Khrystal K Davis,
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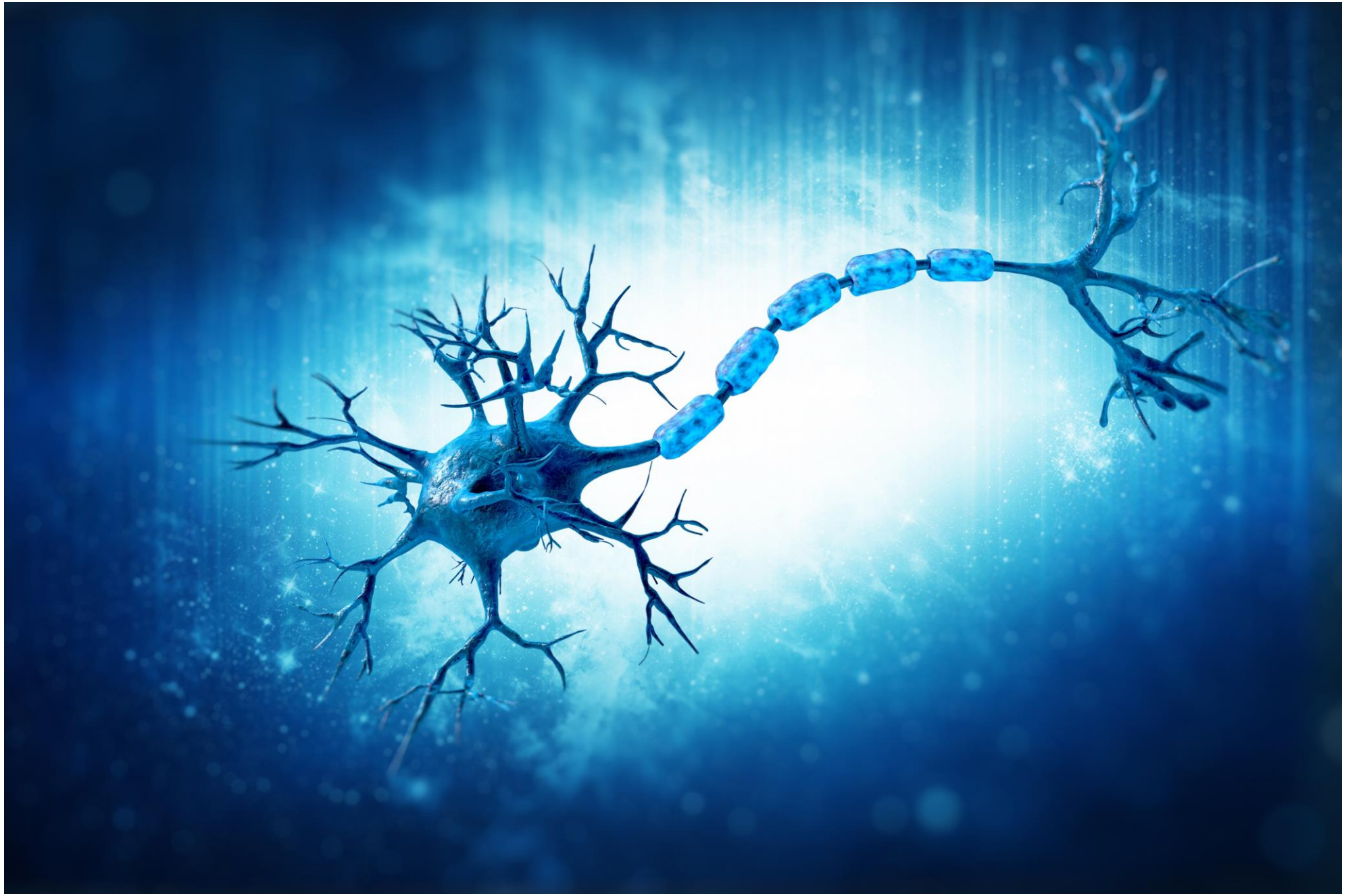

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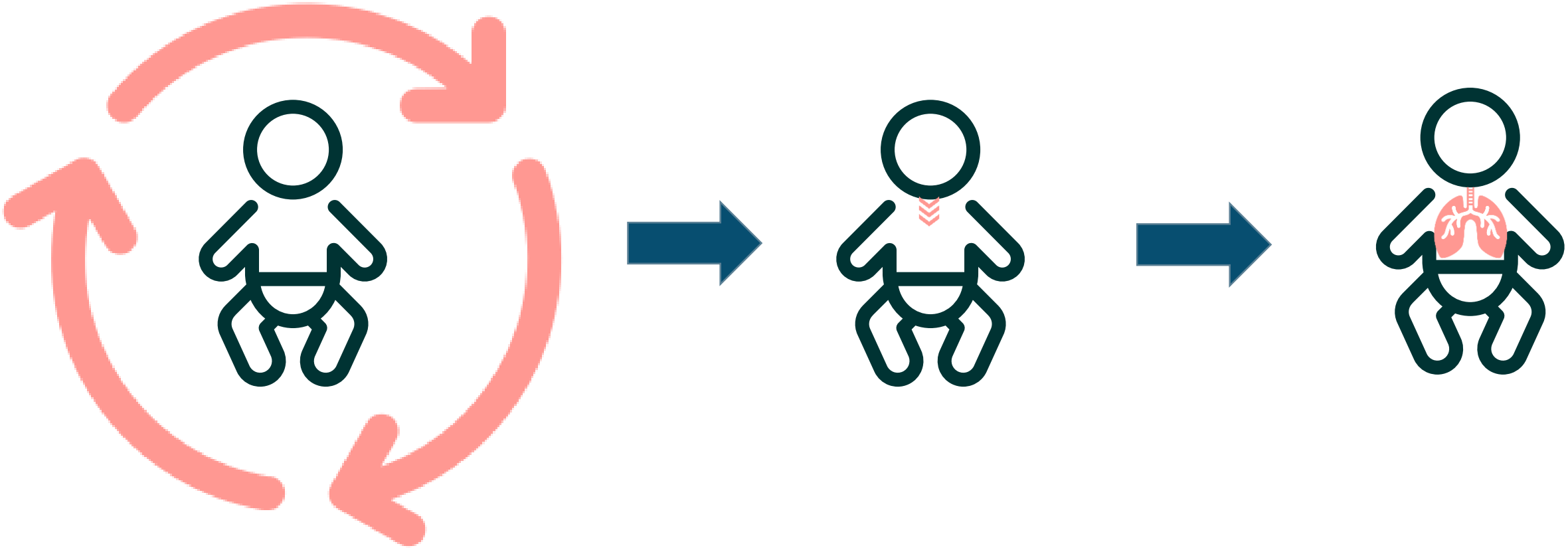

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Advocacy for Hunter

- Diagnosed September 30, 2011
- Obtained chemistry for ASO morpholino
- Manufactured compound
- Located hospital and doctor to provide therapy in Mexico
- First treatment November 22, 2011 (<8 weeks)
- Treatments every 6 weeks for 5 years

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FDA Access Meeting

- May 4, 2016 - FAST closed-door meeting at FDA on the footsteps of the Duchenne public advisory committee meeting
- Asks:
  - Stop placebo trial
  - Provide preapproval access for weakest patients
  - Approve early
  - Approve for all SMA patients

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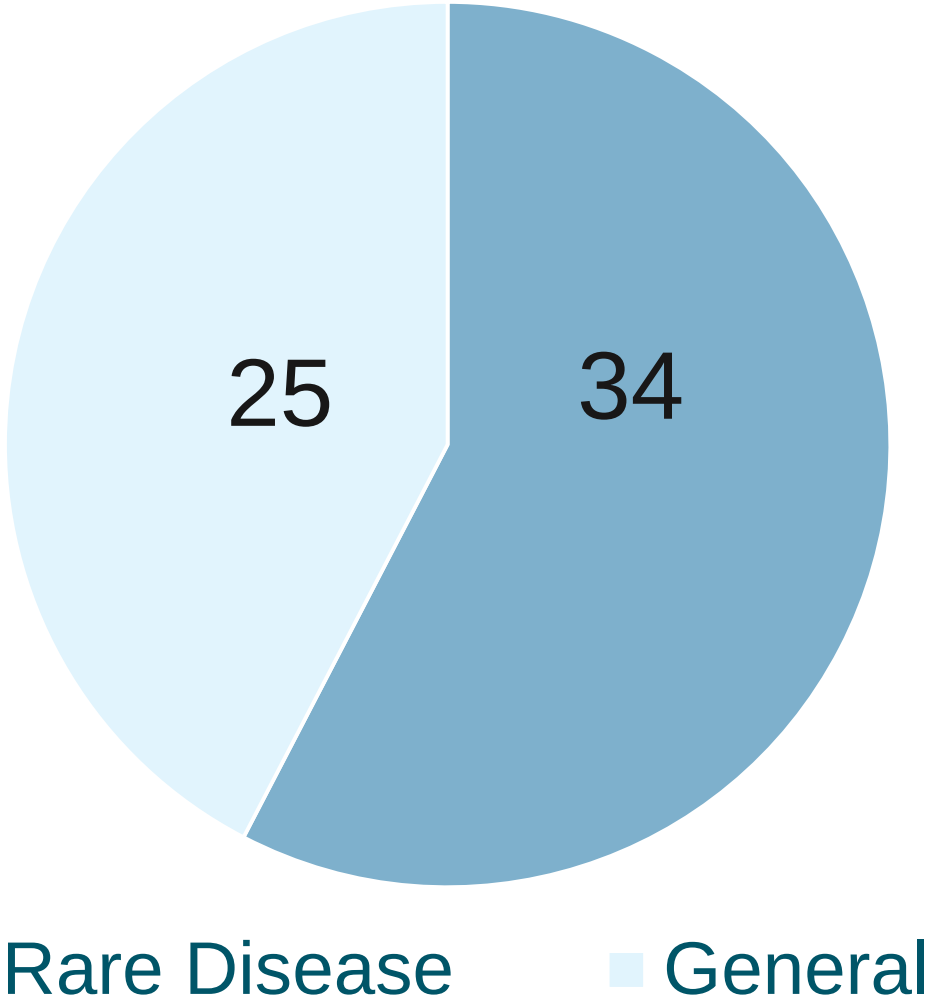
FDA Response

- June of 2016 - Interim look
- August of 2016 - FDA announced primary endpoint met
- August of 2016 - Placebo trial stopped
- August of 2016 - Spinraza EAP for Type 1 patients
- December 23, 2016 - Approved for pediatric and adult SMA patients
- Fastest FDA approval to date

2018 FDA Approvals

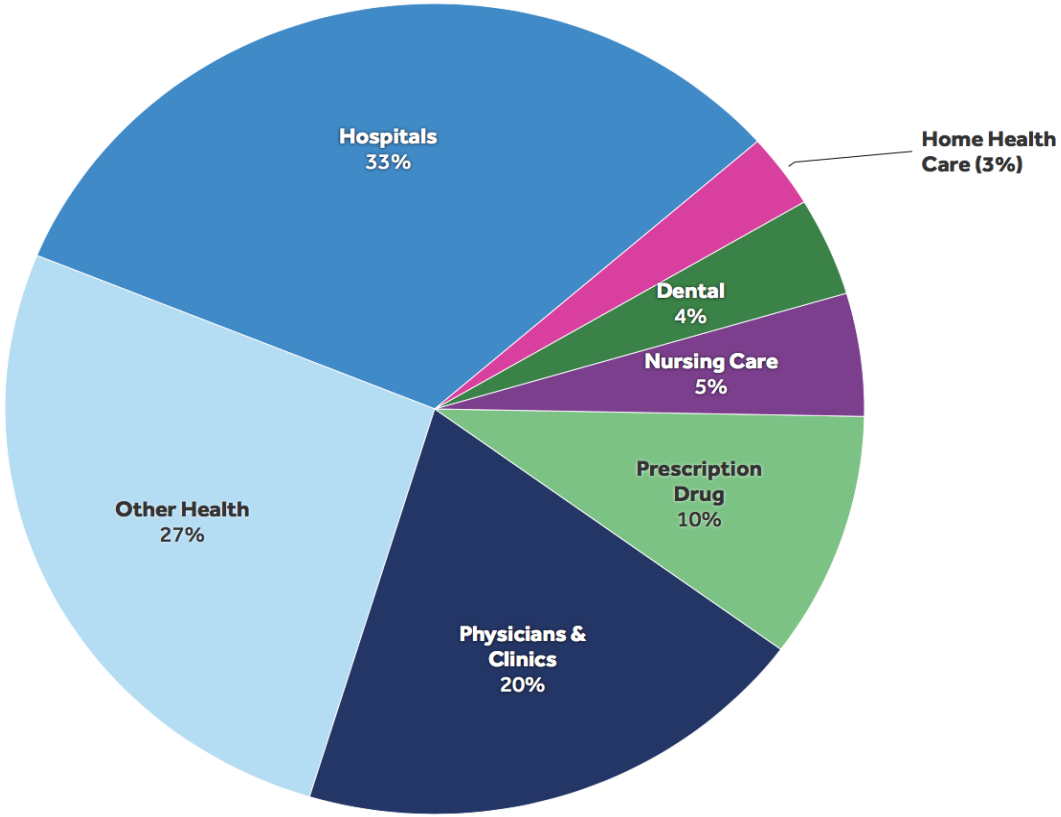


SMA As a Case Study

- Leading genetic killer of babies without treatment
- First approved therapy - ASO, Biogen's Spinraza
  - $750,000 the first year – loading doses
  - $375,000 for subsequent years – dosed every 120 days
- Second approved therapy – GRT, Avexis's Zolgensma
  - One-time IV-infusion over 60 minutes
  - $2.1 million reimbursed over 5 years
- Third contender – Small molecule, Genentech's Risdiplam
  - Approval & price to be determined

Hospital and physician services represent half of total health spending

Relative contributions to total national health expenditures, 2017



Source: Kaiser Family Foundation analysis of National Health Expenditure (NHE) data • Get the data • PNG

Peterson-Kaiser
Health System Tracker

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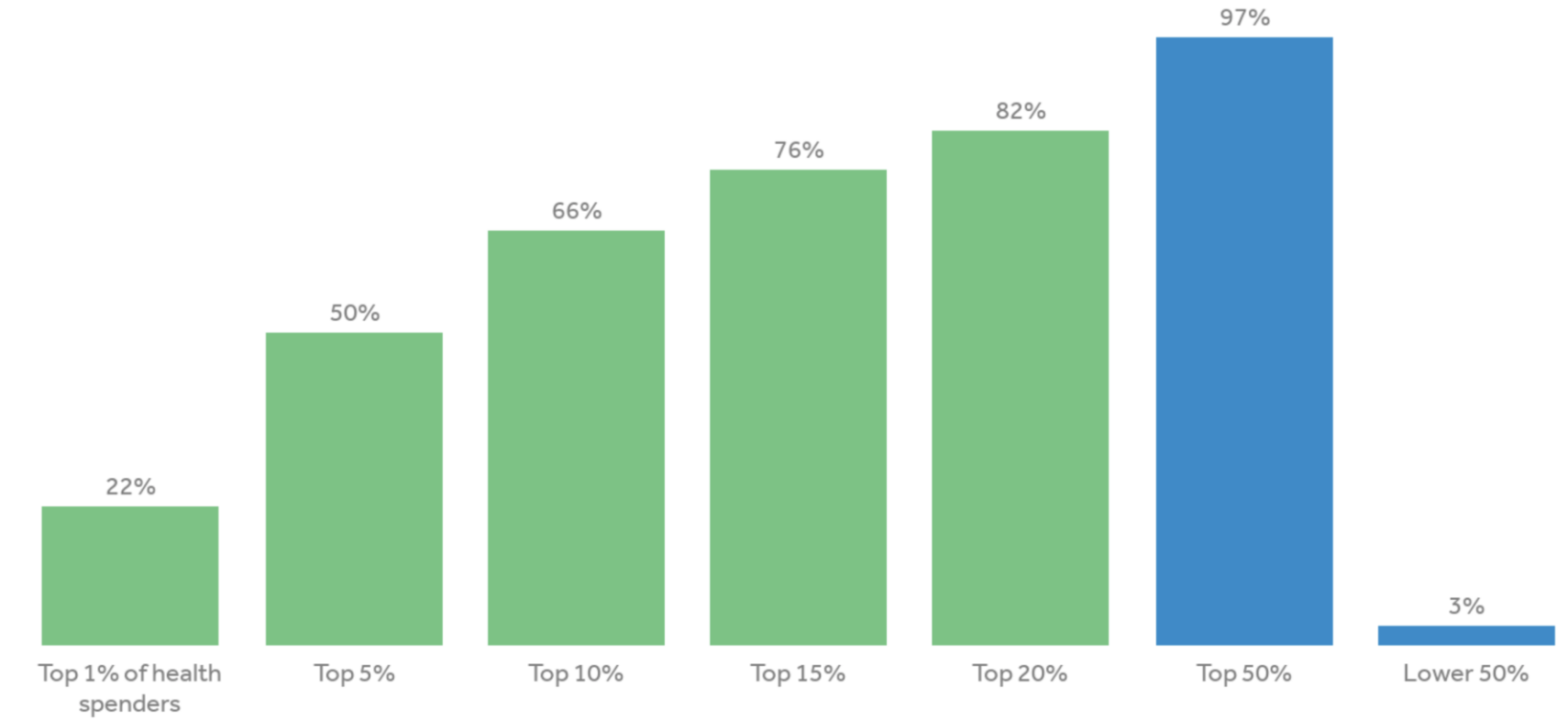
Contribution to total health expenditures by individuals, 2016



Source: Kaiser Family Foundation analysis of Medical Expenditure Panel Survey, Agency for Healthcare Research and Quality, U.S. Department of Health and Human Services
• Get the data • PNG

Peterson-Kaiser
Health System Tracker

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Medical Services & Pharmaceuticals

- Spending for medical services continues to outpace that for pharmaceuticals despite innovative therapies approved for rare diseases with unmet needs.
- Rare disease patients are provided access to medical services to treat acute medical situations caused by their diseases such as respiratory failure for SMA.
- Patients have a much harder time accessing approved treatments to maintain their baseline health to avoid acute medical crises.
- Hunter's treatments helped him maintain his baseline health and have been instrumental in preventing lengthy hospital stays.

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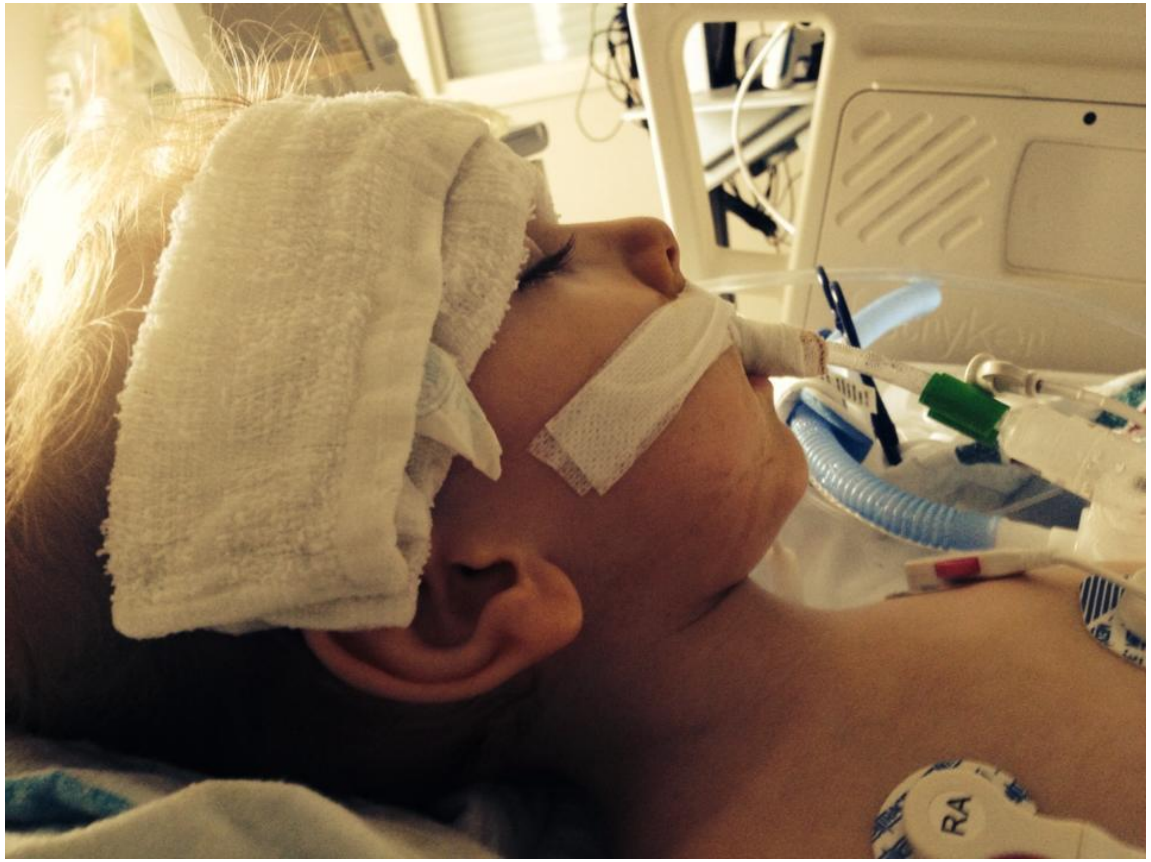

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ICER

- Lengthy hospital stays before Spinraza
  - Costs - $1.5 million
  - Emotional toll on family
  - Time away from other children
  - Curtis returned from India business development trip
    - Missed work
    - Lost business
- Discussed the overall economic impact of SMA
- QALY failed to capture the economic impact of Hunter's hospitalizations to our family, company, and community
- No hospitalizations since beginning Spinraza
- Little impact on our insurance – 80/20 letters

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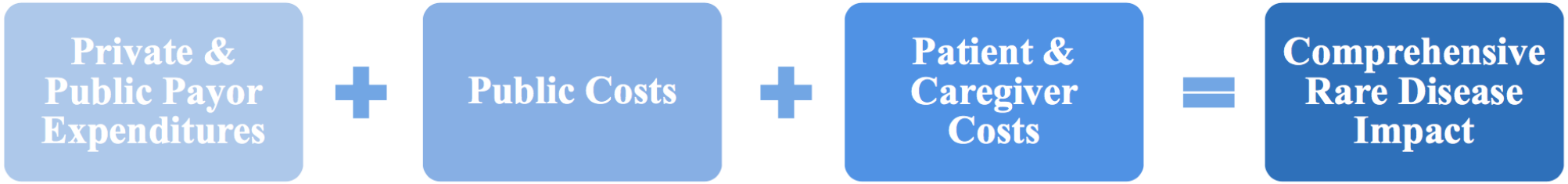
Comprehensive Rare Disease Impact Analysis



Includes:
- Hospital Care
- Physician & Clinical Services
- Home Health Care
- Durable Medical Equipment
- Medical Supplies
- Prescription Medication

Includes:
- Educational Accommodations
- Educational Interventions
- Therapies (Speech, Occupational, & Physical Therapy)
- Nurses
- Personal Care Attendants

Includes:
- Loss of Income
- PTO Used for Caregiving
- Insurance Deductibles
- Insurance Copays
- Home Modifications
- Accessible Vehicles
- Uncovered Equipment, Supplies, & Medication

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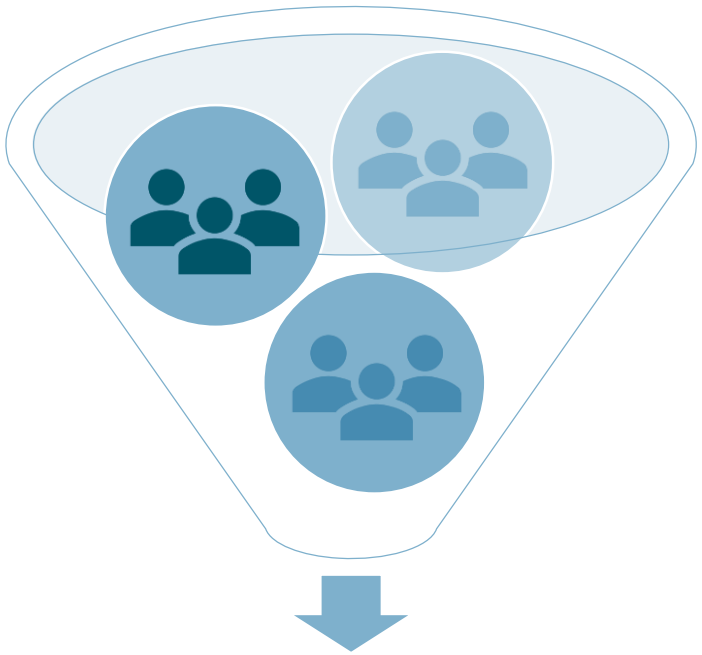
External Price Controls Don’t Work

Technology & Innovation Are the Answer

- As of 2018 US is the largest producer of crude oil
- By 2025 US crude oil production will exceed Saudi Arabia and Russia combined

Innovation in Rare Disease Trial Design



Inclusion Data for FDA Label

- Include patients from all segments of the disease population the therapy is intended to treat
- If a separate trial is designed to collect robust efficacious data, run the trials concurrently
- Inclusion trial data is needed in label packet for payors to extend access to patients from all segments of the disease population

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Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities – Questions and Answers

Guidance for Industry and Review Staff

JUNE 2018

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Final

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Clinical Trials Guidance Documents

Issued by: Center for Drug Evaluation and Research
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research
Office of the Commissioner

This guidance provides answers to common questions regarding firms' communication of health care economic information (HCEI) regarding their prescription drugs and medical devices to payors, formulary committees, or other similar entities with knowledge and expertise in the area of health care economic analysis (collectively referred to as payors). This guidance also addresses common questions relating to dissemination to payors of information about medical products that are not yet approved or cleared for any use and dissemination to payors of information about unapproved uses of approved/cleared medical products.

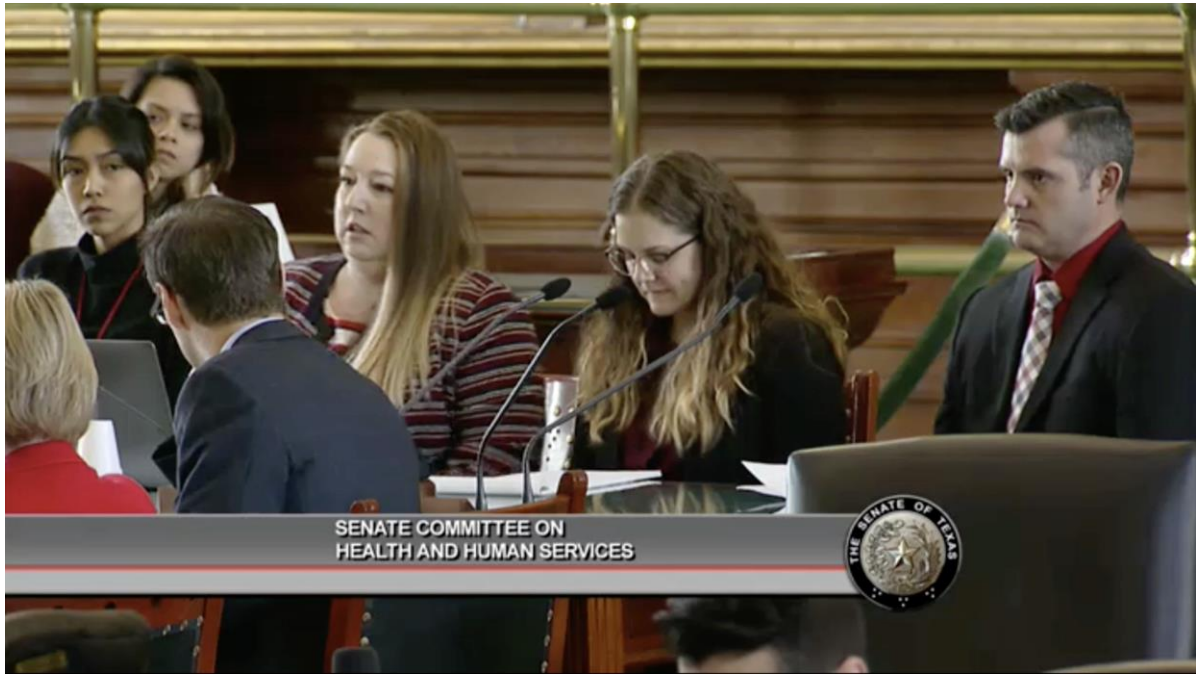
Content current as of:
11/02/2018

Regulated Product(s)
Biologics
Drugs
Medical Devices
Administrative / Procedural

- Discussions between manufacturers and payors regarding health care economic information do not constitute preapproval marketing activity
- Manufacturers can go to payors early and often
- Patient advocates should go to payors too
- Ask for an advisory committee
- Educate the payors on how the therapy positively impacts the patient community
- When appropriate, discuss the overall economic impact of the disease and how the therapy could reduce costs

Texas Senate Testimony for Newborn Screening

- Many rare disease treatments work best when provided presymptomatically.
- Most babies and children continue to be treated following the onset of symptoms, greatly diminishing their chances at achieving an average childhood.
- Presymptomatic diagnosis and treatment will afford rare disease patients the best health outcomes.
- Addressed both the cost of the therapy and the overall economic impact of the disease when left untreated.
- Presymptomatic treatment and diagnosis should prevent many patients from being eligible for Medicaid waivers making private insurers responsible for the cost of the therapy.



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#NotWorthles

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