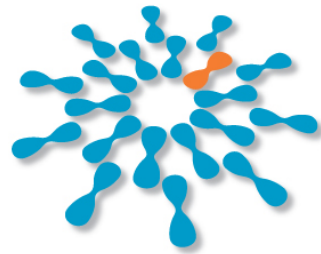


Emerging Commercialization Strategies for Addressing the Needs of Ultra-Orphan Patients

Accelerate Product Launch and Profitability by Building Regulatory Requirements into Distribution

August 24-26, 2009 | Ameristar Casino | St. Louis, MO

Conference Presentation



NORD

National Organization for Rare Disorders

The New Administration & The Impact on Orphan Drug Development: The Good, Bad and Ugly

**Diane Edquist Dorman
Vice President, Public Policy**

Background...

- 343 orphan products for 200 rare diseases
- Treat an estimated 11 to 14 million men, women and children
- 30 million in the U.S. affected by nearly 7,000 known rare diseases

Do The Math...

- Millions of Americans without any treatment specific to their rare disease
- 6,800 rare diseases have no treatments

The Good ...



Healthcare Reform...

- **Lifting annual & lifetime caps**
- **Comparative effectiveness research**
- **Guaranteed issue & renewal**

Healthcare Reform...

- **Catastrophic Coverage**
- **Pre-existing conditions**
- **Genetic Non-discrimination**
- **Phase-out of**



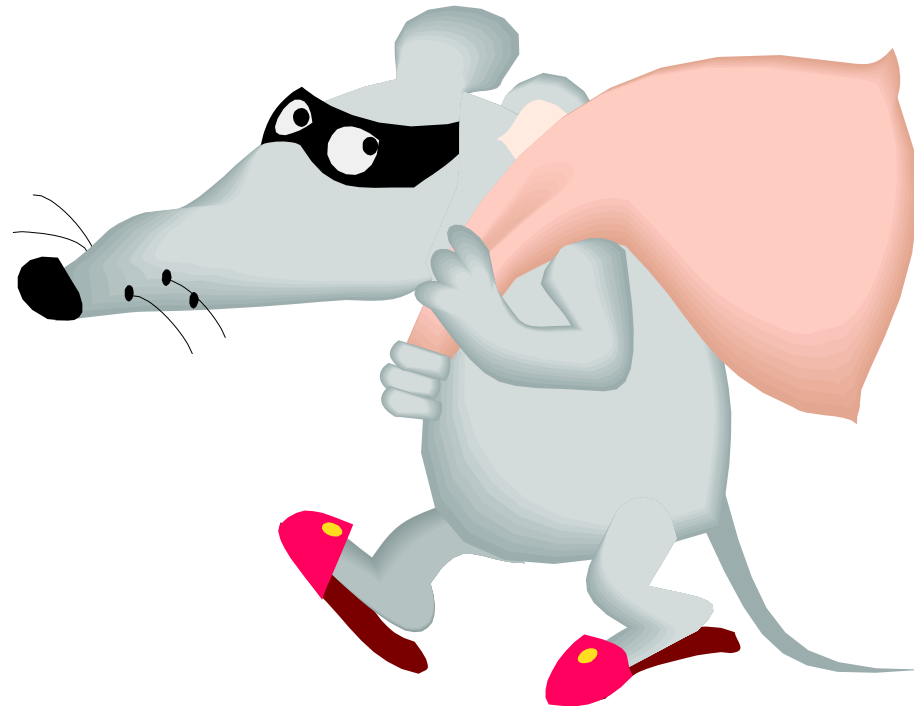
NORD's FOB Agenda...

- **Safety with therapeutic outcomes that don't differ from innovator**
- **Fair exclusivity**
- **Unambiguous, transparent regulatory pathway**

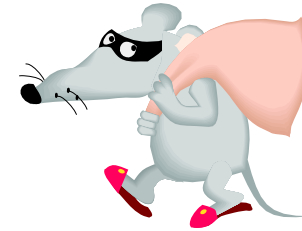
- **Access to biologics that cost less**
- **Allow FDA flexibility and authority to determine what data it needs**
- **FDA must have unobstructed framework to use its scientific expertise**
- **Decouple litigation and review**

- **Timely resolution to patent disputes**
- **Prohibition of frivolous lawsuits that delay competition**
- **Predictability**

The Bad...



- **No healthcare reform**
- **No biosimilars legislation**
- **Regulatory roadblocks – recognizing surrogate endpoints and biomarkers**
- **CER = cost-effectiveness**
- **Medicare 2-year waiting period**



The Ugly...



- **Some orphans cost \$200,000 or more annually**
- **Insurers are targeting orphan biologics**
- **Patients denied access to life-saving treatments**
- **High co-pays and co-insurance create financial burden**

Challenges To Industry...

- **FDA Advisory Committee conflict of interest rules**
- **Regulatory roadblocks – recognizing surrogate endpoints and biomarkers**
- **Reimbursement – drugs, biologics, humanitarian-use devices and medical foods**
- **Lack of harmonization with the EU**
- **Federal scrutiny**

Challenges To Patients...

- **Diagnosis can take years**
- **Cost of care can be financially devastating**
- **Prior authorization**
- **Quality Adjusted Life-Years**
- **Specialty tiers**
- **Evidence-based outcomes**
- **High co-pays/co-insurance**
- **Out-of-pocket costs**

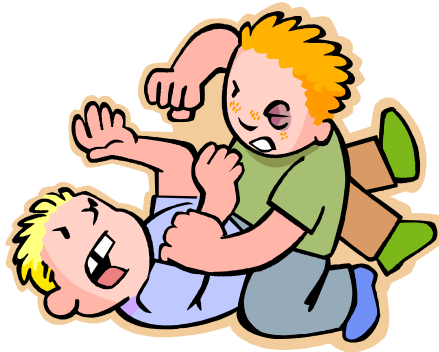
Conclusion...

Competing Interests...

- **Patients**

- **Regulators**

- **Doctors**



- **Insurers**

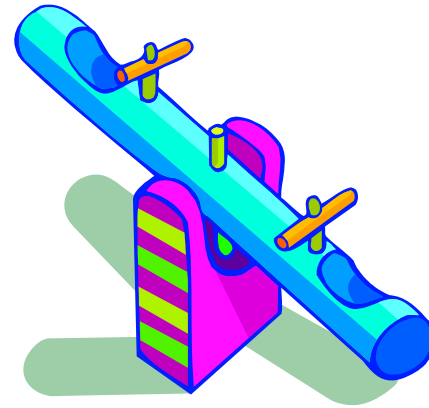
- **Consumers**

- **Industry**

- **Policymakers**

Finding Balance...

- **Social pressures**
- **Paying for hope**
- **Cost of care**
- **Distributive justice vs. beneficence**



- **Balance between access and increased innovation**
- **343 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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