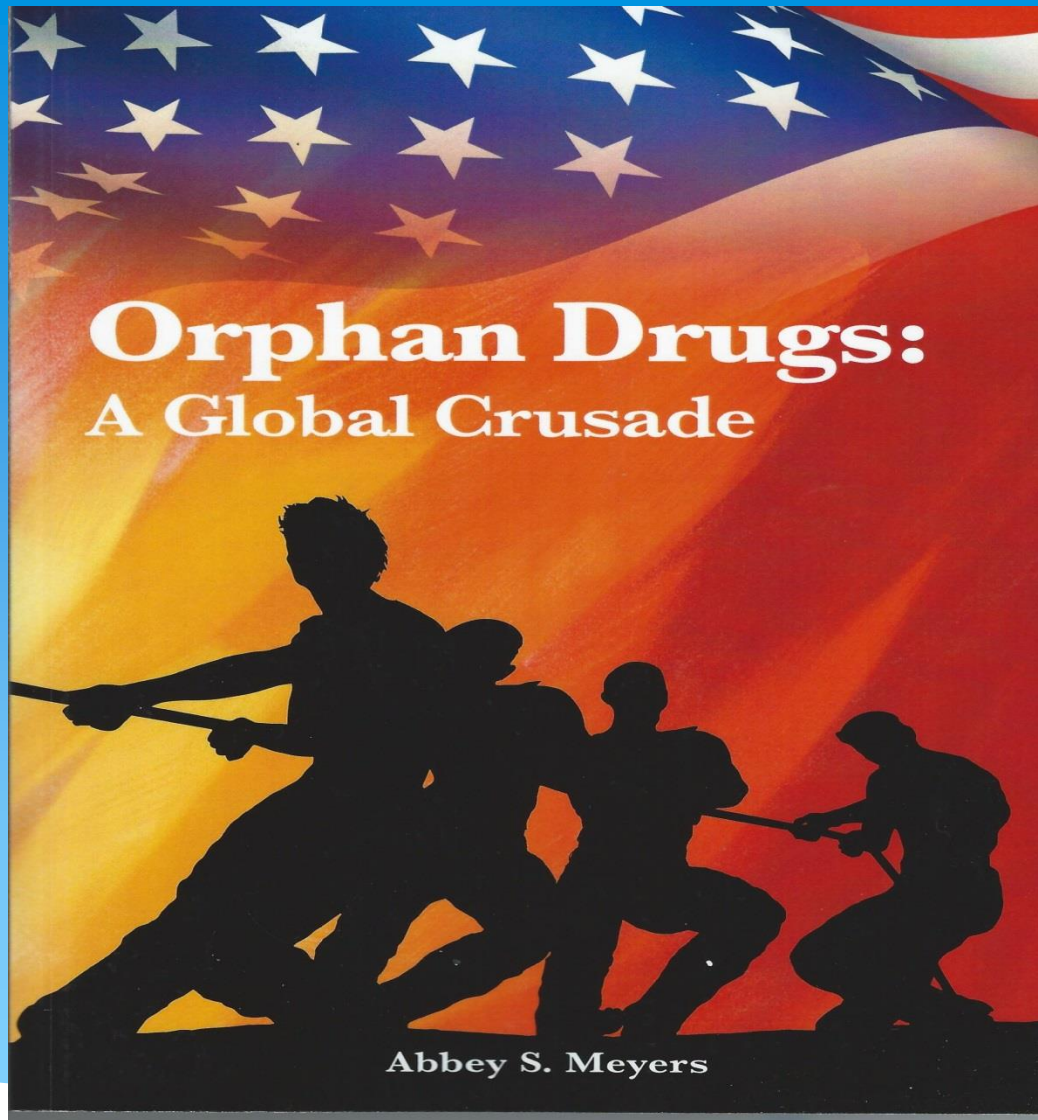




NGO COMMITTEE FOR  
**RARE DISEASES**



A Committee of **CoNGO**



CoNGO  
The Conference of NGOs  
in Consultative Relationship  
with the United Nations



# Orphan Drug Origins

- \* 1960's Thalidomide Tragedy
- \* Kefauver-Harris Amendments: Safety & Efficacy
- \* Increased Costs of R&D
- \* Pharma Mind set: Potential Profits= Size of Market



# Orphan Disease Issues

- \* The problem did not effect one family with one rare disease
- \* It effected many families with that rare disease
- \* It effected many families with many rare diseases
- \* Healthy families should be concerned because no one is immune from a rare disease



# Orphan Drug Act of 1983

- \* The Economic Problem was Solved With An Economic Solution
- \* Financial incentives to entice companies into developing treatments for rare diseases
  - \* Tax credits
  - \* 7 years exclusive marketing rights
  - \* Other incentives to help make development quicker and less expensive



# U.S. Orphan Drugs Today

- \* More than 500 Orphan Drugs are on the U.S. Market Today
- \* Each Year approximately 1/3 of all new drugs that reach the American Market are Orphan Drugs
- \* Thousands More Designated Orphan Drugs Are In Various Stages of Development



# Due to Success of the U.S. Orphan Drug Program

- \* Rare Disease NGO's around the world have enhanced their strength by working together
- \* Scientists and companies are studying rare diseases
- \* Rare disease patients are benefiting from extraordinary medical advancements
- \* These advancements can be applied to common disease treatments



# International Challenges

- \* Cost of orphan drugs and pricing disparities
- \* Differences between National Health Programs and pharmaceutical policies
- \* Need for special distribution networks
- \* Importation barriers



# Next Steps

- \* Continue to encourage the engine of scientific discovery
- \* Insure that patients will have access to treatments
- \* National or Regional purchasing pools funded by small taxes on other drugs sold in that area?
- \* Cooperative agreements with local pharmaceutical groups?

Other?

