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Global Pediatric Development: We Are Making Progress

PMDA Perspective

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Agenda

- PMDA Paediatric Drugs WG
- Collaboration at Paediatric Cluster
- Future Challenges



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PMDA Paediatric WG (1)

- Established in November 2011
- Consists of 20 members (as of June 2014)
 - Including paediatricians, physicians and pharmacists from the Office of New Drugs and Office of Safety etc.
- Routine internal meeting



PMDA Paediatric WG (2)

- Task
 - Promote industries and investigators to develop medicinal products for children
 - Exchanging views with domestic stakeholders (HCPs, industry group,)
 - Strengthen collaboration with foreign regulatory agencies for development of paediatric medicines
 - Analyzing and clarifying issues raised in past reviews and cases of consultations



PMDA Paediatric Drugs WG Activities

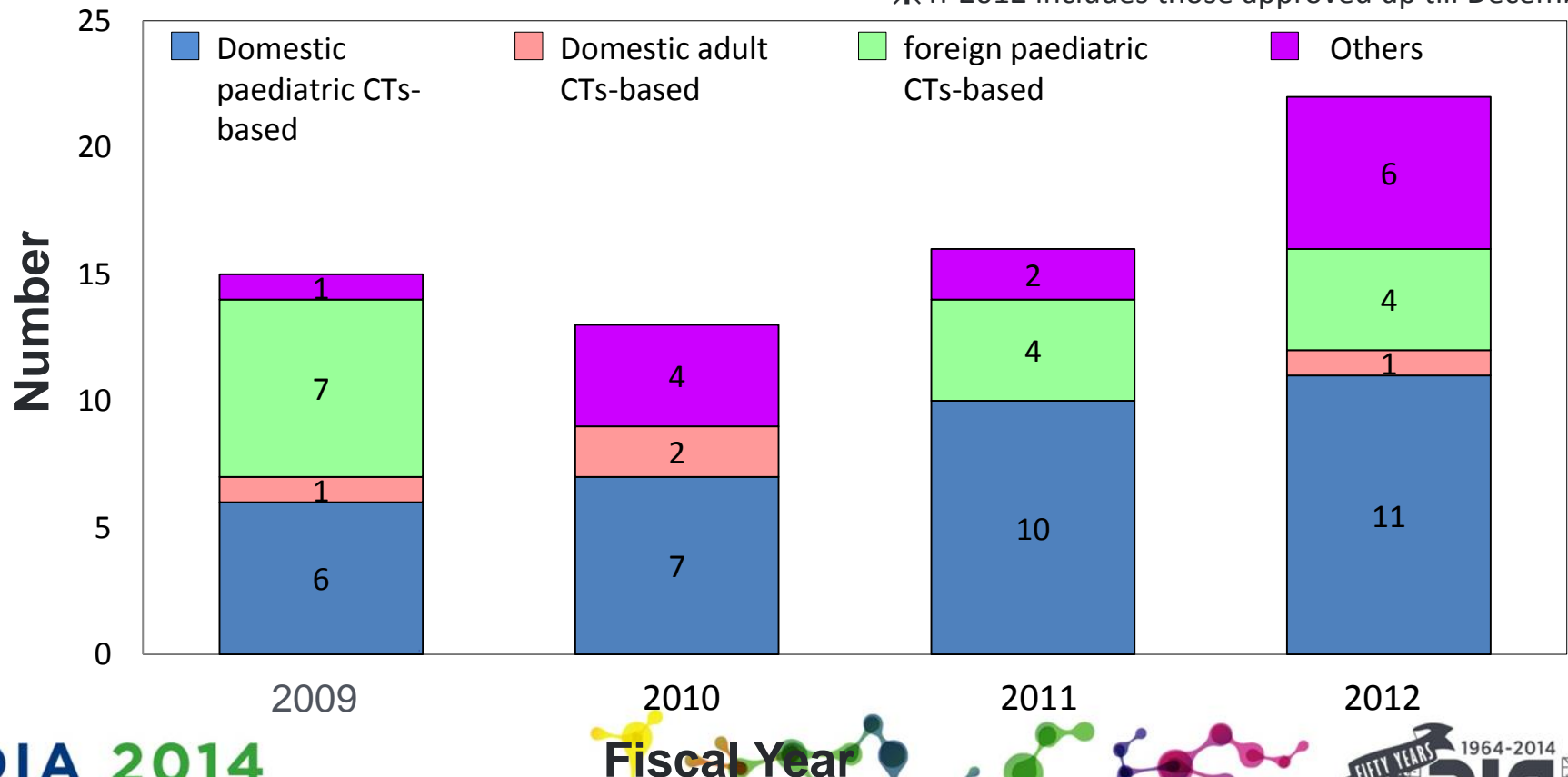
- Analysis of the NDA data in Japan
- PMDA 1st Internal Workshop
 - Internal brainstorming on paediatric drug development



Result of Analysis: Development Strategy

- Review of 66 drugs were approved for paediatric dosage between Apr. 2009 and Dec. 2012
- No drug was approved based on the results of MRCT

※Yr 2012 includes those approved up till December.



PMDA 1st Internal Workshop

Based on our analysis, we held the first internal workshop for paediatric drugs development in February 2013.

We discussed topics such as:

- Extrapolation of efficacy from other population (Japanese adults, foreign children ,etc.)
- Timing of paediatric drug development in Japan



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Paediatric Cluster

- Routine teleconference once a month
 - Exchange opinions between EMA, FDA, PMDA, Health Canada and TGA
 - Clinical trial design on developing products
 - Safety concerns on developing/approved products
 - General issues
 - Development of guidelines
- etc.



Case 1 : Somatropin (1)

- A draft French manuscript
 - Increase in mortality due to bone tumors and cerebral vascular accidents in patients treated with human growth hormone compared to a generally healthy French population
 - EMA, FDA and PMDA are in the process of safety evaluation.
 - FDA has issued a Safety Alert, and EMA and French Regulatory Agency have issued press releases on somatropin products.



Case 1 : Somatropin (2)

- EMA, FDA and PMDA shared followings;
 - Data the agency has
 - Local data
 - Scientific articles
 - Current consideration
 - Action plan and the timeline

- Followed up at the later TCs



Case 2 : Oseltamivir (1)

- Safety concern
 - Accumulation of ICSRs of ‘Abnormal behaviour’ in Japan
 - Studies are conducted to assess the causality in Japan
 - Post marketing observational studies by MAH
 - Epidemiological studies by investigators
 - Non-clinical studies by MAH/investigators
 - Established the Advisory Committee for the assessment



Case 2 : Oseltamivir (2)

- PMDA shared followings;
 - Japanese data
 - Abstract of our review report
 - Including our conclusion
 - Timeline of the assessment



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Paediatric drug development

- In less populated countries/regions (such as Japan), paediatric domestic studies of a certain scale leading to high level of evidences is often difficult to perform.
- In Japan, sponsors show reluctance to take action aggressively on drug development for children due to lack of paediatric regulations like EU and US.
- Paediatric development in Japan hits the wall?



Better medicine for children!

- Create new path for the better medicines for the paediatric population
 - Collaboration is very important
 - Beyond the borders
 - Regulators, industries, HCPs, researchers
 - Countries/regions



The better medicine for children!

- Accelerating multi-regional pediatric studies
- Development of new technologies and its utilization
 - New analytical methods (e.g. modeling & simulation)
 - Biomaker
 - High Sensitive Assay
- Efficient data collection
 - Global database ?
 - Off-label use?



Thank you

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