



Research Opportunities through Orphan Designation

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Introductions

- Background (Burn Prevention and Scar Research)
- Recap - 2016 Activities
- 2017 Initiatives and Goals

Orphan History

- Rare diseases/medical conditions are individually infrequent but collectively common
- 7000 rare diseases affecting 25 million people
- Like orphans, they require special care

Criteria

- To secure “Orphan Designation” must show (with data) that treatment is promising
- Condition has fewer than 200,000 people in US

Economic Incentives

- Market exclusivity for 7 years
- Tax Credits – accrue 50% of \$ spent as tax credit
- Fee Exemptions – for review of disease by FDA
- OOPD (Office of Orphan Products Development) support at review divisions

A Success Story!

- 12 orphans before 1983
- Over 535 orphans approved today
- Treat about 200 rare diseases
 - Increasing overlap
 - Over 7,000 rare diseases

Orphans are Different!

- Transformative rather than incremental
- Often 1st therapies for dz
- Effective size and public health imperative speeds approval
- Patients are paramount

Finances

- Can be comparatively small investments
- Can yield high returns on orphan indications
- Revenues relatively small, ROI relatively large
- Many models: ERT, Patient-sponsored, Academic start-up, Mid-sized
- Good things happen! i.e., – Botox, Gleevec

Seeking Orphan Designation

- FDA OOPD (Office of Orphan Products Development)
- Big Questions:
 - What is an orphan disease?

You propose ... they decide!

- When is it best to apply?

As soon as possible! (Once medical rationale and prevalence criteria are met)

Advantages of Starting Early

- Optimizes tax credit benefit
- Optimizes assistance from OOPD
- Optimizes value with investors
- Immediately augments asset value

Medical Rationale Criteria

- Clinical data (60%)
- Animal model data (35%)
- In vitro data (5%)

Prevalence Data

- Sources
 - US Gov
 - CDC/NCHS (National Center for Health Statistics)
 - NCI/SEER (Surveillance, Epidemiology, and End Results)
 - Medical Literature
 - Claims data
 - Many others
- Must be US Data!

OOPD Responses

- 60% orphan designations granted
- Only half (30%) on first round
- 40% held in perpetual abeyance (awaiting info)
- > 1% denied

OOD Summary

- Huge increases in asset value, \$400K - \$4M
- Comparatively small fee to get one (\$40K – 10-100X ROI)
- Apply as early as possible to max benefits

For Additional Discussion

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