
Navigating Uncharted Waters:
A Fresh Perspective on Rare Disease Treatment Launches
Keep reading for the latest trends in the rare disease space
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Why Pharma Companies are Focusing on Rare Diseases

+7000 rare diseases have been identified, with more being discovered every day

While each disease affects few people, collectively many lives are affected.

>90% of rare diseases are without an FDA-approved treatment

The FDA has approved >600 drugs since the passage of the Orphan Drug Act

Most rare diseases are genetic or have a genetic component

All pediatric cancers are rare

Sources: National Organizations for Rare Disorders (NORD); National Human Genome Research Institute; Global Genes

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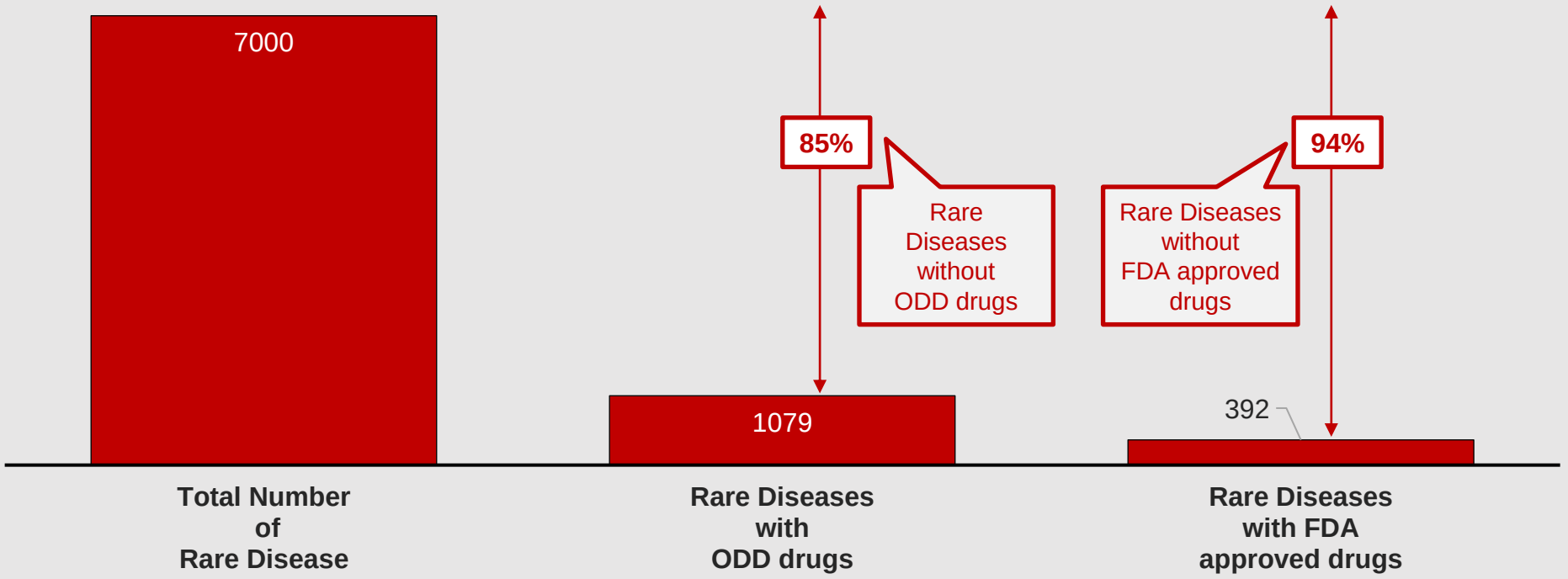
The FDA Recognizes the Significant Opportunity in Rare Diseases

54% of novel drug approvals in 2022 (20 out of 37 by CDER) were for rare diseases, yet more work is needed

Low Approval Rate	Slow Development	Lack of Investment
Only 6% of orphan drugs in development reach approval, half the success rate of drugs overall (12%)	Orphan drug development takes 4 years longer than drugs for common conditions	~85% of rare diseases lack ODD, while ~94% lack FDA-approved drugs, signaling insufficient research and investment

Number of Rare Diseases with Orphan Drug Designation (ODD) or FDA Approval (1983-2022)



Sources: Lewis J. Fermaglich, “A comprehensive study of the rare diseases…”, 2023; PhRMA, “2012-21 A Decade of Innovation in Rare Diseases”, 2022; FDA is Working to Bridge Gaps, 2019; FDA, “New Drug Therapy Approvals”, 2022;

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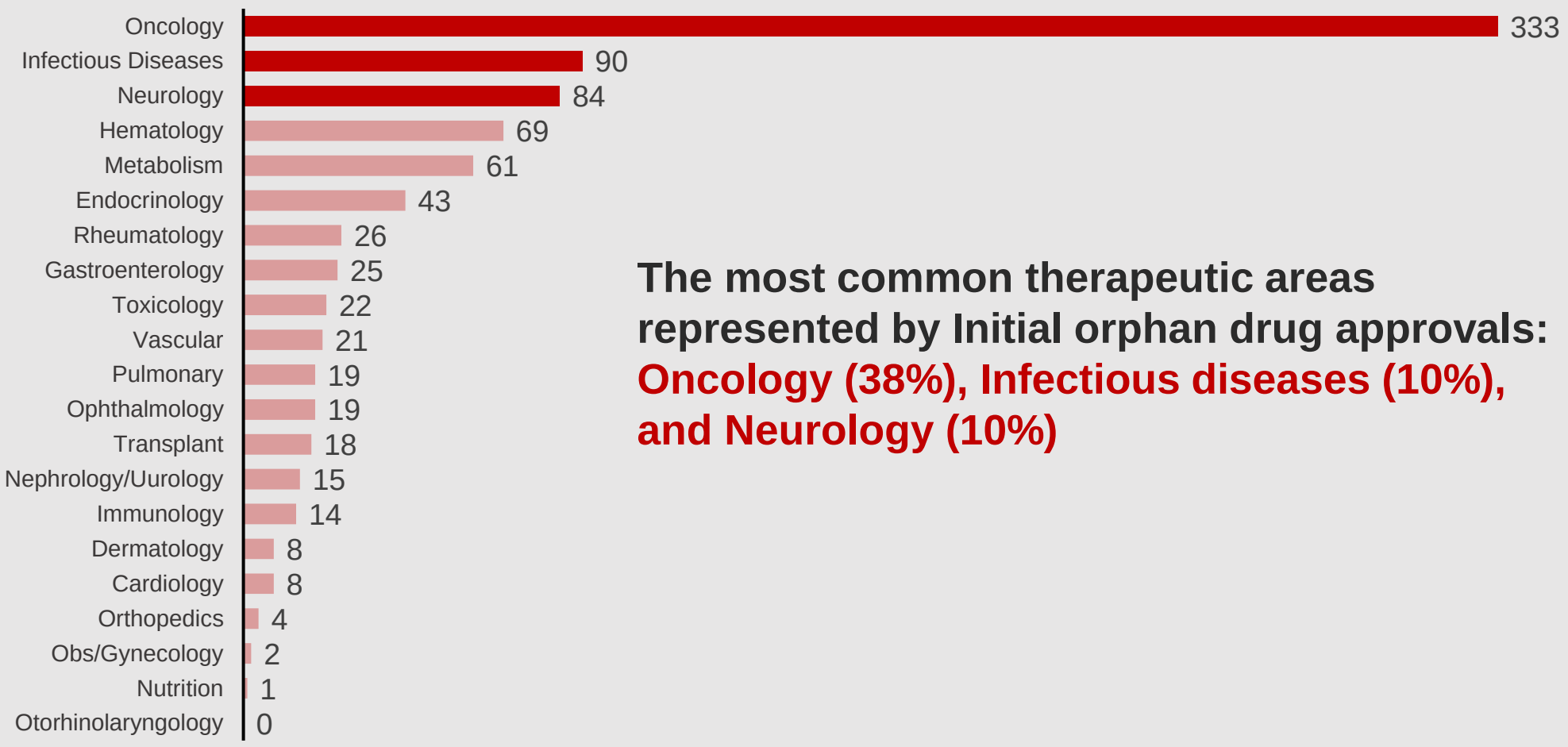
Oncology Therapies Dominate the Orphan Drug Landscape

What Therapeutic Area Are You Focused On?



The most common therapeutic areas represented by Initial orphan drug approvals: Oncology (38%), Infectious diseases (10%), and Neurology (10%)

Sources: Lewis J. Fermaglich, “A comprehensive study of the rare diseases…”, 2023

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Orphan Drug Designation Provides Many Opportunities

Are You Taking Advantage of *All* the FDA Incentives?

- **25% Tax Credits for Applicable R&D Cost**
- **Financial Grants**
- **Fee Reductions and Waivers**
- **Increased Access to Regulatory Agencies**
- **Accelerated Time to Market – Priority Review**
- **Extended Periods of Market Exclusivity**

Sources: Life Science Leader, “Orphan Drug Incentives & Innovations On The Rise”, 2017; US FDA, “FDA is Working to Bridge Gaps”, 2019

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Stepwise Approach to Navigate Rare Disease Drug Development & Commercialization

How Can You Get to Market Faster and Stronger?

Preclinical

- Understanding the disease
- Identifying and validating drug targets
- Developing preclinical models

Clinical

- Identifying trial sites
- Geographic dispersion and small patient population
- Recruiting and retaining patients
- Validated outcome measures
- Ethical considerations – pediatric trials

Regulatory

- Meeting FDA requirements
- Need for additional data
- Obtaining marketing approval

Commercial

- Raising awareness of the new drug
- Improving access to the new drug
- Delayed Diagnosis
- Recouping the cost of drug development
- Generating a profit

Sources: The challenges of clinical trials in rare diseases, Jemima E, 2022

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