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Promotion of Pediatric Drug Development by Industry, Government and Academia – What Has Changed, What Has Been Done and What Is Necessary for the Further Progress?

From the perspective of the PMDA

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Actions by the PMDA over the Past Year -1-

- (Action by MHLW) Draft of Revision of Act on Pharmaceuticals and Medical Devises (PMD Act)
  - ✓ Establishment of “Specific Use Drug Designation System”
- PMDA Asia Training Center (ATC) & U.S. FDA Pediatric Review Seminar
- Contribution to JPMA Pediatric Drug Development Workshop

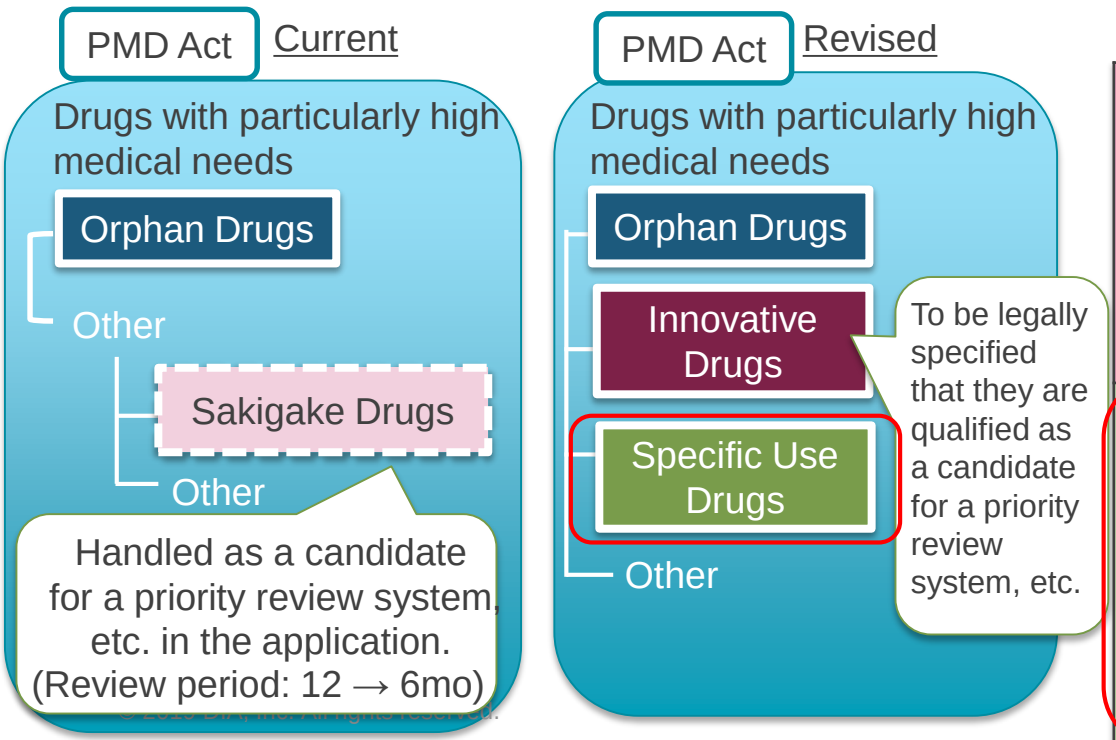
Legislation of “Sakigake Designation System” and “Specific Use Drug Designation system”

○ A system to designate drugs/medical devices/regenerative medical products whose action mechanisms are clearly different form those approved in Japan/other countries as “**innovative drugs**” shall be defined by law, and the designated products shall be clearly qualified by law to be a candidate for a priority review system, etc.

○ A system to designate drugs addressing significant unmet medical needs such as drugs with no indication for pediatric patients as “**specific use drugs**“ shall be defined by law, and the designated products shall be clearly qualified by law to be a candidate for a priority review system, etc.

○ It shall be legally stipulated that specific use drugs (limited to those for the small number of patients) are eligible for tax benefits and subsidies to promote development as well as existing orphan drugs.

(*) Tax benefits have already been included in the 2019 tax reform plan.



Designation requirements

Innovative drugs	1. Having clearly different action mechanisms from products approved in Japan/other countries. 2. Markedly useful in that particular use. ➢ Assuming the same drugs for the current “Sakigake designation system”.
Specific use drugs	1. Being used for the treatment of specific diseases (*). * • Being used to treat pediatric diseases but dosage and administration for children not stipulated. • Infectious diseases caused by drug-resistant bacteria, etc. 2. The need for specific use drugs is significantly unmet. 3. Markedly useful in that specific use.

PMDA ATC & U.S. FDA Pediatric Review Seminar (July 8-11, 2019)

- To promote pediatric drug development globally
- To raise the level of skill related to pediatric drug development in Asian and other countries/regions

- This seminar is intended for regulatory authority officials who are engaged in the review of pediatric drug development programs.
- This seminar covers current pediatric guidelines and practices in the United States and Japan, and provides the opportunity for the participants to share current pediatric guidelines and practices in their respective countries and regions.
- Case study sessions on pediatric drug development programs are also planned for small group discussions among the participants.
- Face to face meetings between the U.S. FDA, the PMDA and participants are took place if they want.

JPMA Pediatric Drug Development Workshop (July 12, 2019)

- Keynote Speech
  The BMS's Approach for Pediatric Oncology Drug Development
  Kiyoshi Hashigami, Bristol-Myers Squibb
- Drug Development of Pediatric Oncology Area
  - Drug Development of Pediatric Oncology Area in Japan
    Chitose Ogawa, National Cancer Center Hospital
  - Recent Topics of Pediatric Oncology Drug Development
    Michiyo Sakiyama, PMDA
  - Regulatory and Ethical Considerations: Pediatric Oncology Trials
    Donna Snyder, FDA
- Pediatric Extrapolation
  - ICH E11 (R1) and ICH E11A
    Shinichi Kijima, PMDA
  - From the Point of View of Pharmaceutical Companies
    Masako Saito, Pediatric Extrapolation Discussion Team, JPMA
- Panel Discussion

Actions of the PMDA over the Past Year -2-

- Patient Centricity WG as one of the Projects across Multi-offices in the PMDA (established in May 2019)

- Other activities of Pediatric Drugs WG in the PMDA
  - Lecture at symposiums in academic conferences
    Japan Society of Developmental Pharmacology and Therapeutics
    Japan Society for Neonatal Health and Development
    Japanese Society of Kawasaki Disease
    The Japanese Teratology Society etc.
  - Invited Articles (Japanese)
    Current Therapy, Japanese Pharmacology & Therapeutics,
    PHARM STAGE, Pediatrics of Japan, Japanese Journal of Pediatrics etc.
  - Scientific Publication
    “Pediatric drug regulation: International Perspectives” Pediatric Anesthesia, 29: 572-582, 2019. coauthored with the U.S. FDA, the EMA, the Health Canada and the CNDA
  - PMDA Internal Meeting for Pediatric Extrapolation
  - Pediatric Cluster Teleconference between the U.S. FDA, the EMA, the Health Canada, the TGA and the PMDA (monthly)
    etc.

What Is Necessary for the Further Progress?

- Industry-Government-Academia should work closely together more than ever.

- Enhance the incorporation of the voice of patients/ parents of patients in pediatric drug development
  - Engagement of patients/ parents of patients into pediatric drug development
  - Provision of adequate and timely information to patients/ parents of patients

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