FDA U.S. FOOD & DRUG ADMINISTRATION

Pediatric Exclusivity

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Presented by:
Kristiana Brugger

Disclaimer:

This presentation reflects the views of the author and should not be construed to represent FDA's views or policies.

Overview

- History/Purpose of BPCA
- Pediatric Exclusivity: The Basics
- PPSRs and Written Requests
- Requirements to “Fairly Respond”
- Recap
- Resources

Pediatric Exclusivity: What Is It?

- Financial incentive to study drugs in children
  - "information relating to the use of a new drug in the pediatric population may produce health benefits in that population"
- Voluntary
  - vs. PREA studies (required)
- 6 months of exclusivity added to existing patent or exclusivity life
  - "listed patent"
  - NCE, 3-year, orphan exclusivities

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Problem: drugs not labeled for children

- Historically:
  - Children rarely included in clinical trials
  - Few pediatric indications, dosing, safety information
  - Few pediatric formulations
  - Off-label use common
- Children = “therapeutic orphans”

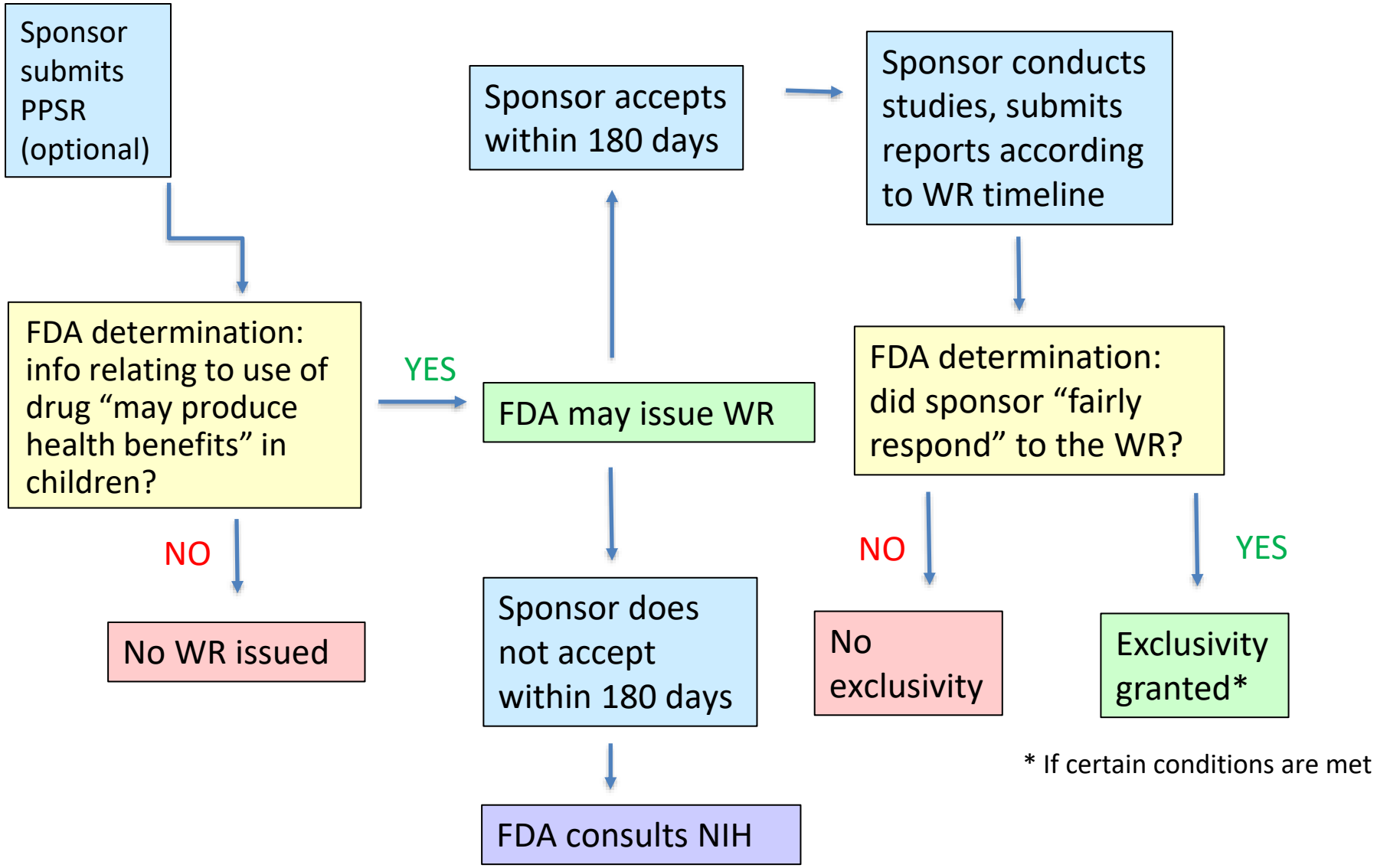
(One) Solution: Best Pharmaceuticals for Children Act

- Enacted 2002
  - codified at section 505A of the FD&C Act (21 U.S.C. 355a)
- Created pediatric exclusivity as an add-on incentive for drugs with existing patent or exclusivity
- FDA determines which drugs deserve the incentive
  - Established standard for written request: "may produce health benefits"
  - FDA determines whether sponsors "fairly respond"
  - Determination must be made at least 9 months before expiration of patent/exclusivity
- Intent: more drugs studied, labeled for use in children

Contrast: Pediatric Research Equity Act

- Enacted 2003
  - Codified at section 505B of the FD&C Act (21 USC 355c)
- Pediatric studies required
  - Original applications or supplements for
    - New active ingredient
    - New indication
    - New dosage form
    - New dosing regimen
    - New route of administration
  - Original applications for
    - Oncology drugs for an adult indication with a molecular target determined to be “substantially relevant to the growth or progression of a pediatric cancer”
    - Studies conducted on the pediatric cancer, not the adult cancer
- Intent: more drugs studied, labeled for use in children

BPCA Process: Overview



* If certain conditions are met

Proposed Pediatric Study Plans (PPSRs)

- Sponsor submission to FDA
  - Describes proposed studies
  - Requests issuance of written request (WR)
  - Optional: sponsor could still receive WR without PPSR
- Reviewed by Pediatric Review Committee
- FDA has 120 days to respond
  - Issuance of written request, or
  - PPSR inadequate letter

Written Requests

- FDA may issue WRs if "information relating to the use of a new drug in the pediatric population may produce health benefits in that population"
  - May include multiple indications, approved or unapproved
  - May be issued at any time during drug development
- Required for pediatric exclusivity
- Reviewed by Pediatric Review Committee
- FDA consults with sponsor
- Sponsor must accept/reject within 180 days

Written Requests (cont'd)

- FDA takes into account "adequate representation of children of ethnic and racial minorities"
- FDA includes neonates, or explains why neonates are not included in study request
- WR includes a request for sponsor to propose labeling resulting from studies

Studies Conducted Pursuant to WR

- Conducted with “appropriate pediatric formulation” for each age group under study
- Submitted according to the timeline set forth in the WR
- FDA has 180 days to review the studies and make an exclusivity determination

Exclusivity Determination

✓Were the studies…?

- conducted using appropriate pediatric formulation
- “conducted in accordance with commonly accepted scientific principles and protocols”
- submitted in accordance with WR timelines
- reported in accordance with filing requirements

Exclusivity Determination (cont'd)

- ✓ Did the sponsor "fairly respond"? (*Amgen v. HHS)*
  - Prong 1: Sponsor meets all terms, **or**
  - Prong 2: Sponsor provides "clinically meaningful information across all age groups and uses cited"

- ✓ As of determination date, are there at least 9 months of patent/exclusivity life left?

Additional Expectations, Requirements

- FDA must order study information to be added to labeling, whether positive, negative, or inconclusive
- Labeling supplements with this pediatric information get priority review (6 month target date)
- Sponsors of a safe and effective pediatric formulation who fail to market that formulation within 1 year of exclusivity grant must be listed on public list
- FDA required to provide certain information about pediatric labeling, reviews of pediatric studies, exclusivity grants

Challenge Question #1

1) Which of the following **is necessary** for FDA to issue a written request?

A) A proposed pediatric study request (PPSR) from the sponsor

B) A determination that information about the drug "may produce health benefits" in pediatric populations

C) A user fee equivalent to that required for an original marketing application

Challenge Question #2

2) Which of the following **is not necessary** for FDA to grant exclusivity?

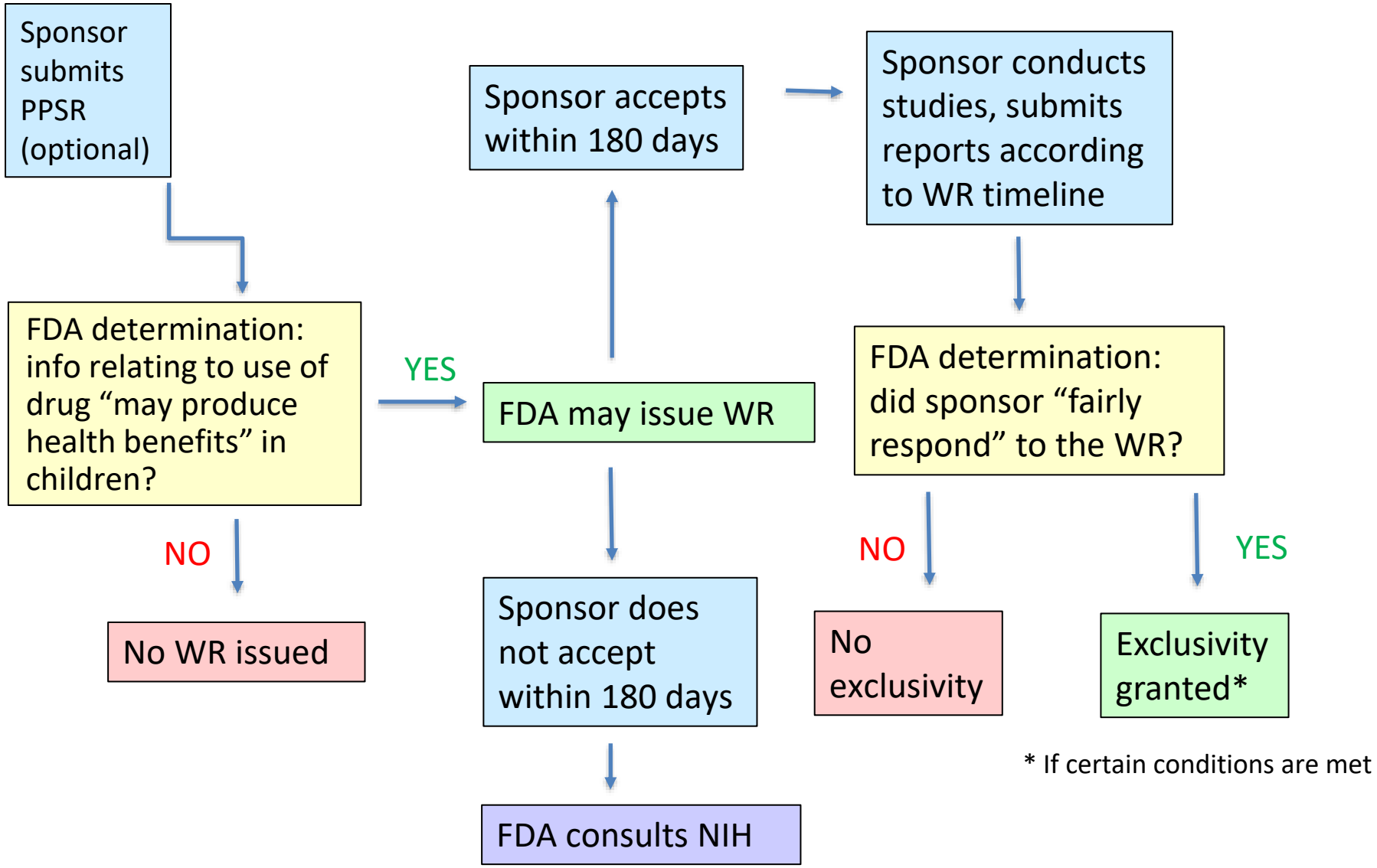
A) 9+ months remaining of qualifying patent or exclusivity life

B) FDA determination that sponsor "fairly respond[ed]" to the written request

C) Studies conducted using appropriate pediatric formulation

D) Studies submitted within one year of timeline specified in written request

Pediatric Exclusivity Process: Recap



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Suggested further reading:

- https://www.fda.gov/science-research/science-and-research-special-topics/pediatrics
- https://www.fda.gov/drugs/development-resources/best-pharmaceuticals-children-act-bpca

Thank you!


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