Obstacles and Dollars

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Rare diseases, genetic or not, and the patients suffering from them, are the real orphans in medical sciences and drug development

Financial obstacles to rare disease resolution

- Grant funding for research in rare diseases is limited i.e. not attractive for academic centers
- ROI is highly uncertain for larger companies
- Regulatory agencies have shown interest but actions don’t follow the words because of lack of $ resources. The focus on public safety is now overshadowing the Agency’s role in making new treatments available in a controlled environment

Possibly coming to the financial rescue could be:

- Small Pharma companies:
  - Treatments for rare diseases have high prices and low hurdles for reimbursement. Not attractive to large pharma but attractive to small pharma
- Venture Capital Funds:
  - Investment in development of orphan drugs may be lucrative if there is potential for accelerated development and approval processes: exit strategy and IRR

The Regulatory Puzzle

- The “words” exist in regulatory guidelines to provide development incentives for orphan drugs
- Reality is not always in line with the words
- The conventional development paths don’t work (time and cost)
- The risk – benefit debate: real versus political

Some Solutions

- Extension of market exclusivity beyond current limits to reduce pressure on ROI.
- Conditional or provisional approvals
  - Annually renewable after status report
  - Control system to avoid off-label use
- Extended authority for FDA orphan drug group
  - To evaluate and approve drugs independently from the other divisions
  - To impose tight commercialization rules