第31回日本心血管インターベンション治療学会 学術集会
CVIT2023

8月4日（金）16:40-17:30
Town Hall Meeting　持続可能な市販後RWD基盤構築に向けて

RWD collection to enable rebalancing of the development of medical devices for children and rare diseases

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COI Disclosure

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The authors have no financial conflicts of interest to disclose concerning the presentation.

Medical devices(MDs) for children and orphan diseases

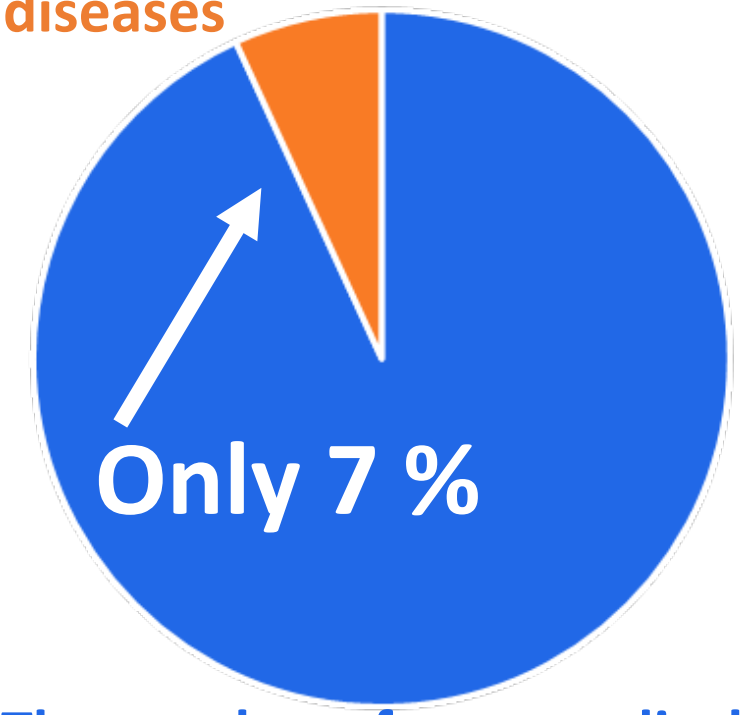
- Pediatric medical devices(MDs)
- MDs for orphan diseases

The burdens for development ;

- ***The market is too small.***
- ***Specific conditions are required.***
- ***The number of patients is limited.***

Only 0～4 pediatric MDs and MDs for orphan diseases were approved per year[1,2].

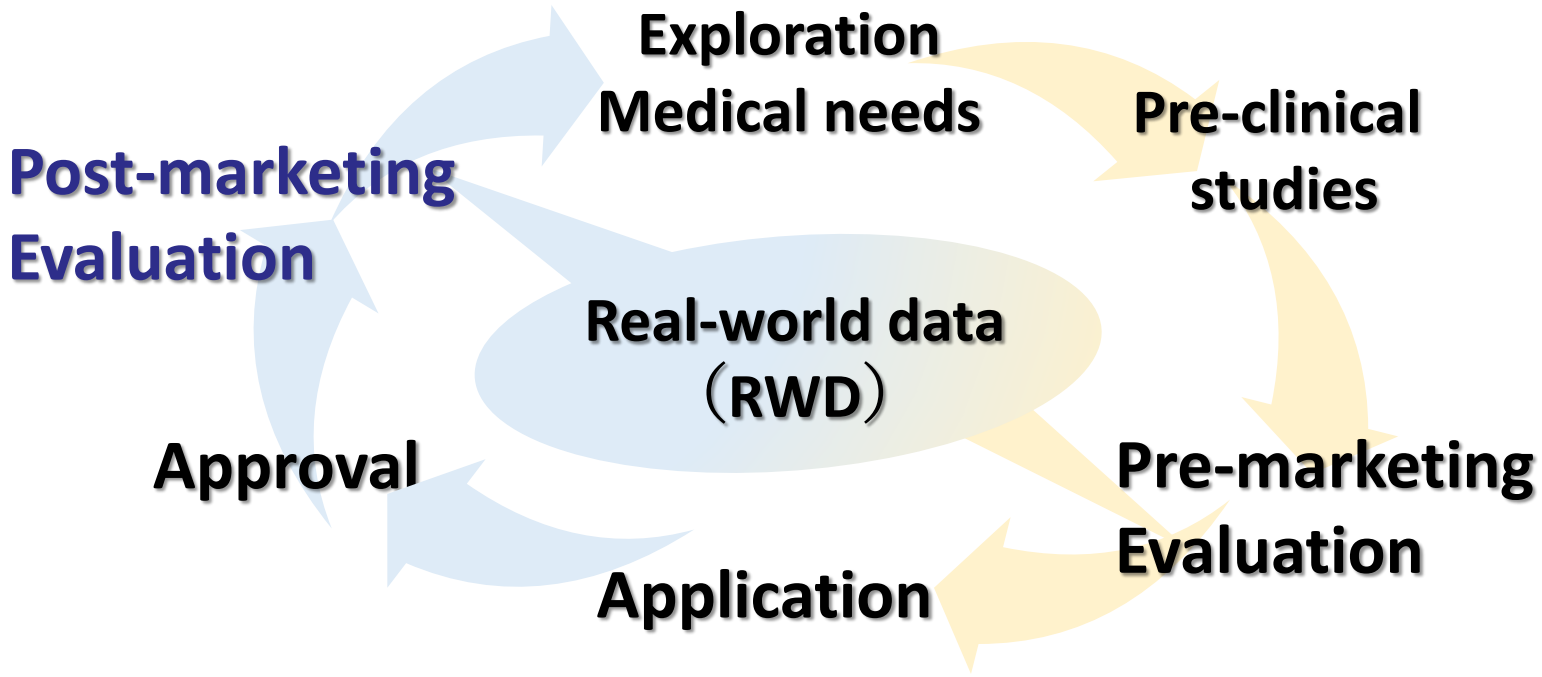
The number of pediatric MDs and MDs for orphan diseases from 2008 to 2019



The number of new medical devices from 2008 to 2019

[1] Takahashi S, et al., *J Artif Organs*. **2021,** 24, 90-101.
[2]https://www.nibiohn.go.jp/nibio/part/promote/files/ph_orphanlist_medicaldevice_JP.pdf

Possibility of promoting the development of pediatric and orphan MDs by utilizing RWD



Utilization of RWD through pre- and post-marketing phase is often effective for development of medical devices required repeated improvements and medical devices for orphan disease.

Administrative notification No.1117-1, November 17, 2017

Trends of RWD utilization in Japan

2016 Clinical Innovation Network (CIN)* was constructed as a infrastructure for registry utilization

*The Japanese framework for accelerating use of RWE in development of medical products

2018 MHLW revised the Ministerial Ordinance on GPSP* to use real-world data in PMS, complying with the data reliability

*Good Post-marketing Study Practice

2019 A new consultation category about reliability of the registry data for the application to PMDA was launched.

2021 Guidance for the utilization and reliability of registry was published.[1]

- Basic principles on Utilization of Registry for Applications

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

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

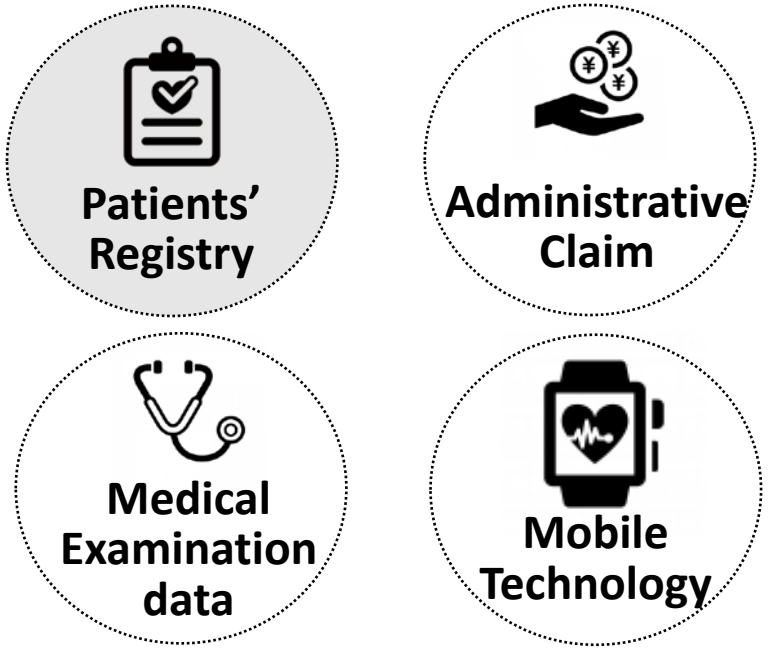
PSEHB/PED Notification No.0323-2, PSEHB/MDED Notification No.0323-2, March 23, 2021

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

Guidance for the utilization and reliability of registry

Source of RWD

- National / International
- Academic / Sponsor
- Procedure / Medical Device



Purpose of Utilization in regulatory use

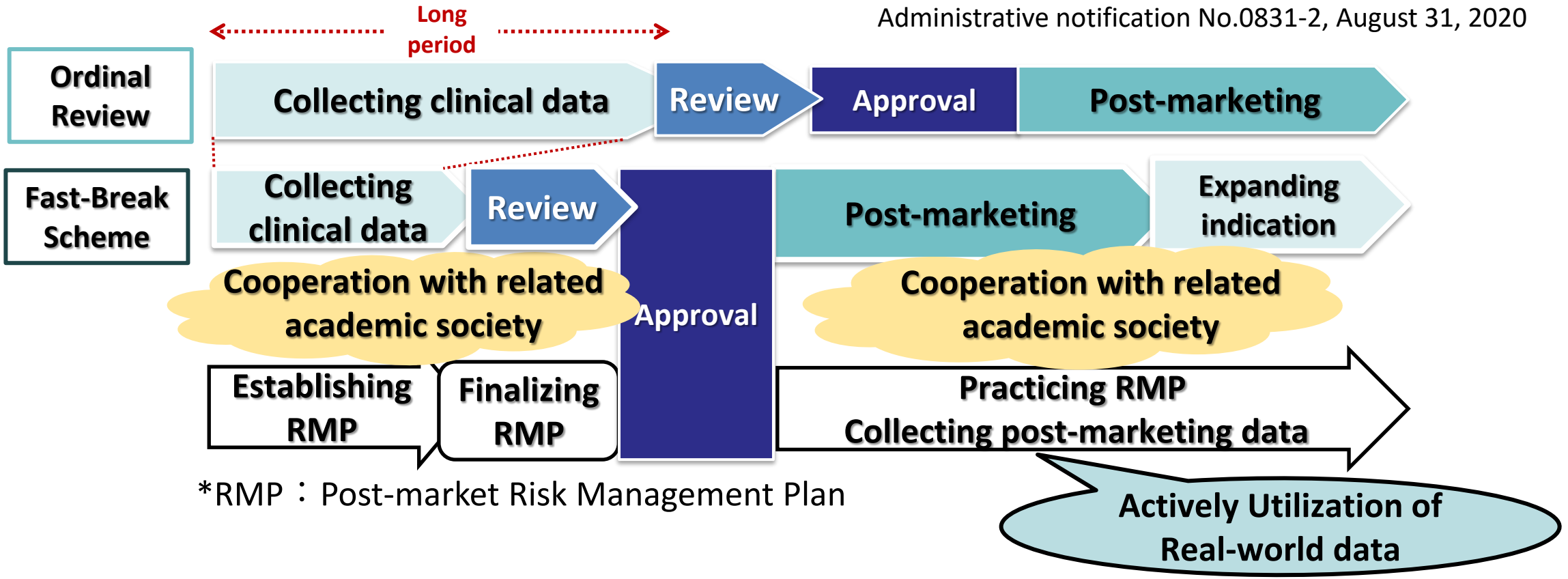
1. **External control of clinical trials**
2. **Primary data or complement of clinical trials**
3. **Efficacy and/or safety evaluation of conditionally approved items**
4. **Post-marketing surveillance for safety measures**

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

Utilizing RWD in post-market evaluation of conditionally approved items

Accelerate approval of MDs of high clinical needs by balancing the pre- and post-market requirements, based on the lifecycle management of the MDs.

Administrative notification No.0831-2, August 31, 2020



*RMP：Post-market Risk Management Plan

Utilizing RWD in post-market surveillance

Brand-new Drug/Device → Development → Approval → Post-marketing → Post-marketing Evaluation

Institutions

Data entry

Registry holder

Data provision

Contract

Industry

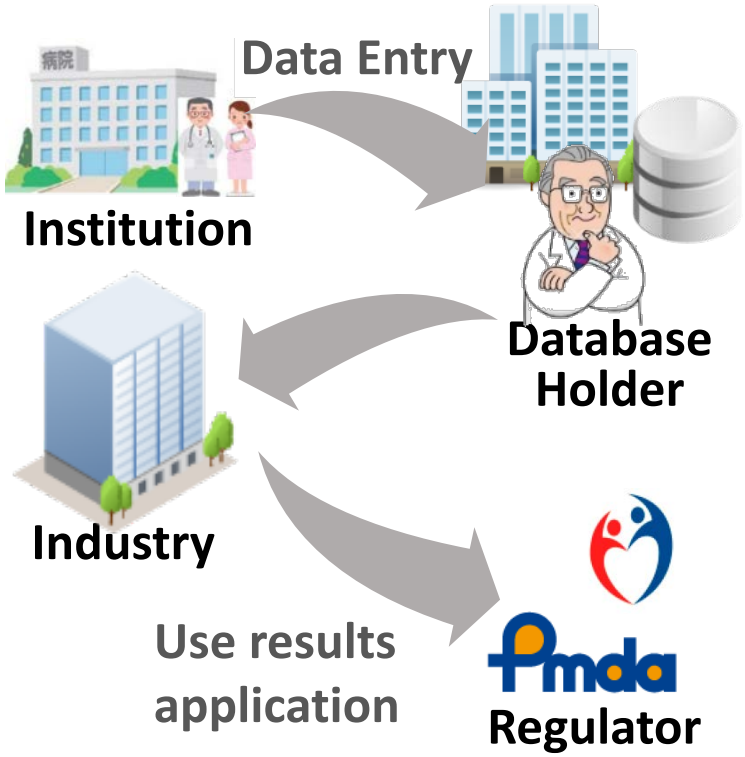
PMS application using the database

Regulator

Please utilize general consultation and face-to-face consultations at an early stage for discussing the sufficiency of evaluation items.

Points to Consider for Ensuring the Reliability of post-marketing data base study for MDs

It shows the points to consider for applicants' ensuring the reliability in utilization of data in the data/documents for Use-results evaluation od MDs.



Applicants shall be responsible for the following duties as usual;

- Preparation of application data/documents for Use-results evaluation of medical devices

- Ensuring reliability of application data/documents

1. **Selection of Medical information Database（MID） and contract with DB holders**
2. **Quality management of medical information collected from the information sources**
3. **Analysis utilizing medical information extracted from MID**
4. **Storage of records related to preparation of application data/documents for Use-results evaluation of medical device.**

PSEHB/PED Notification No.1219-4, December 19, 2018

Consultations for development and utilization of registry

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

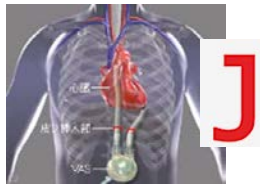
Number of cases[1] (FY2019~FY2022)

	Consultation Category	Consulter	Objective	
1	**Development of Registry Data**	**Registry holder (mainly academic society)** 	- General consideration of development strategies for registry - Methods of ensuring the data reliability of registry for marketing approval/PMS applications	3 4
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	

[1]https://www.pmda.go.jp/files/000263261.pdf

Examples of Post-Marketing Surveillance Application using RWD

2010. 6.～
J-MACS



J-MACS
Japanese registry for Mechanically Assisted Circulatory Support

Implantable Ventricular Assist System
Implantable Ventricular Assist System EVAHEART
DuraHeart Left Ventricular Assist System
Jarvik 2000 Implantable Ventricular Assist Device
HeartMate II Left Ventricular Assist System
HeartMate 3 Left Ventricular Assist System

2018
GPSP has revised
to use RWD

2013. 1.～
JCIC-Registry

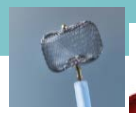
JCIC REGISTRY

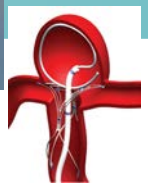
Prosthetic Material for Vessel Embolization
AMPLATZER Piccolo Occluder

2014. 7.～
Japan TVT Registry

Japan Transcatheter Valve Therapies
JTVT

Transcatheter Aortic Valve Implantation (TAVI)
Edwards SAPIEN XT, Edwards SAPIEN 3
CoreValve, CoreValve Evolut R, CoreValve Evolut PRO





2020. 7.～
JSNET

JSNET

Prosthetic Material for Vessel Embolization
Woven EndoBridge Device (Terumo)
PulseRider (J＆J)
Pipeline Flex Flow Diverter System（Medtronic）
Surpass Streamline (Stryker)

Key consideration

Efficient Development

- An early access to new devices that guarantees safety
- *Consistent efficacy and safety clinical evaluation system for medical devices* ***pre and post marketing***
  ⇒Utilization of RWD
- ***Development support*** that makes the best use of the current system

Securing Industries profitability

- Support for ***overseas expansion*** aiming at large market
- ***Consideration of reimbursement prices*** and additions specifically for pediatric medical devices and consultation on insurance prospects

Industry-government-academia collaboration

- Strengthening ***industry-government-academia collaboration system*** specializing in the development of pediatric medical devices
- Strengthening the system of ***related academia***

Promote development of pediatric and rare diseases medical devices

Thank you for your kind attention!

Pharmaceuticals and Medical Devices Agency

For more information, please visit our website:

URL : http://www.pmda.go.jp/