National MPS Society
Support for Families. Research for a Cure.

Advocacy Committee

June 20, 2018

Orphan Drug Act Review (from NORD)
and
In District Lobby Days

Make Sure Congress Hears from the MPS Community This Summer!

Rare Disease Legislative Advocates (RDLA) is hosting In-District Lobby Days to facilitate meetings for rare disease advocates across the country with members of Congress in their local offices during the 2018 summer in-district work period (July 31st - Sept. 4th).

Registration for In-District Lobby Days is FREE and open through July 4th. You will be provided the option to specify you availability and the distance you are willing to travel for a meeting.

All In-District Lobby Day participants will be invited to a webinar on Thursday, July 26th, at 2pm EST to go over key legislative issues and tips for successful meetings. Legislative asks will be available on RDLA's website by late July.

Check out the MPS Society's Advocacy page for resources.

The Orphan Drug Act of 1983

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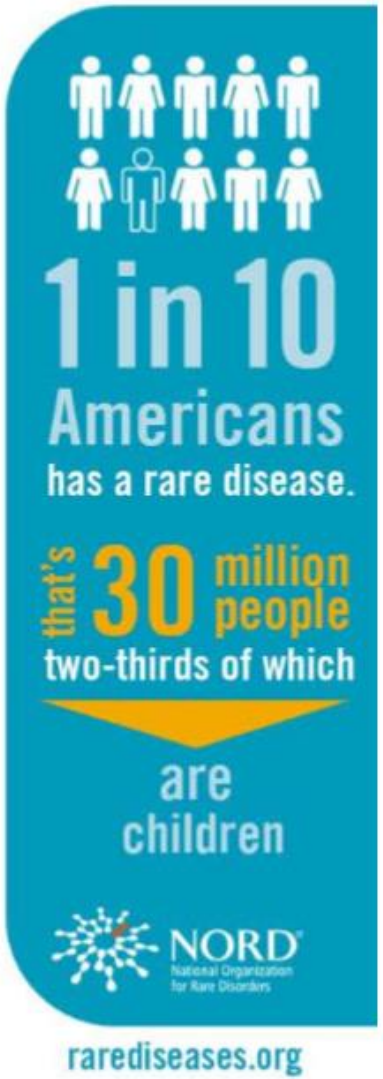
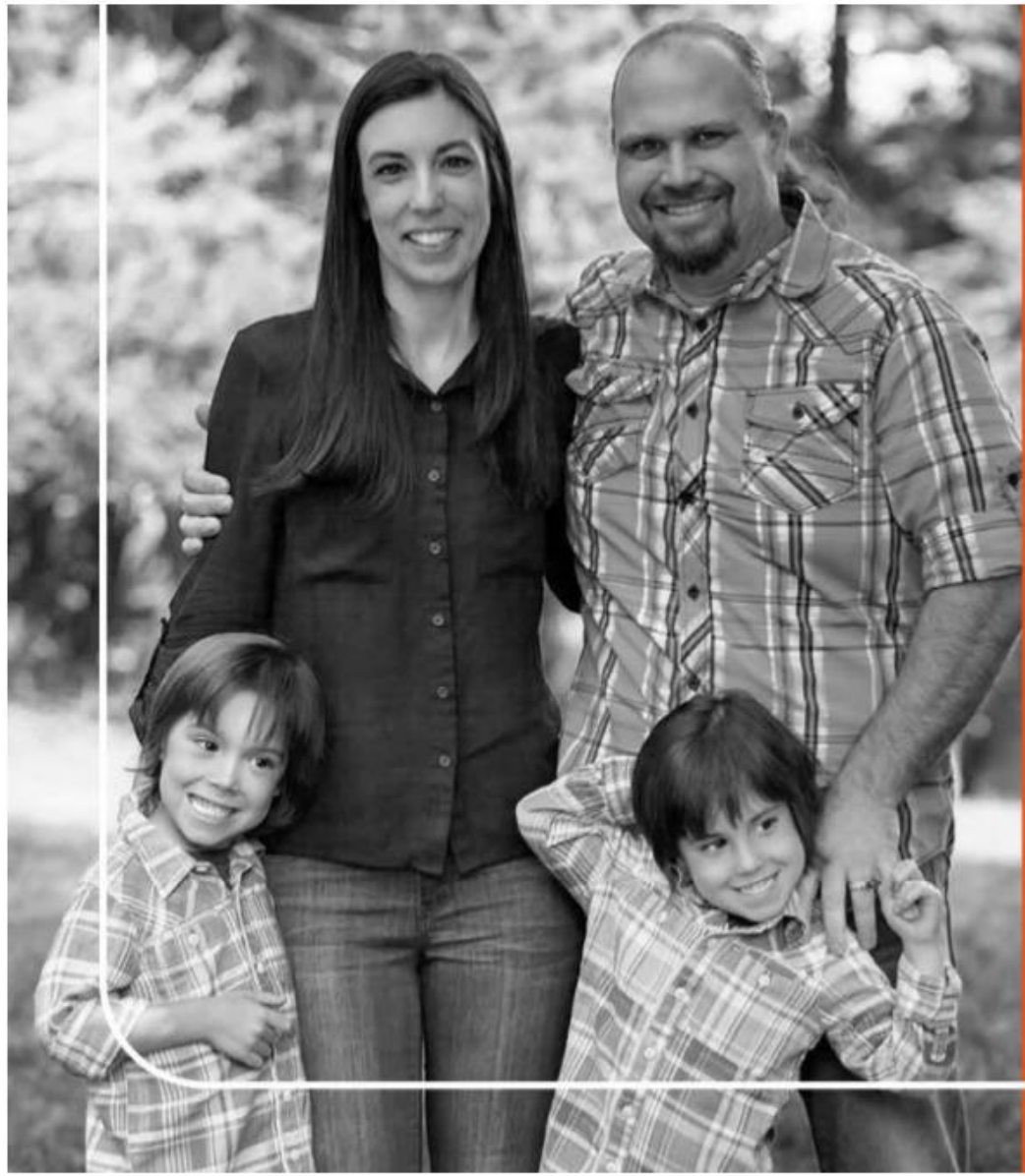
The Orphan Drug Act

- In the decade before 1983, only 10 new treatments were brought to market by industry for diseases that today would be defined as rare.

- A total of 34 orphan therapies approved by FDA were in existence.





What Incentives Are in the Bill?

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Orphan Drug Designation


Daily News

1983

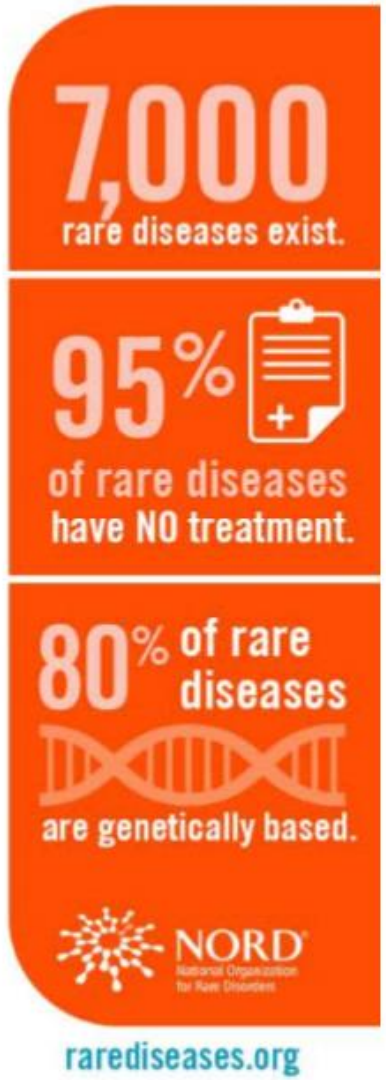
Congress Passes
Orphan Drug Act

First, the drug must meet the qualifications of:

- Small molecule drugs (Section 505 of the FD&C Act)

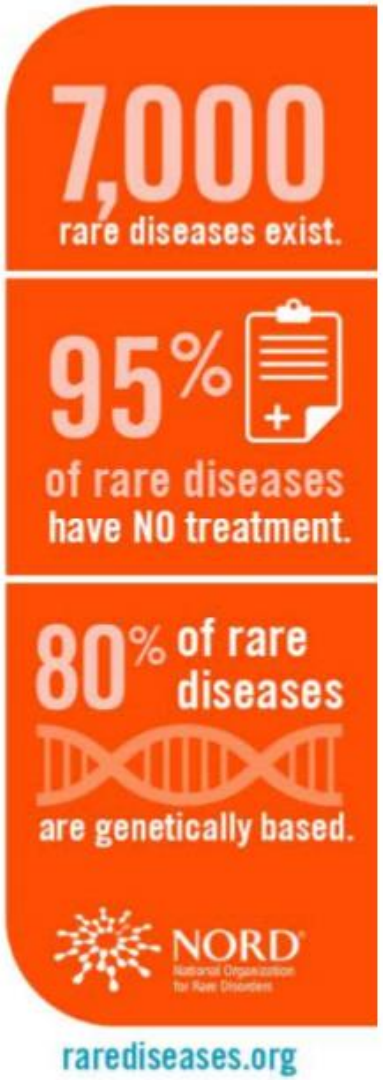
OR

- Large molecule biologics (Section 351 of the Public Health Services Act)

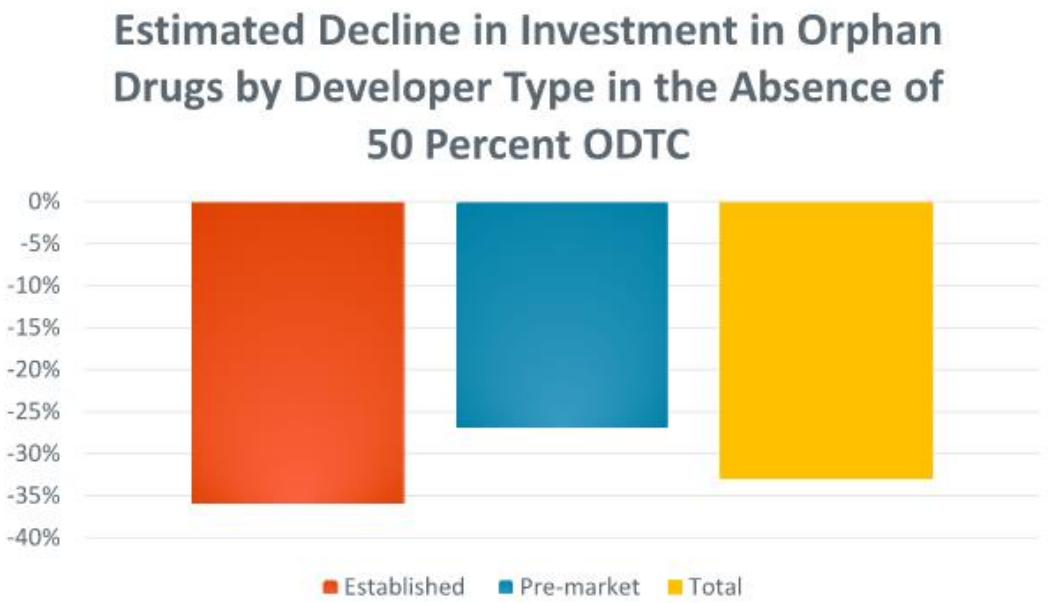


Orphan Drug Designation

- FDA determines whether the drug is “intended to treat a rare disease or condition.”
- Defined by:
  - “Affects less than 200,000 people in the U.S.”
  - “Affects more than 200,000 people in the U.S., but there is no reasonable expectation a company will recover costs through sales.”
- Usually determined in early clinical testing stages.



Orphan Drug Tax Credit



- Allows companies to claim a tax credit of up to ~~50%~~ 25% of their qualified clinical testing expenses.
- Not all companies opt to take the ODTC, instead choosing to take the R&D credit.

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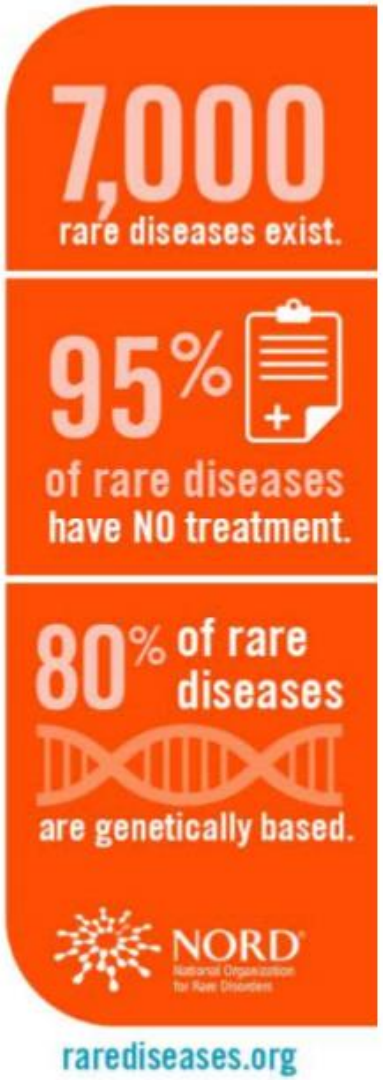
Orphan Products Clinical Trials Grants Program

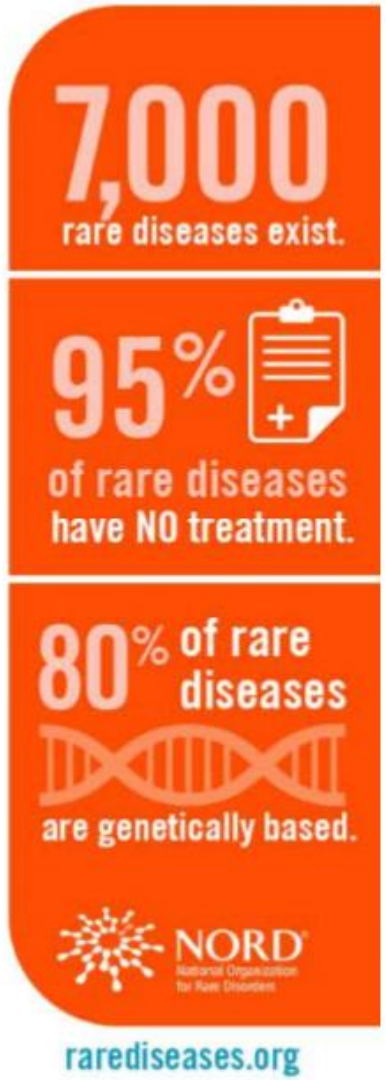
- Approximately $15 million in grants per year.
- Received over 2,500 applications (100 per year).
- Reviewed over 2,300 applications.
- Funded over 700 studies.
- Over 60 drugs on the market benefited from funding from the Orphan Grants Program.



User Fee Exemption

- Added in 1992
- FY2017 User Fees –
  - $2,421,495 for an application requiring clinical data
  - $1,210,748 for an application not requiring clinical data or a supplement requiring clinical data
  - $304,162 program fee

PRESCRIPTION DRUG USER FEE ACT



The Orphan Drug Act

Figure 3. Available ODA assistance during development timeline for orphan drugs

Source: Joseph A. DiMasi and Henry G. Grabowski, "The cost of biopharmaceutical R&D: is biotech different?" *Managerial and Decision Economics*, (John Wiley & Sons, Ltd, 2007); EY analysis.

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What Other Incentives Exist for Orphan Drug Development?

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Other Incentives for Orphan Drug Development

- Incentives that "exempt" orphan drugs from certain requirements or programs, including:
  - Affordable Care Act Pharmaceutical Fee,
  - Pediatric Research Equity Act Requirements (for non-oncology drugs), and
  - 340(b) pricing.

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Other Incentives for Orphan Drug Development

- Priority Review Vouchers are awarded for products that treat:
  - Rare pediatric diseases, or
  - Tropical infectious diseases.

Credit: Jonah's Just Begun

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Other Incentives for Orphan Drug Development

- FDA will often review orphan drugs more quickly and flexibly:
  - Over 80 percent of orphan drugs qualified for an “expedited review pathway.”
  - Nearly 80 percent of orphan drugs were reviewed “flexibly” by FDA.

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Have these Incentives Worked?

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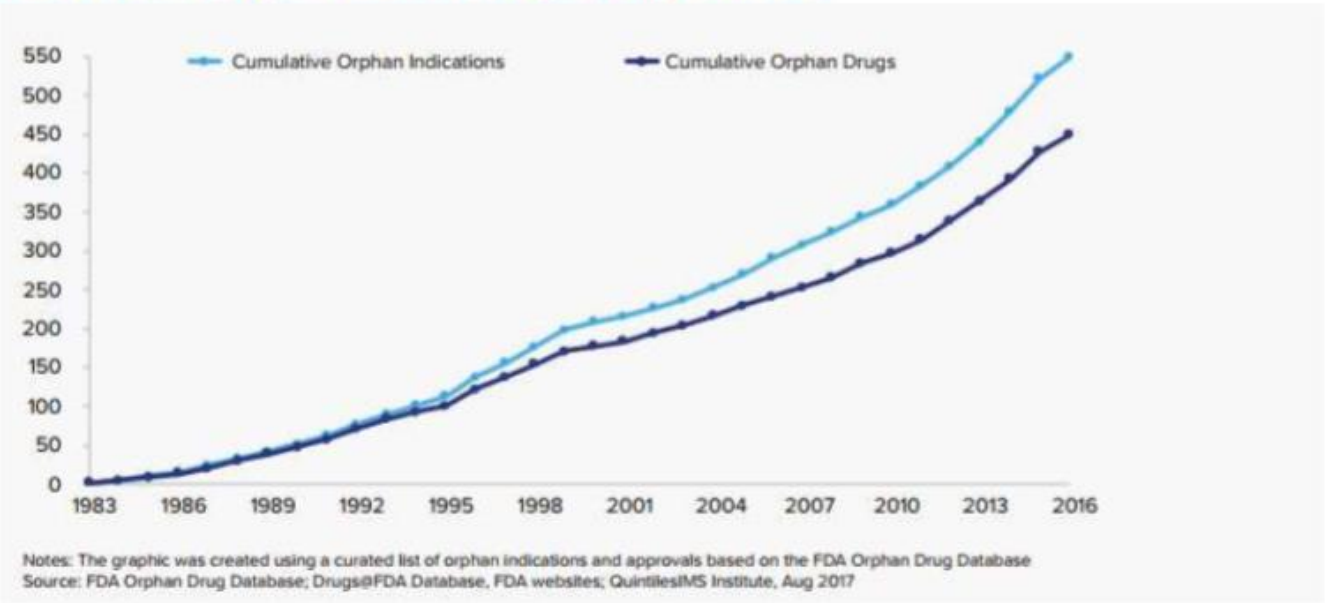
Have These Incentives Worked?

Exhibit 3: Number of Orphan Indications and Orphan Drugs 1983–2016



Notes: The graphic was created using a curated list of orphan indications and approvals based on the FDA Orphan Drug Database
Source: FDA Orphan Drug Database; Drugs@FDA Database, FDA websites; QuintilesIMS Institute, Aug 2017

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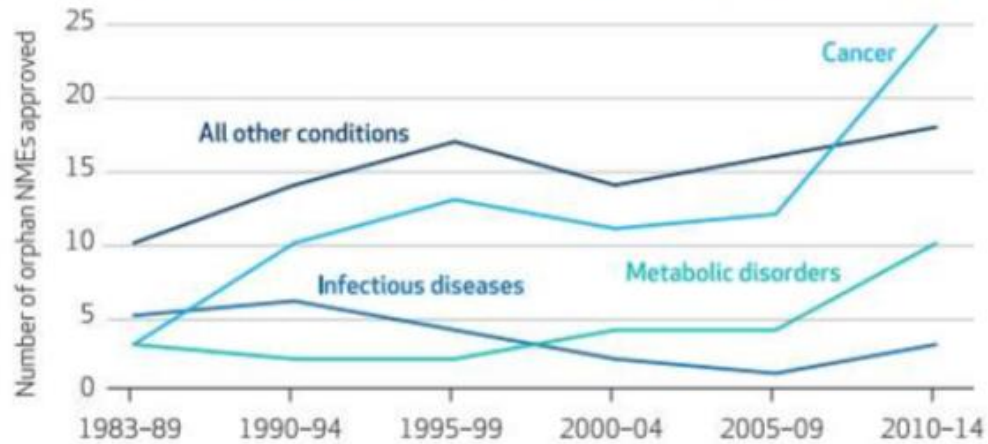
Have These Incentives Worked?

Number of orphan new molecular entities (NMEs) approved, by therapeutic category



SOURCE Authors' analysis of drug approval data from the Food and Drug Administration. **NOTE** The first time period, 1983–89, is two years longer than the other time periods, to account for the fact that the study period could not be divided evenly into five-year periods.

Credit: Kathleen Miller and Michael Lanthier "Trends in Orphan New Molecular Entities, 1983 – 2014: Half Were First in Class, And Rare Cancers Were the Most Frequent Target", Health Affairs, March 2016

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Have These Incentives Worked?

Orphan new molecular entities (NMEs) approved during 1983–2014, by therapeutic category

Therapeutic category	Number of orphan NMEs: All	Number of orphan NMEs: With genetic origin	Examples of conditions treated
Antidote	7	0	Lead poisoning, radiation poisoning
Cancer	74	0	Leukemia, mesothelioma, multiple myeloma, renal cell carcinoma
Cardiovascular diseases	8	0	Pulmonary arterial hypertension
Endocrine disorders	7	0	Cushing's disease, growth failure
Gastrointestinal disorders	7	1	Cholesterol biliary stones, short bowel syndrome
Hematologic disorders	12	3	*Hereditary angioedema*, essential thrombocythemia
Immunologic disorders	11	4	Castleman's disease, *cryopyrin-associated periodic syndromes*, organ transplant rejection
Infectious diseases	21	0	HIV, malaria, tuberculosis
Metabolic disorders	25	23	*Gaucher disease, hyperphenylalaninemia, mucopolysaccharidosis 6, urea cycle disorders*
Muscular or skeletal disorders	2	0	Dupuytren's contracture, Paget's disease
Neurologic disorders	20	1	*Huntington's disease*, multiple sclerosis, narcolepsy
Pulmonary diseases	9	2	*Cystic fibrosis*, idiopathic pulmonary fibrosis, respiratory distress syndrome
Renal or urinary disorders	6	1	*Nephropathic cystinosis*, secondary hyperparathyroidism

SOURCE Authors' analysis of drug approval data from the Food and Drug Administration. **NOTE** Diseases in italics were classified as genetic conditions.

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Credit: Kathleen Miller and Michael Lanthier "Trends in Orphan New Molecular Entities, 1983 – 2014: Half Were First in Class, And Rare Cancers Were the Most Frequent Target", Health Affairs, March 2016

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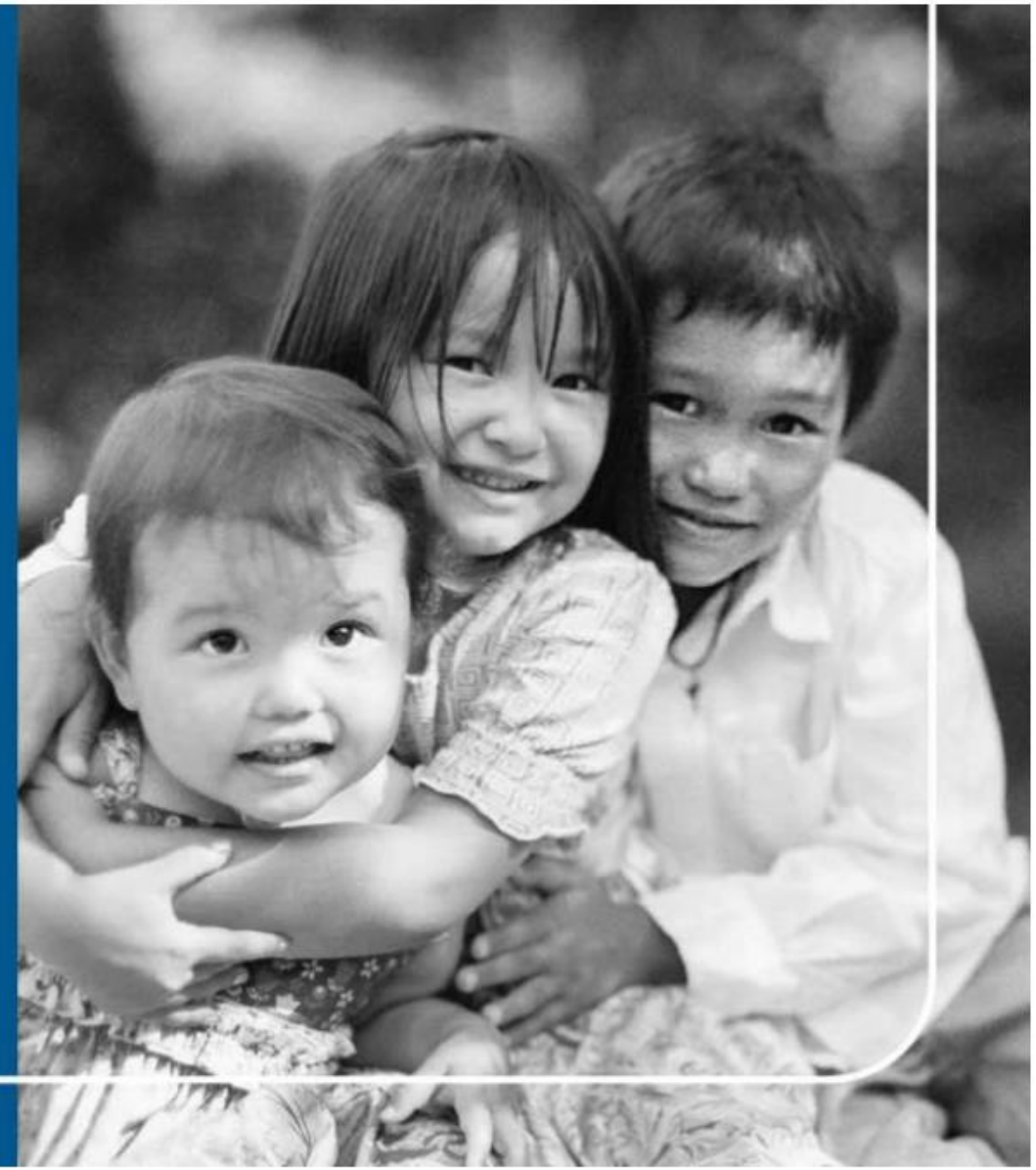
Countering Myths



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5 MYTHS About Orphan Drugs & the Orphan Drug Act

NORD® RARE INSIGHTS™

MYTH 1:
7 of the 10 Top-Selling Drugs in the United States Are Orphan Drugs

FACT: These drugs have multiple indications, both orphan and non-orphan. For example, Humira has 12 indications, four of which are orphan. Of its $13.6 billion in total sales in 2016, only 3.8% were for orphan indications.

MYTH 2:
Blockbuster drugs are protected from competition by seeking added orphan indications and reaping the benefit of market exclusivity for the entire drug.

FACT: If a drug is already on the market and the company gains approval for an additional orphan indication, the benefit of seven-years of exclusivity under the Orphan Drug Act applies only to the new orphan indication, not the entire drug.

MYTH 3:
Orphan Drugs Are a Major Contributor to Rising Drug and Healthcare Costs.

FACT: Of the total drug sales of $450 billion in the US in 2016, only 7.9% were for orphan designations of approved drugs.

MYTH 4:
Specialty Drugs Are the Same as Orphan Drugs

FACT: The two are not the same. Specialty drugs are defined by special requirements (i.e., for storage or handling); how they are administered (i.e., by a professional or as an infusion); and how much they cost. While an orphan drug may be a specialty drug, not all specialty drugs are orphans.

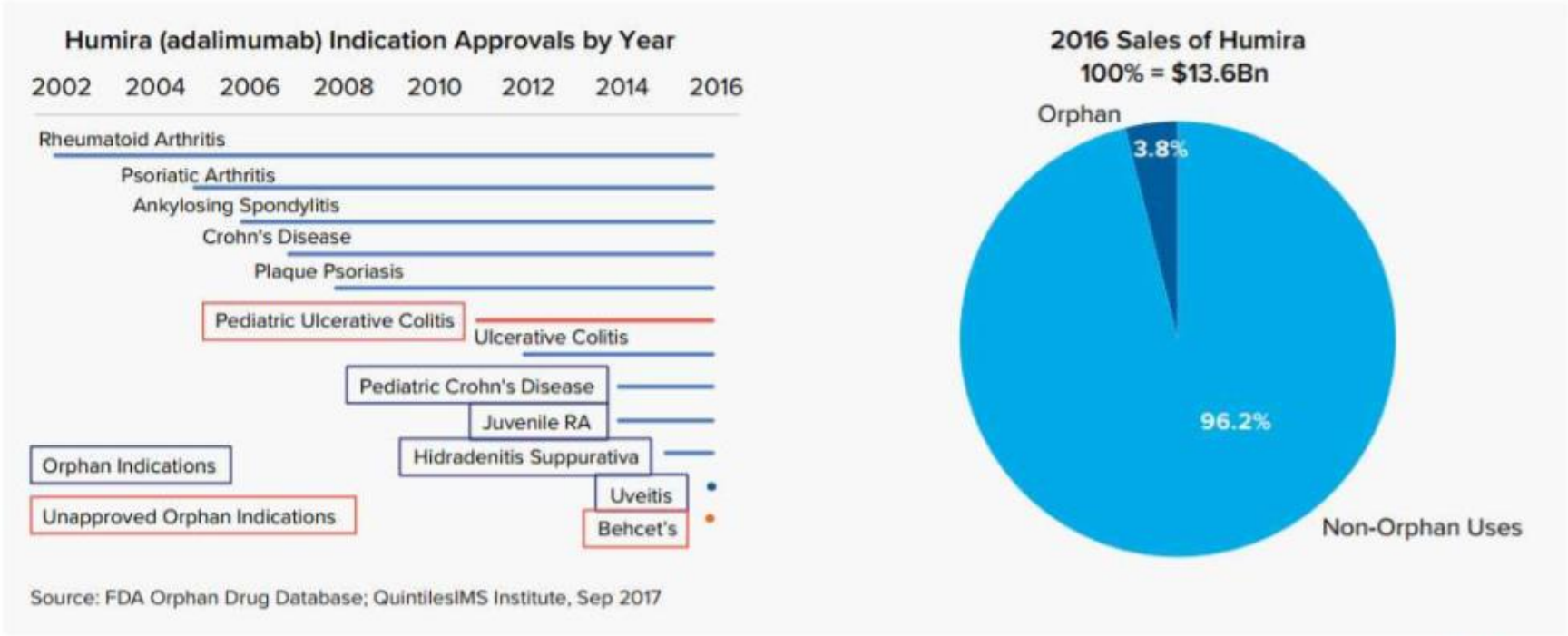
MYTH 5:
The benefits of the Orphan Drug Act distort the marketplace and bias research away from diseases affecting more people.

FACT: Studying rare diseases has led to increased understanding of the body's biochemical pathways and to major breakthroughs in discovering how our genes interact with other factors to cause disease. The Orphan Drug Act has helped drive innovation in many fields within medicine, including cancer treatment.

MYTH 1:
7 of the 10 Top-Selling Drugs in the United States Are Orphan Drugs

FACT: These drugs have multiple indications, both orphan and non-orphan. For example, Humira has 12 indications, four of which are orphan. Of its $13.6 billion in total sales in 2016, only 3.8% were for orphan indications.

Countering Myths



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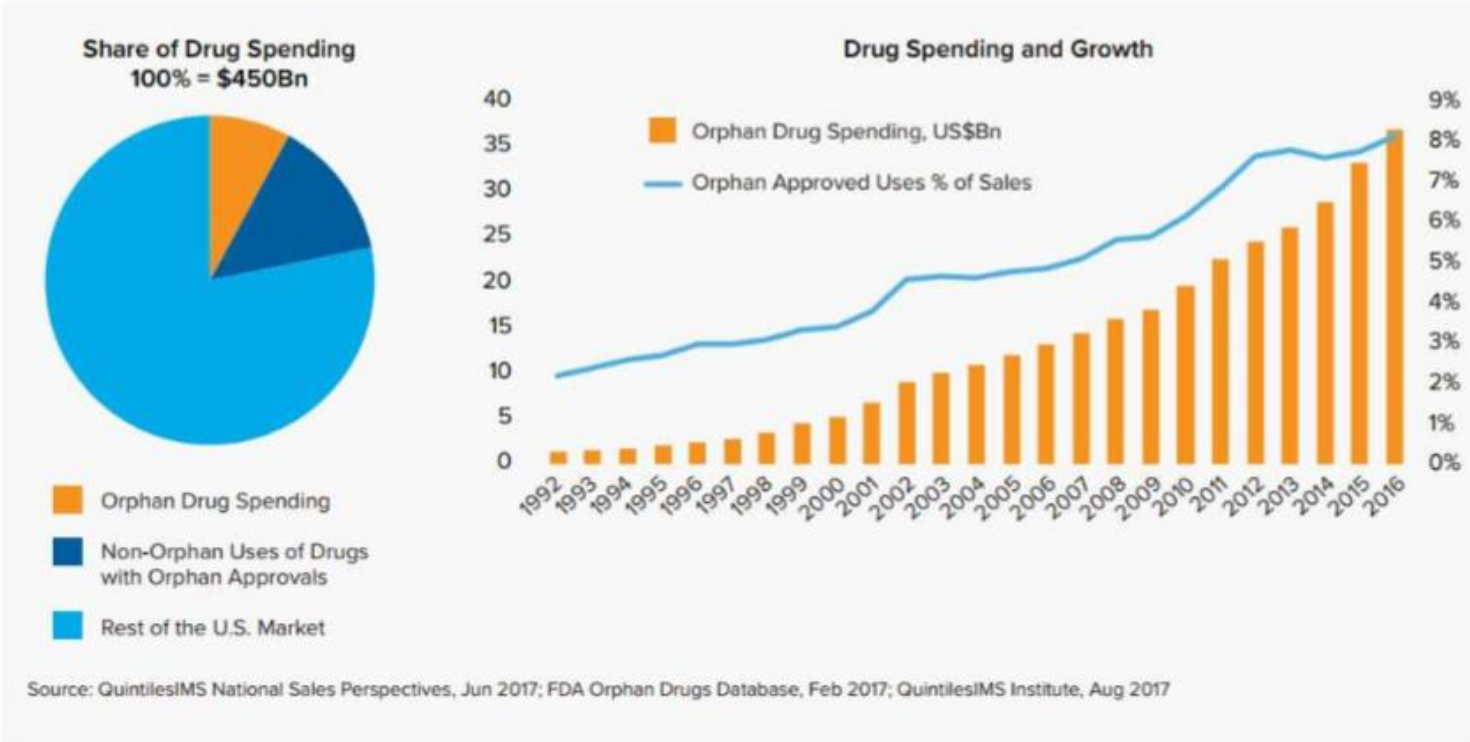

Source: QuintilesIMS National Sales Perspectives, Jun 2017; FDA Orphan Drugs Database, Feb 2017; QuintilesIMS Institute, Aug 2017

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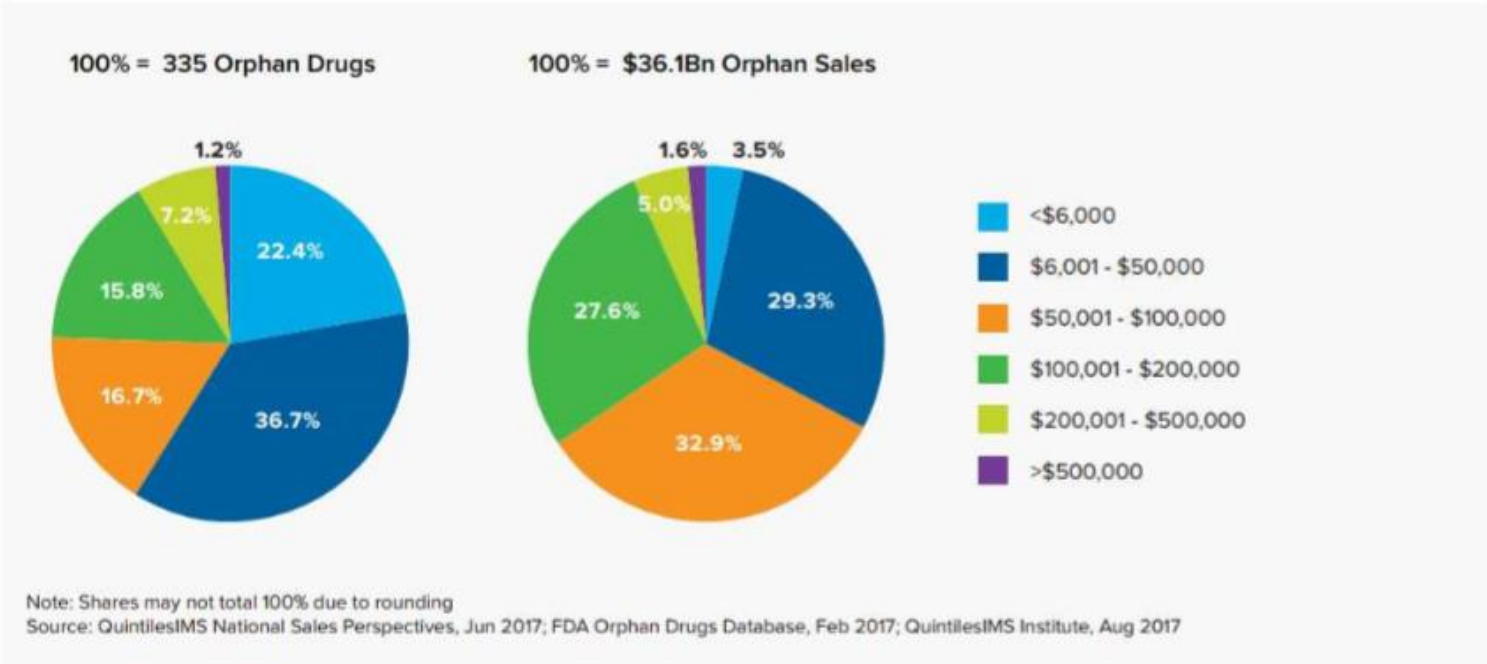
Countering Myths



Note: Shares may not total 100% due to rounding
Source: QuintilesIMS National Sales Perspectives, Jun 2017; FDA Orphan Drugs Database, Feb 2017; QuintilesIMS Institute, Aug 2017

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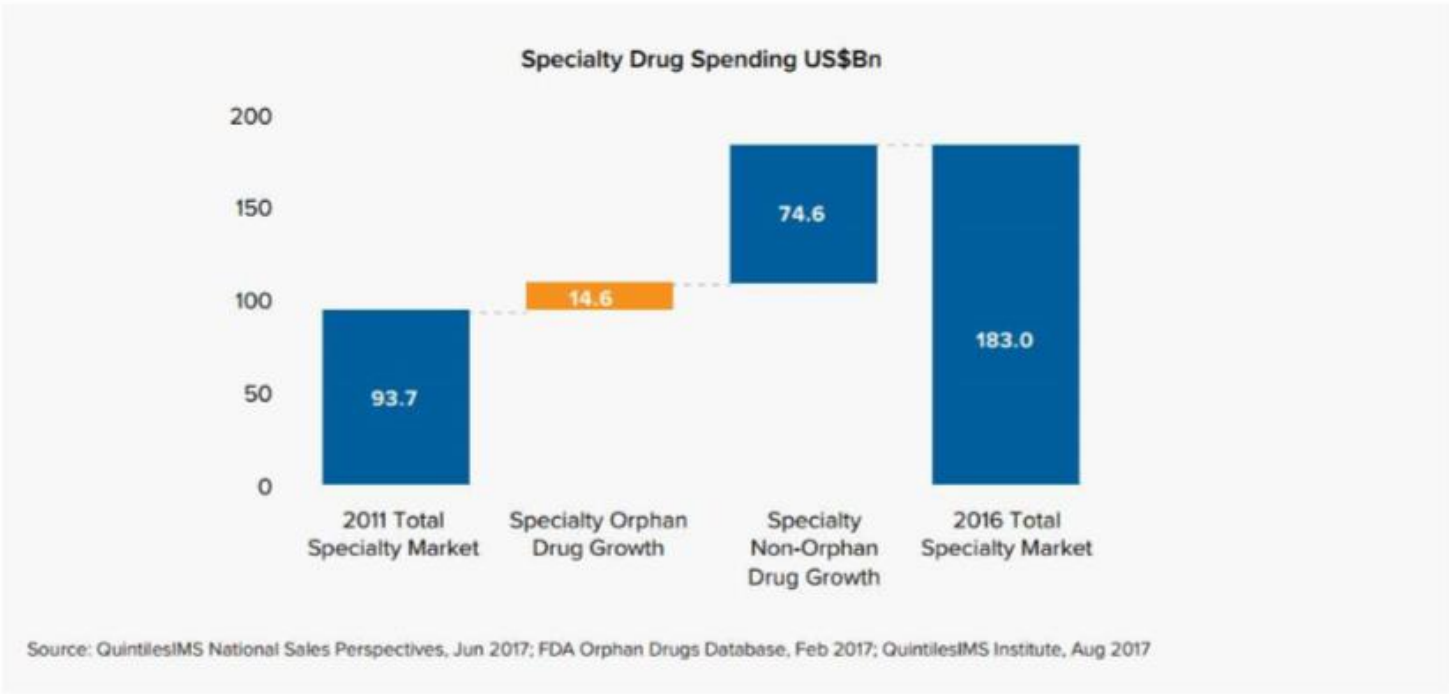
MYTH 4:
Specialty Drugs Are the Same as Orphan Drugs

FACT: The two are not the same. Specialty drugs are defined by special requirements (i.e., for storage or handling); how they are administered (i.e., by a professional or as an infusion); and how much they cost. While an orphan drug may be a specialty drug, not all specialty drugs are orphans.

Countering Myths



Source: QuintilesIMS National Sales Perspectives, Jun 2017; FDA Orphan Drugs Database, Feb 2017; QuintilesIMS Institute, Aug 2017

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Lastly, if you want to learn more about the 5 Common Myths of the Orphan Drug Act, you can visit

https://rarediseases.org/advocate/rareinsights/5-myths-orphan-drugs-orphan-drug-act/