# Orphan Drug Exclusivity

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Celebrating 40 Years: An In-Depth Examination of the FDA Orange Book – October 28, 2020

# Learning Objectives

- Define “orphan drug”
- Describe the scope of orphan-drug exclusivity
- Identify when clinical superiority needs to be demonstrated
- Locate orphan-drug exclusivity information

# Orphan Drug Act

- Passed in 1983
- Drugs or biologics for rare diseases
- Created a designation process
- Financial incentives

# Definition of Rare Disease

- Disease affects < 200,000
- Disease affects > 200,000, but no expectation of cost recovery
  - Very uncommon

# Orphan Incentives

- 25% tax credit for qualified clinical trials
- Waiver of user fee
- Grants to support studies of orphan products
- Potential for 7-year orphan drug exclusivity (ODE)

# Eligibility for ODE

- Orphan drug designation
- Approval for orphan disease
  - Approved indication often narrower than designation
- Must be for first approval of drug for indication
  - Clinical Superiority

# Scope of ODE

- 7 years from approval
- FDA cannot approve another sponsor's
  - Application
  - For the same drug
  - For the same use or indication
- Unless subsequent drug is clinically superior

# Application

- NDA: 505(b)(1) or 505(b)(2)
- ANDA
- BLA

# Same Drug

- Same active moiety; or
- Same principal molecular structural features
- Unless clinically superior

# Same Use or Indication

- Indication defined by marketing approval
- Does not protect other indications within the rare disease or condition

# Clinical Superiority Definition

- Significant therapeutic advantage:
  - Greater effectiveness
  - Greater safety
  - Major contribution to patient care

# Clinical Superiority Scenarios

- To “break” existing ODE:
  - Clinically superior to drug with ODE
- To be eligible for ODE:
  - Clinically superior to all same drugs approved for same indication

# ODE Exceptions

- Insufficient quantities
- Waiver

# ODE Review

- 7 years from approval
- FDA cannot approve another sponsor's
  - Application
  - For the same drug
  - For the same use or indication
- Unless subsequent drug is clinically superior

# ODE on Orphan Website

https://www.accessdata.fda.gov/scripts/opdlisting/oopd/

## Search Orphan Drug Designations and Approvals

FDA Home › Developing Products for Rare Diseases & Conditions

**Results for Approved Products**

Return to Orphan Products Designation Search Page

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Number of Orphan Drug Approvals: 1

| # | Generic Name | Orphan Designation | Designation Date | Marketing Approval Date | Exclusivity End | Approved Labeled Indication | Exclusivity Protected Indication* |
|---|---|---|---|---|---|---|---|
| 1 | givosiran | Treatment of acute hepatic porphyria | 08/29/2016 | 11/20/2019 | 11/20/2026 | GIVLAARI is indicated for the treatment of adults with acute hepatic porphyria (AHP). | Indicated for the treatment of adults with acute hepatic porphyria (AHP). |

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*Exclusivity Protected Indications are shown for approvals from Jan. 1, 2013, to the present.

# ODE in Orange Book

**Patent and Exclusivity for: N212194**

Product 001
GIVOSIRAN SODIUM (GIVLAARI) SOLUTION EQ 189MG BASE/ML (EQ 189MG BASE/ML)

**Patent Data**

| Product No | Patent No | Patent Expiration | Drug Substance | Drug Product | Patent Use Code | Delist Requested | Submission Date |
|---|---|---|---|---|---|---|---|
| 001 | 8106022 | 12/12/2029 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 8546143 | 01/09/2022 | DS | | U-2672 | | 12/12/2019 |
| 001 | 8828956 | 12/04/2028 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 9133461 | 05/14/2033 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 9150605 | 08/28/2025 | DS | DP | | | 12/12/2019 |
| 001 | 9631193 | 03/15/2033 | | | U-2672 | | 12/12/2019 |
| 001 | 9708610 | 01/01/2024 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 9708615 | 03/08/2024 | DS | | | | 12/12/2019 |
| 001 | 10119143 | 10/03/2034 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 10125364 | 03/15/2033 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 10131907 | 08/24/2028 | DS | DP | U-2672 | | 12/12/2019 |
| 001 | 10273477 | 03/08/2024 | DS | | | | 12/12/2019 |

INDICATED FOR THE TREATMENT OF ADULTS WITH ACUTE HEPATIC PORPHYRIA (AHP)

| | sivity Code | Exclusivity Expiration |
|---|---|---|
| | | 11/20/2024 |
| 001 | ODE-273 | 11/20/2026 |

# Challenge Question #1

**ODE means that FDA cannot approve another sponsor's application for:**

A. The same drug for any indication

B. The same drug for the same indication

C. The same drug for the same orphan disease

D. Any drug for the same indication

# Challenge Question #2

**ODE lasts for how long?**

A. 3 years from designation

B. 7 years from designation

C. 3 years from approval

D. 7 years from approval

# Summary

- First step: Orphan-drug designation
- ODE: FDA cannot approve another sponsor's application for same drug for same indication
- Clinical Superiority
- OOPD website and Orange Book

U.S. FOOD & DRUG ADMINISTRATION

# Questions?

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