7th APAC RA Session

Regulatory landscape for "Access to Innovative Medicine" in Asia
-"Conditional Early Approval (CEA)" Systems in Asia

Conditional Early Approvals in Taiwan

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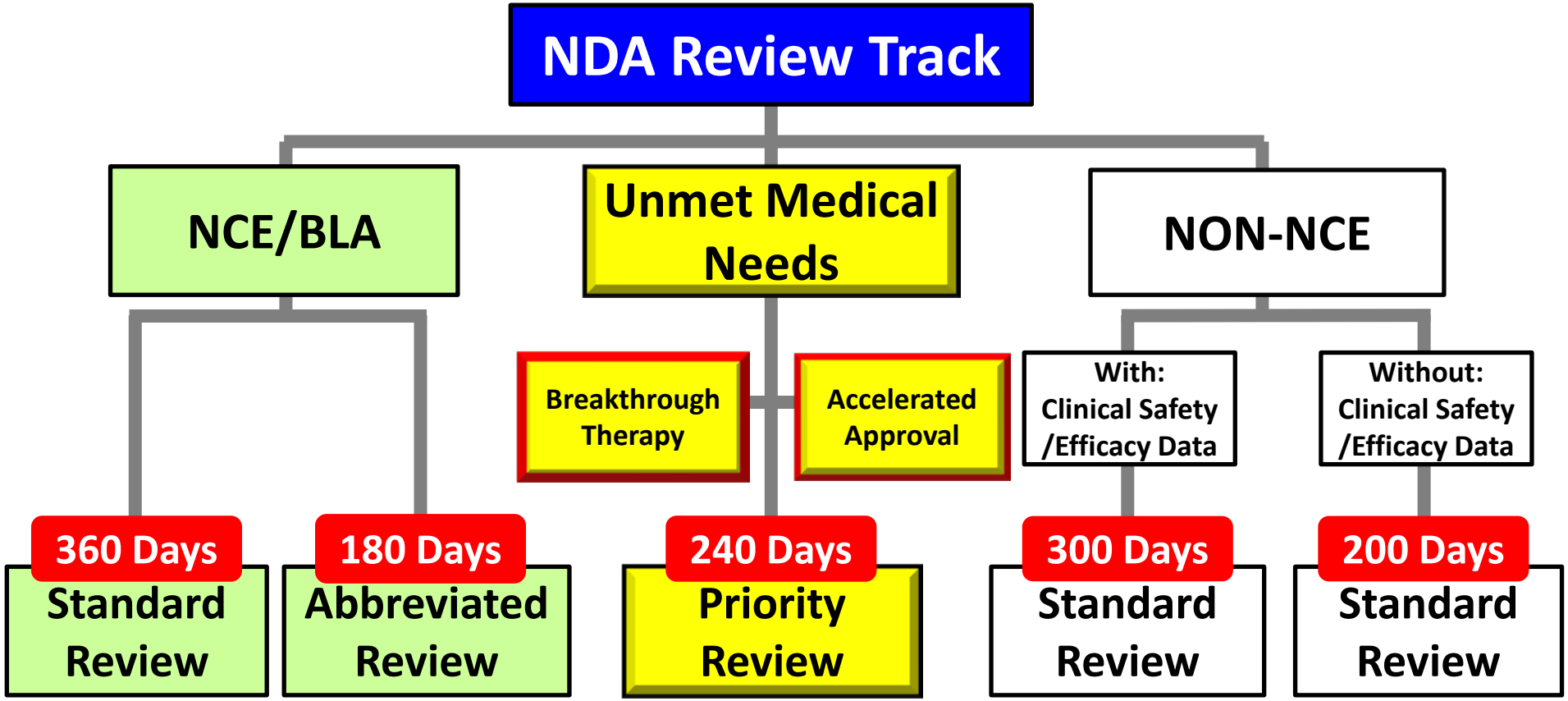
食品藥物管理署
FDA Food and Drug Administration
http://www.fda.gov.tw/

Expedited Programs for New Drug Application



- **Priority Review** meets the following criteria
  - new drug
  - unmet medical needs (serious disease or life-treating disease)
- **Abbreviated Review** meets the following criteria
  (1) NCE; (2) US FDA, EMA, MHLW approved (2 out of 3)

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Designations of Accelerated Approval (AA) and Breakthrough Therapy (BT)

	Accelerated Approval (Oct. 2013 ～)	Breakthrough Therapy (Feb. 2018 ～) NEW
Criteria	**1. New Drug** **2. Indication** A. with major advance for serious diseases for unmet medical need B. infectious disease C. orphan drug designation in the A10 countries or difficult to acquire/manufacture	**1. NCE or New indication for serious diseases/rare diseases** **2. Substantial Improvement** a. An effect on surrogate endpoint b. Efficacy predicable by surrogate / intermediate clinical endpoint c. Better safety profile **3. Clinical trial(s) in Taiwan**
Advantage	**1. Priority review** **2. Surrogate end point or small number of subjects**	**1. Priority Review** **2. Surrogate end point** **3. Regulatory consultation** **4. Rolling Review**
Requirement	**Post-approval Confirmatory Trials**	**Report to TFDA every 3 mon**

Short Presentation Questionnaire

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

1. Does 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 using the 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?

Facts about Conditional Early Approval -1

Q

Are phase 3 confirmatory clinical data the essential requitment for submission ?

A

For submissions with accelerated approval or breakthrough therapy designation：

1.phase 3 confirmatory clinical data are not essential for submission.

2.Post-marketing phase 3 confirmatory clinical data should be required before license renewal (5 year). It could be acceptable to gather the multiregional data.

Q

How do Taiwan utilize the assessment reports of new drugs approved through the accelerated/expedited processes in preceding countries?

A

1.Assessment reports can be submitted as supportive documents.

2.Abbreviated Review Mechanism has been established in Taiwan.

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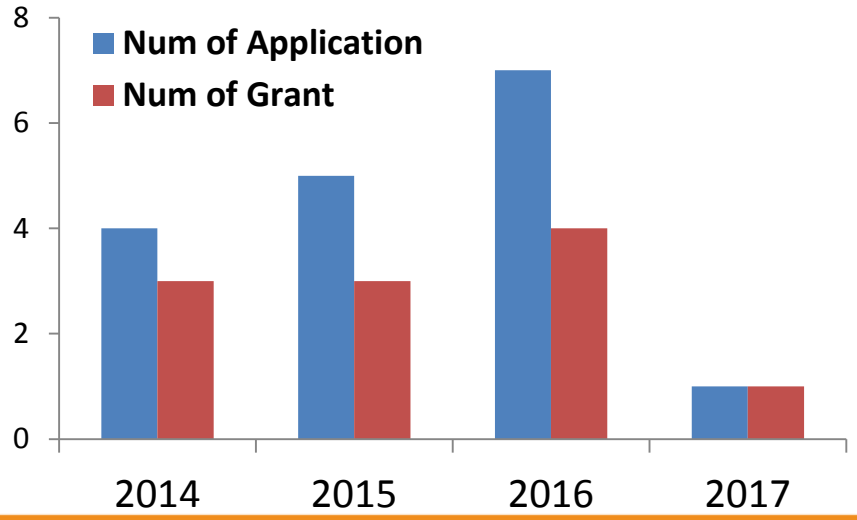
Facts about Conditional Early Approval -2

Q How many drugs have been approved with accelerated approval designation?

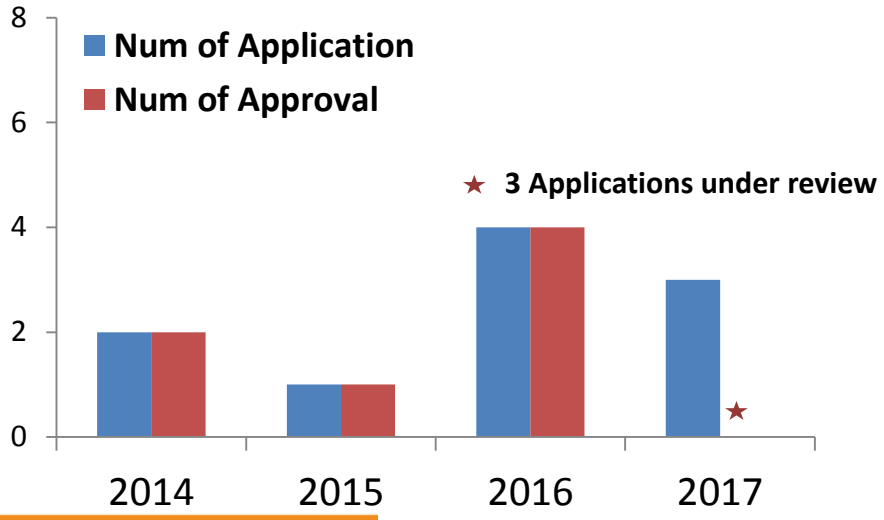
A

10 applications have been granted with accelerated approval designation.
-7 Drugs have been approved with accelerated approval.
-3 applications are under review.

Designation of Accelerated Approval



NDA Approval by Accelerated Approval



Drugs Approved with Accelerated Approval (AA)

Drug	Indication	Post-approval Commitment
Darzalex Concentrate for solution for infusion	Cancer	✔
Tecentriq	Cancer	✔
Tagrisso Film-coated Tablets	Cancer	✔
SirturoTablet 100mg	MDR-TB	✔
Imbruvica Capsules	Cancer	✔
Sylvant® Powder for Concentrate for Solution for Infusion	MCD	✔
Blincyto for Injection	Cancer	✔

MDR-TB：Multidrug-resistant tuberculosis;
MCD：Multicentric Castleman's Disease

Question 1:
Dose your economy plan to introduce a Conditional Early Approval System (CEA)?

- Taiwan has announced CEA to approve new drugs for unmet medical needs.
  - Accelerated Approval designation was introduced in October 2013.
  - Breakthrough Therapy designation was introduced in February 2018.

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

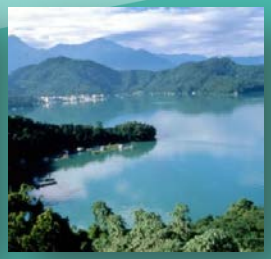
For submissions with accelerated approval or breakthrough therapy designation：

- Phase 3 confirmatory clinical data are not essential for submission.
- Post-marketing Phase 3 confirmatory clinical data should be required before license renewal.

Question 3:
What is your opinion using the foreign data in the post marketing evaluation of Conditional Early Approval systems?

- It could be acceptable to gather the multiregional data in the post-marketing evaluation of Conditional Early Approval systems.
- Using real world data for Conditional Early Approval is under discussion.



Thank You for Your Attention

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