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, 4 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 can be protected from competition by seeking an added orphan indication 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 U.S. 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.

Resources: 1. QuintilesIMS Institute. Orphan Drugs in the United States: Providing Context for Use and Cost. (2017) 2. National Organization for Rare Disorders. Trends in Orphan Drug Costs and Expenditures Do Not Support Revisions to the Orphan Drug Act: Background and History. (2017)
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NORD® National Organization for Rare Disorders