Regulations and Approvals Session

Aim of part-2 in RA session

April 10, 2018

JPMA (APAC RA-EWG)

Osamu Inagaki

APAC
Asia Partnership Conference of Pharmaceutical Associations

Expedited Pathways (US, EU and Japan)

	US	EU	JAPAN
SAKIGAKE/ Breakthrough Therapy/ PRIME	- Rolling Review - Designation required *All Fast Track designation features plus additional features apply	- Early dialogue with applicant - Accelerated assessment (150 days) applied - Designation required	- Rolling Review (SAKIGAKE Comprehensive Consultation) & 6-month review period - Designation required
Fast Track	- Rolling Review - Designation required	—	—
Priority Review/Accelerated Assessment	- Reduce review period from 10 months to 6 months - Designation required	- Accelerated assessment reduces the typical review time to 150 days from 210 days. - Designation required	- 9-month review period - Designation required

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Kazuhiko Mori; 14th DIA Japan Annual Meeting 2017

Expedited Pathways (US, EU and Japan) (Cont.)

	US	EU	JAPAN
Jinsoku (Rapid) Review	—	—	- Specific designation by MHLW
Conditional marketing authorization	—	- Allows for the early approval of a medicine on the basis of less complete clinical data than normally required.	- (Limited to Regenerative medicine Products)
Accelerated Approval	- Approval on a surrogate or intermediate clinical end point	—	- (Standard practice)

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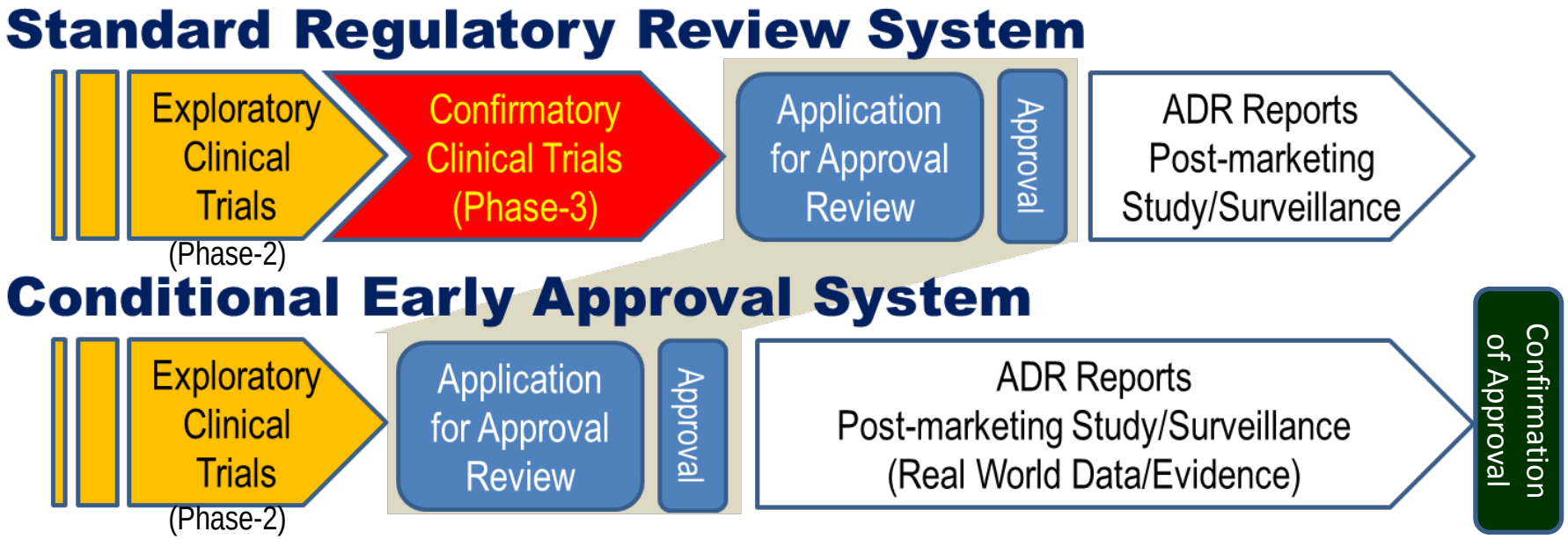
Expedited Pathways

Pathway types	Features	Accelerate: Development	Accelerate: Review
SAKIGAKE Breakthrough Therapy (PRIME)	Rolling Review Project Manager to lead the review (Priority consultation) （Surrogate or intermediate clinical endpoints?）	△	○
Fast Track	Rolling Review to reduce review period	-	○
Accelerated Approval	Approved on surrogate or intermediate clinical endpoints	○	-
Priority Review Accelerated Assessment	Reduced review period	-	○
Conditional Early Approval	**Approved on exploratory clinical data Post-marketing reconfirmation of safety and efficacy**	○	-

"Conditional Early Approval"
= Approval without Confirmatory Clinical Data?



At Submission/Approval Stage	Standard Approval Process	Conditional Early Approval Process
Confirmatory Clinical study (Phase-3) data	○	×
Review report after the approval	**Full Assessment** (*with efficacy and safety data*)	**Partial Assessment(?)** (*efficacy and safety: from post-marketing study/survey?)*

Aim of part-2 session

APAC Mission

"Expedite the launch of innovative medicines for the people in Asia"

How to accelerate/secure the access to innovative medicines approved through the CEA pathway in various Asian economies?

Challenges:

- Differences in the conditional early approval (CEA) system itself
- Differences in the data requirements for submission/approval
  - ✓ *With/without confirmatory clinical (=Phase-3) data*
  - ✓ *Domestic patient data*
- Referentiability of the review report of CEA-approved products
- Usage of post-marketing data from other economies

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Part-2. "Conditional Early Approval (CEA)" Systems in Asia

10:50~12:05 75min

Chair: John Lim (Duke-NUS CoRE), Masayoshi Shibatsuji (PMDA)

Presentation

・Explanation of this part	Inagaki (JPMA)	5 min
・Introduction of the CEA system in Japan	Shibatsuji (PMDA)	15min
・Introduction of the CEA system in Malaysia	Ramli Z. (NPRA)	10min

Panel Discussion with short presentation

・Short presentation: Views on CEA System in the economy 5minx3
SH Kim (NIFDS), YC Lin (TW-FDA), Juliati D (NADFC)

・Panel discussion 30min
Ramli Z (NPRA), SH Kim (NIFDS), YC Lin (TW-FDA),
Juliati D (NADFC)

Ramli Z (NPRA/Malaysia), SH Kim (NIFDS/Korea), YC Lin (TW-FDA/Taiwan),Juliati D (NADFC/Indonesia),

Short Presentation Questionnaire

Please include your opinion on the following three questions in your short presentation during the panel.

1. Dose your economy plan to introduce a Conditional Early Approval System (CEA)?
   - Otherwise, do you plan to introduce any other expedited / accelerated approval system? If yes, what are they?

2. What is your opinion on the new drug submission without confirmatory clinical data?

   In the case of the products approved through Conditional Early Approval pathways?
   In the case of medical products for rare / orphan diseases?
   - By any chance, will you (or your economy) request confirmatory clinical data in the submission dataset? If no, in what kind of situations?

3. What is your opinion of using foreign data in the post marketing evaluation of Conditional Early Approval systems?
   - Will it be possible to gather the post-marketing evaluation data through multi-regional collaboration?

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