

NORD

National Organization
for Rare Disorders



Adopting Orphan Drugs

U.S. Chamber National Foundation

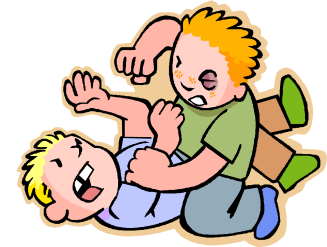
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Diane Edquist Dorman, Vice President

Unique Challenges...

- **Small patient populations internationally dispersed**
- **Few researchers**
- **Natural histories lacking**
- **Limited funding**

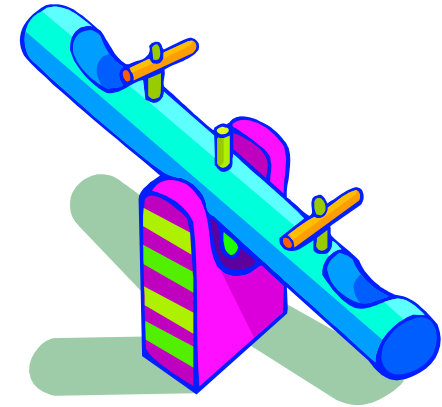
Competing Interests...



- Patients
- Doctors
- Consumers
- Policymakers
- Regulators
- Insurers
- Industry

Finding Balance...

- **Social pressures**
- **Paying for hope**
- **Cost of care**
- **Distributive justice vs. beneficence**
- **Ethical issues**
- **Emotional influences**



Challenges To Development...

- **Overcoming regulatory hurdles**
 - **Insufficient expertise**
 - **Small clinical trials**
 - **Recognizing surrogate endpoints**

- **U.S. Congress**
 - **Healthcare reform**
 - **Biosimilars**
 - **Cost controls**

- **Insurers**

- **Prior authorization**
- **QALY**
- **Specialty tiers**
- **Evidence-focused outcomes**
- **High co-pays/co-insurance**

- **Public Interest**
 - **Mistrust of industry**
 - **High costs**
 - **Suspicion of FDA process**

Striking A Balance...

- **Access and innovation**
- **339 orphans treat 11 to 14 million**
- **Millions more without hope**
- **Rare disease patients willing to take on greater risk**

Thank You...

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