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CHARTING NEW HORIZONS

Regulatory Updates regarding Pediatric Drug Development in Japan and International Collaboration

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Today's Agenda

- Regulatory updates regarding pediatric drug development in Japan

- Topics of international collaboration
  - Latest information about ICH E11A
  - PMDA Asia Training Center and U.S. FDA Pediatric Seminar

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Pediatric Regulations In Japan

- There is no regulation mandating pediatric drug development.
- ICH-E11 and ICH E11(R1)
- Several frameworks to enhance pediatric drug development have been implemented so far.
  - Extension of re-examination period (revised in 2020)
  - Drug Price Premium for Pediatric Use (5~20%) (updated in 2024)
  - Council for unapproved and off-label drugs with high medical needs
  - Pediatric clinical trial network
  - Considerations for clinical evaluation of drugs in pediatric patients (10 or 12 years of age and older) who can be evaluated together with adults
  - Specific Use Drug Designation System

Regulatory Updates in Japan

- **New Approach to Enhance Pediatric Drug Development**
  - Planning of the Pediatric Drug Development Program during Development of Drugs for Adults

- **PMDA Regulatory Affairs Consultation Center dedicated to Pediatric and Orphan Drugs**
  - Consultation on Confirmation of the Pediatric Drug Development Program

- **New Drug Pricing System**
  - FY 2024 Drug Price System Reform to promote pediatric drug development

Planning of the Pediatric Drug Development Program during Development of Drugs for Adults

Applied from April 1, 2024

(PSB/PED Notification No.0112-3. dated January 12,2024, partially revised in March 29, 2024)

https://www.pmda.go.jp/files/000268523.pdf (English translation by the PMDA)

- Encourage pharmaceutical companies to
  - prepare a pediatric drug development plan
  - confirm with the PMDA before submitting new drug application for adult use
  - proceed with the development without delay based on the prepared development plan
- Not only the implementation of clinical trials in Japanese children, but also using clinical data from adults and non-Japanese pediatrics, real-world data, and modeling & simulation, etc. should be considered and confirmed by the PMDA

When pharmaceutical companies develop pediatric drugs in accordance with this new approach

Incentives related to the drug price

Consultation on Confirmation of the Pediatric Drug Development Program

Starts from July 1, 2024

- Dedicated to confirmation of the Pediatric Drug Development Program based on the new approach

- Consultation prerequisites
  - The pharmaceutical company is planning to conduct a pediatric clinical trial and has already conducted scientific consultation for adult indication with the PMDA regarding clinical trial design, etc..

    AND
  - The adult use is before filing an application for approval.

Consultation on Confirmation of the Pediatric Drug Development Program

Starts from July 1, 2024

- Exceptions
  - Regarding the validity of the clinical trial design and the sufficiency of the application data package
  - Regarding pediatric drug development plan for drugs with different indications between adults and children
  - Regarding the utilization of real-world data, modeling & simulation, etc., without conducting pediatric clinical trials

→ **Existing scientific consultation**

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Latest Information about ICH E11A “Pediatric Extrapolation”

- Completed Regional Consultation 28 Oct 2022
  - 1182 comments received

- Current work plan is intended to deliver final guideline by the end of the second quarter of 2024

- ICH E11A Expert Working Group acknowledges the need to develop training materials
  - Expected to be completed 6 months after the publication of the final guideline

To Enhance Pediatric Drug Development

- Because of a decrease in the number of children in Japan as well as in major advanced countries/ regions, it becomes more and more difficult to conduct pediatric clinical trials of a certain scale leading to high level of evidences in single countries.
- Global collaboration is absolutely imperative.
- Participation from emerging countries/ regions is also essential.

Create new path for better medicines for children

- ✓ Global Harmonization
- ✓ Beyond the borders
  - – Regulators, Industries, Health Care Professionals, Patients/ Patients' Care Givers
  - – Countries/ Regions

PMDA Asia Training Center & U.S. FDA Pediatric Review Seminar

Once a year since 2018

- To promote pediatric drug development globally
- To assist capacity building related to pediatric drug development in Asia and other countries/regions

- For regulatory authority officials who are engaged in the review of pediatric drug development programs
- Current pediatric guidelines and practices in the United States and Japan
- Current pediatric guidelines and practices in participants' respective countries and regions
- Case study and small group discussions among the participants
- Face to face meetings between the U.S. FDA, the PMDA and participants
- A lecturer from the EMA

Summary and Future Perspectives

- The environment for pediatric drug development in Japan is improving through various efforts.
- It is expected that pediatric drug development will gain further momentum with the latest new approach and new initiatives in Japan.
- PMDA strongly supports pediatric drug development.
  - Accelerating multi-regional pediatric clinical trials
  - Global Collaboration and Harmonization
  - Involving emerging countries/regions

Better medicines for children!

Thank You

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Website of PMDA Pediatric Drugs Working Group:

https://www.pmda.go.jp/english/rs-sb-std/rs/0007.html