ICORD 2009:

Inching towards Integration--- the NIH and FDA seek IOM Input on a National Rare Disease Policy

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Research and Regulation Separate yet Symbiotic

Research Impulse

- Creative
- Peer-governance
- Output: transformative ideas
- Require patients for data generation

Regulatory Impulse

- Standardized
- Statutory governance
- Output: transformative actions
- Utilize data to make best decisions in service to patient

Problems

- Funded research data often insufficient/ inadequate for regulatory action. GMP, GCP, GLP are often ignored.
- Rare disease clinical trails are unconventional: smaller size, alternative methods, unique situations. Regulatory bodies strength in standardization can hamper advancement.

IOM: Highest Hopes

Deliver an integrated vision of rare disease research and rare disease drug regulation.

Other FDA hopes from the IOM

- Analysis of existing FDA data to optimize drug development.
  - Determinants of maturation from designation to marketing approval
  - Best way to find/use abandoned orphans of promise.
- Means of advancing drugs for neglected tropical diseases.

Summary: the IOM Process and the Future

- Big thinking
- Many inputs
- High hopes