How Can We Generate Robust Real-World Evidence from Real-World Data?

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Why RWE Medical Devices and Technologies?

- The safety and effectiveness of medical devices (implants or non-implantable technologies) Intertwined with interventional procedures affects ¼ of U.S. GDP
- Aggregate Profits of device companies are reported to be comparable to drug companies
- There are over a 1.5M device adverse events reported to US regulator and the number of reports now exceed those for drugs

Global MDEpiNet

Existing MDEpiNet Chapter

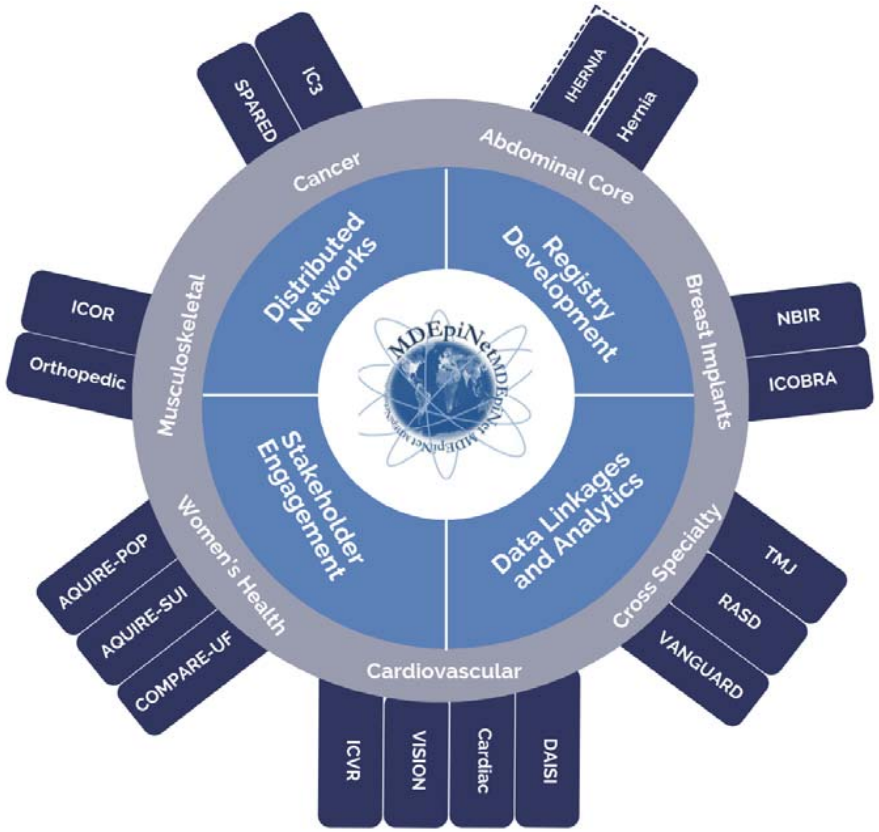
Future MDEpiNet Chapter

Academic Centers

Data Sources

Real-World Evidence: building Coordinated Registry Networks

MDEpiNet's Data & Infrastructure Platform:



- Facilitate and/or leverage national investments in registries, administrative data, EHRs and other relevant data systems and their linkages (dual purposing)
- Minimize the costs & resource requirements for research and surveillance
- Provide timely information on performance of devices (e.g. active surveillance) for patients, physicians, regulators & other stakeholders

RWE: Registries and Coordinated RWE Networks

"Organized system that continuously and consistently collects relevant data in conjunction with routine clinical care, evaluates meaningful outcomes and comprehensively covers the population defined by exposure to particular device(s) at a reasonable generalizable scale (e.g., international, national, regional, and health system) with a primary aim to improve the quality of patient care." - International Medical Device Regulatory Forum

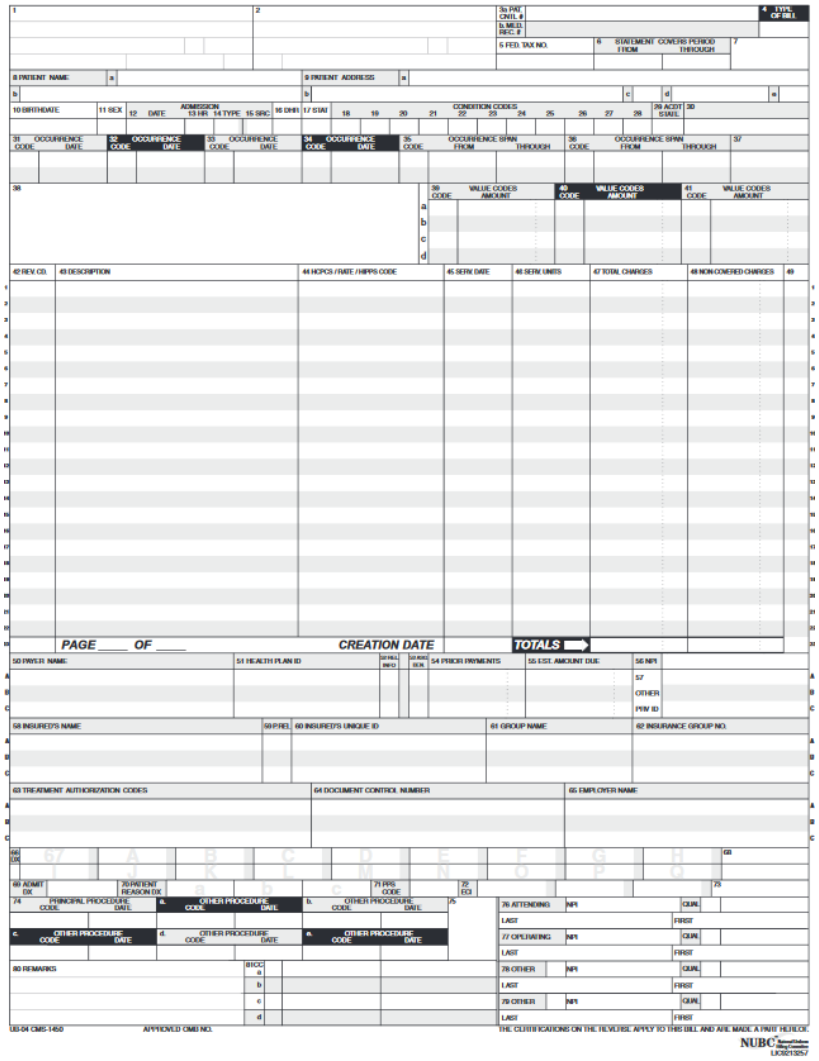
1. Device data: the registry contains sufficient information to uniquely identify the device. Ideally, the unique device identifier would be included, but when unavailable, the registry would include a combination of identifiers (catalogue number, manufacturer, description)
2. Quality improvement system: is part of a healthcare delivery improvement system or evolving into one as device technologies are diffused into practice and need continuing evaluation (including outlier identification).
3. Beneficial change: has established mechanisms to bring about beneficial change in healthcare delivery through stakeholder participation, ownership, and intergration into the relevant healthcare systems.
4. Efficiency: the registry is embedded in the healthcare delivery system so that data collection occurs as part of care delivery (i.e., not overly burdensome, not highly complicated, not overly costly) and integrated with workflow of clinical teams.
5. Actionable data: the registry provides actionable information in a relevant and timely manner to decision makers.
6. Transparency: the governance structure, data access, and analytical processes of the registry are transparent.
7. Linkability: information in the registry can be linked with other data sources for enhancement, including adequate follow-up achievement.
8. Total device lifecycle: the registry can serve as infrastructure for seamless integration of evidence throughout the device lifecycle.

- International Medical Device Regulatory Forum's (IMDRF) vision and definition of Registry is comprehensive
- RWE Network aims to achieve the IMDRF vision with registries and other RWE data sources to address limitations of any single registry

Qualifiers to define the impact, value and sustainability of the medical device registry:

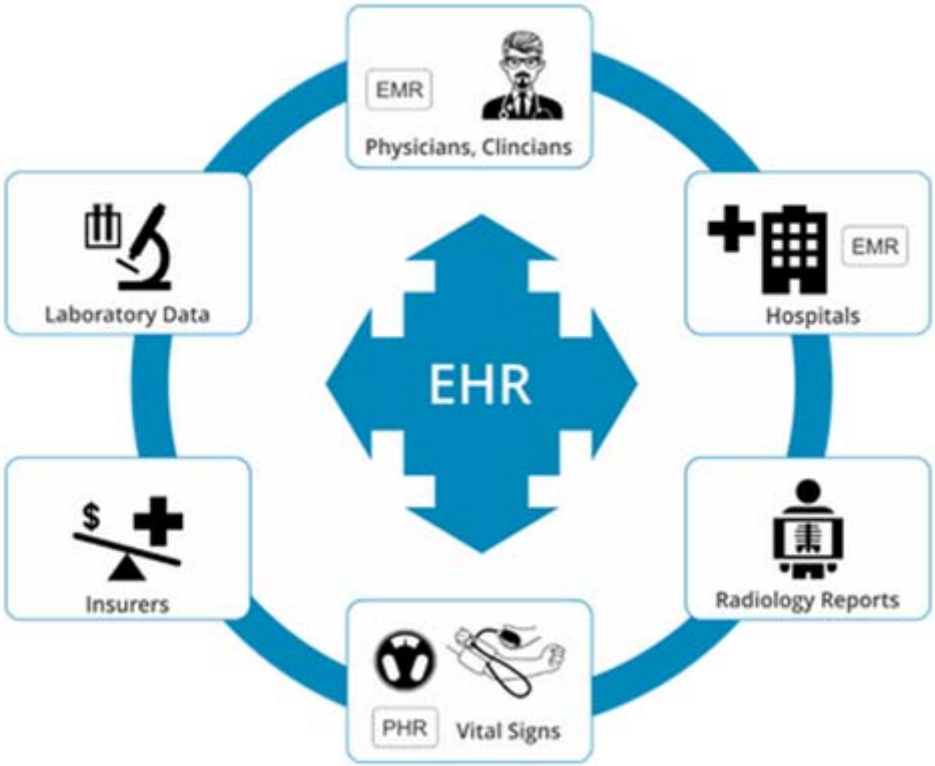
1. DEVICE: Has sufficient device information (unique device identification)
2. QUALITY SYSTEM: Is part of contin[illegible]d (including outlier identification).
3. BENEFICIAL CHANGE: Has establi[illegible] delivery through stakeholder particip
4. EFFICIENCY: Data collection is em[illegible] work flow of clinical teams. (Not ov
5. ACTIONABLE DATA: Provides acti[illegible] makers.
6. TRANPARENCY: the governance st
7. LINKABILITY: Can be linked with ot[illegible] achievement.
8. TOTAL DEVICE LIFE-CYCLE: Can [illegible] throughout the device life cycle.

RWE: Claims Data



- Data collected for billing purposes
- Trained coders enter the data on a standardized forms Nationally
- Diagnoses and Procedures are recorded using standardized, CPT, ICD-9 and ICD-10 codes (CMS and private payer guidance)
- Limited device identification via codes
- Good follow-up data in fee-for-service Medicare and some State data efforts

RWE: Electronic Health Records



- **Patient Management Component**
  - Enable patient registration, admission, and discharge
- **Clinical Component**
  - Electronic documentation, nursing component, and computerized physician order entry used for documentation (e.g. op-notes)
- **Laboratory Component**
  - Capture lab data and integration with billing
- **Radiology Information System**
  - Managing patient work flow, ordering, images.
- **Billing System**
  - Claims are generated & submitted to insurance carriers

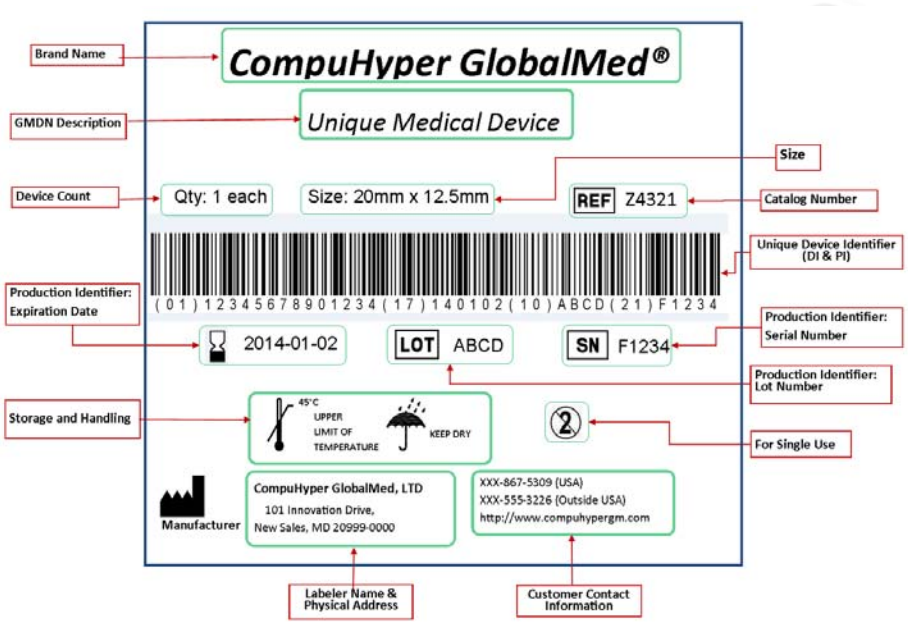
RWE: Patient-Facing Mobile Apps

- Engaging patients and developing patient-facing mobile apps
  - Helps address patient preferences
  - Develop partnership
  - Obtain further efficiencies

Key Issue: Device Information

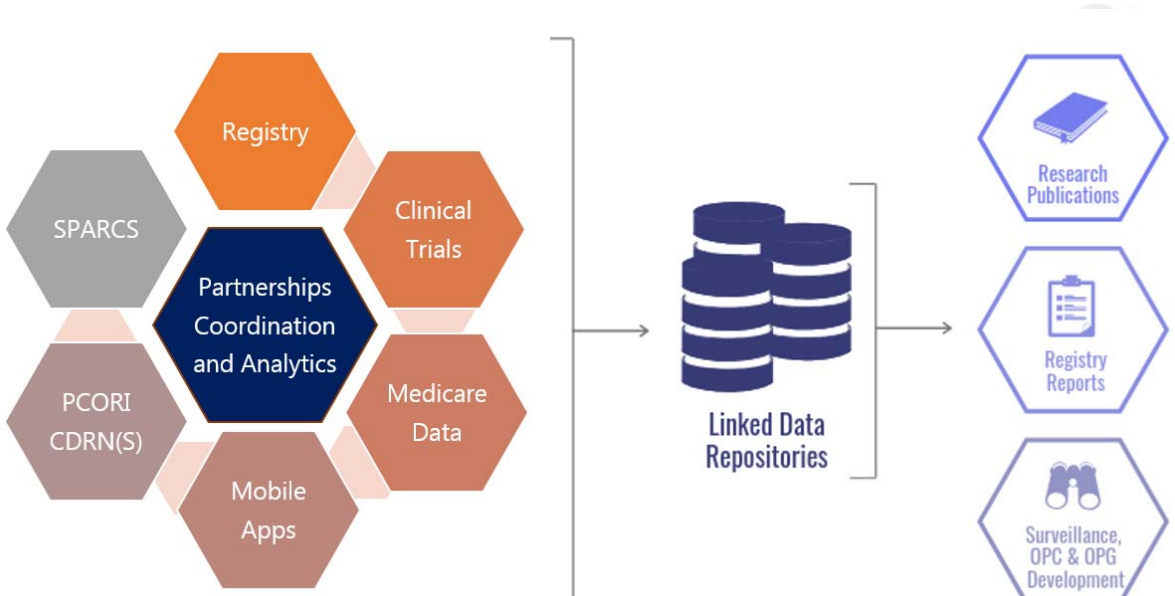
Unique device identification (UDI):

- The FDA rules require manufacturers to assign unique identifiers to their marketed devices
- The precise identification of medical devices and their attributes is essential to evaluation
- The FDA's AccessGUDID, a public portal of the Global Unique Device Identification Database (GUDID), serves this purpose



Example: Patient with Abdominal Aortic Aneurism Undergoing Surgery

- Patient: How can I learn if my hospital/surgeons are excellent and using good devices ensuring that my implant will last?
- Manufacturer: How can we efficiently obtain data for our devices and conduct research to obtain indications and labeling changes?
- Regulator: How can we get safety signals, conduct confirmatory studies and understand off-label use & impact on outcomes?



MDEpiNet International Chapters/Centers

- Big-Data Centre in Sydney, Australia
- National Center for Global Health and Medicine in Tokyo, Japan
- UKE, GermanVasc, Hamburg, Germany
- Charters at planning stage in
  - Canada
  - Netherlands
  - The United Kingdom

CENTRE FOR BIG DATA RESEARCH IN HEALTH

NCGM

MDEpiNet Creating the Discipline of Device Research: Please Send Your Best (And Good) Studies!

BMJ Surgery, Interventions, & Health Technologies

Recent progress to shorten premarket evaluation and improve patient access to medical devices by the Pharmaceuticals and Medical Devices Agency of Japan

Nobuhiro Handa, Shinichi Takae, Kensuke Ishii

To cite: Handa N, Takae S, Ishii K. Recent progress to shorten premarket evaluation and improve patient access to medical devices by the Pharmaceuticals and Medical Devices Agency of Japan. *BMJ Surg Interv Health Technologies* 2019;**1**:e000014. doi:10.1136/bmjsit-2019-000014

Received 03 June 2019
Accepted 24 June 2019

The Pharmaceuticals and Medical Devices Agency (PMDA) of Japan advances the fundamental principle that the safety and effectiveness of all medical products must be reasonably assured before they are approved. However, strict premarket evaluation often delays patients from gaining access to some innovative products. To shorten the premarket evaluation, several initiatives and pieces of legislation, including forerunner designation (the Sakigake fast-track development and review system)[1] and "Conditional

has the potential to facilitate rebalancing between premarket and postmarket conditions.[3] The registry model is helpful because the development of medical devices is known to follow a total product life cycle that registries can address.[4] The initiatives of the Clinical Innovation Network led by the Ministry of Health, Labour and Welfare (MHLW) and the PMDA, in collaboration with academia and device manufacturers, facilitate registry development, which is useful for regulatory

ありがとうございます

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