7th Annual International Conference for Rare Diseases and Orphan Drugs

ICORD
International Conference on Rare Diseases & Orphan Drugs

Regulatory Aspects of Orphan Drugs – FDA Perspective

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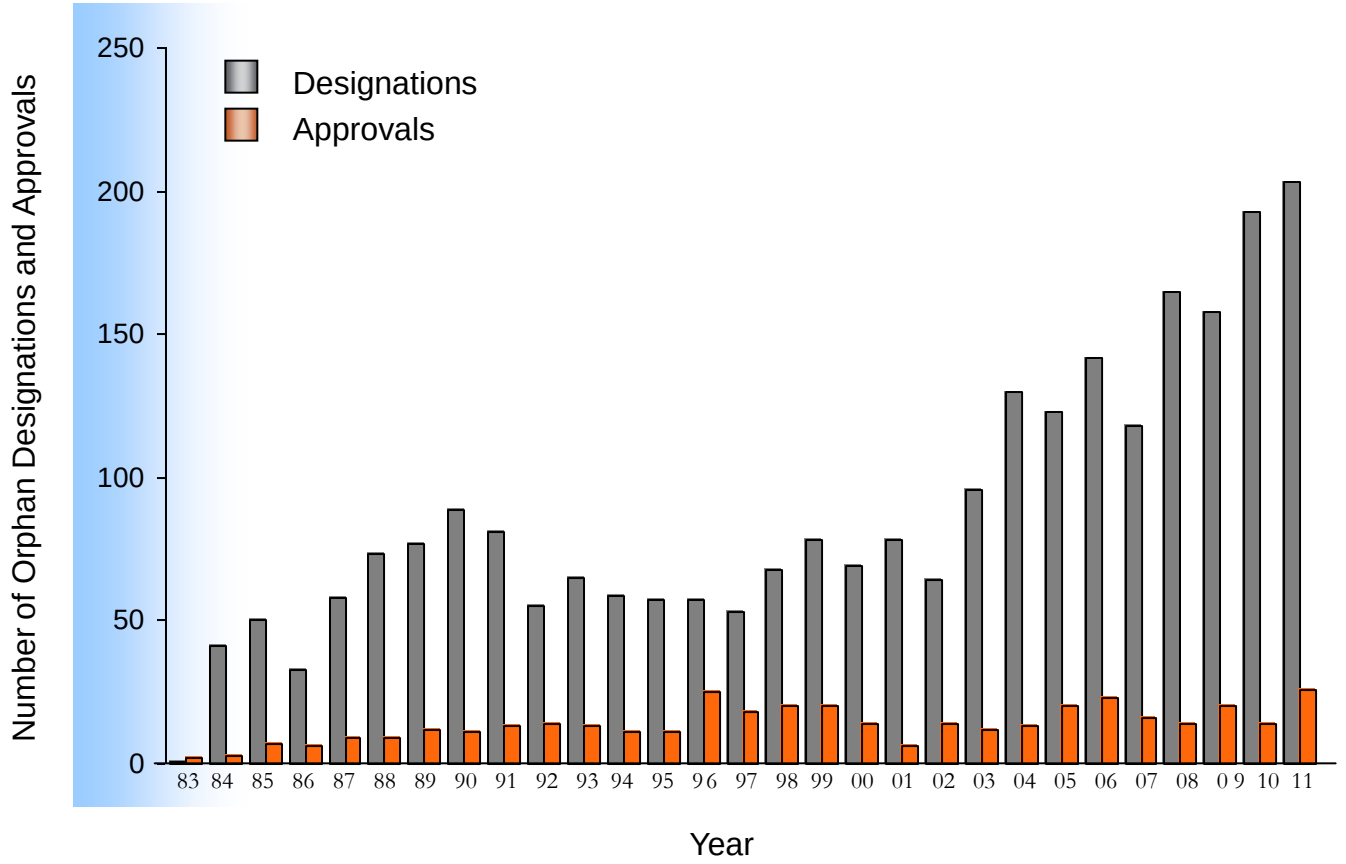
Recent Orphan Drug Successes

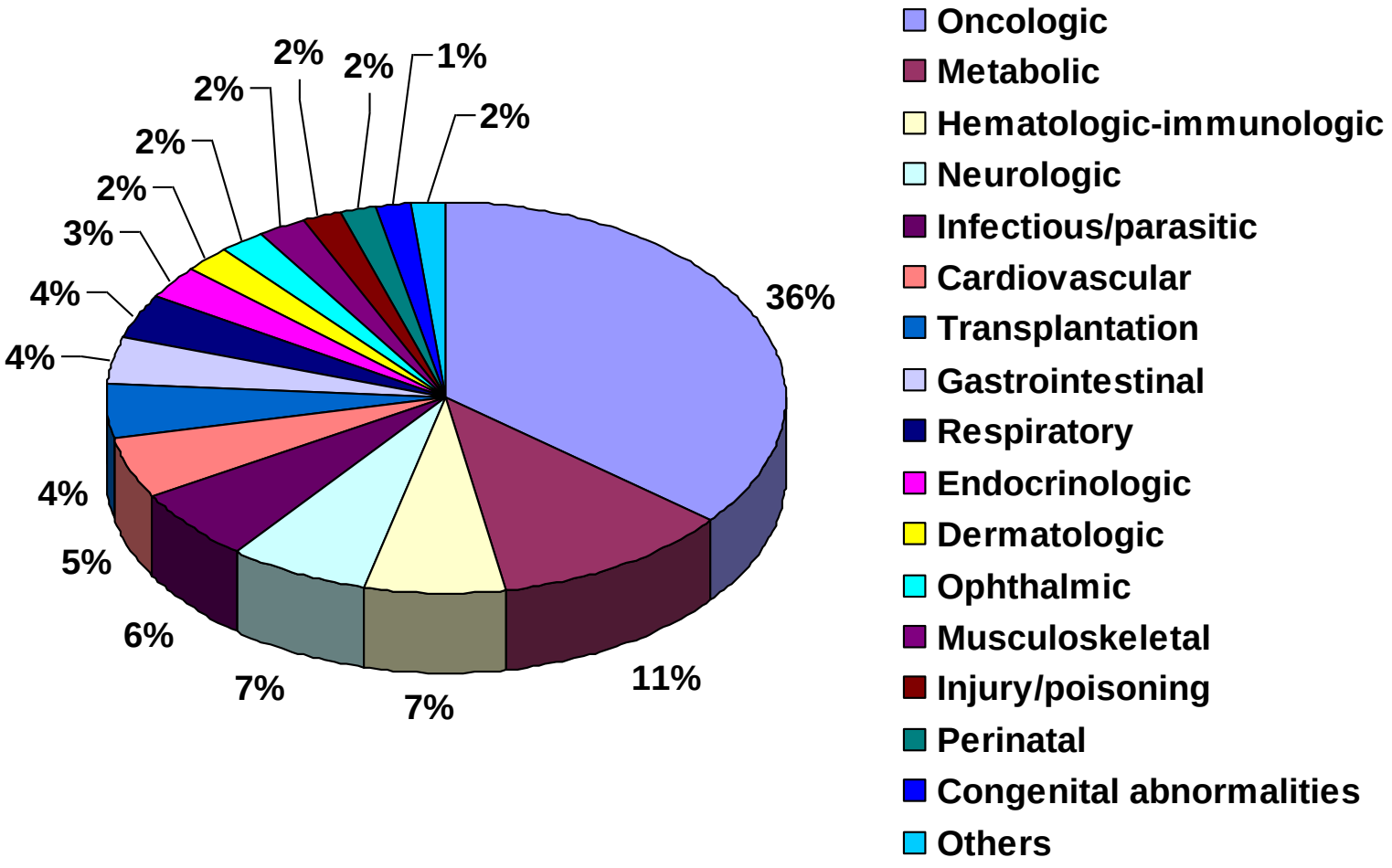
- Since 2006, FDA/CDER has issued ~90 approvals for ~80 different rare disease indications
  - Includes new drugs, new biologics, efficacy supplements
  - Wide variety of clinical development programs
    - ~2/3 of approvals based on “non-traditional” clinical development programs
      - » single study 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



Diseases/Conditions Targeted by Designated Orphan Drugs*



Size of populations that orphan drugs serve (1983-2011)