

Report from Japanese national grant research to promote the efficient development of pediatric medical devices

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- Outline -

1. Purpose of research and research organization
2. Main research subjects
3. Results of research
4. Special symposium, “Medical Device Day for Children”
5. Conclusion

Purpose of our research project and research organization

Finding burdens and solutions for

- ***accelerating global clinical trials*** of pediatric medical devices and
- ***utilizing real-world-data efficiently*** for regulatory use of pediatric medical devices

1. For accelerating global clinical trials, we will find

- Problems in the hospitals,
- Burdens which sponsor has, and
- Issues which regulatory has to consider

2. For utilizing real-world-data, we will think of

- Business continuity of registry and
- Measure for assuring minimum required quality and quantity of registry data for regulatory use

GOAL



Propose issues and solutions for effective development of pediatric medical devices

Research organization

Principal Investigator:

Dr. Kisaburo Sakamoto

Academia



地方独立行政法人 静岡県立病院機構

静岡県立こども病院

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国立研究開発法人

国立成育医療研究センター

National Center for Child Health and Development

and Japanese pediatricians

Government



Collaboration with HBD activities

Main research subjects

Basic investigation of issues

- Scoping Review and
- Questionnaire survey to pediatricians and industries

Finding issues and solutions for accelerating global clinical trials

- Comparison of GCP inspection procedures by PMDA and FDA

Finding issues and solutions for utilizing real-world-data efficiently

- Hearing survey to the registry holders in Japan and the U.S.

Special symposium:

“Medical Device Day for Children”

- Discussion among industries, academia and regulatory



What can we do for accelerating effective development of pediatric medical devices?

Main research subjects

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What can we do for accelerating effective development of pediatric medical devices?

Basic investigation of issues using scoping review and questionnaire

- **Purpose** of basic investigation is to capture
 - current situation of medical devices for children,
 - general burdens in development of medical devices for children, and
 - proposed solutions for accelerating development of pediatric medical devices
- **Method**
 - Scoping Review: review 588 relevant articles
 - Questionnaire survey
 - to **pediatricians**: council members of Japanese Society of Pediatric Cardiology and Cardiac Surgery, 327 persons
 - to **industries**: organizations belonging to the Japan federation of Medical Devices Associations, 60 persons

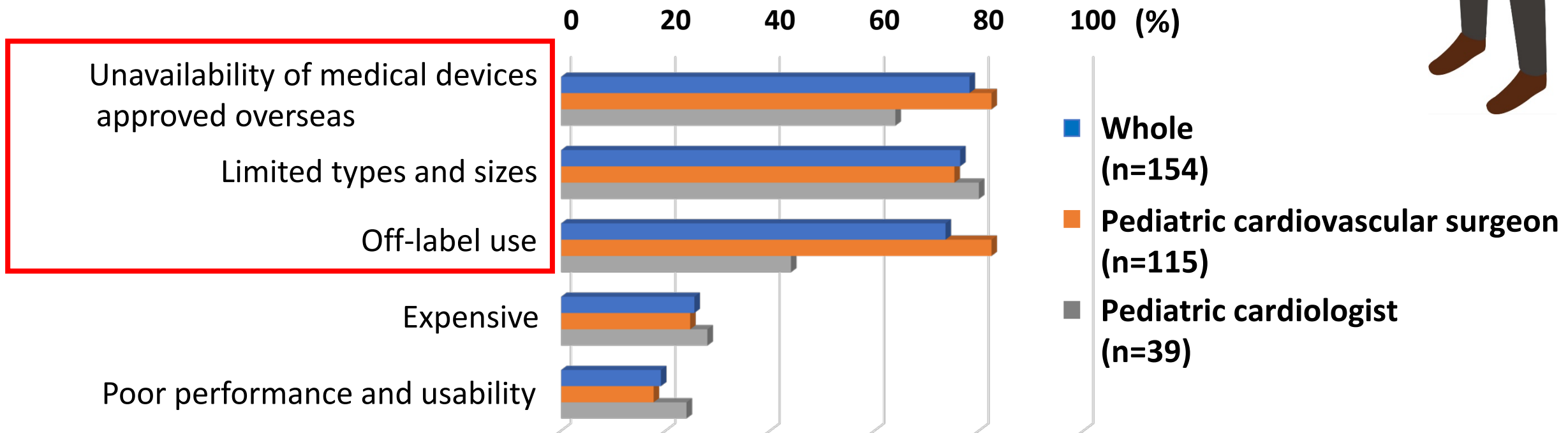
Results of questionnaire to pediatricians

- Response rate: 47.1% (154/327)
- Cardiovascular surgeons: 39 (25.3%)
- Pediatric cardiologists: 115 (74.7%)

Approximately 70% were dissatisfied with the pediatric medical devices currently available in Japan.



Reasons for dissatisfaction with medical devices for children available in Japan



Results of questionnaire to industries

- The number of respondents: 60 persons
- Research and development: 41%
- Pharmaceutical affairs: 38%



1. Reason of starting development

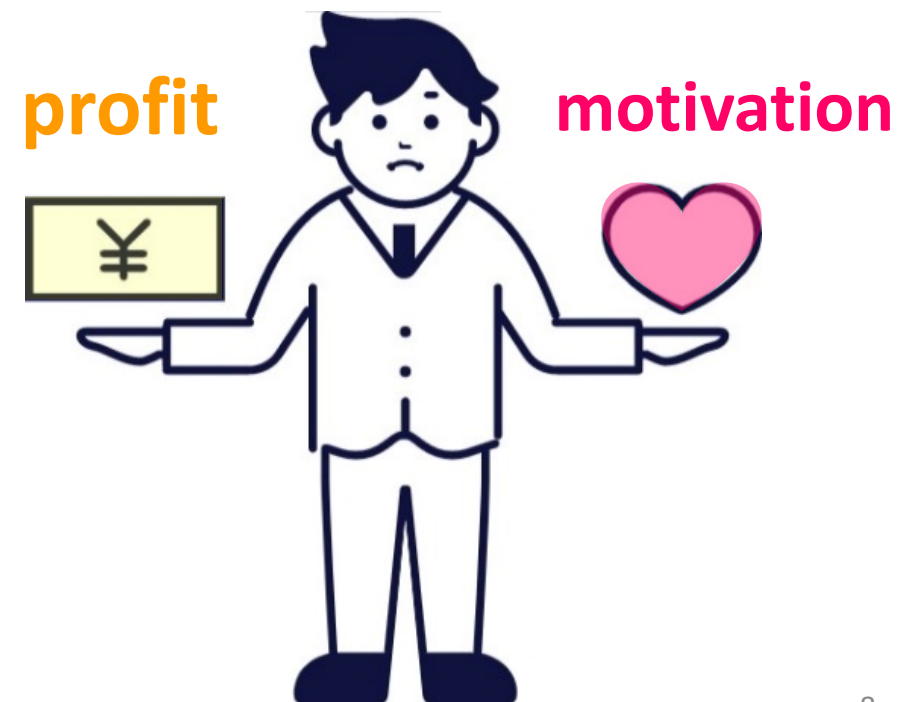
Requests from medical practice and academia	30%
Social contribution	30%

2. Obstacles of pediatric medical devices development

Revenue is difficult to predict	24%
Development risk is high	13%
Conducting clinical trials	11%

3. Effective measures for cost reduction

Reduction of examination fee	18%
Acceptance of overseas clinical data	16%
Publication of guidelines	15%



Summary of basic investigation of issues

Main burdens

1. Universal issues particular to children

- ✓ Small market size
- ✓ Disease rarity
- ✓ Abundant size variations
- ✓ Necessity of long-term prognosis surveillance considering growth
- ✓ Difficulty of conducting a clinical trial and lack of experience of clinical trial in pediatric field

2. Profitability issues for industry

- ✓ Insufficient of incentives in insurance reimbursement for pediatric medical devices

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Special symposium:

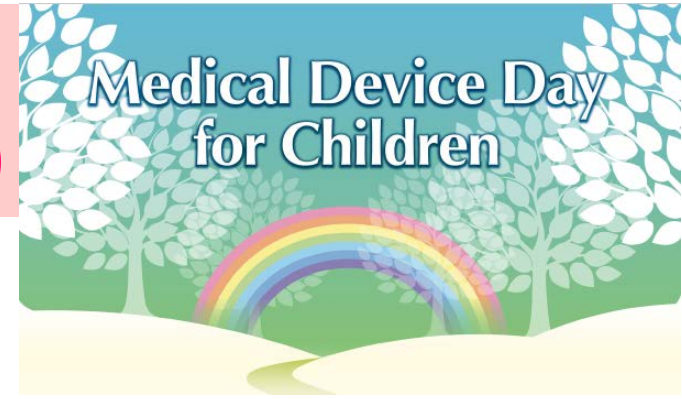
“Medical Device Day for Children”

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What can we do for accelerating effective development of pediatric medical devices?

Special symposium:
Medical Device Day for children (Oct. 10th, 2021)



Purpose:

- Exchanging idea among academia, industry and registry
- Understanding the burdens from a business perspective
- Getting a clue to find solutions for raising industry's motivation

Attendee:

- **About 150 people** from academia, industry and government

Main subject for discussion:

- What is necessary to establish the development and continuous provision of pediatric medical devices as a business?

<https://www.pmda.go.jp/files/000242991.pdf>

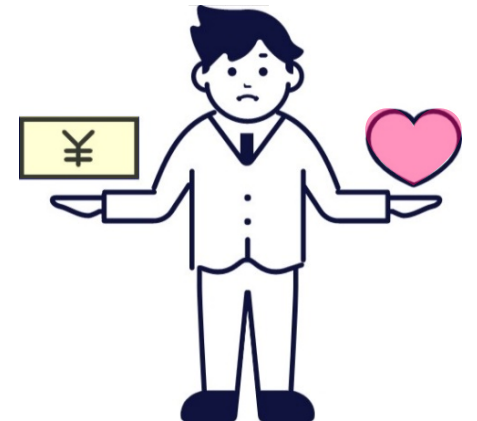
Special symposium: *Medical Device Day for children*

Real opinions from industries

- The development of pediatric medical devices is done **in a volunteer spirit**, because it is not expected to be profitable.
- Domestic market size is limited and **introduction to international market is important**. However, it is very costly and it is also difficult to predict profit.
- **Regulatory harmonization is a key** for promotion of the pediatric medical device development.

Expected support

- ***To promote the regulatory harmonization through HBD activities***
- ***To extend the market scale by overseas expansion***



Conclusion

Basic investigation of issues

Accelerating global clinical trials

Utilization of real-world-data

Special symposium

- ◆ Burdens
 - ✓ Universal issues particular to children
 - ✓ Profitability issues for industry

- ◆ Keywords for raising industry's motivation
 - ¥ **Enough profit** (enough insurance reimbursement)
 - ♥ **Contribution to society**



We will present a proposal for accelerating the development of pediatric medical devices, taking into account the efficient development and the ingenuity for market expansion.





Thank you for listening.

*If you have any questions, please contact us.
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