The 21st DIA Japan Annual Meeting 2024

Toward a Well-Being Future
in which Each and Every One of Us Has 'Ikigai'

October 27-29, 2024 | Tokyo Big Sight

S08

Regulatory updates regarding pediatric drug development in Japan

本邦における小児用医薬品開発に関連する規制の最近の状況

Takaaki TANINOKUCHI

Pharmaceuticals and Medical Devices Agency (PMDA)

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This presentation is incomplete without accompanying verbal commentary.

Today’s Agenda

- Recent New Drug Approvals in Japan

- Regulatory updates regarding pediatric drug development in Japan

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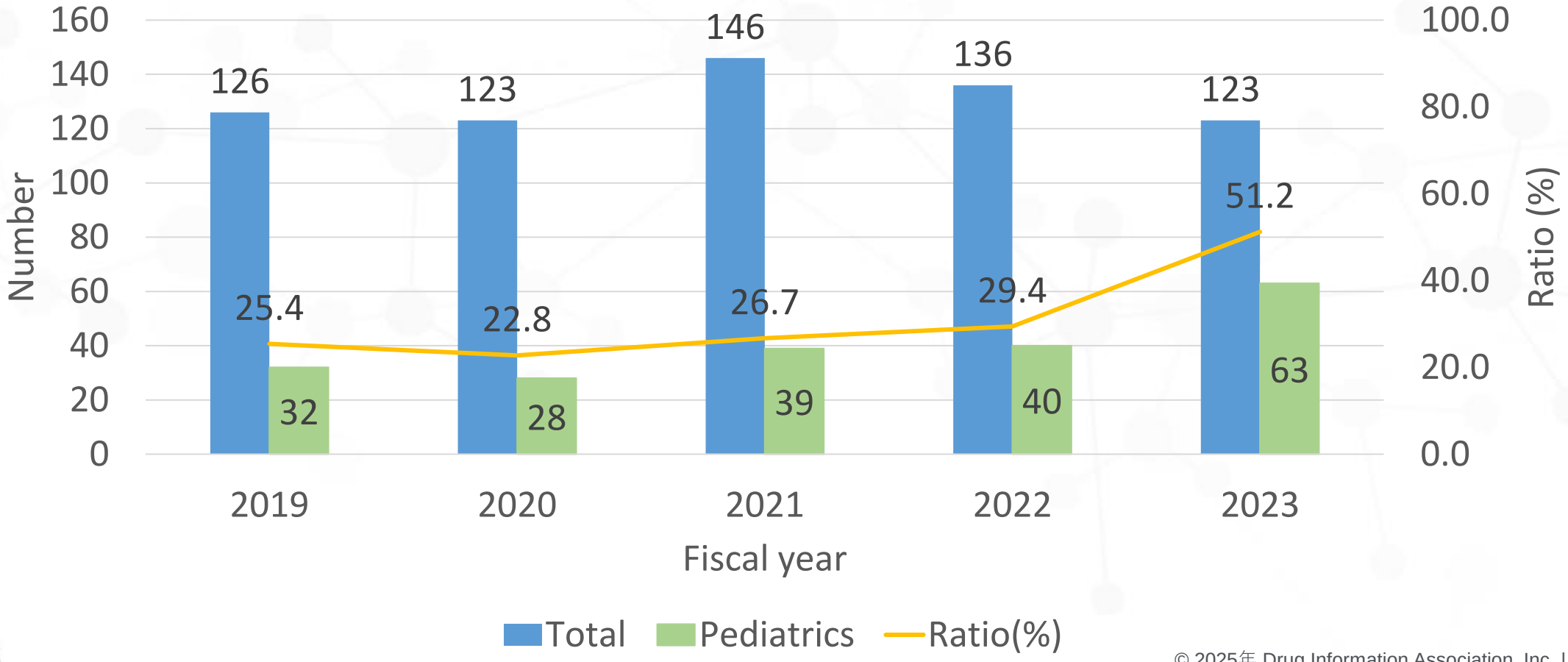
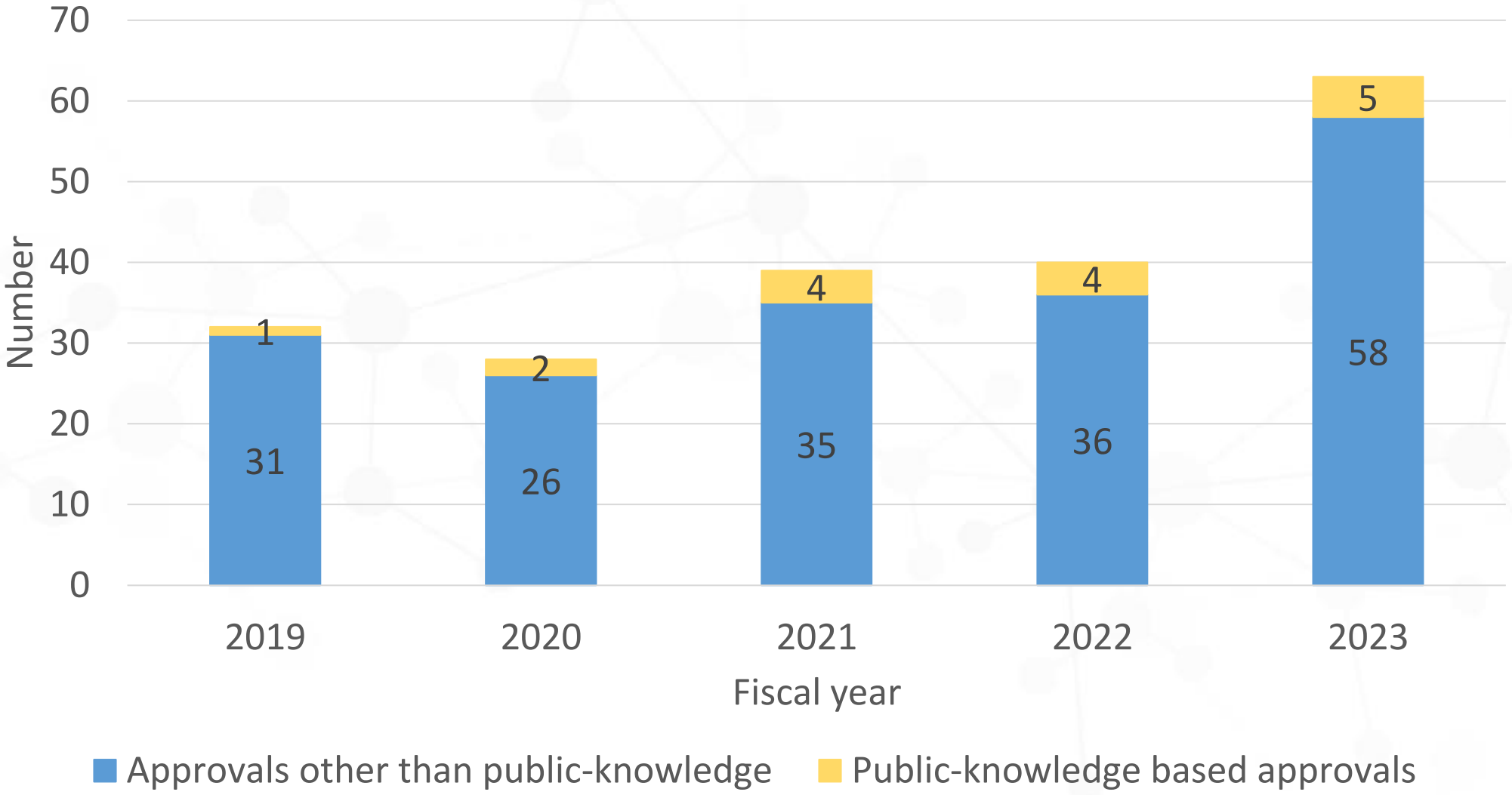
Recent New Drug Approvals in Japan

- The PMDA Pediatric Drugs Working Group surveyed drugs with pediatric dosage and administration from the list of approved Drugs on the PMDA website.

https://www.pmda.go.jp/english/review-services/reviews/approved-information/drugs/0002.html



Basis of Pediatric Approvals



Pediatric Regulations In Japan

- Pediatric drug development is not mandated in Japan.
- ICH-E11 and ICH E11(R1)
- Several frameworks to enhance pediatric drug development have been implemented so far.
  - Drug Price Premium for Pediatric Indication Use (5~20%)
  - Extension of re-examination period (revised in 2020)
  - Committee for unapproved/off-label drugs with high medical needs
  - The designation system for drugs for specific use
  - Announcement of Considerations for clinical evaluation of drugs in pediatric patients (10 or 12 years of age and older) who can be evaluated together with adults
  - Establishment of Pediatric clinical trial network

Re-examination period

- Post-marketing safety and efficacy for new drugs are reviewed for a certain period as shown in the table below after the approval in Japan.
- As generic drugs are not approved prior to the re-examination of new drugs, re-examination period is similar to the exclusive sales period for new drugs.

Re-examination period	Drug Type
10 years	Orphan Drugs, Drugs that need to be surveyed by pharmacoepidemiological method
8 years	Drugs with new active ingredients
4 – 6 years	New combination drugs, Drugs with a new route of administration
4 years	Drugs with new indications, Drugs with a new dosage

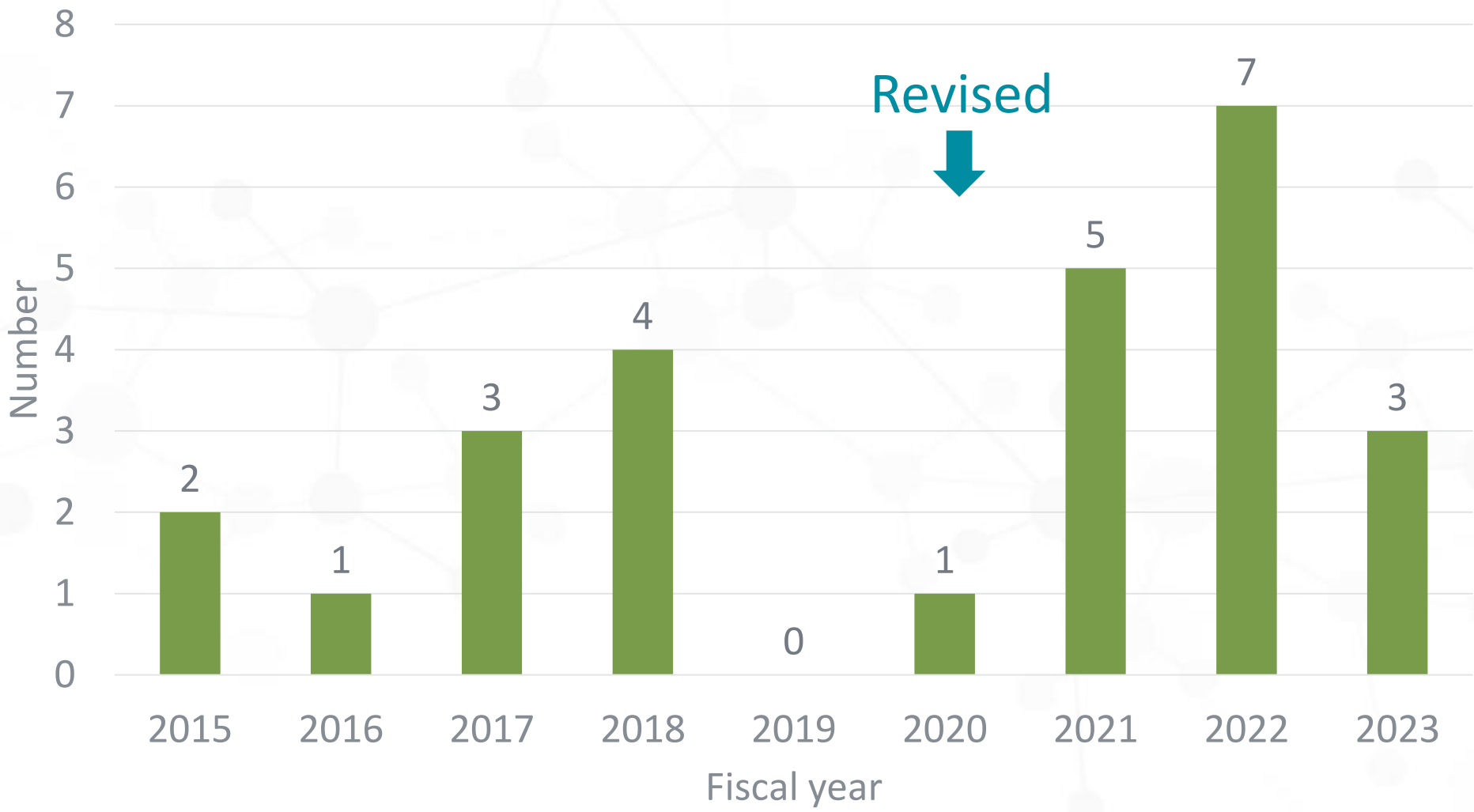
Extension of re-examination period

- The re-examination period can be extended to 10 years, if a dose-finding study in pediatrics is planned **under review or after approval**

Revised (Aug. 2020)

- If all of the following apply, the re-examination period can be extended to 10 years
  - At the time of approval, a dose-finding study in pediatrics is clearly required
  - The development plan for finding dosage in pediatrics is submitted **by the end of the review on marketing approval**
  - The planned clinical study is started without delay

Drugs with extended re-examination period with pediatric development



Today’s Agenda

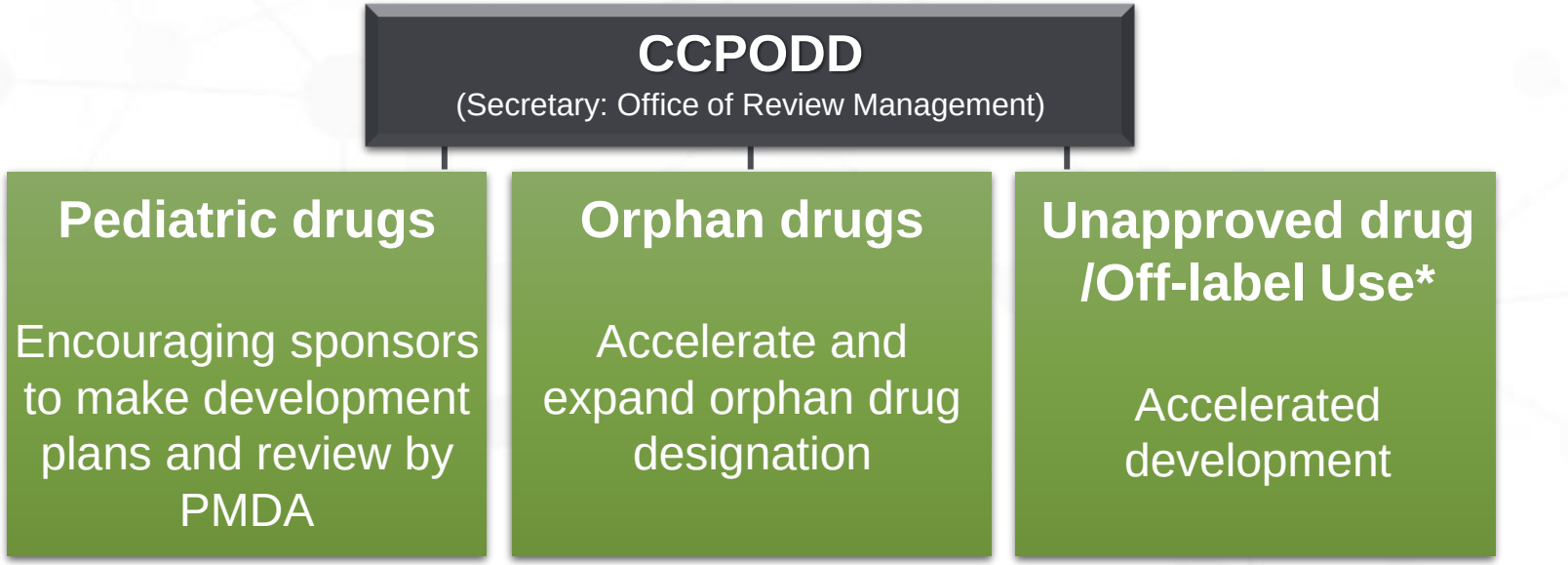
- Recent New Drug Approvals in Japan

- Regulatory updates regarding pediatric drug development in Japan

Consultation Center for Pediatric and Orphan Drugs Development (CCPODD)

Established on July 1, 2024

- For promoting development and introduction of medicinal products for pediatric and rare diseases and providing the necessary consultation, the Consultation Center for Pediatric and Orphan Drugs Development (CCPODD) is established.



* By the Evaluation Committee on Unapproved and Off-label Drugs with High Medical Need. (Independent of the Center)

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

Applied on April 1, 2024

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

When companies develop pediatric drugs in accordance with this approach

Incentives related to the drug price

Consultation on Confirmation of the Pediatric Drug Development Program

Started on July 1, 2024

- This consultation is only for confirmation of the Pediatric Drug Development Program

- Consultation prerequisites
  - The applicant have already had scientific advices on the product for adult indication with the PMDA regarding clinical design, etc..

    AND
  - The adult use is before submission of NDA.

Consultation on Confirmation of the Pediatric Drug Development Program

Started on July 1, 2024

- Exceptions
  - The validity of the clinical trial design and the sufficiency of the application data package
  - Pediatric drug development program for the drug with different indications between adults and children
  - Not conducting pediatric clinical trials (the utilization of real-world data, modeling & simulation, etc.)

→ **Existing scientific consultation**

Gate Opening Summit for Innovative Drug Discovery

Held on July 30, 2024

"Prompt Delivery of Novel Drugs to Patients"

- Eliminate the current drug loss (start development by FY2026 for drugs that treat diseases for which treatments are unavailable in Japan)
- Aim for 50 pediatric drug development programs and 150 approvals of orphan drugs (cumulative number from FY2024 to FY2028)

https://www.cas.go.jp/jp/seisaku/souyakuryoku/shiryou/1-1_e.pdf

Summary and future perspectives

- The environment for pediatric drug development is improving through various efforts in Japan.
- It is expected that pediatric drug development will gain momentum further with with the latest new approach and new initiatives in Japan.

Questions?