Regulatory Aspects of Orphan Drugs – FDA Perspective

Debra Lewis, OD, MBA
Deputy Director
Office of Orphan Products Development
U.S. Food and Drug Administration

4 February 2012
Recent Orphan Drug Successes

• Since 2006, FDA/CDER has issued ~90 approvals for ~80 different rare disease indications
  • Includes new drugs, new biologics, efficacy supplements
  • Wide variety of clinical development programs
    – ~2/3 of approvals based on “non-traditional” clinical development programs
      » single study with or without supporting evidence
      » “other” (e.g., case series, historical control)
Orphan Designation- Update

- 3660+ Designation requests
- 2550+ Products have received orphan designation
- 395+ Drugs brought to market
  - About 1/3rd of all New Molecular Entities (NMEs) are now orphans

![Graph showing number of orphan designations and approvals over the years](image)

**Legend:**
- **Designations**
- **Approvals**

**Year**
- 83  84  85  86  87  88  89  90  91  92  93  94  95  96  97  98  99  00  01  02  03  04  05  06  07  08  09  10  11

**Number of Orphan Designations and Approvals**
- 0  50  100  150  200  250
Diseases/Conditions Targeted by Designated Orphan Drugs*

- Oncologic: 36%
- Metabolic: 11%
- Hematologic-immunologic: 7%
- Neurologic: 7%
- Infectious/parasitic: 6%
- Cardiovascular: 5%
- Transplantation: 4%
- Gastrointestinal: 4%
- Respiratory: 4%
- Endocrinologic: 3%
- Dermatologic: 2%
- Ophthalmic: 2%
- Musculoskeletal: 2%
- Injury/poisoning: 2%
- Perinatal: 1%
- Congenital abnormalities: 1%
- Others: 2%
Size of populations that orphan drugs serve (1983-2011)

US prevalence (in thousands) for diseases for which products received an orphan designation