

Rare Disease Issues State by State

Newborn Screening



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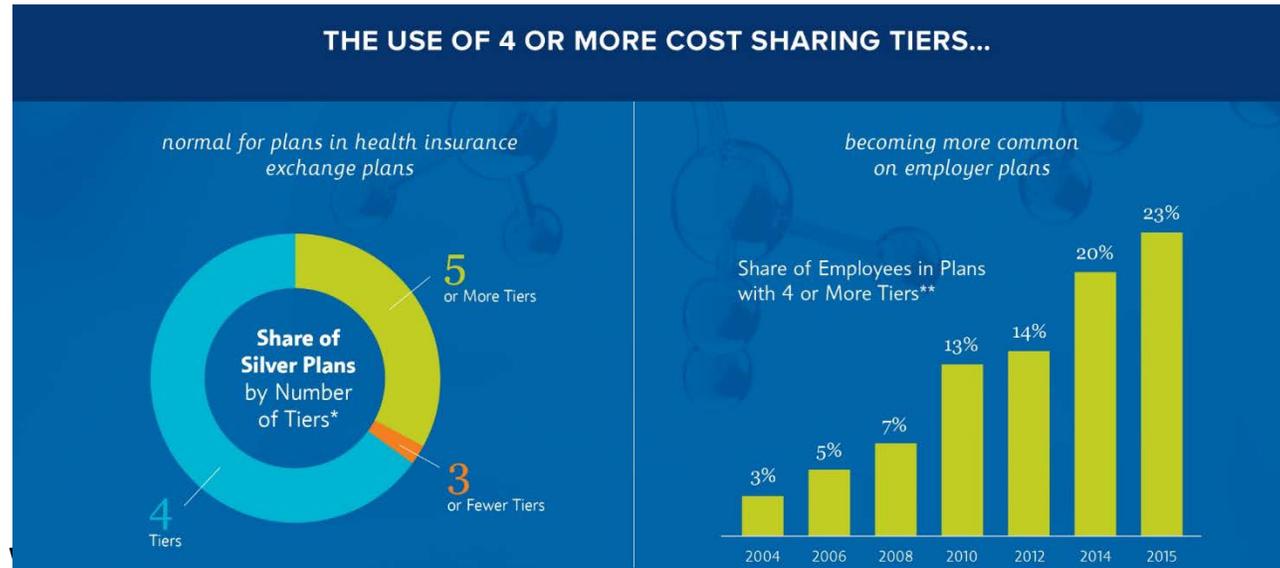
Supporting Rare Disease Patients Through State Policy

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State Policy Impacting Rare Disease Patients

- **Right to Try** – allows for access to investigational drugs before they are approved by FDA
- **Specialty Tier Co-Pay** – requires patients to pay a percentage of the cost of drugs
- **Step Therapy** – requires patients to “fail first” on a cheaper therapy to reduce the cost for health insurers
- **Medical Foods** – provides coverage or financial assistance of medical foods for metabolic disorders
- **Rare Disease State Advisory Councils** – Creates a state-level advisory council
- **Newborn Screening** – allows states to screen for life-threatening diseases preventing death and disability
- **EXPERT Act** – allows for experts to be involved in the Medicaid decision-making process

Specialty Tier -Out of Pocket



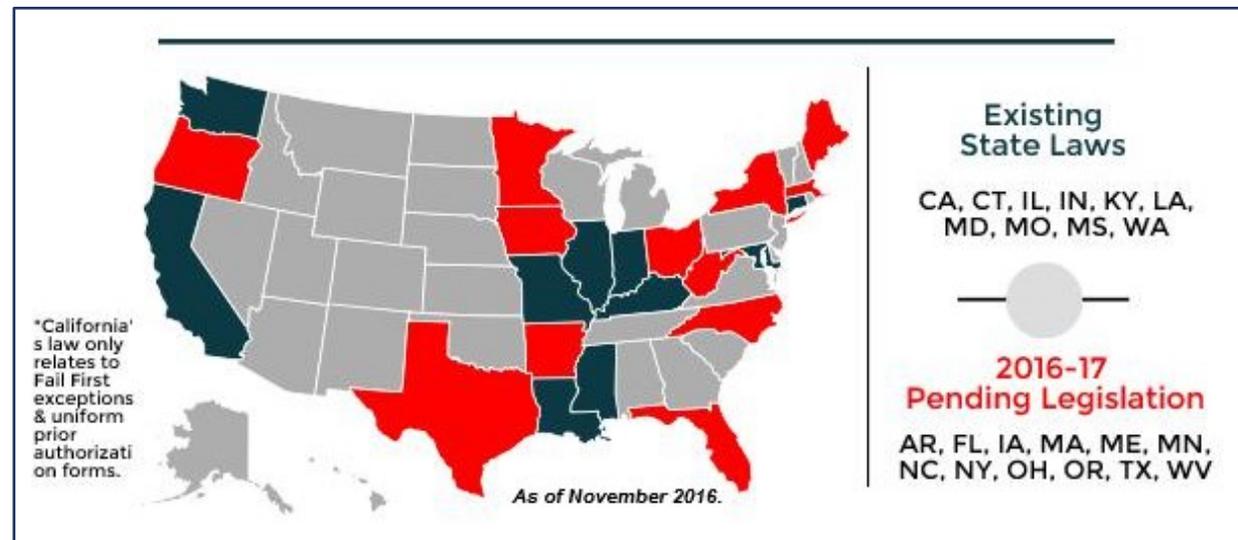
with co insurance as high as 40%, states have responded by passing new laws and regulations limiting how much cost sharing can be charged for any drug

- Cap amount of cost sharing for all drugs at a certain level (e.g., \$150 per 30-day supply)
- Require that a subset of plans have a co-pay only model for drugs and limit overall cost sharing to 1/12 of the MOOP

Currently, only 9 states have any comprehensive policy in place: CA, CO, DE, LA, ME, MD, MT, NY, and VT

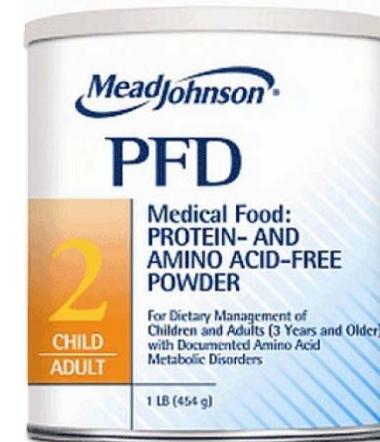
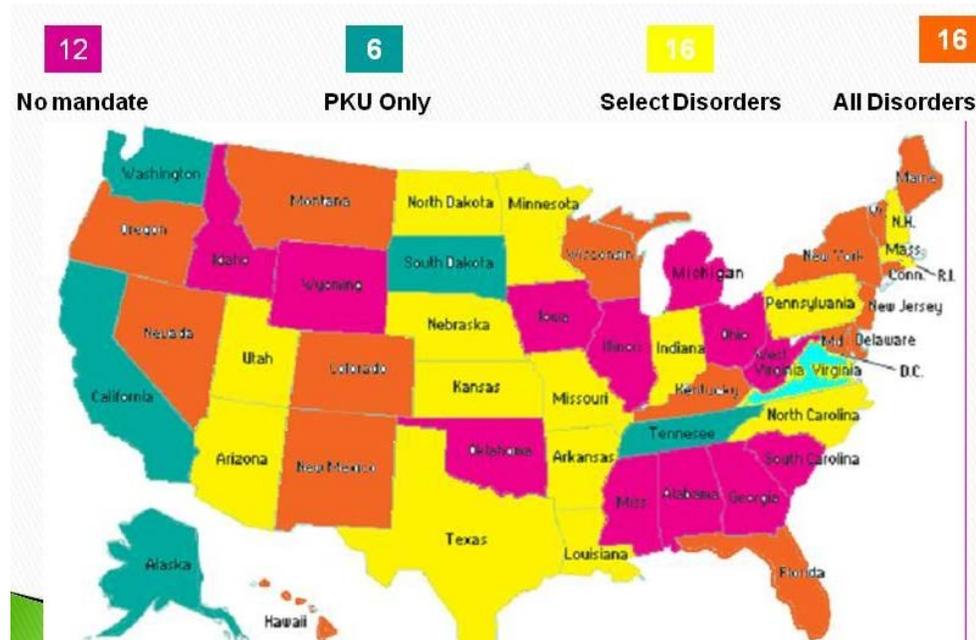
2017 National Landscape on Step Therapy/Fail First Reform

- Increase oversight of insurers' use of Step Therapy/Fail First
- Make sure Step Therapy/Fail First process is safe for patients
- Establish minimum exceptions rules and ensure transparency in exceptions process
- Currently, 11 states have passed legislation to reform Step Therapy practices to protect patients (CA, CT, IL, IN, KY, LA, MD, MO, MS, WA, and WV) and policy changes are in the works in 10 others (AR, FL, MA, ME, MN, NC, NY, and OH) Bills are also expected in Iowa, Texas and Oregon, in 2017



Medical Foods

- Some metabolic diseases treated through diet
 - PKU can cause server mental disabilities within a few weeks
- Medical Foods are expensive and are not always covered by insurance
 - Georgia now has a program to cover medical foods if someone doesn't have insurance, but it's not a mandate
 - Wyoming now has a state mandate



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State Rare Disease Advisory Councils

- Four states have created Rare Disease Advisory Councils (aka Commission or Task Force)
 - **Connecticut, Illinois, North Carolina & Rhode Island**
- These are official advisory bodies intended to increase awareness of rare disease within state government.
Some common features:
 - Require that a rare disease survivor and caregiver be on the council itself
 - Broad representation from different medical disciplines and state departments
 - Specific purpose is to make recommendations to the governor and/or legislature about ways to improve care for rare disease community
 - Often features a collaboration with local universities
- In 2017, rare disease advocates are seeking to build councils in new states:
 - **Georgia, Kansas, Massachusetts, Michigan**

For more information, please contact Tim Boyd at NORD (tboyd@rarediseases.org)



Sibling 2
3.6 yrs old

Treatment
From Birth
(normal spine)



Sibling 1
7 yrs old

Treatment
from 3 yrs old
(deformed spine)



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NEWBORN SCREENING



- We can do better!
- Universal screening will ensure that no baby is left behind
- If all states screen for what is recommended by experts, we can save lives and money

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NEWBORN SCREENING



- A federal committee of physician experts recommends a screening for a panel (RUSP) of life threatening rare diseases
- States can take up to 8 years before beginning screening for a disease after it's recommended
 - Most states have to pass new legislation to implement
 - Patients and parent advocates spend their limited time and resources testifying in front of each legislature
- Some states screen for nearly 60 diseases while other screen for as few as 29
- The legislative delays result in delays in diagnosis and irreversible mental and physical health challenges and even death
- Babies should not live or die based on their zip code

Newborn Screening Legislation Saves Lives and Money

- 30% of children with rare diseases won't live to see their 5th birthday
- It takes an average of 7 years for rare disease patients to receive an accurate diagnosis



Boys pictured above with ALD – left diagnosed and treated early

- Treatment can halt the progression of the disease – but cannot reverse the damage
- Delays in treatment can result in decline in cognitive abilities, costly surgery, the need for occupational therapy, special needs education, etc.
- End of life care for children with ALD can exceed 3 million dollars per child
- California saves \$9.32 for every dollar spent on newborn screening

California Newborn Screening Legislation – SB 1095

- Largest number of babies born in the US per year
- New legislation was needed every time a new disease was added to the federal recommended screening panel (RUSP)
- Introduced by Dr. Richard Pan in February 2016
- Requires that California screen for a disease within two years of its addition to the RUSP
- Supported by more than 120 patient organizations
- Passed unanimously through the Assembly and Senate
- Requires screening of MPS I and Pompe disease by September 2018
- **Governor Jerry Brown signed on September 16, 2016**
(7 months after introduction)



No it was not that easy: Challenges we face:

- Cost and feasibility of screening
 - Procuring new kits and reagents
 - Hiring new staff
- Cost of treatment
 - Enzyme replacement therapy would be partially covered by Medicaid, so concerns about effect on state budget
- Hospital Association concerns
 - Would hospitals be required to procure new equipment?
- Department of Public Health concerns
 - Timeframe for implementation

Florida



- EveryLife is now focused on passing similar legislation in Florida
- Screens 215,000 infants each year for every RUSP disease except for X-ALD, MPS I, and Pompe (4th highest birthrate)
- Taking a tailored approach
 - California bill would be unconstitutional in Florida
- Genetics & Newborn Screening Advisory Council
 - Meets twice a year and comprised of 15 members
 - Recommended X-ALD, but Governor did not include it in his budget
- This legislation would require the Council to review new diseases added to the RUSP within one year, and enable Florida to screen for any disease on the RUSP within one year of approval by the Council

Moving the Florida bills forward



FL SB 1124: Newborn Screenings

- Senator Lauren Book (D, Fort Lauderdale)

FL HB 963: Newborn Screenings

- Representative Heather Fitzenhagen (R, Fort Myers)



Important Dates:

- **March 7th** – Session begins
- **April 25th** – Last day for committees to meet and consider bills
- **May 5th** – Last day of legislative session
- **Governor must sign or veto legislation within 15 days of passage**

How you can help!

- **Submit patient stories** –illustrate why newborn screening saves lives and money
- **Share action alerts to Florida contacts to write legislators**
- **Sign your organization on in support** – join the many patient organizations supporting this effort.
- **Meet with legislators and testify** – Florida patients and advocates will be instrumental in demonstrating the need for robust screening in the state.
- **Provide economic data in support of newborn screening** – robust economic data on how newborn screening saves money by avoiding costly surgeries, misdiagnoses, mental health support, developmental care, and other healthcare costs helped us make the case to California’s Legislature.
- **Join our Community Congress Working Group!**

www.RareScreening.org