

ICEOS 2015  
Boston



The Children's Hospital of Philadelphia®

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**Center  
for  
Thoracic Insufficiency  
Syndrome**

# Health Outcomes as seen by Congress

**RM Campbell, MD**

Director , Center for Thoracic Insufficiency Syndrome

Division of Orthopaedics, CHOP

Professor of Orthopaedic Surgery

University of Pennsylvania Perelman School of Medicine



# Disclosures

- Grant Support
  - NORD
  - NIH R21
- Medical Advisory Committee member National Organization of Rare Disorders (NORD)
- Advocate for inventors/companies trying to develop safe and effective devices for children

# What does Congress want about Health Care?

- Address real needs
- Minimize fraud
- Stretch the dollars
  - ACA Quality/Value/Cost Issues
    - Penalize for re-admissions
      - 74% PA hospitals
      - 97% NJ hospitals
      - US \$420 million in penalties
    - Saves money



# What does Congress think about Health Care for children?



- Politically sensitive
- Children's Health Policy has been relatively spared from cutbacks
- Will this continue?
- Will ACA Quality/Value penalties negatively impact children's care?
- How can we help guide the process?

# Advocacy

At best, it may work 10% of the time, but if you don't do it, the success rate is 0%.



# How does Congress write the rules about Health care?

1. A Bill is proposed by a champion
2. Co-sponsors are recruited, the more the better, especially bipartisan
3. Interested parties, through lobbyists or public meetings, contribute
4. The proposed Bill goes to markup
5. Hearings are held to gain support
6. The Bill proceeds to a vote

# Who are the players in children's health care legislation?

- The members of Congress
  - Their support staff
- The organizations that champion the needs of children
  - Their lobbyists
- Public opinion
- An opportune moment

# What's hot on Capitol Hill?

## Medical Device Users Fee Act

### MDUFA

- Reauthorization 2017
  - Must pass, so popular means to “piggyback” companion bills
- Pediatric Device Safety and Improvement Act of 2007 renewed in 2012 with MDUFA reauthorization



# MDUFA reauthorization

- Improvements needed in custom device availability, HDE pathway, and compassionate use exemption pathways
- Risk tolerance spectrum for patients with limited treatment options
- AAOS/POSNA/MSTS

# What's really Hot on Capitol Hill

## 21<sup>st</sup> Century Cures Act

- Initiative began 2014
- Fred Upton R-MI, Diana DeGette D-CO
- Better, faster, safer, and more innovative approaches to treat diseases
- Passed House of Representatives 389-40

# Status

## The Players:

- House Energy and Commerce Committee and the Senate Health , Education, Labor and Pensions Committee
- The Administration
  - Senate health innovation proposal pending
    - Possible bill passage next year

# 21<sup>st</sup> Century Cures Act

## FDA Medical Devices

- Establish priority review program for breakthrough medical devices for serious diseases
- Third party quality assessment
- Registry data, peer reviewed studies, foreign data usable
- Improved classification panel review

# Centralized IRBs

- Will be made available for multi-institution medical device trials
- Poster Children for problems with multiple IRBs and studies
  - VEPTR device trial 1990-2004
  - BRAIST Study 2013

# Humanitarian Device Exemption (HDE)



- Began with 1990 Safe Medical Devices Act
- FDA approved
  - Proven safety/probable efficacy
- Manufacturers originally could not make a profit, but this changed with 2012 MDUFA reauthorization
- Limited to 4,000 patients/yr
- Will increase to 8,000/yr

# NIH

- 5 year funding for NIH Innovation Fund
- Increased travel funds to scientific meetings for NIH
- Supports high risk/high reward research

# NIH Pediatric Activities

- National Pediatric Research Network
  - Consortium of research institutions to pool resources and coordinate activities for treatment of rare diseases and birth defects
- Appropriate age groupings for research studies



# Natural History

- Public/private partnership to analyze and store data on natural history of diseases, especially rare diseases

# Protecting FDA Device User Fees from Sequestration

- Fees are \$246,000 for PMAs (2013)
- \$5,000 for a 510(k) (2013)

# Sunshine Law Exemptions

- CME activities
- Journals, medical textbooks, reprints

# Health Information Technology

- Interoperability
- Hardship exemptions for practices with incompatible EMR systems

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advocate  
for the health of  
children and that of their  
families



Thank You!

