Utilization of RWD in Pediatric Medical Device Development

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On behalf of **JCIC registry working group**

and **AMED Fujii research group**

Features of Pediatric Medical Device in Japan

- **Small market** due to the rarity of the diseases
- **Variable kinds and sizes** of devices are required
- Difficult to perform clinical trials due to small number of patients and too many institutions
- **Precise post-marketing surveillance (PMS)** tends to be required as a condition of device approval (as a means of assessing **safety and effectiveness** in the real world)

Utilization of Academic Registry Data for Pediatric Device Development

JCIC registry (2013-) operated by National clinical database (NCD)

JCIC: Japanese society of Congenital Interventional Cardiology

- Pediatric interventional cardiac catheter (PICC) procedures from nationwide 92 institutions (~**4500 procedures** annually)
- 146 variables/case (blanks are not permitted)
- **Meticulous collection of complications**
- **Strict error-check at data entry**

→Ensuring high-quality data on PICC procedures

Utilization of Academic Registry Data for Pediatric Device Development

- Needs Discovery (mainly from off-label use)

  Stent: 470 off-label uses/3 years

  Static Balloon Atrial Septostomy: 247 off-label uses/3 years

  → Approval of TMP PED® for static BAS in Japan

- Increase market predictability by identifying needs

- Use for PMS (regulatory use)

  <u>Cost reduction</u>

  Reduction of input burden

PMS for pediatric medical devices

using JCIC registry～direct use of registry data for PMS

PMS
for pediatric medical device

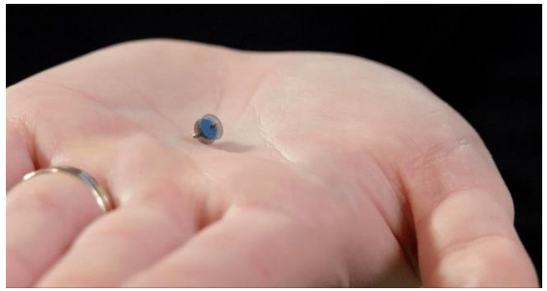
Pmda JAPAN

Become compliant with GPSP(Good Post-marketing Study Practice)
Already fulfilled the minimal requirement for audit traits

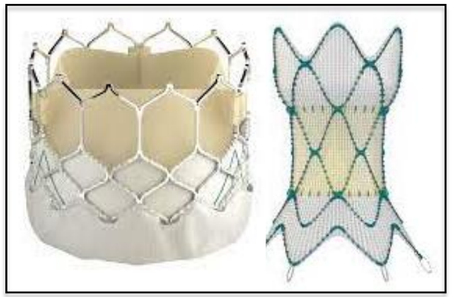
= Development of universal platform for PMS on pediatric medical devices
→Faster and more efficient collection of PMS data at lower cost

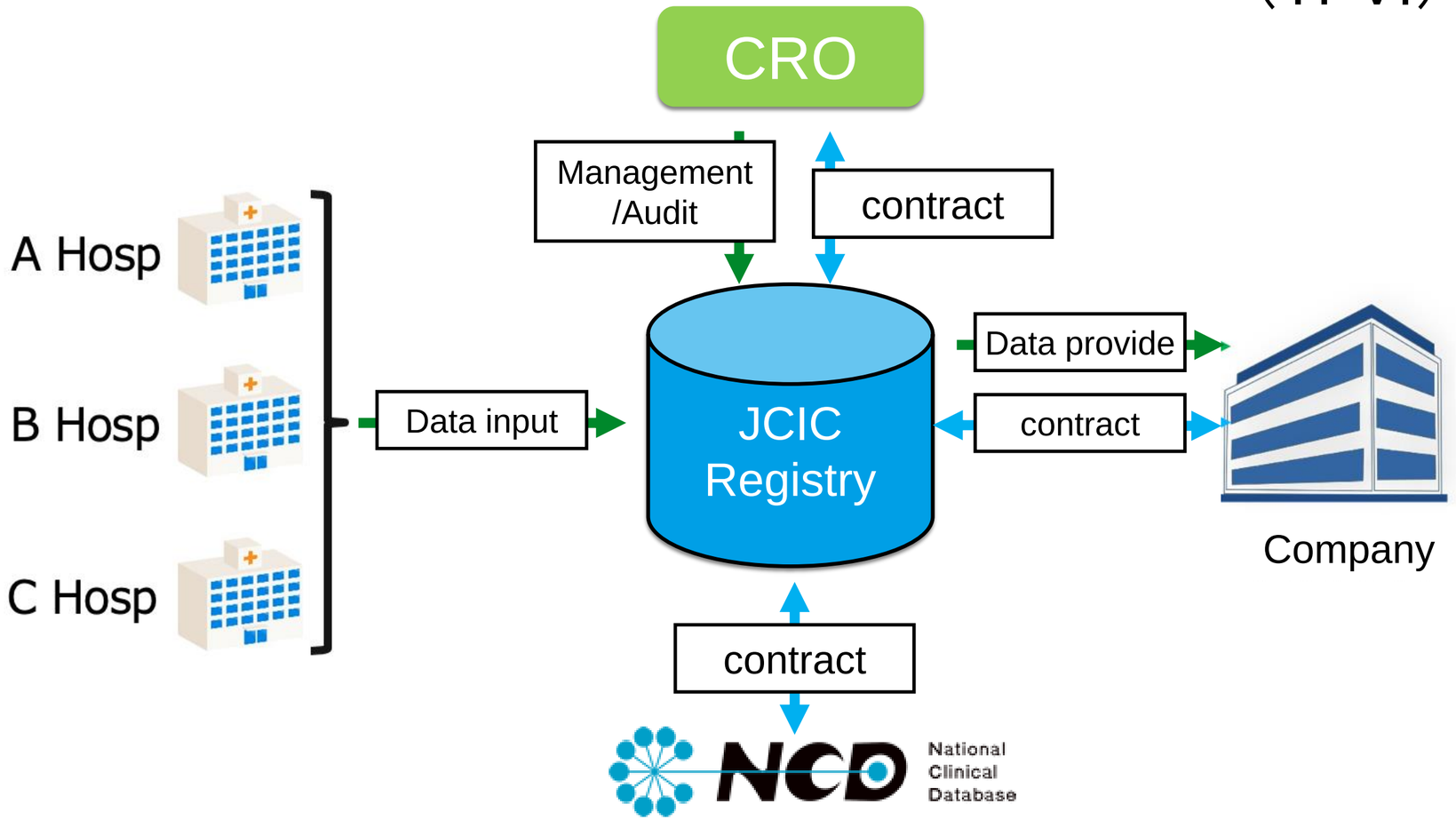
✓ Ongoing Registry-PMS

Piccolo Occluder



Transcatheter Pulmonary Valve Implantation （TPVI）

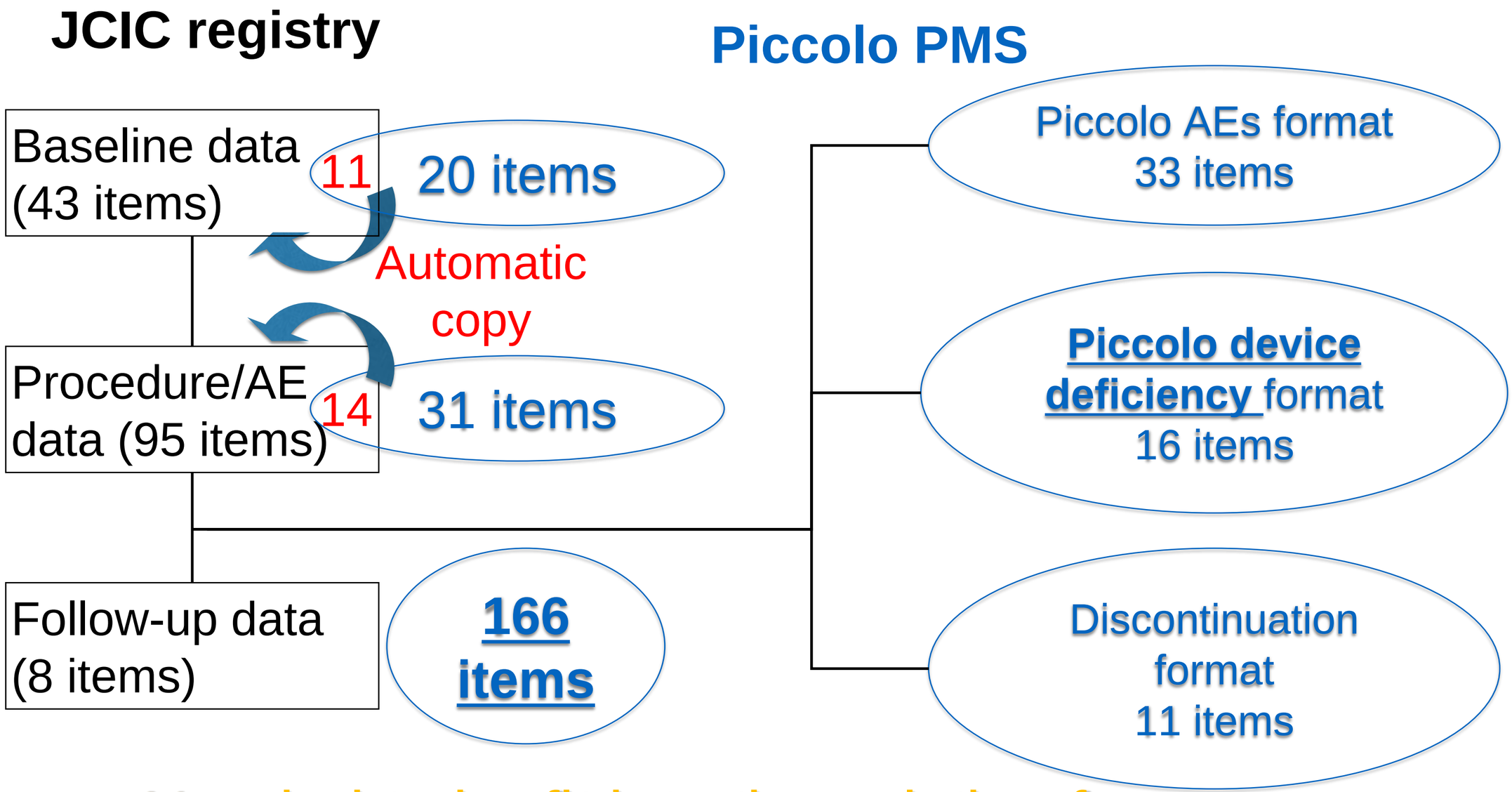




Registry-PMS required

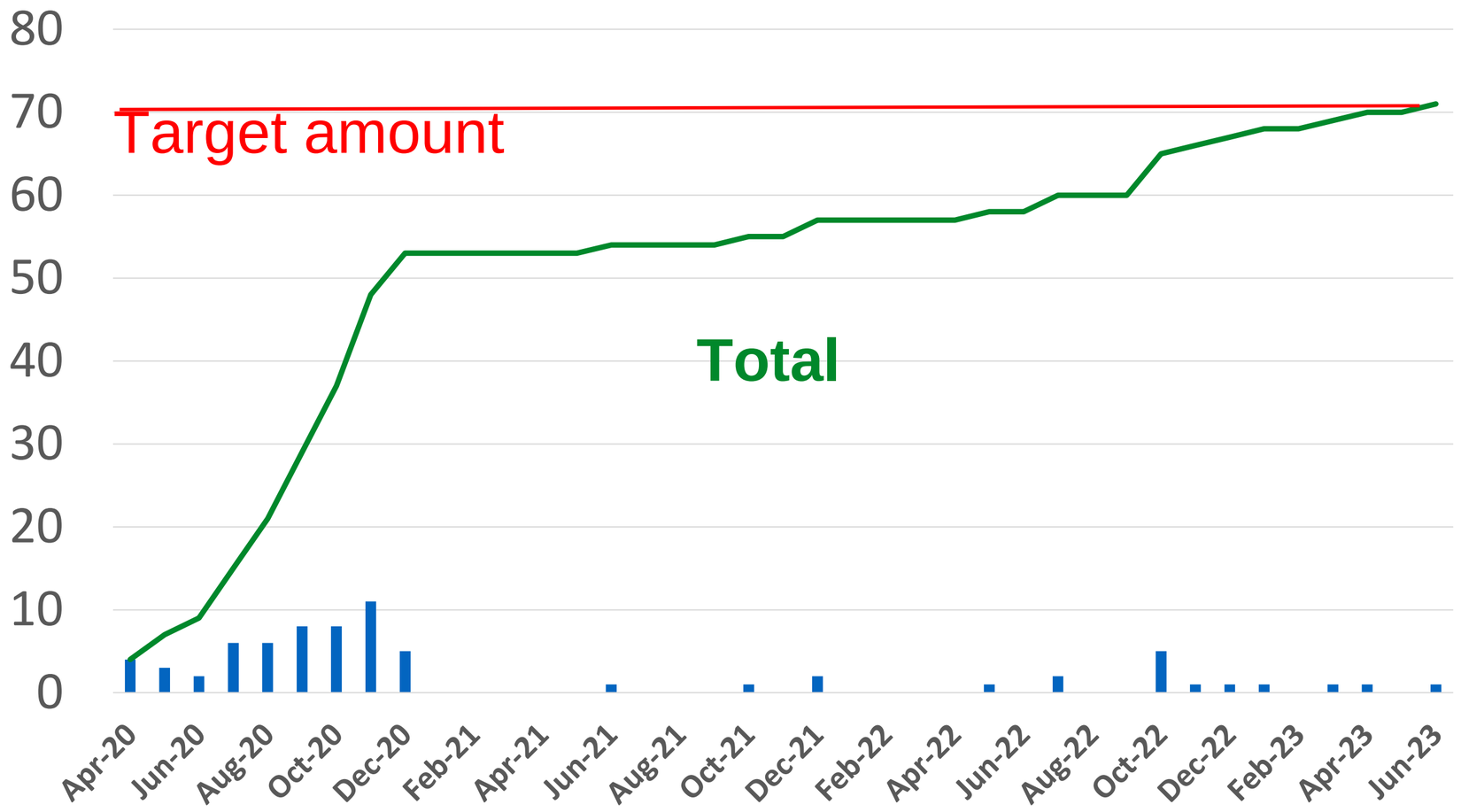
- Preparation of SOPs
- Patient Consent by Opt-out
- Progress management using shipping records

User-friendly data entry system



Needed to be fit into the existing format

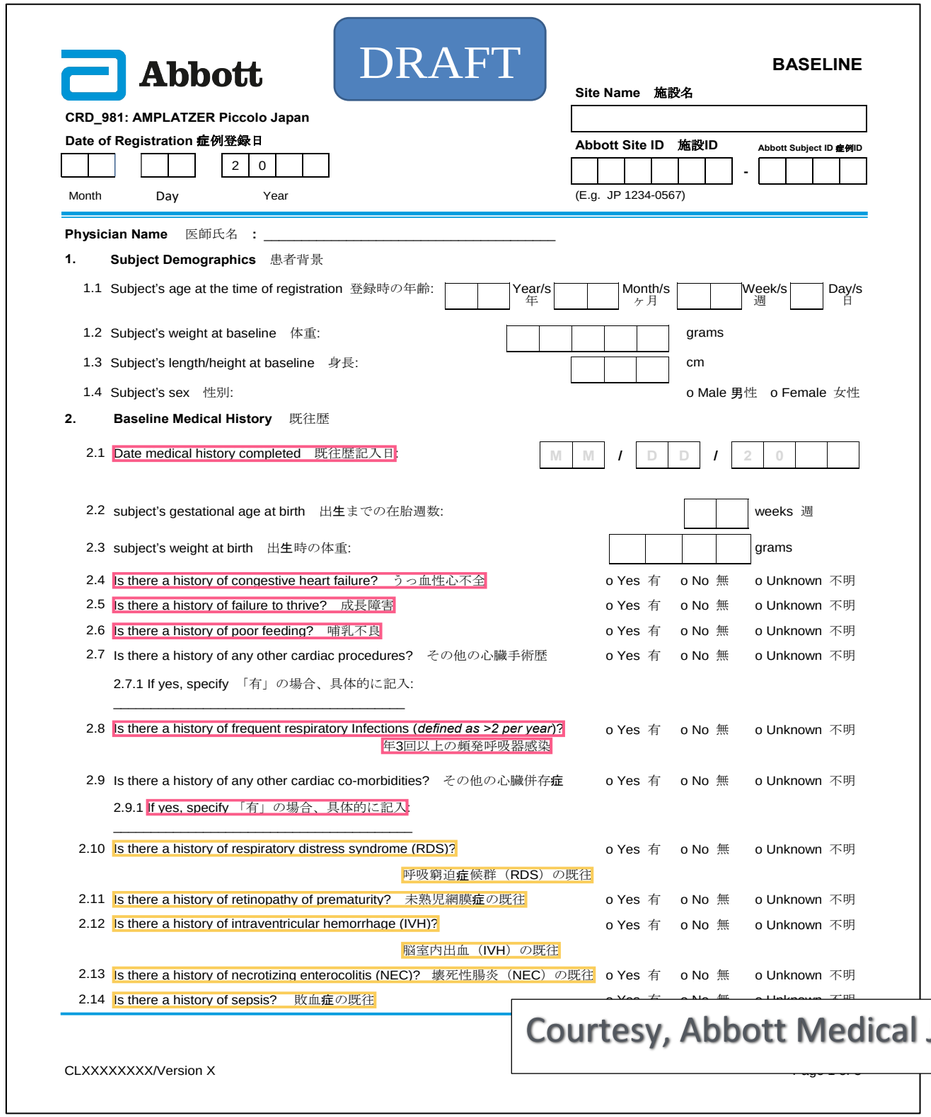
Progress Chart and Actual Cost for Piccolo Occluder



Cost for the system: ~$ 100,000

Total cost: ~60% of conventional PMS cost

Challenge : Too many survey items



Abbott

DRAFT

BASELINE

CRD_981: AMPLATZER Piccolo Japan

Site Name 施設名

Date of Registration 症例登録日

Abbott Site ID 施設ID

Abbott Subject ID 症例ID

Month Day Year (E.g. JP 1234-0567)

Physician Name 医師氏名 ：

1. Subject Demographics 患者背景

1.1 Subject's age at the time of registration 登録時の年齢: Year/s 年 Month/s ヶ月 Week/s 週 Day/s 日

1.2 Subject's weight at baseline 体重: grams

1.3 Subject's length/height at baseline 身長: cm

1.4 Subject's sex 性別: o Male 男性 o Female 女性

2. Baseline Medical History 既往歴

2.1 Date medical history completed 既往歴記入日: M M / D D / 2 0

2.2 subject's gestational age at birth 出生までの在胎週数: weeks 週

2.3 subject's weight at birth 出生時の体重: grams

2.4 Is there a history of congestive heart failure? うっ血性心不全 o Yes 有 o No 無 o Unknown 不明

2.5 Is there a history of failure to thrive? 成長障害 o Yes 有 o No 無 o Unknown 不明

2.6 Is there a history of poor feeding? 哺乳不良 o Yes 有 o No 無 o Unknown 不明

2.7 Is there a history of any other cardiac procedures? その他の心臓手術歴 o Yes 有 o No 無 o Unknown 不明

2.7.1 If yes, specify 「有」の場合、具体的に記入:

2.8 Is there a history of frequent respiratory Infections (*defined as >2 per year*)? 年3回以上の頻発呼吸器感染 o Yes 有 o No 無 o Unknown 不明

2.9 Is there a history of any other cardiac co-morbidities? その他の心臓併存症 o Yes 有 o No 無 o Unknown 不明

2.9.1 If yes, specify 「有」の場合、具体的に記入

2.10 Is there a history of respiratory distress syndrome (RDS)? 呼吸窮迫症候群（RDS）の既往 o Yes 有 o No 無 o Unknown 不明

2.11 Is there a history of retinopathy of prematurity? 未熟児網膜症の既往 o Yes 有 o No 無 o Unknown 不明

2.12 Is there a history of intraventricular hemorrhage (IVH)? 脳室内出血（IVH）の既往 o Yes 有 o No 無 o Unknown 不明

2.13 Is there a history of necrotizing enterocolitis (NEC)? 壊死性腸炎（NEC）の既往 o Yes 有 o No 無 o Unknown 不明

2.14 Is there a history of sepsis? 敗血症の既往 o Yes 有 o No 無 o Unknown 不明

CLXXXXXXXX/Version X

Courtesy, Abbott Medical Japan

- **Too many** items increase the cost and input burden (input/error check/query)
- **Too different** from academic survey items

× 21 pages !!

Minimizing survey items

Essence of PMS

1. Efficacy
- Clinical outcomes
- Device performance
- Procedure-related endpoints

2. Safety
- AEs related to device or procedure
- Unknown AEs

Research on the Improvement of the Environment to Promote the Development of Pediatric Medical Devices
2023-2025 PI Dr. Takanari Fujii

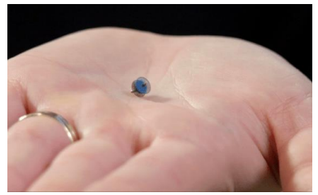
→ Reduction of survey items by 60-70%?

Remaining Issues for Registry-PMS

- **Templatization of SOPs**

- **Relatively high maintenance costs** (progress management and manual check): Increased PMS costs due to extended Registry-PMS due to missed targets.

  e.g. **Piccolo Occluder for 2.5kg** has not reached the target of 32 cases.

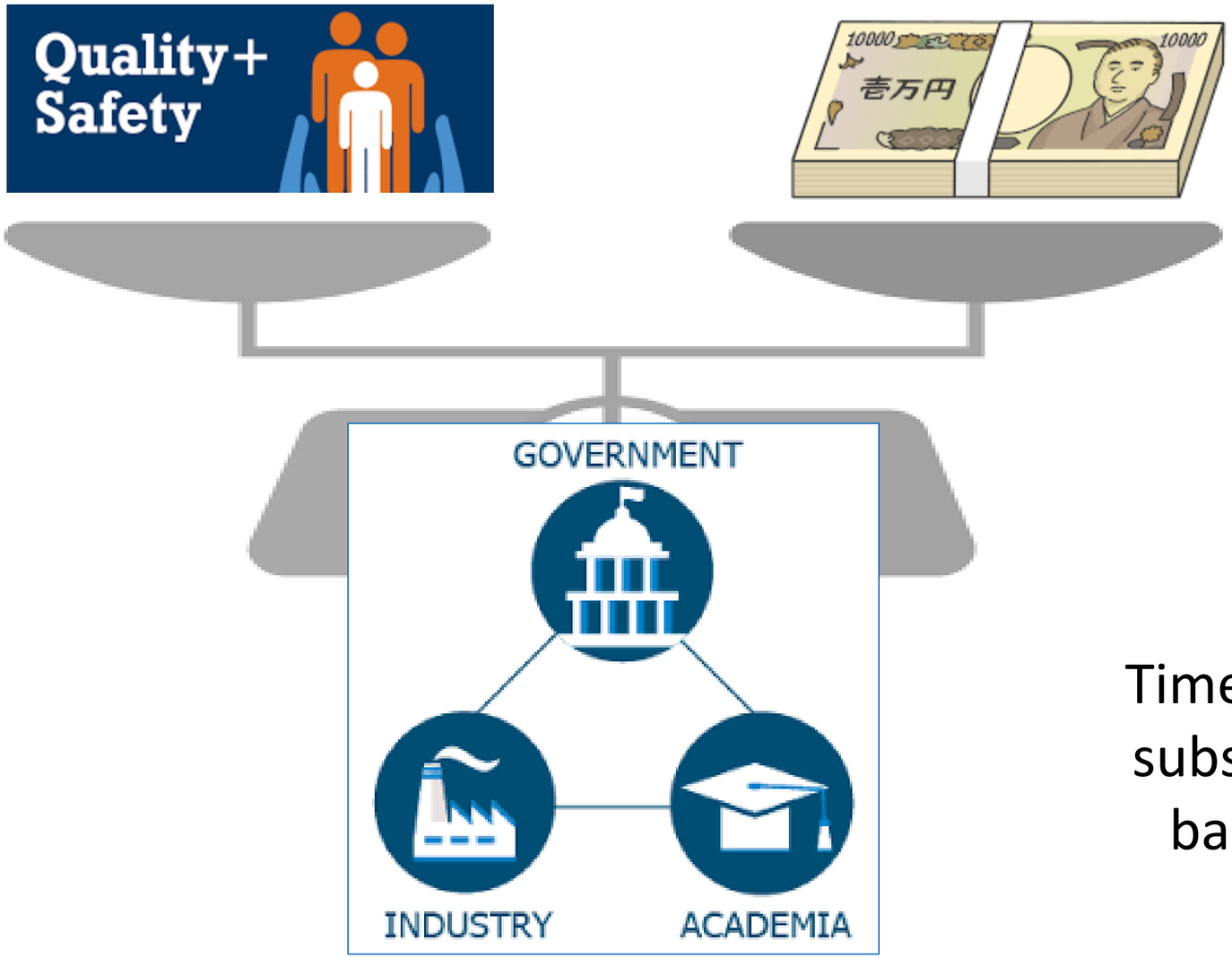


- **PMS cost reduction is small** compared to total application cost.

  e.g. Increased costs due to nonclinical-data requested for **CP stent applications** (lack of historical raw data).

- **Cost estimates** for Registry-PMS (or conventional PMS) not yet available.

Development of small-market medical devices



- ✓ Improved efficiency
- ✓ Cost reduction

achieved by cooperation b/w government and academia

Time to think about more deregulation and subsidies for small-market medical devices based on their actual cost for approval?

Summary

- Registry-PMS enabled an efficient data collection by direct use of registry data and system
- Further cost reduction may be possible once developing the minimum dataset for PMS
- Still a long way to go to develop medical devices for children