FDA Considerations for Growing Spine Implants

Susan K. Cummins, MD, MPH, FAAP
Center for Devices & Radiological Health
Food & Drug Administration

Today's Talk

- Paul's Questions
- Does Scoliosis qualify as a rare disease?
- Medical Device Regulation Lightning Round
- Susan's Answers
- Resources for you

Paul's Questions

- What is the future of the 510K process?
- What is an HDE and how does it differ from a PMA?
- How to succeed despite orphan market challenges?
- How to minimize "pain" in the regulatory process?
- How can professional societies contribute?
- What would be the process for a hypothetical "self-powered growing implant" or a simpler "rib anchor"?
- How do we use device experience from other countries?
- What might be the changes in the US regulatory mechanisms in the years ahead?

Is Scoliosis Rare?

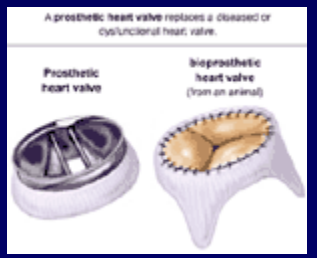
- Short answer: No---
- Categorized by: age of onset & etiology
- Idiopathic most common, by age of onset:
  - Infantile: < 3 years
  - Juvenile: 4 to 9 years
  - Adolescent: 10 years to skeletal maturity
- Early Onset: before 5 years, all etiologies
- A disease continuum; not a rare disease or condition

Medical Device Regulation Lightning Round

Device Classification

Risk-Based Paradigm

Medical devices are classified and regulated according to their degree of risk

Class I	Class II	Class III
510(k) or Exempt	510(k) or Special Controls	PMA Approval
		

Class III Medical Device

- Support or sustain human life
- Prevent impairment of human health
- Potential for unreasonable risk of illness or injury
- Requires Pre-Market Approval prior to marketing

Types of CDRH Submissions

- *For Clearance or Approval*
  - Premarket Notification Submission 510(k)
  - Premarket Approval Submission (PMA)
  - Humanitarian Device Exemption (HDE)
- *For use in clinical studies to support clearance or approval*
  - Investigational Device Exemption (IDE)

510(k) Clearance

- Established in 1976 with the medical device amendments
- Marketing pathway for more than 90% of medical devices
- "Substantially equivalent" to predicate device
  - Same as device currently marketed
- Mostly Class I and II, few Class III devices
- 510(k) is a ***clearance*** (not an approval)

2010-2011: Evaluation of the 510(k) Regulatory Program

- *2010:* Internal evaluation by CDRH
  - 55 Recommendations to strengthen program
  - *Jan 2011:* CDRH announced 25 actions to implement 47 of 55 recommendations
- *2011:* External evaluation by Institute of Medicine
  - Recommended elimination of 510(k) program
  - FDA does not support ending the 510(k) program
  - FDA response to IOM report due in 2011

Pre-Market Approval (PMA) Class III Medical Devices

- FDA scientific & regulatory review process to evaluate safety & effectiveness
- Required before device can be legally marketed
- Approval based on conclusion that sufficient valid scientific evidence to assure device is safe & effective for its intended use

Investigational Device Exemption (IDE)

- IDE approval allows:
  - Lawful interstate shipment of the investigational device for use in clinical studies to support:
    - a 510(k) submission
    - a PMA submission
    - a HDE submission

HDE Pathway Begins with HUD Designation

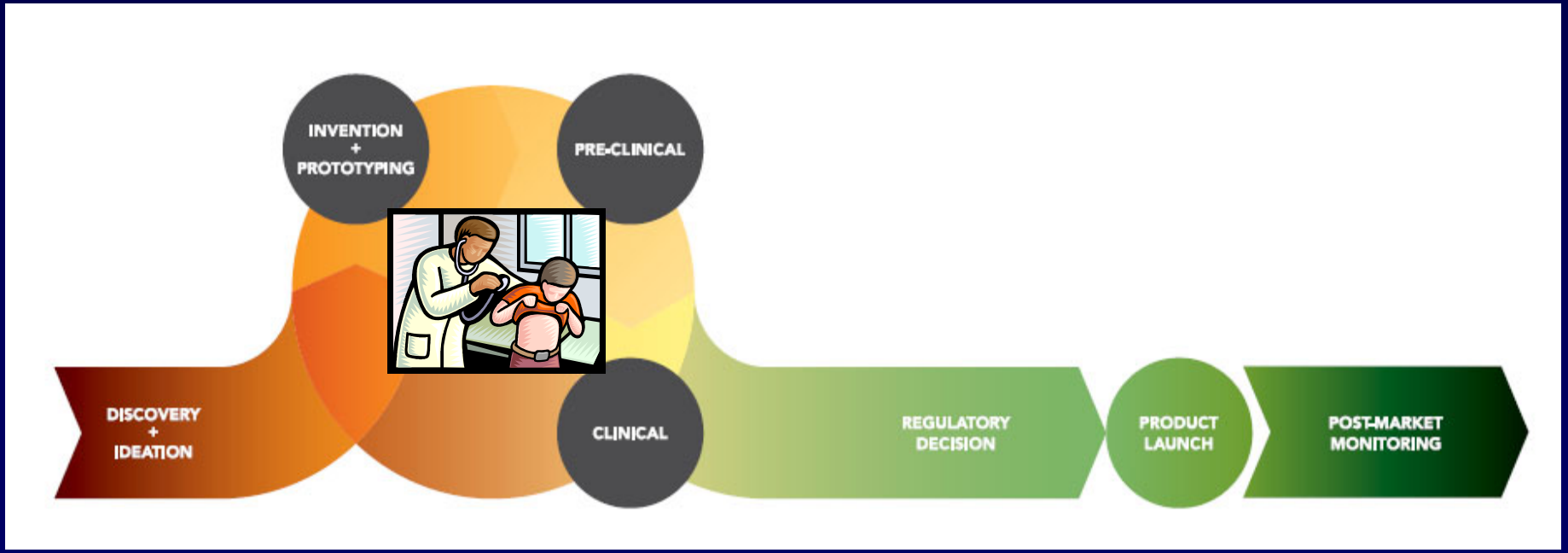
- Humanitarian Use Device (HUD)
  - Intended to treat a disease or condition affecting fewer than 4,000 individuals in the United States annually
  - No comparable device available
  - Determined by Office of Orphan Products Development
  - Profit for Pediatric HDE devices allowed
- HDE review & approval performed by CDRH

HDE vs. PMA

- Both are marketing approvals
- Both subject to post-market Medical Device Reporting (MDR) requirements
- Approval thresholds differ:
  - PMA: safety and **effectiveness**
  - HDE: **probable benefit** outweighs the risks
    - No regulatory definition of probable benefit

Tips for Optimal Device Development (Susan's Answers)

Product Development Life Cycle



Tips for Optimal Device Development

- Do your Homework!!
- Come Early & Come Often
- Do Excellent Clinical Studies
- Design for the patient population you intend to treat
- Yes FDA can accept Outside the United States (OUS) data, however...

How Can Professional Societies Help?

- Strengthen scientific underpinnings to enable your device development efforts
  - Develop & validate effectiveness & safety endpoints
  - Develop registries for post-approval studies
  - Strengthen research networks to support therapeutic studies for rare diseases
  - Establish performance measures
  - Establish trial design models

Susan's Answers to Paul's Questions

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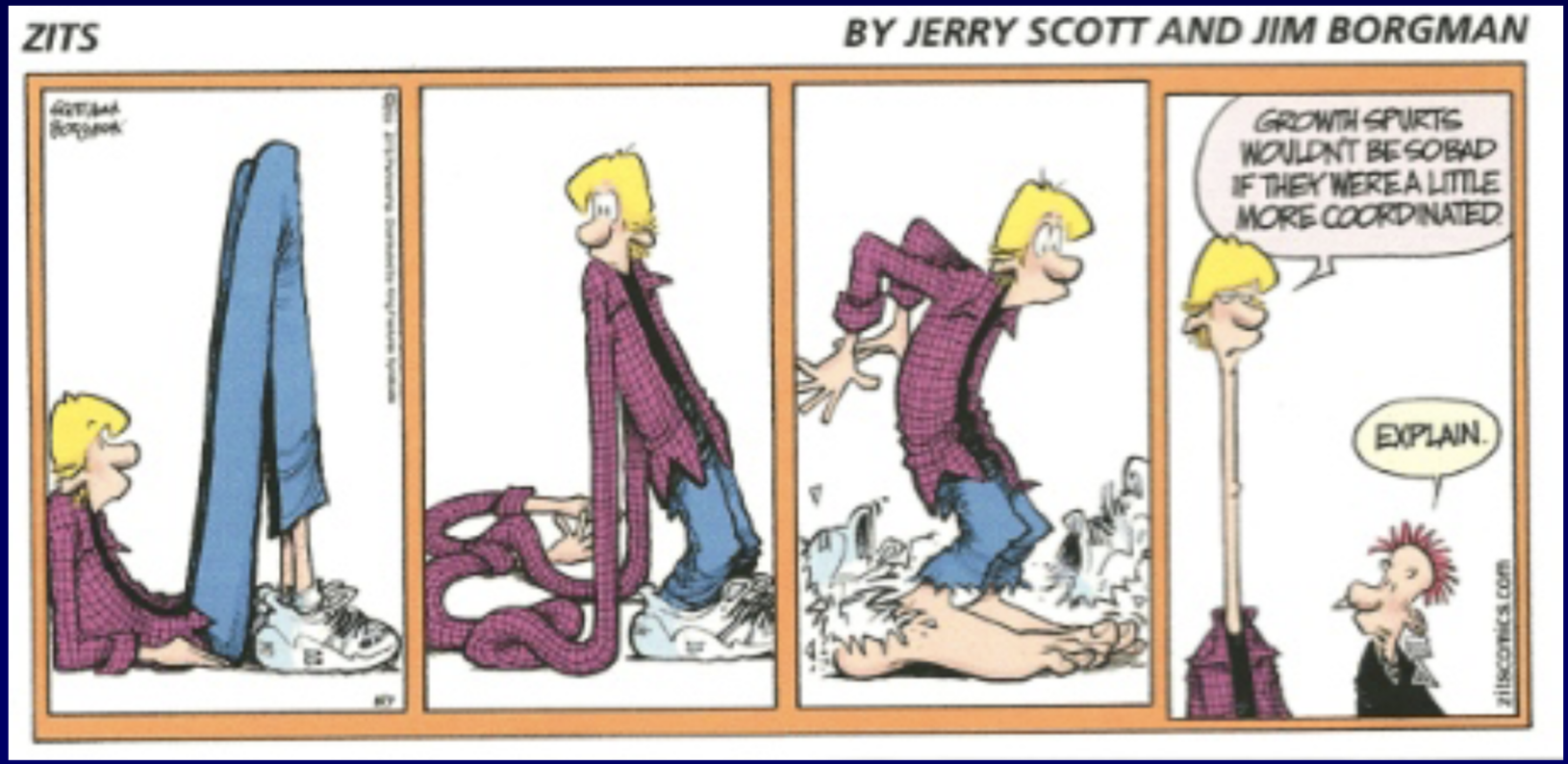
CDRH: Spinal Device Review Branch

What Complicates Pediatric Device Development...



Thank you!

- Be sure you get a handout of FDA resources
- How to get in touch:

Susan Cummins, MD, MPH
susan.cummins@fda.hhs.gov
301-796-2177