NORD
National Organization for Rare Disorders

Pediatric Devices for Rare Diseases January 8, 2014

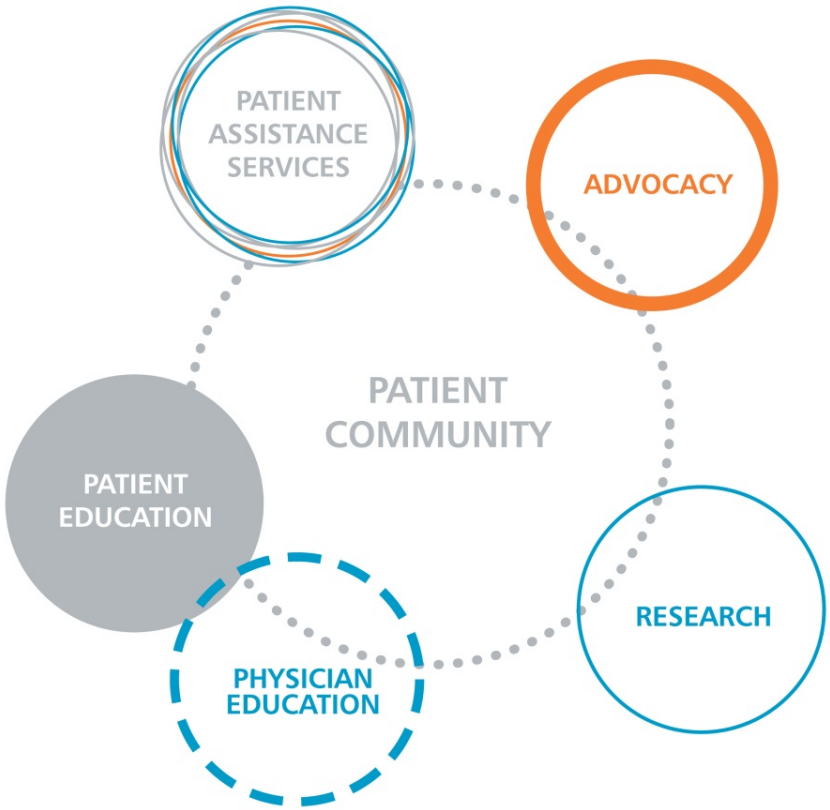
What Can Be Done?... Incentives & Otherwise

NORD's Guiding Principles…

- Awareness and recognition of the challenges faced by people living with rare diseases and the costs to society;
- A nation where people with rare diseases can secure access to diagnostics and therapies that extend and improve their lives;
- A social, political, and financial culture of innovation that supports the basic and translational research necessary to create diagnostic tests and effective therapies for all rare disorders;
- A regulatory environment that encourages development and timely approval of safe and effective diagnostics and treatments for individuals with rare diseases

What We Do…

- Advocate
- Educate
- Connect
- Support Innovation
- Promote Access
- Provide Assistance



Rare Diseases…

- Over 7,000 known rare diseases
  - 80% affect children
  - Chronic, life-threatening, life-altering
- Of the 350 most “common” rare diseases, 27% die before their 1st birthday (NATURE|Vol 466|8 July 2010)
- Just over 400 orphan products approved treating few rare conditions

Challenges…

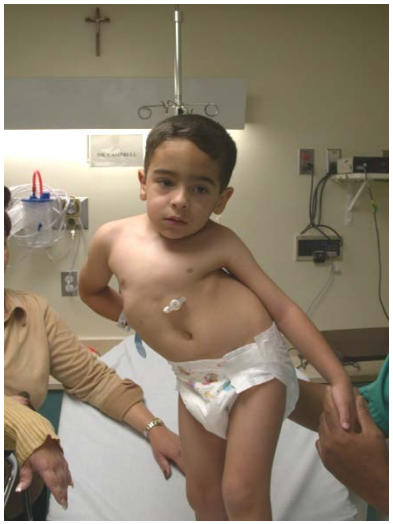
- Identifying needs in the pediatric populations
- Clearance/approval vs. reimbursement
- Required IRB review of humanitarian use devices
- Coordination between Centers

Challenge #1

Needs Assessment

Challenge #1…

- Identifying needs in the pediatric populations



Challenge #2…

- Clearance/approval vs. reimbursement
  - Studies typically don’t collect or include outcome demonstrating value
  - Labeling may be confusing or unhelpful to payers
  - Lack of transparency

Challenge #2

Reimbursement

CDRH Entrepreneurs in Residence

Team #2
Streamlining the Path to Reimbursement
2012-2013

- Small companies have limited experience and resources to navigate the reimbursement process
- Process is fragmented
- Engage innovators early during the process

- Awareness of differences & potential overlap in evidentiary requirements
- FDA approval/clearance does not support reimbursement
- Evidence required by FDA differs from public/private payers

- Coding, payment & bundling
  - Obtaining coverage is only part of the process for obtaining reimbursement
  - Coverage process is distinct and separate from coding, payment and bundling processes

Challenge #3

IRB of Record

- IRB review
  - Manufacturers must submit report to the IRB of record whenever a HUD may have caused or contributed to death or serious injury
  - Payers view HUDs as experimental
  - Access/reimbursement is delayed or denied

Challenge #4

Stakeholder Coordination

- Coordination between CDER, CDRH, other stakeholders
  - Orphan Products Research Grants Program
  - Pediatric Device Consortia Grants Program
  - Rare Disease Council
  - Increased cooperation
  - Outreach to stakeholder community

Finding a Balance…

- *Innovation*
- *Sustainability*
- *Access*
- *Affordability*

Thank You!

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