

ICORD 2009:

Facilitating Cooperation in Regulatory Efforts

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International Collaborative Regulatory Actions

- Designations
 - The joint form
 - Annual reports
 - Exchange
- Grants
- Neglected tropical diseases
- The Science of Small Clinical Trails
- Search for Forgotten Orphan Drugs of Special Promise---We must be accountable in holding the People's data.

The search for forgotten orphan drugs of promise

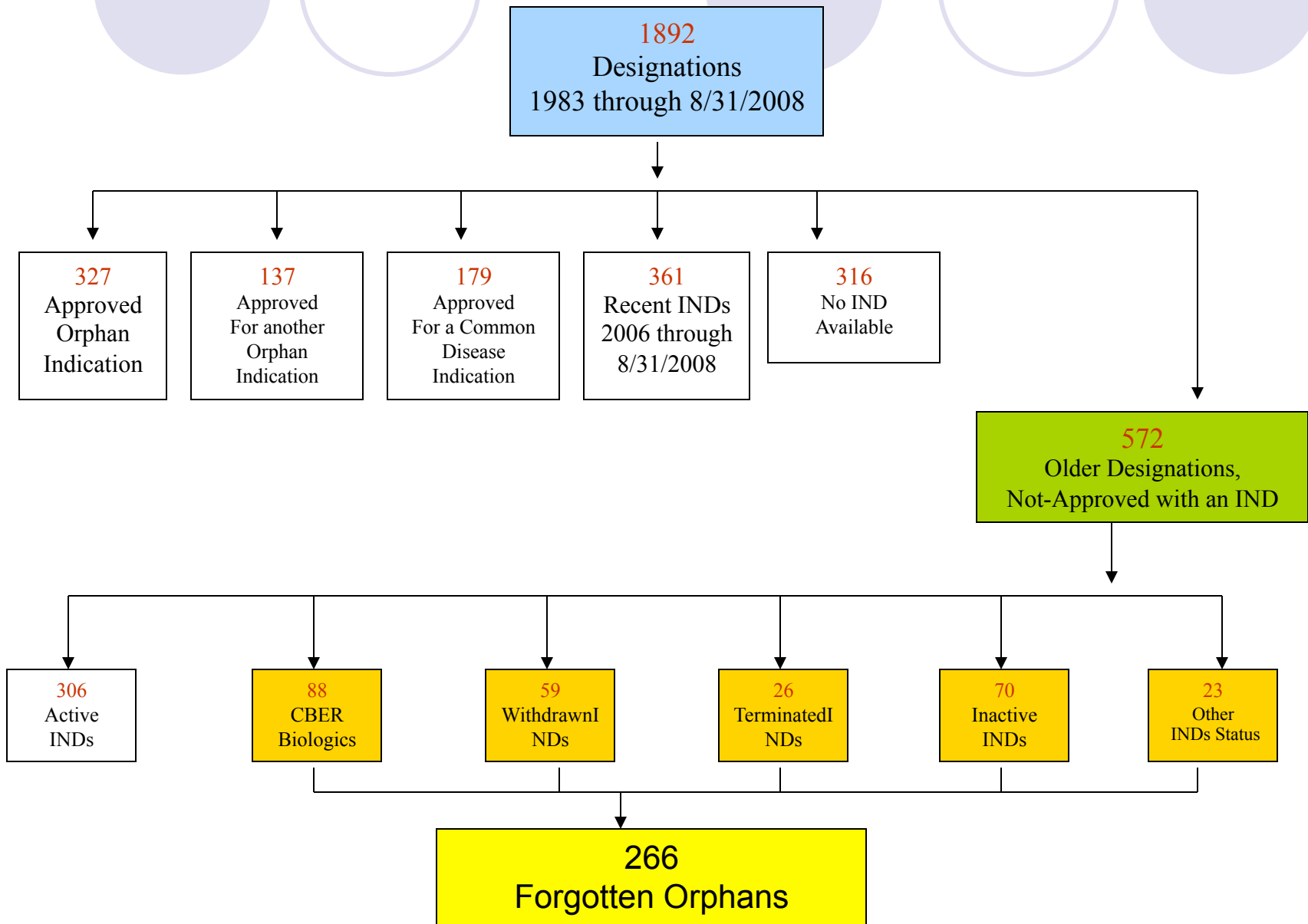
- 25 years! 1,892 orphan designations as of August 2008 (1,960+ now) and 334 marketing approvals---what happened to the rest?
- Some were junk.
- Some were toxic.
- Some were forgotten.
- Drug development is not a strictly Darwinian process where the fit (i.e. safe/effective) always win.

Definition: Forgotten Orphan Drugs?

- Some IND* data was submitted to FDA
- IND was begun before Jan 1 2006
- No record of recent activity on the IND.
- Hasn't been approved for
 - its designated orphan indication,
 - another orphan indication
 - or a common disease
 - (i.e. not on the market).

*Investigational New Drug, a file of incoming data submitted to FDA.

Forgotten Orphans Waiting at FDA



Plan: Re-examine Forgotten Orphans

- Study design
 - Study enrollment
 - Study results for Efficacy
 - Study safety signals
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- Then...if there are forgotten orphans of great promise; reinvigorate them!

Summary: Rare Diseases Erase National Boundaries

- International body of science
- International pharmaceutical industry
- International network of patients
- To be effective proponents of developing drugs for rare diseases, regulators must work internationally.