Introduction of the Japanese guidance "To ensure reliability when using registry data for approval applications”

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HBD East Think Tank 2021 Day2: Jan.20-21, 2022
7) Sustainable Development Goals in Real-world Data Collection

Agenda

1. Examples of real-world data (RWD) utilization in regulatory decision making of medical devices

2. PMDA's actions for effective utilization of RWD

3. What are the SDGs in RWD collection?

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RWD Utilization to Evaluate Clinical Outcomes of Medical Devices

Source of RWD

- **Registry**
  - National / International
  - Academic / Sponsor
  - Procedure / Medical Device
- **Administrative Claim**
- **Medical Record**
- **Mobile technology**

etc…

Usage of RWE

- **Clinical Evidence of New Device**
  - Primary source / Complementary data
- **Clinical Evidence of Indication Expansion**
  - Primary source / Complementary data
- **Postmarket Clinical Evidence**
  - Post-marketing Surveillance
  - Post-approval Study(Conditional Approval)
  - Revision of the package insert
- **Control Arm of Clinical Trial**
- **Objective Performance Goal**

etc…

Examples of RWD Utilization in Medical Device Regulatory Decisions in Japan

Source of RWD

- **Registry**
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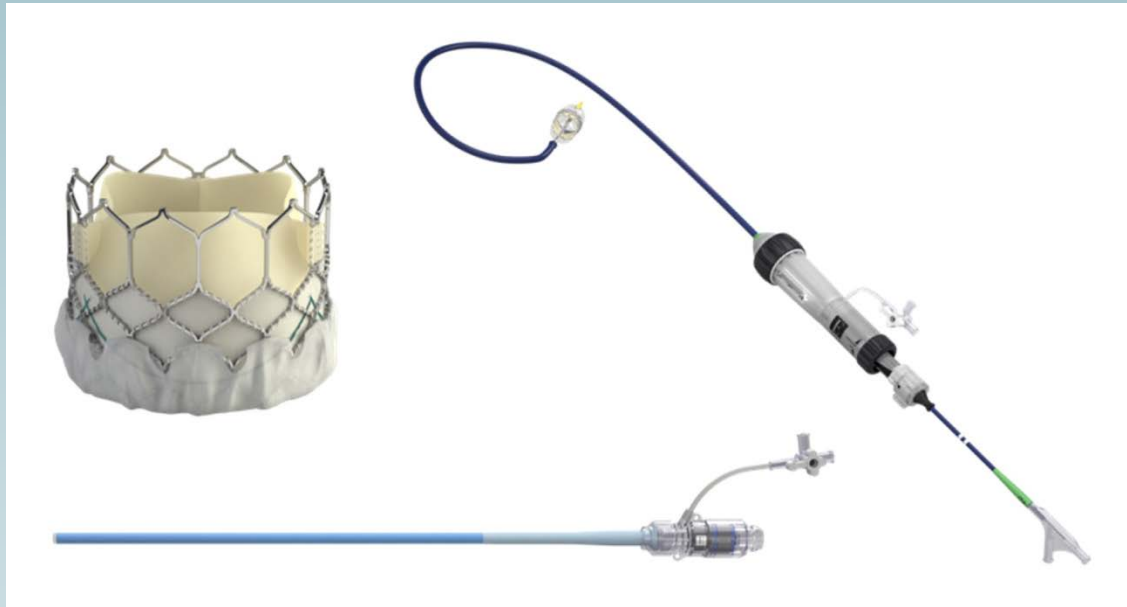
RWD Utilization Case Examples (Complement of Clinical Trial)

Edwards SAPIEN 3

（Edwards Lifesciences Limited, Approved in 2021）



https://www.pmda.go.jp/files/000243046.pdf

Description

- Prosthetic heart valve system used for transcatheter aortic valve implantation
- Clinical evidence for indication expansion: patients at low-risk for surgical aortic valve replacement
  - ✓ Global clinical trial（PARTNER 3 trial）
  - ✓ Japanese cohort（PARTNER 3 trial）
  - ✓ National Registry (Current AS Registry)
  - ✓ International Registry (TVT Registry)

Registry Data is used for complement of Clinical Trial Data

- **Current AS Registry** is utilized for explanation of extrapolation
- **TVT Registry** is utilized for safety explanation of subclavian/axillary access

Review report (Japanese only) ;
https://www.pmda.go.jp/medical_devices/2021/M20210416001/170492000_22800BZX00094_A100_1.pdf

RWD Utilization Case Examples (Post-marketing Surveillance)

New Device

Development → Market Approval → Post-marketing

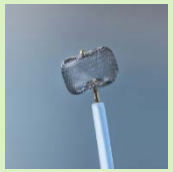
Post-marketing Evaluation

Device-specific registry

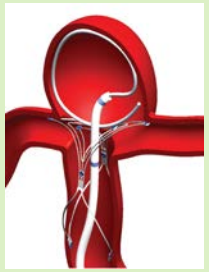
JSNET 特定非営利活動法人 日本脳神経血管内治療学会

PMS application using Registry

Woven EndoBridge Device
(Terumo Corporation) July 2020-




PulseRider
(Johnson & Johnson K.K.)
March 2021-


Database Holder

data provision

contract


Industry

PMS application




Regulator

data entry


Institutions

Photos are From the Web Page of Terumo.co.jp, PMDA and Am J Neuroradiol Jan 2016, 37:130 –35

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1. Examples of real-world data (RWD) utilization in regulatory decision making of medical devices

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What are the principles of reliability of RWD Used in Medical Device Regulatory Decisions?

Source of RWD

- **Registry**
  - National / International
  - Academic / Sponsor
  - Procedure / Medical Device
- Administrative Claim
- [illegible]

How to secure the reliability of RWD?

etc…

Usage of RWE

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Development of Guidance
-Utilization and Reliability of Registry Data-

1. Basic principles on Utilization of Registry for Applications

PSEHB/PED Notification No.0323-1, PSEHB/MDED Notification No.0323-1, March 23, 2021

- **It shows the principles for applicants utilizing registry data to explain the efficacy and/or safety in documents of clinical data for the applications.**

2. Points to Consider for Ensuring the Reliability in Utilization of Registry Data for Applications

PSEHB/PED Notification No.0323-2, PSEHB/MDED Notification No.0323-2, March 23, 2021

- **It shows the points to consider for applicants' ensuring the reliability in utilization of data from the registries as a clinical data in the data/documents for the applications.**

https://www.pmda.go.jp/english/rs-sb-std/rs/0023.html

(English translation is available.)

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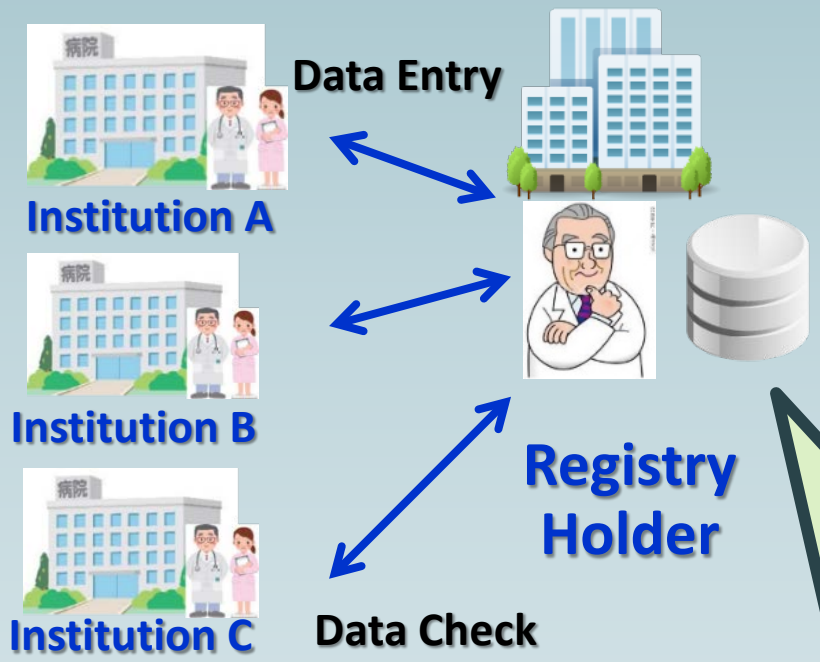
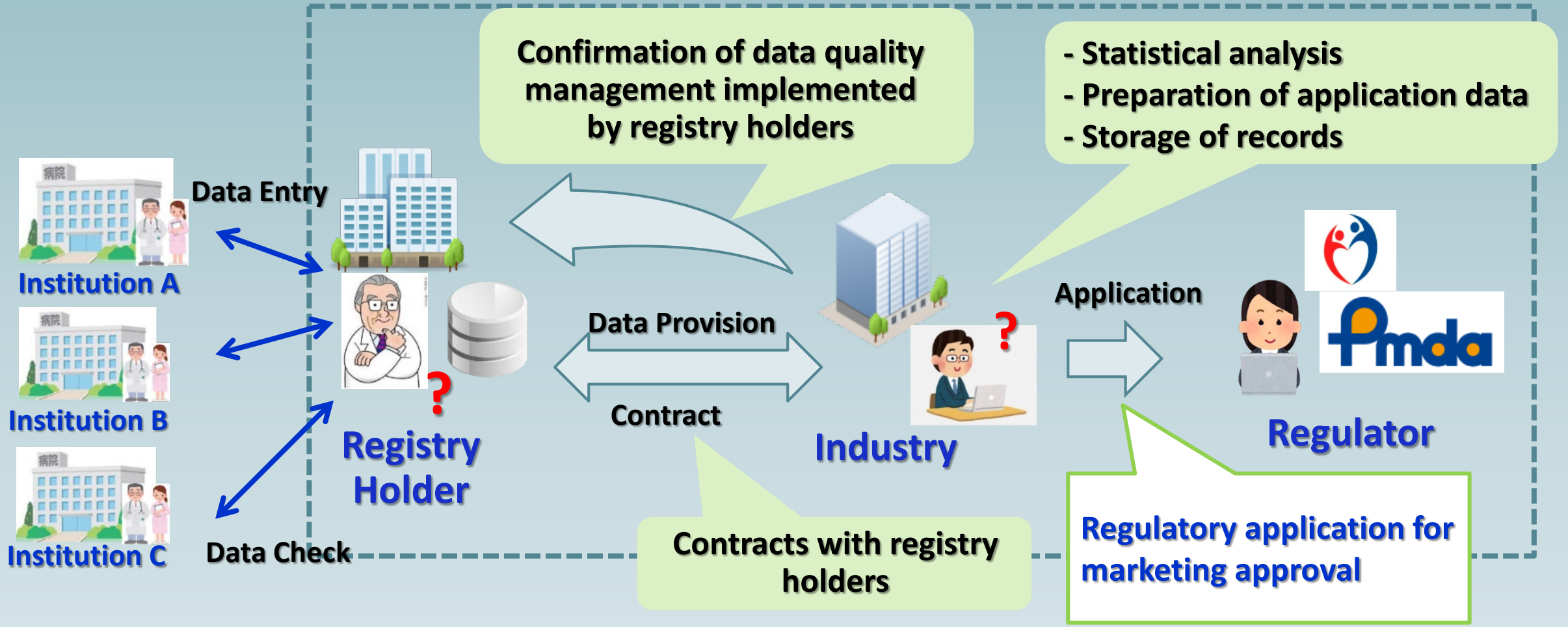
Points to consider for the registry utilized as application data/documents for marketing approval



1. **Governance by registry holders**
   - Establishment of operation and management system
   - Policy on securing transparency
   - Policy on access to registry data
2. **Computerized system**
   - Quality management of the computerized system
   - Security of computerized system
3. **Quality Management of registry data**
   - Data collection methods
   - Handling of collected registry data
   - Monitoring
   - Quality management for data migration from hospital information system, etc. to a computerized system
4. **Quality Assurance for Registry**
5. **Data Extraction and Datasets Preparation**

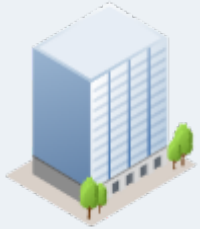
Concepts of ensuring the reliability in utilization of registry data as application data/documents for marketing approval



PMDA has launched a new consultation system about reliability of the registry data.

Two types of PMDA Registry Consultations

(since 2019)

	Consultation Type	Consulter	Objective
1	**Development of Registry Data**	**Registry holder (mainly academic society)** 	- General considerations of development strategies for registry - Methods of ensuring the data reliability of registry for marketing approval/PMS applications 
2	**Quality of Registry Data**	**Industry** 	- Check and specify the status of data reliability of registry for marketing approval/PMS applications corresponding to the individual new Device 

Implementation of PMDA Registry Consultation Meetings

Consultation Type		FY 2019		FY 2020	
		Drug	Device	Drug	Device
1	Development of Registry Data	2	1	1	3
2	Quality of Registry Data	0	0	2	1

https://www.pmda.go.jp/files/000241310.pdf

Industries (Applicants) and registry holders are encouraged to use the PMDA consultations to discuss any basic matters in an early stage, taking into account the purpose of utilizing the registry.

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What are the SDGs in RWD collection?

Institutions

- ✓ Keeping staff for entering data
- ✓ Keeping doctor's motivation for establishing RWE

Data Holders

- ✓ Appropriate endpoints and evaluation period
- ✓ Securing human resource and robust system to keep data quality

To collect valuable RWD for US/Japan

- ✓ Endpoints
- ✓ Evaluation period
- ✓ Reliability of data
- ✓ Cost-saving

Industries

- ✓ Making efficient use of RWD
- ✓ Sponsorship

Government

- ✓ Building infrastructure for sustainable RWD collection
- ✓ Accompanied consultation

Conclusion

1. The collaboration between stakeholders is important to accelerate the utilization of RWD.

2. We need to continue discussing for more effective RWD utilization in regulatory decision making .

Thank You

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