

MAKING A DRUG FOR A RARE COMMUNITY

What is a rare disease?

In the United States, a rare disease is defined as a disease that is found in < 200,000 people. There are about 7,000 rare diseases known today and 1 in 10 Americans is affected by one of these rare diseases. 85-90% of rare diseases are considered “serious or life-threatening”.

What is an orphan drug?

In the United States, an orphan drug is defined as a drug that is intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S.

Treatments for rare diseases:

- Approximately 95% of rare diseases do not have a treatment option
- Food & Drug Administration (FDA) has approved 770 Orphan Drugs
- Currently more than 560 medicines in development for rare diseases

Key points about developing a medicine for a rare disease:

- Criteria/standards for safety and effectiveness are not different from those for other medicines
- Orphan Drug Act (ODA) was passed in 1983 and provides incentives for research and development of medicines for rare diseases
- Complexities of developing a medicine for a rare disease can include:
 - Small patient numbers can lead to difficult in clinical trial recruitment
 - Finding physicians with expertise in the disease to participate in clinical research trials may be difficult
 - Natural history of some rare diseases is not well-understood or well-documented leading to discussions with FDA about how to define effectiveness and safety

Potential roles for patient advocacy groups in development of medicines for rare diseases:

- Understanding the natural history of rare disease, coordinating patient registries
- Providing input into clinical trial planning & sharing information about ongoing clinical research with patients
- Participate in educating stakeholders about disease, burden of illness and unmet need
- Many others!!

Sources:

PhRMA (www.phrma.org/rare-disease)

Food & Drug administration (FDA) (www.fda.gov/about-fda/office-special-medical-programs/office-orphan-products-development)