OBSTACLES TO TRANSLATION

REGULATORY CHALLENGES

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What is an Orphan Product??

- Defined by law as a drug/biologic that treats a population of <200,000
- Or which will not be profitable for 7 years following development and FDA approval

Who is the OOPD?

- The Office of Orphan Products Development was founded in FDA 1982 to assist in the development of orphan drugs
- The Orphan Drug Act was signed by President Reagan in 1983

OPD MISSION

To assist and encourage the identification, development, and availability of safe and effective products for people with rare diseases/disorders.

INCENTIVES OF THE ORPHAN DRUG ACT

- Designation
- Protocol Assistance
- Tax Credits for Clinical Development
- 7 years Exclusive Marketing following FDA approval - Indication Specific

INCENTIVES OF THE ORPHAN DRUG ACT (continued)

- Waiver of PDUFA fee (Prescription Drug User Fee Act) FY 2006 $767,400
- OOPD Grants Program – Bench-to-Bedside
- Assistance of OOPD Staff

Designation Process

- Designation can be obtained by researcher or pharmaceutical sponsor
- Apply to OOPD
- Tips for Designation on OOPD website – www.FDA.gov/Orphan
- Include analysis of population served and literature references with the articles

OBSTACLES TO PRODUCT DEVELOPMENT

- Basic Science
- Natural History of the Disease
- Appropriate endpoints
- Geographic Dispersion of Patients
- Small # of Patients
- Phenotypic vs Genotypic description of disease
- Registries

Dermatological Orphans

- Approved – 8

  *Vaccinia Immune Globulin (Human) Intravenous

  * *Thalomid*

  * *Proleukin*

Dermatological Orphans

- In Development – 82
  - *epidermolysis bullosa (3)
  - *pemphigus vulgaris
  - *hypohidrotic ectodermal dysplasia
  - *mycosis fungoides (3)
  - *congenital ichthyosis
  - *dermatitis herpetiformis

Dermatological Orphans

- In Development – 82

  *xeroderma pigmentosum
  *Melanoma (30)
  *Kaposi's sarcoma

Approval of Orphan Products

- Same standards for development, review and approval as products for more common diseases
- Must be as safe and as effective
- FDA must also be flexible
- Clinical Trials are generally smaller

Grants Process

- Annual RFA
- Next Receipt Date – March 14, 2006
- Phase I, II, III Clinical Trials
- Maximum funding $350,000 per annum – includes indirect costs
- Three year funding/competitive renewal

Dermatological Grants

- Chlorambucil vs Placebo in Progressive Systemic Sclerosis
- Tretinoin for Stevens-Johnson Syndrome Patients
- Nicotinamide/Tetracycline vs Prednisone in Bullous Pemphigoid
- Photofrin II Photodynamic Therapy of Severe Psoriasis

Dermatological Grants

- Infusion of C1-Inhibitor in Hereditary Angioneurotic Edema
- Methotrexate-Azone Gel in Mycosis Fungoides
- Hormonal Regulation of Infantile Hemangiomas
- T4N5 Liposome Lotion for Xeroderma Pigmentosum

HUD Program

- For Medical Devices
- Devices whose use is <4,000/year
- Used under IDE
- Must have IRB approval
- Less stringent efficacy requirements

Next Horizons

- Pharmacogenomics
- Cures vs therapies
- Better understanding of disease
- Targeting of disease

Twenty Four Years Experience

- 284 Orphan Drug Approval
- 14,000,000+ patients treated
- 1553 products designated
- 69 grants currently funded
- 40 products approved that have been grant funded

www.FDA.gov/orphan

800 300 7469

301 827 3666

The Orphan Staff

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