Small Clinical Trials for Orphan Drugs: How are these received at FDA?

Timothy R. Coté, MD MPH

Director, FDA Office of Orphan Products Development, FDA

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FDA Policy

- People with rare diseases are entitled to the same evidentiary standards as people with common diseases.
- FDA policy is a non-policy; no differences are officially recognized.
- Sensitivities and common sense does exist.

Problems

- Few patients means smaller trials.
- A “Lourdes-like effect” is unlikely to be missed. But few drugs do this.
- Patients with rare diseases are locked out of helpful drugs with modest effects.
- Safety issues are entirely different for otherwise fatal illnesses.
- Reproducibility is an unaffordable luxury.

OOPD Role

- Advocate for the development of orphan drugs.
- Grant orphan status when criteria are met
- Make grants.
- Not involved in the contemplative process of evaluation for safety/efficacy.
- Happy to hand-hold!
- OOPD is the focal point for policies (and non-policies) on clinical trails for rare diseases.

Education

- Body of knowledge of alternative study designs for rare diseases.
- FDA reviewers are largely unaware.
- Curriculum development underway.
- Certification envisioned.
- Slow process: one mind at a time.

New Policy Development

- Clinical trials for rare diseases are different:
  - Different challenges
  - Different rewards
- Arguably, there is a need for an FDA-wide policy for clinical trials for rare diseases.
- But this could be a very long argument.

Summary

- Current FDA policy on review of small clinical trials: No special policy.
- OOPD is the focal point for all issues orphan, but mindful of limits in authority.
- Educational efforts will be our first initiative in this area.
- Special policies for rare diseases are probably warranted but will require considerable time.