Rare Disease Issues State by State

Advocating for Rare Patient Access in Medicaid

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Two-Hat Sue

• ED of CFRI, cystic fibrosis patient support and advocacy group formed in 1975 by a group of parents of young children with cystic fibrosis (CF). Based in Palo Alto, CA but with 20,000 global constituents.

• Over 26 year volunteer history with CFRI, while in other executive positions at nonprofits before becoming ED in 2013.

• Added 10 new education programs and support services for the CF community including an advocacy program, Many Voices ~ One Voice, to increase advocacy activism across the nation on issues impacting the CF community.
Have worked with Medicaid funded state insurance programs

• in CA, OR, AK, TX, TN, and UT to alleviate barriers to accessing FDA approved medications.

• Second Hat: Mother of a beloved adult daughter with cystic fibrosis.
Cystic Fibrosis – A Rare Disease

• CF is a silent disease – not well known to those outside the CF community – like many of the 7,000 identified rare diseases.

• Jerry Cahill and Up for Air documentary of his life with CF pre and post double lung transplant.
Advocating for Rare Patient Access in Medicaid

• Focus is on advocating for rare diseases that have FDA approved drugs but are experiencing barriers in accessing them through their state Medicaid programs.

• Rare Disease Community Ice Breaker – Oh yeah!!!
What is Cystic Fibrosis?

• Most common fatal genetic disease in North America

• Affects ~ 30,000 in the United States and 70,000+ worldwide

• Both parents must carry the genetic mutation to produce a child with CF

• 25% chance their child will have CF

• 1 in 31 people are a silent carrier of the gene. Guaranteed there are many carriers in this room today.
Cystic Fibrosis - Continued

• The genetic defect produces thick mucus which clogs ducts in the body, resulting in damage to the major organs in the body, most often the lungs, but also to the endocrine, reproductive and digestive systems.

• CFers take 7-20 medications daily & do airway clearance therapy multiple times a day, may be on in-home IV treatments, supplemental oxygen and have many hospitalizations due to infections.
I sometimes need oxygen to breathe.
Having CF is a full-time job. As the disease progresses, more time is spent on therapies to stay as healthy as possible.
The primary cause of death is pulmonary failure due to inflammation and infections that destroy the lungs.

• Median age of death is approximately 29 years old.

• CF community is fortunate that there are many different medications to treat the disease.

• But there is still no cure.
My Daughter Victoria’s Story

• Successful liver transplant 1997 at age 12
• End-stage CF 2 years ago
• Admitted herself to her CF care center with the remnants of a cold in June 2015
• Four days later: pulmonary failure and life support for 11 days. Death was imminent.
• Victoria received a double lung transplant exactly 4 weeks after admission (in July 2015.)
• She is now 32 and able to enjoy her life again with her husband and my grand pups.
Roadblocks to Accessing rare disease drugs denied by state Medicaid programs

• FDA approved drug does not guarantee access to rare disease therapies, particularly in state Medicaid programs. WHY?

• State Medicaid programs have extremely powerful Drug Utilization Review Boards or Pharmacy and Therapeutics committees that determine medication coverage
DUR Boards and P&T Committees

- Medicaid coverage decisions for therapies are often made through opaque processes and **without input** from experts in their rare disease states.

- These boards rarely include rare disease patients, clinicians, or advocates.

- Many lack clearly defined processes for coverage decisions.

- Usually do not provide opportunity for public or clinical experts to provide input.
Road Rage

• Many state Medicaid programs will put restrictions on access not consistent with FDA criteria.

• Decisions left to those that typically do not have rare disease expertise – thus the need for policy changes, perhaps like the EXPERRRT Act followed by the FDA.

• Current system can lead to policies that are inconsistent with real-world clinical practice and harmful to patients.
How to Reduce Barriers to Access

• Monitor state Medicaid sites for their meetings and agendas
• Advocate for access, hope and health
• Increase awareness of negative impact
CFRI’s Method

• Be proactive and persistent
• Contact multiple individuals
  • DUR Boards, P&T Committees, Medical Directors and state and federal legislators
• Email
• Letters
• Phone calls
• Personal meetings
1. **Educate** them about CF

2. **Serve as** a CF rare disease resource/expert

3. **Give specifics** on harm caused to the CF population for whom the drug is intended

4. **Ask them** to remove the barrier to access

5. **Offer to give** testimony during their review process – the letter might serve as public testimony
6. Find a CF advocate in that state to testify if they have a public forum

7. Ask CFRI’s constituents in state to send letters – if needed

8. Follow up

9. Continue to offer assistance to find solution

10. Be persistent!
CA. GHPP and CCS Medicaid

- Added restrictive clauses to Orkambi not in FDA criteria for access
- Contacted medical director for a meeting
- Brought 17-year-old CF patient to tell his story
- Stressed urgency to act now to save lives
End Result

• Called meeting of CA centers, CFRI and medical director in Dec. 2016

• Dropped restrictions for children to age 20
• Agreed to work on same for ages 21+
• Centers to supply data to prove success
• Meeting in March to review
Not Always a Happy Ending

• Need advocacy movement to push Congress to encourage state Medicaid entities to incorporate the EXPERRT Act

  • A. Develop an open, transparent procedure for coverage policy determinations

  • B. Include public, patient, clinicians, and manufacturer perspectives during the drug review process
• C. Include on the DUR and P&T committees relevant expert specialists for that particular disease group and drug

• D. Make the draft policy public

• E. Ask for public testimony on the draft policy

• F. Return the policy to the Drug Utilization Board for a final review and vote BUT only after incorporating steps a – e.
• We have our work cut out for us.
• Rare disease community is powerful if we all come together as one
• Never give up
• To give up is to remove hope from the lives of those with rare diseases
• Be persistent
• Stay focused
• Stay hopeful
Thank you!  
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