

# Trans-Atlantic Opportunities for Orphans

Timothy R. Coté, MD MPH

Director, Office of Orphan Products  
Development, FDA

Presented May 20, 2008

ICORD, Washington, DC



# It's a new day!

- 25 years of the FDA Orphan Drug Act, hugely successful.
- Thriving Orphan Drug activities in Europe.
- But 6,000 rare diseases, a tiny sliver with treatments.
- Shared goal: More designations, better designations.
- What can we do together?



# Why collaborate?

- After all, you have your rules, we have ours...
- But the diseases are shared.
- The companies are shared.
- The rules are of a shared “spirit”.
- The science is the same science.
- The goal---more designations, better designations---is shared.



# Shared Interests

 Nuts and Bolts

 Processes

 Dreams and Ideas



# Shared Interests: Nuts and Bolts

- The new submission form.
- Strong encouragement for cross-submission as regular admin action.
- Thru-put alignment.
- Annual reports.



# Shared Interests: Processes

- Monthly teleconferences
- Guidance alignment
- Simplification of submission.
- “Exchange student” approach.
- Embracing academic ventures



# Shared Interests: Dreams and Idea

- Rescuing “abandoned/forgotten” orphan drugs.
- Grants program: Do you want one?
- New initiative: Orphan drugs for tropical diseases, synergistic with EMEA Article 58, FDA priority review vouchers.
- Bigger orphan drug world.



# Summary: The friendship continues

- Marlene left me a wonderful set of new friends, some still not yet met!
- We are open to your ideas and have some of our own.
- FDA engagement with EMEA on orphan drugs is strongly rooted, poised to grow.

