

Accelerating Therapies for Rare Diseases

The First 25 Years

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October 16, 2010



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Expertise Orphan Products & Rare Diseases

In the Beginning - 1983...

- Consumer Groups had the initial interest – pushed for passage of the ODA
- Big Pharma less than enthusiastic
- Little knowledge as to the scope of rare diseases
- Incentives well conceived but uncharted territory

1986

- 100th Designation granted – today (2010) more than 2000 granted
- Small and medium sized companies emerging
- AZT approved as an orphan drug for HIV
- Biotech products and companies emerging

And then--

- Orphan products were on the “cutting edge”
 - Pegylation
 - Liposomal encapsulation
 - Monoclonal antibodies

No population “too small”

Excellent safety profiles

Initial Treatment INDs were primarily orphan products

Increasing **Interest** in Orphan Products – 1990's

- International expansion
 - Japan
 - Australia
 - EU
 - Many more
- Issues of Cost arose – also Biotech became more important

Orphan Devices

- First define them
- 1990 – Safe Medical Devices Act legislated
- Issues –
 - No profit except for pediatric devices
 - Insurance reimbursement
 - Is 4000 ceiling a correct number
 - What about orphan drugs for which an invitro device is needed for appropriate use

The Human Genome

- Opened many discoveries as to the “cause” or mechanism of diseases
- Diseases began to have many causes – phenotype gives way to genotype
- Personalized medicine has its beginning
- More and more rare diseases are described

Safety

- Erythropoietin and other orphan products show safety signals
- REMS
- For small populations, totality of safety parameters are not known
- Cost – especially with REMS remains an issue

Are Incentives Sufficient??

- 2000+ designations - <400 approvals
- More than 7000 rare diseases
- Concern that pace of approvals and discovery of drugs insufficient
- Funding issues – for FDA and FDA Orphan Grants Program

Incentives

- Is NIH interest sufficient – TRND program
- Tax credits – worthy if profit being made
- Increasing interest on the part of larger companies
- Enlarge the grants program – need dollars
- Other incentives – voucher program, exclusivity for biological products

Next Steps

- National plan for rare disease research and therapeutic development
- Focus by NIH and FDA on orphan products
- Use of biomarkers and surrogate endpoints
- Better understanding of the challenges of small population studies
- **Consistency** – both within OOPD and CDER

THANK YOU

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