A specialty healthcare system's perspective

Rare and Orphan drugs: Stop excessive pricing and impact on children with uncommon and complex conditions

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Becker's CEO Summit
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Disclosure

- I have no significant financial interest or other relationship with (1) the manufacturer of any commercial product(s) and/or provider(s) of commercial services discussed in this presentation and (2) with any commercial supporters of the referenced activity.
- However, in my current role I advocate for regulations and policy that focus on reasonable access to effective treatment options for children, especially those who have complex, traumatic or rare conditions.

Todays Aim

- Using Gillette's experience, describe how complex conditions and diversity of pediatric population make orphan drug market dynamics even more complicated
- Illustrate how the current state of orphan drugs market journey impact children and their families– and front-line providers
- Explore how health systems can lead in developing system improvements to balance cost and access to these drugs

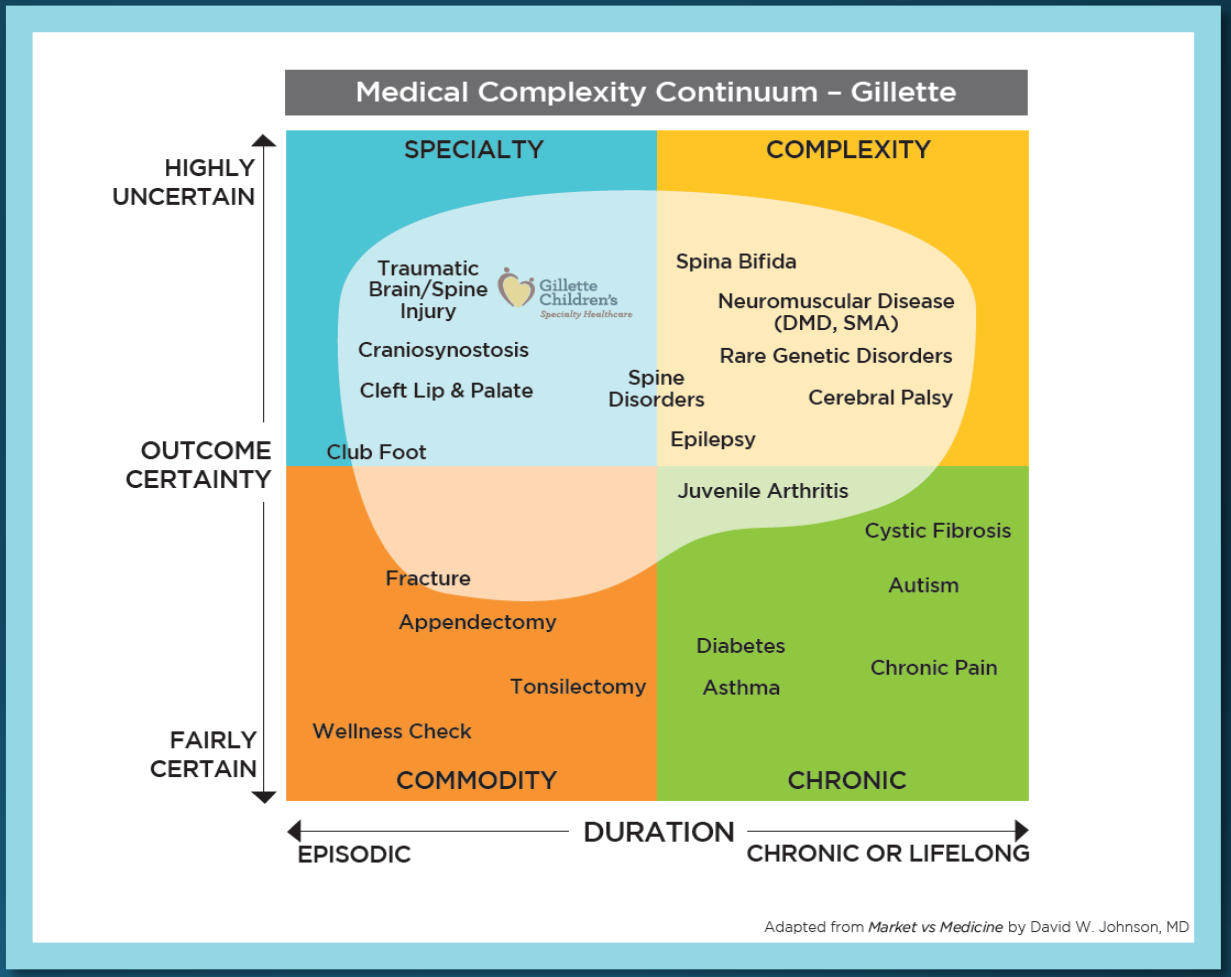
Gillette Children's Specialty Healthcare

- Founded in 1897
  - By Minnesota Legislature as the nation's first hospital dedicated to serving children who have disabilities
  - Skeletal deformities
- Today
  - Free-standing, independent nonprofit hospital and clinic, 15 locations
  - Focused on conditions of the neuromuscular and skeletal systems

Focus

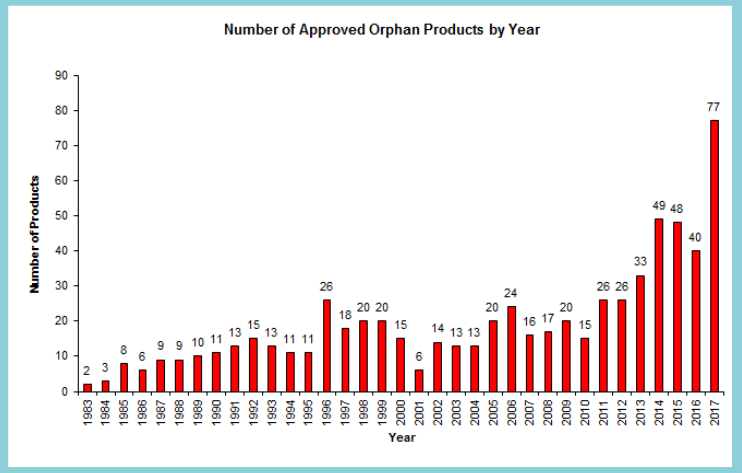
Complex –
Rare –
Traumatic

Potential
Realized



Orphan Drug Act of 1983 – 35th Anniversary

- Worldwide impact
- Focus on treatment options for small cohorts
- Encouraged development of drugs for rare diseases
- Aware of limited potential for profitability



MAKE EVERYTHING AS SIMPLE AS POSSIBLE, BUT NOT SIMPLER

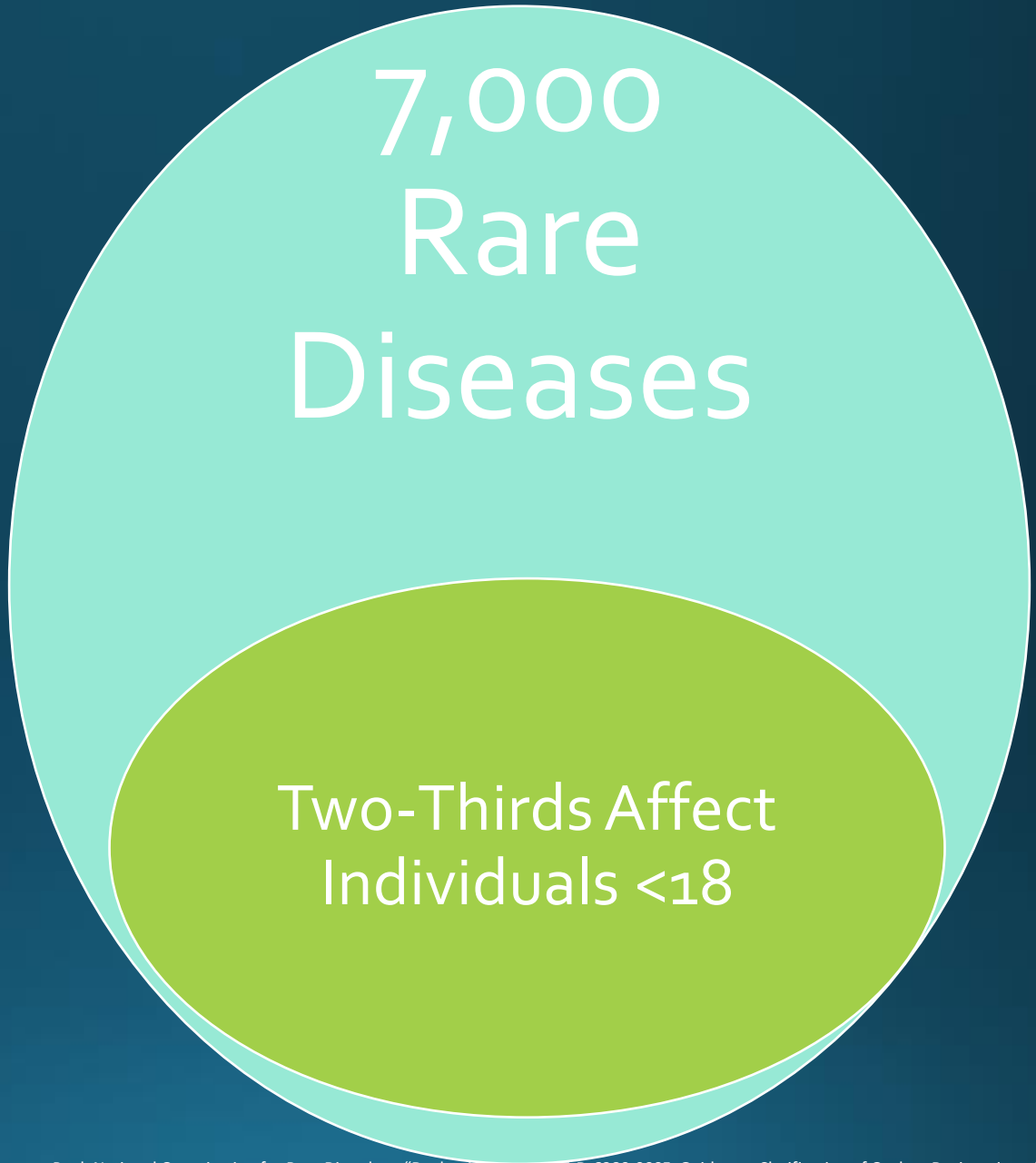
-ALBERT EINSTEIN

Orphan Drug

Drugs approved for small populations of patients with rare diseases

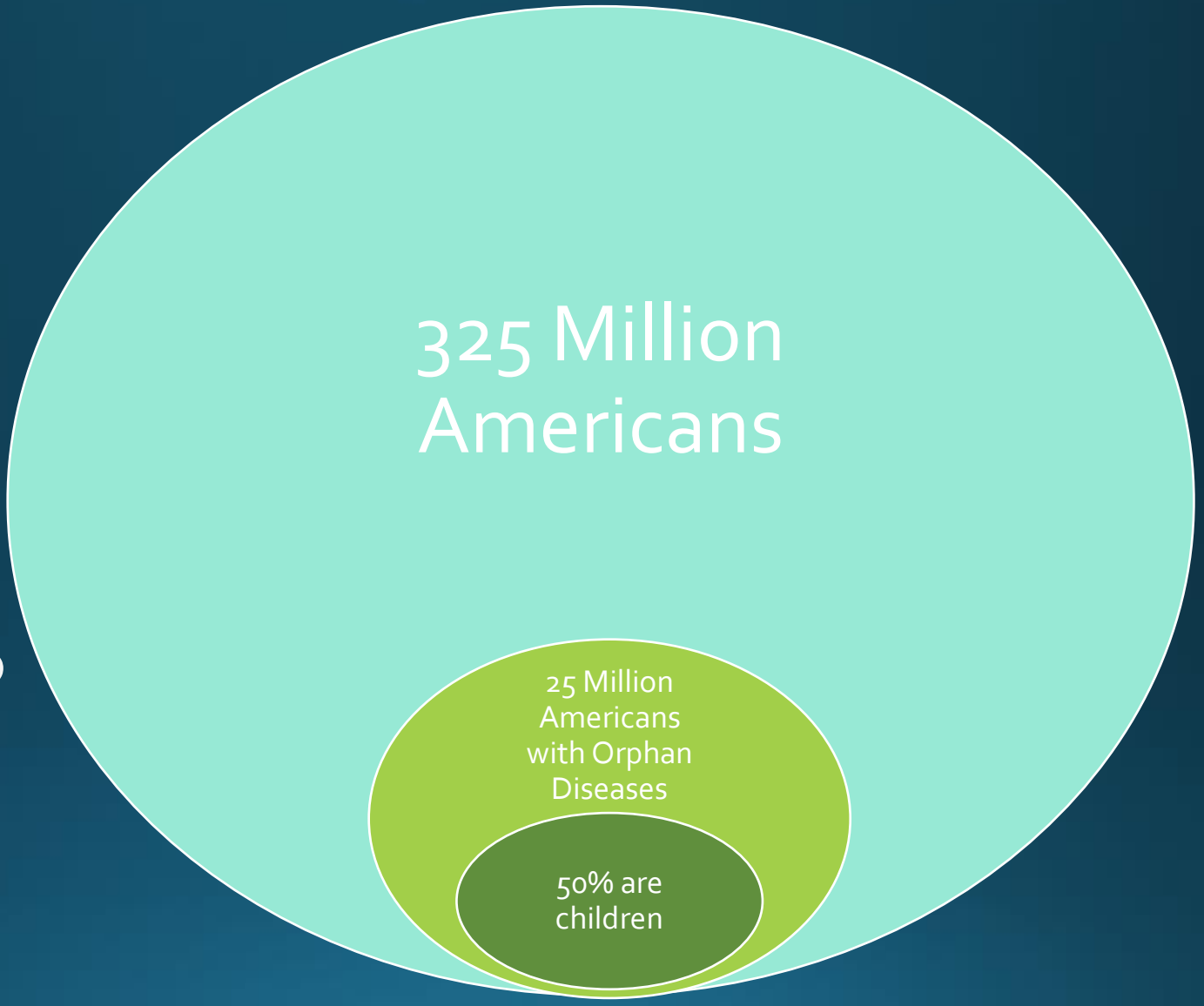
> *Intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug (FDA, 2018)*

How many orphan diseases?



Melmeyer, Paul. National Organization for Rare Disorders. "Docket No. FDA-2017-D-6380-0005: Guidance: Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Disease." Received by U.S. Food and Drug Administration, 18 Feb. 2018, rarediseases.org/wp-content/uploads/2014/11/NORD-Comments-on-FDA-Guidance-Clarification-of-Orphan-Designation-of-Drugs-and-Biologics-for-Pediatric-Subpopulations-of-Common-Diseases.pdf.

Who has an orphan disease?



NIH National Center for Advancing Translational Sciences. Genetic and Rare Disease Information Center. FAQs about Rare Diseases. Updated 2017 November 30. Available from: https://rarediseases.info.nih.gov/diseases/pages/31/faqs-aboutrare-diseases

Global Genes. Rare Diseases: Facts and Statistics. Available from: https://globalgenes.org/rare-diseases-facts-statistics/

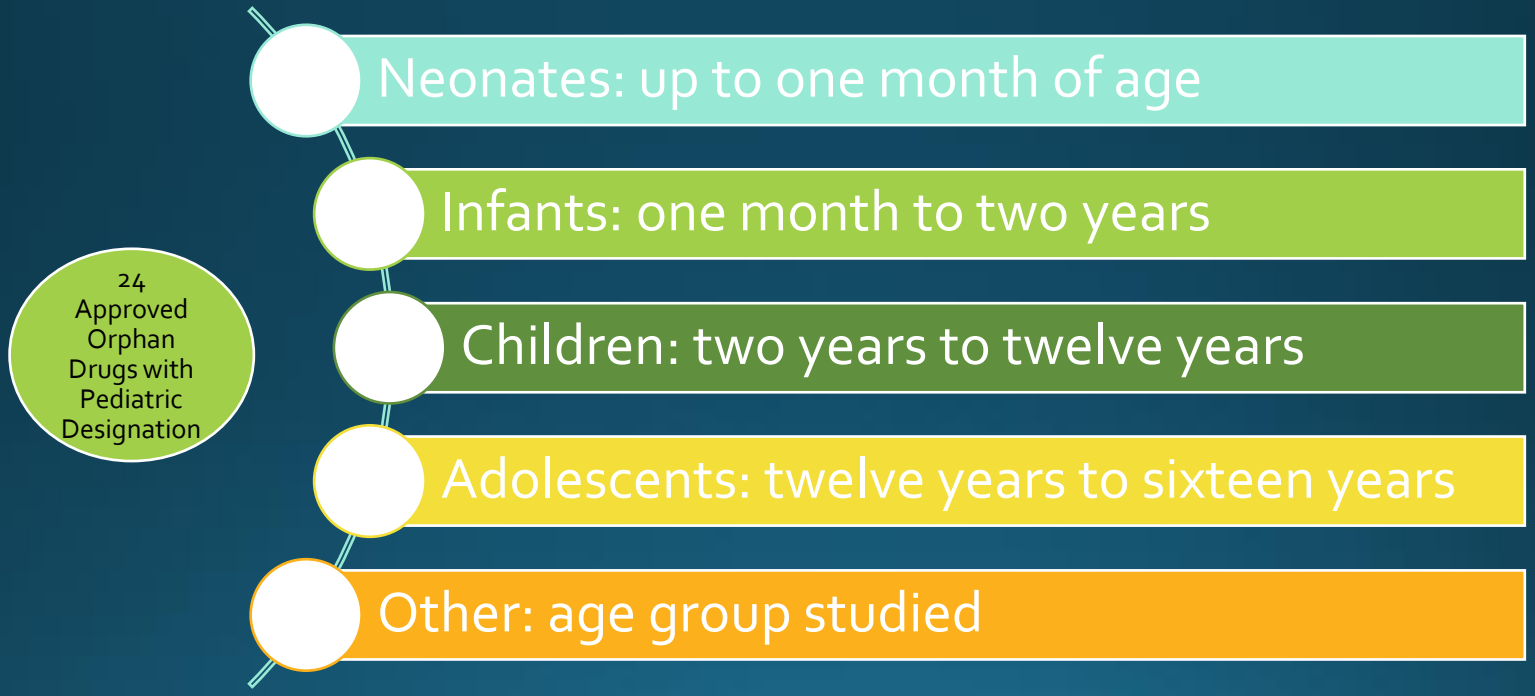
How many orphan drugs?

*Prior to the Orphan Drug Act – 38 approved drugs.

U.S. Food & Drug Administration. Orphan Drug Designations and Approvals. Available from: https://www.accessdata.fda.gov/scripts/opdlisting/oopd/

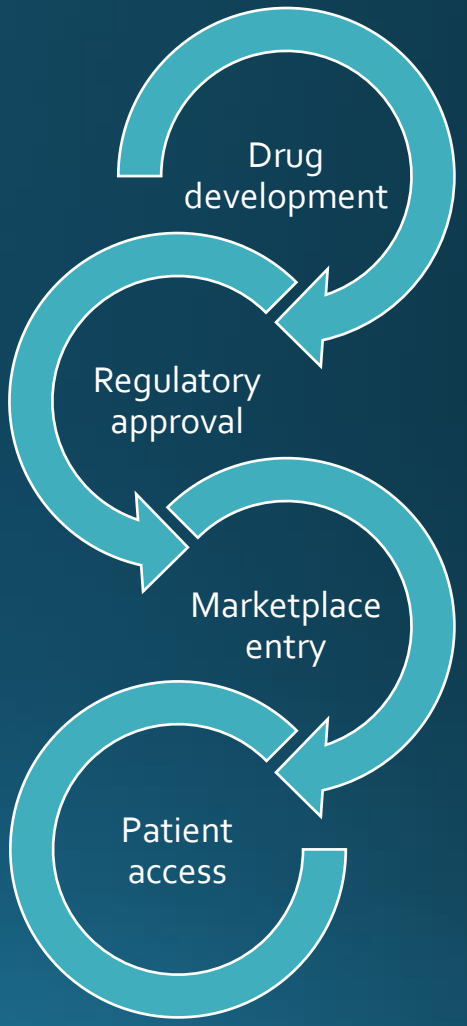
Pediatrics

U.S. Food and Drug Administration – Pediatric Exclusivity Study Age Groups



Areas to discuss

- Call for improvement:
  - Consistent drug development strategy across disease groups / body systems
  - Clear policy for market entry requirements post regulatory approval
  - Consistent process for pediatric patient access
    - Clinical pathway
    - Financial pathway
- Health systems have a role to play in improving access and coverage



Call for Pediatric Focus

Financial Pathways to Access

- Price setting and rate setting by States
- Related price setting and rate setting by Providers and Insurers
- Price for our discussion today: what it costs the patient
  - Co pay
  - Deductibles
  - Prior authorization and related denial impact
  - Self Pay options
  - Assignment of assets to access special coverage

Financial pathways for children to orphan drugs are fragmented, differ by insurer: state or federal government and commercial. Result?

Disparity in Access

Applying value-based concepts from general population to pediatric sized orphan disease and orphan drug groups. Result likely?

Delay in Access

Case Study:

"Miracle Drug" for SMA

Spinal Muscular Atrophy - SMA

- 1 in 10,000 children born with SMA
- Estimated 9,000 US patients
- Gillette: SMA – 110 patients (2017)
- SMA is number one genetic cause of death of infants in the US

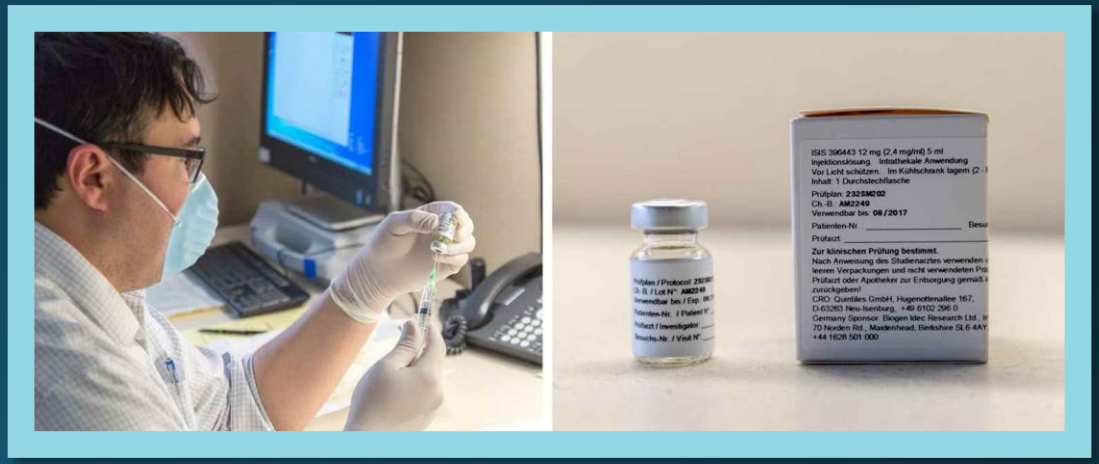
SMA Insights

Classification according to age of onset (ISMAC)	
SMA Type 1	**Acute infantile**
SMA Type 2	**Chronic infantile**
SMA Type 3	**Chronic juvenile**
SMA Type 4	Adult onset

Clinical Classification of SMA			
SMA Type	**Age of Onset**	**Motor Milestones**	**Avg. Age of Death** (limited intervention)
I	**< 6 months**	**Unable to sit without support, poor head control, difficulty swallowing & feeding…**	**<24 months**
II	**< 18 months**	**Can sit but cannot stand, delayed motor milestones, scoliosis, intercostal muscle weakness**	**20's-30's**
III	**> 18 months**	**Can stand and walk; hand tremors, scoliosis, gait and hip weakness**	**Normal life expectancy**
IV	Adolescent or Adult	Retain walking, pain, mild motor impairment	Normal life expectancy

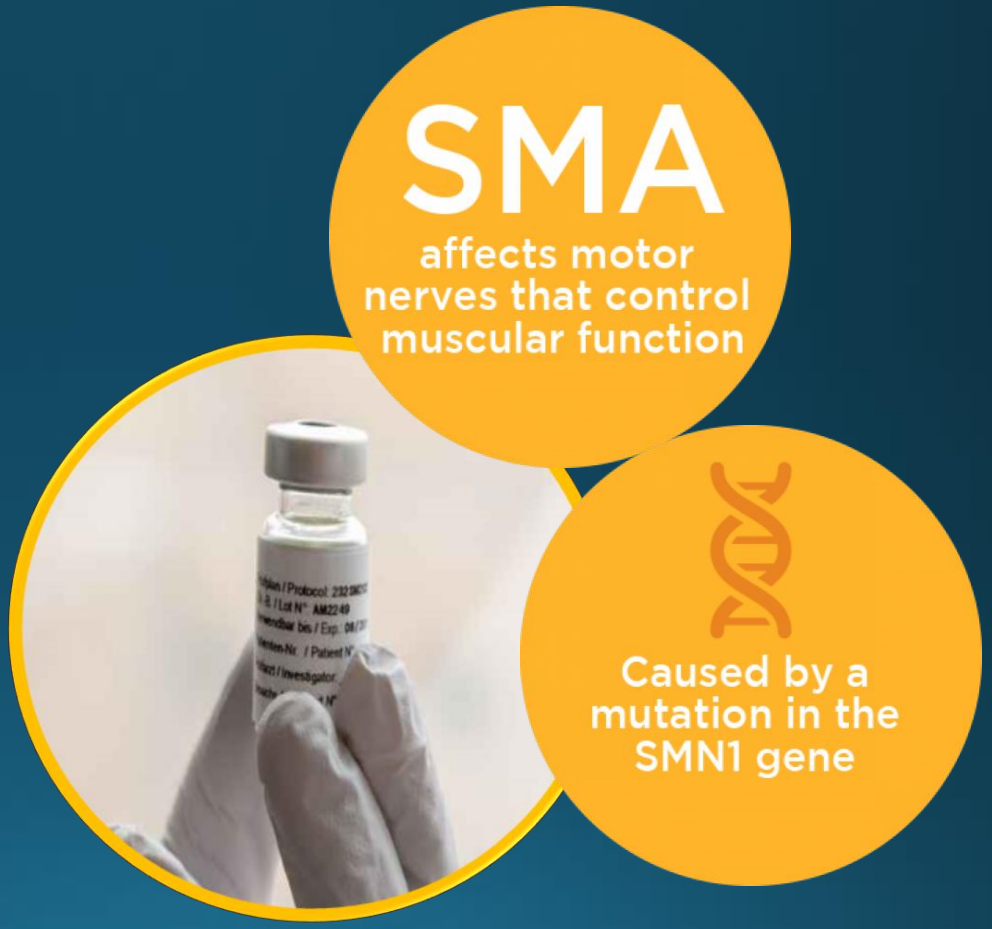
Spinraza for SMA

a *life changer* for some,
a *life saver* for others.

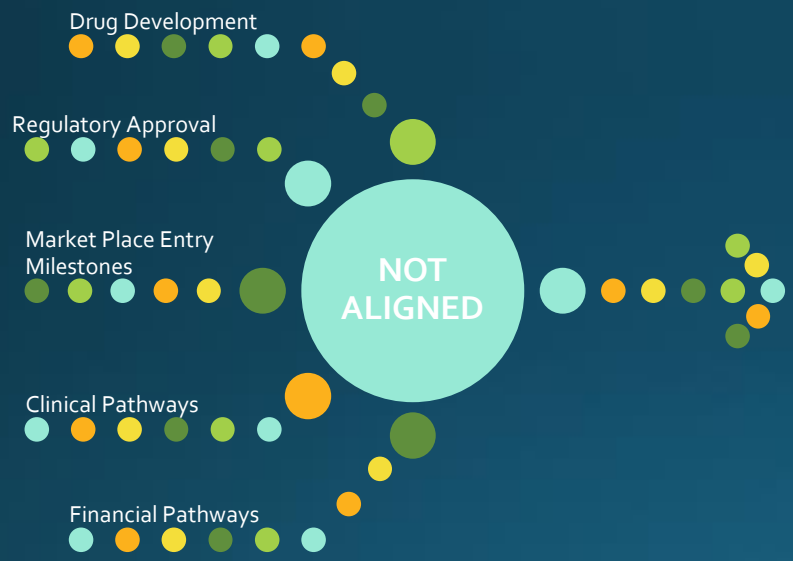


Spinraza – for SMA

Patient Access Challenges



Case Study –
Gillette Children's Specialty Healthcare



- "Embrace" clinical trial
- Spinraza ™, also called nusinersen,
- Per FDA, patients randomly assigned to receive Spinraza ; random controls halted <1 year; outcomes proven
- FDA quickly approved the drug on December 23, 2016
- And then….

Case Study –
Gillette Children's Specialty Healthcare

- Formulary challenges
- Regulatory distribution challenges
- Government payer challenges
- Commercial payer challenges

- 'burden" of process…and cost

- a *life changer*, *life saver* out of reach of patients and their families

Accelerated approval process for orphan drugs is a plus for patients,

only if patients can access the drug

Without price and payer clarity, standard coverage processes, access is limited.

And without access…

The effectiveness of the Orphan Drug Act to expand access is eroded

Spinraza, Gillette Children's, & Minnesota

Neuromuscular Neurologist

- Advocate –
  - MN Medicaid Formulary
  - Contacted each family:
    - Drug
    - Delivery regimen
    - Dosing
    - Expected outcomes
    - Access challenges

Orphan Drugs

A Path Forward for Pediatrics

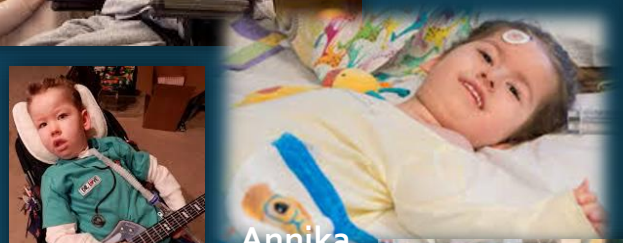
Agree that

Availability  Access

How can we better improve access to orphan drugs for children?

Today's Message

- Accelerated approval of orphan drugs to market creates both opportunities and challenges for children and their families– and front-line providers
- Complex conditions and diversity of pediatric population make orphan drug dynamics even more complicated
- Together we need to create needed system improvements –
  - Regulatory agencies
  - Pharmaceutical manufacturers
  - Health insurance companies
  - Healthcare delivery systems



Annika

Photos from social media outlets

Thank you