Myth Busters

What you think you know about the FDA may not be true

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Audience Participation

- Please pick up the remote on your table and prepare to respond to the statements that follow
  - Please respond with your remote

    True (Fact)

    or

    False (Myth)

#1

FDA's OHOP requires two randomized trials for approval of a new product.

Answer: MYTH

Myth #1: Two Trials

- Two trial requirement
  - One trial may be allowed in the following settings*:
    - High unmet medical need
    - Serious, life-threatening illnesses
    - Difficult to repeat a positive trial

* FDAMA 1997

#2

- FDA's OHOP requires that trials use overall survival as the primary endpoint for approval of a new product

- Answer: MYTH

Myth # 2: Endpoints

	2017 (As of 10/21/17)
Total # New Molecular Entities (NME)	15
Response Rate	9
Progression Free Survival	3
Overall Survival	2
Other	1

#3

- FDA's OHOP requires that trials to support approvals only enroll patient in the U.S.

- Answer: MYTH

Myth # 3: U.S. Enrollment

- Trials are not required to be limited to the U.S. population, or even mostly in a U.S. population…
- However, the results should be relevant to the U.S. population:
  - Patient population
  - Control arm
  - Biomarker data
  - Supportive care measures
  - Available prior/subsequent therapy

4

- FDA's OHOP permits crossover in trials

- Answer: FACT

Fact # 4: Crossover

- Recent approvals where crossover was permitted at time of progression
  - Dabrafenib and trametinib in metastatic melanoma
  - Erlotinib in non-small cell lung cancer
  - Afatinib in non-small cell lung cancer
  - Crizotinib in non-small cell lung cancer

5

- FDA advisory committees, such as the ODAC (Oncologic Drug Advisory Committee), are the final decision makers in drug approval.

- Answer: MYTH

Myth # 5: Advisory Comm

- Advisory committees provide opinions based upon their clinical and scientific expertise.

- FDA generally follows an AC's recommendation, but is not bound to do so.

6

- FDA staff lack scientific and clinical expertise.

- Answer: MYTH

Myth # 6: Expertise

- FDA Review Multidisciplinary Team
  - Clinical
  - Biostatistics
  - Clinical Pharmacology
  - Chemistry and Manufacturing
  - Pharmacology/Toxicology

7

- FDA permits access to investigational drugs (e.g.,compassionate use)

- Answer: FACT

Fact # 7: Compassionate Use

- Steps to gain access to investigational drug:
  - Patient's physician contacts drug company that makes the drug of interest
  - Company agrees to provide drug to patient
  - Single-patient IND (Form 3926) submitted to FDA by physician
  - Application reviewed by FDA staff within 24-48 hours
  - In 2016, FDA approved 99% of all expanded access requests

8

- FDA determines the cost of anticancer agents

- Answer: MYTH

Myth # 8: Drug Cost

- No legal authority over drug costs
- Cost savings with generics and biosimilar products
- Reduced drug development expenses
- Sources of assistance:
  - Drug manufacturer patient assistance programs
  - Ask your health-care provider if your drugs are available in a generic form

9

- FDA takes the patient experience into account in approval decisions and encourages the use of patient-reported outcome measures in clinical trials.

- Answer: FACT

Fact # 9: Patient Experience

- Advisory Committee: Patient Representative
- 2009 Patient Reported Outcomes Guidance
- Patient Representative Program
- PROs in Labeling: Hycela and Imbruvica
- Patient Focused Drug Development Meetings

10

- FDA may publish its reasons for non-approval of a drug

- Answer: MYTH

Myth # 10: Negative Reviews

- By law, FDA is NOT permitted to post reviews or letters to the Applicant for products that do not receive approval

- Reviews and approval letters for approvals ARE posted.

11

- FDA discusses their approval decisions with regulatory agencies around the world prior to taking action.

- Answer: FACT

FACT # 11: Foreign Regulators

- We do talk to each other!
  - Monthly teleconferences with FIVE international regulatory agencies
    - European Medicines Agency
    - Health Canada
    - Pharmaceuticals & Medical Devices Agency (Japan)
    - Swissmedic (Switzerland)
    - Therapeutic Goods Administration (Australia)

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