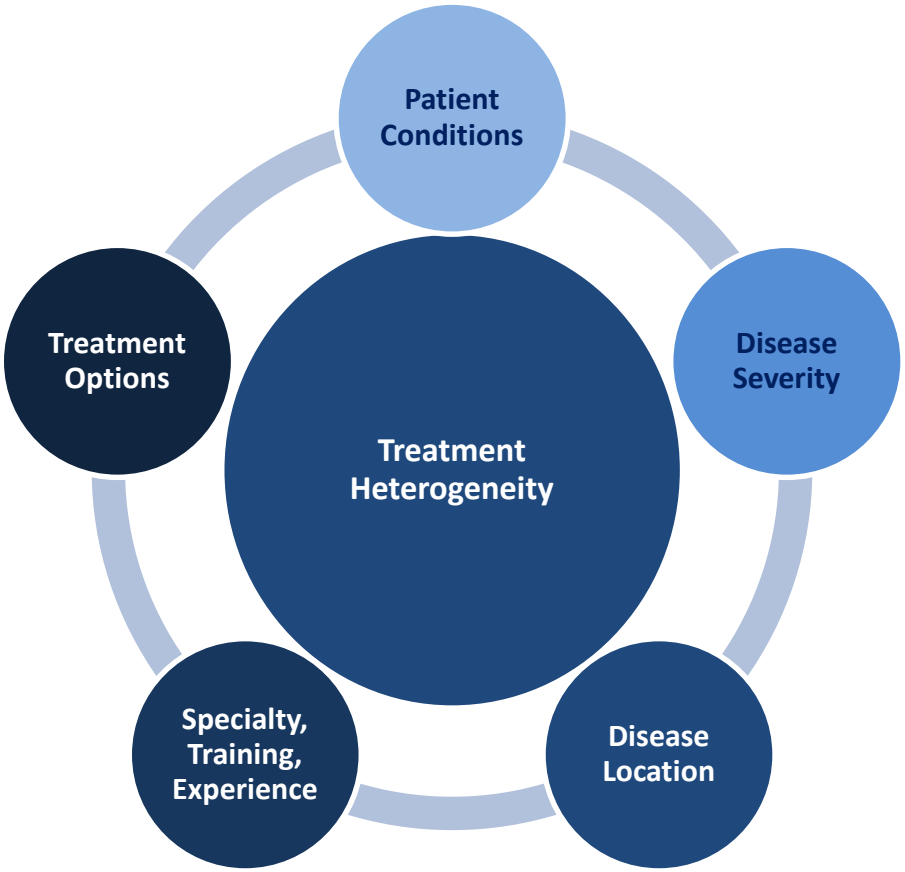
- Control arm for pivotal clinical study
- New indications for approved devices
- Studying new improvements to devices
- Replacing "formal" post approval study – Links other RWD sources
- Adverse event reporting – automated fashion
- Shifts to pre-postmarket balance

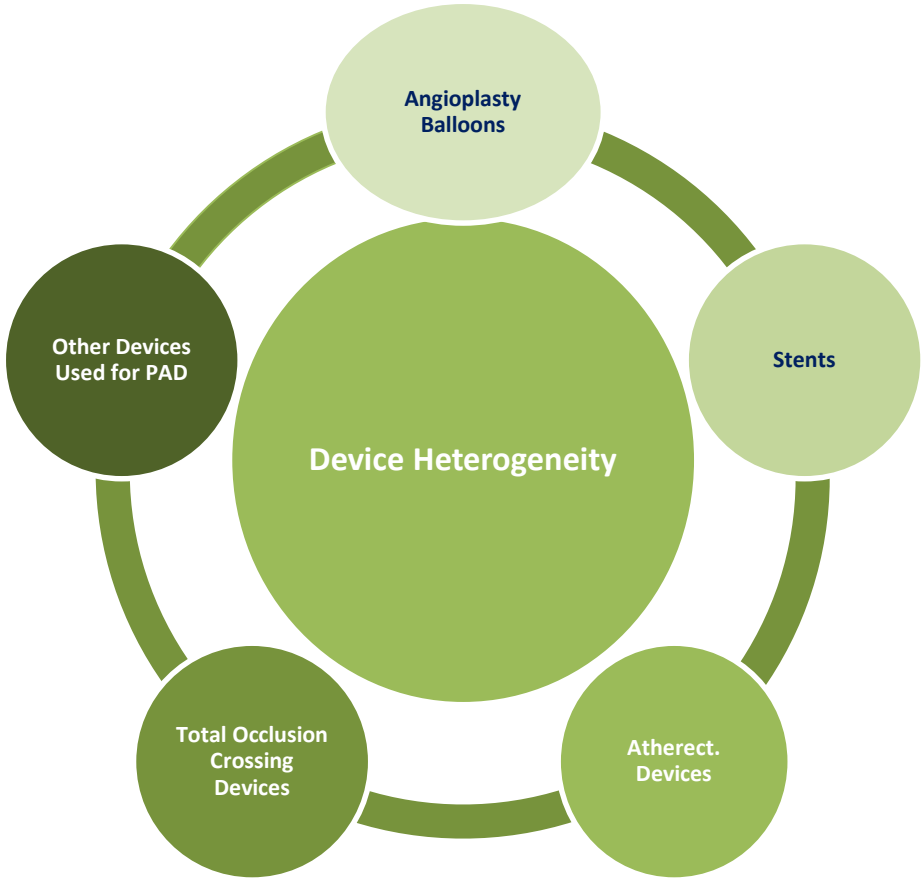
Some Non-Regulatory Uses for RWE

- Informing the community on optimal care
- Identifying needs and gaps
- Market analysis
- Assessing quality of care

Registry Assessment of Peripheral Interventional Devices

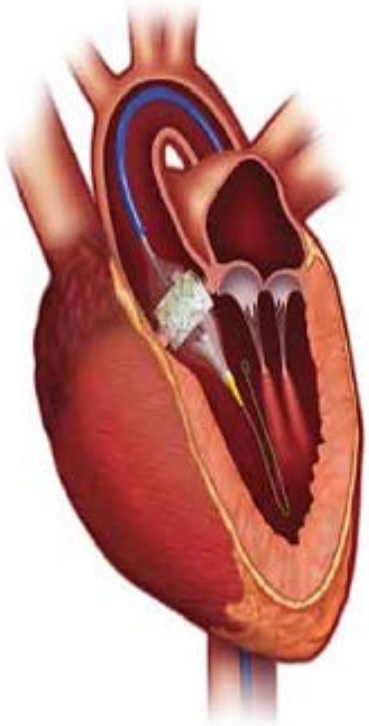
Infrainguinal Arterial Occlusive Disease

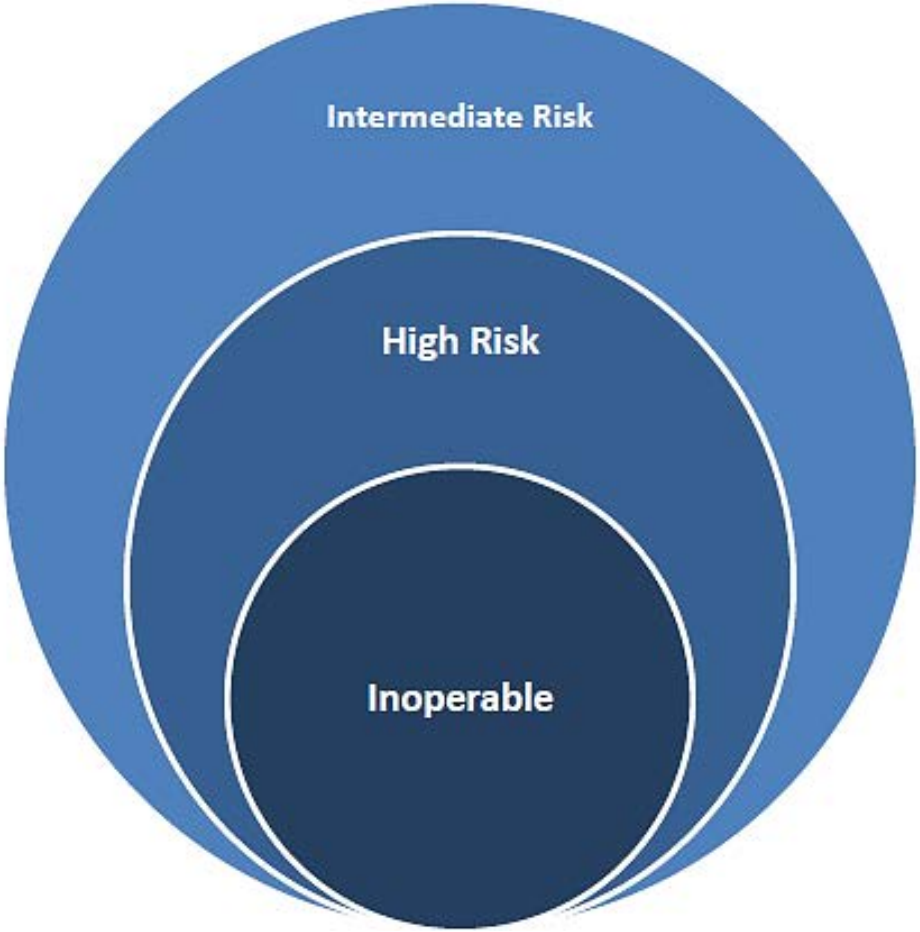




Transcatheter Heart Valves

Use of Registry Data for Post Market Surveillance and Label Expansion





"It is probably true that one of the commonest features of new ideas – certainly of practical new ideas – is their imperfection"

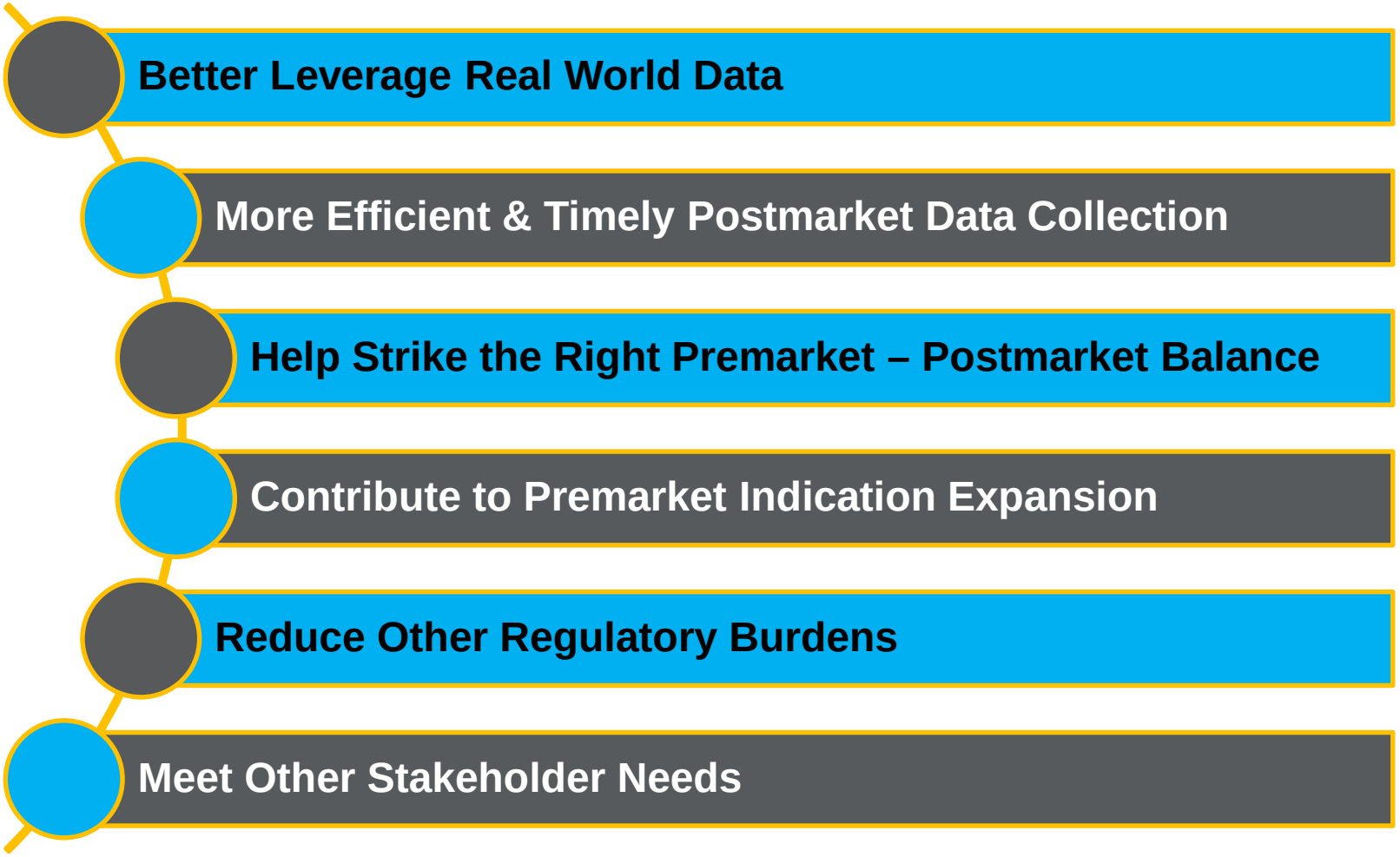
A.J. Youngson

"It is probably true that one of the commonest features of new ideas – certainly of practical new ideas – is their imperfection"

A.J. Youngson


FDA
U.S. FOOD & DRUG
ADMINISTRATION

Potential Benefits of a National Evaluation System for Medical Devices



Quality Systems Approach to RWE

Support NEST - Process

- Engage in broad collaboration and discussion
- MDUFA IV

Work with Stakeholders - People

- CDRH RWE “tactical team”
- Consider new and flexible approaches

Develop and clarify Policies

- RWE guidance – draft to final
- Outreach pre-submission meetings with FDA