

An aerial photograph of a winding asphalt road that curves through rolling green hills. The road is dark and has white dashed lines. The hills are covered in lush green grass, and the overall scene is bathed in a warm, golden light, suggesting late afternoon or early morning. The text is overlaid on the upper portion of the image.

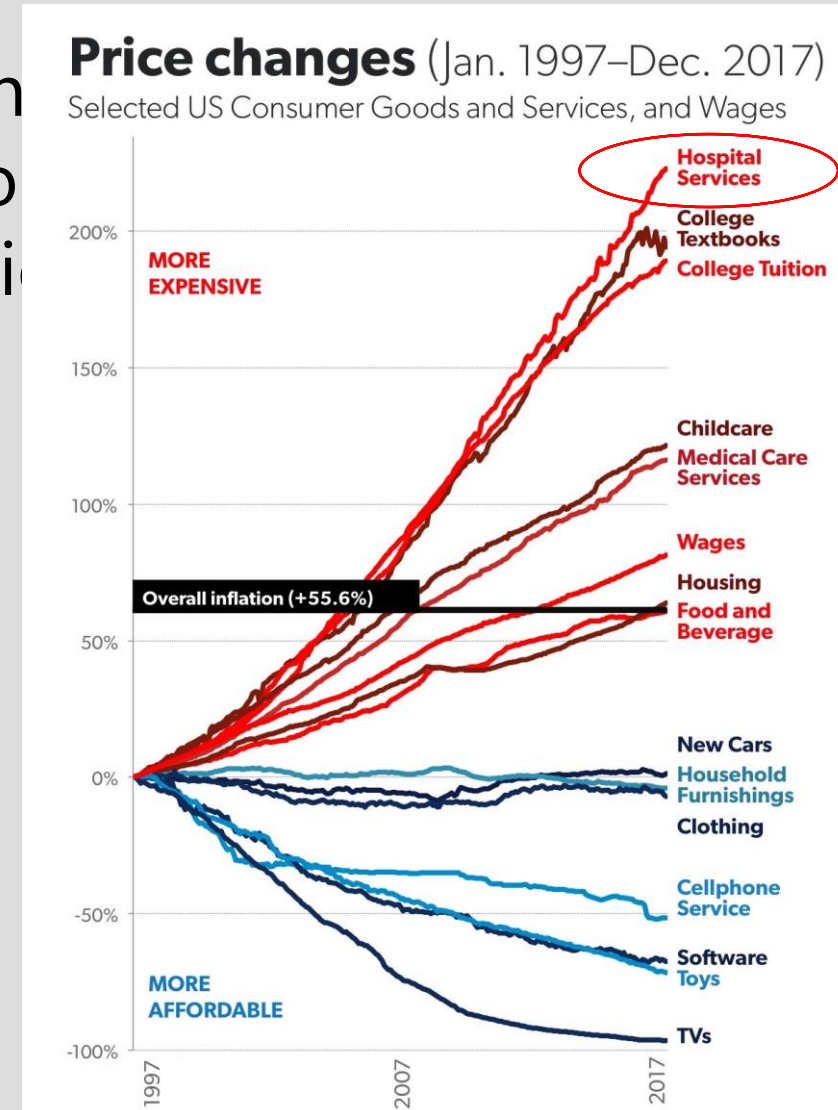
# The Road Ahead: Comparing the Treatment of Rare Conditions with Other Aspects of Care in an Era of Cost Containment

Stephen L. Ondra, MD  
*North Star Healthcare Consulting*

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[@StephenOndra](https://twitter.com/StephenOndra)

# Health spending continues to spiral:

- Around the world, the rate of increase in health spending continues to outpace other key sectors of the economy and is forcing government action
- In the United States;
  - Health spending was \$2.7T in 2016
    - Government directly covered 40% of the population and 50% of health spending
    - Medicaid was 19% of the total health spend
  - Health spending projected at \$5.7 T by 2026
    - Government will directly fund 48% of the population and 60% of overall healthcare spending
    - Medicaid will rise to 21% of the total spend



There is a global shift in reimbursement away from the traditional volume driven *Fee-for-Service* models and towards more outcome driven *Fee-for-Value* paradigms

- Initially catalyzed by government, *(the ACA created CMMI in the United States)* this shift is increasingly being driven by the private sector payers, employers, and some providers
- Similar efforts are taking place worldwide to address the issue of the unsustainable rise in the cost of healthcare

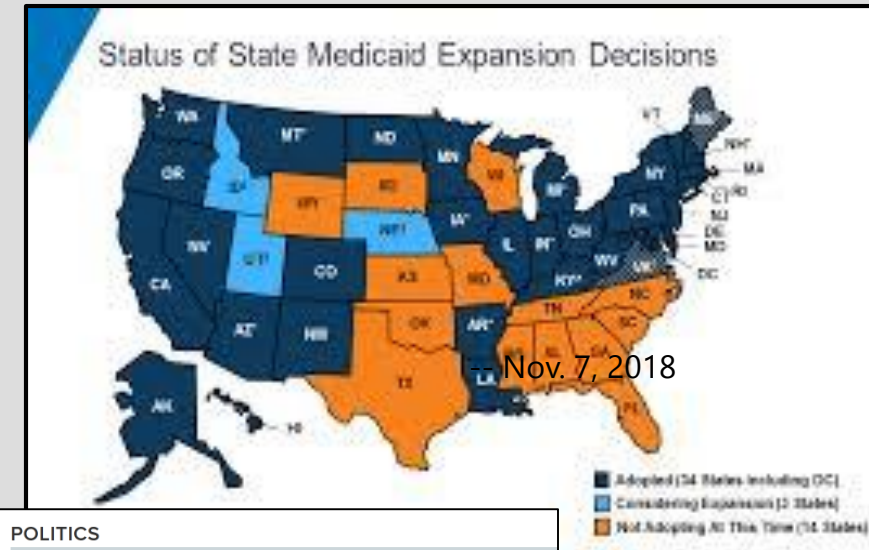


# New challenges for populations with rare conditions:

- The pressure on state legislatures and the Federal government to contain costs;
  - Likely be addressed by expanding Managed Medicaid, similar to Medicare Advantage

- 75% of all rare conditions are in the pediatrics

- Changes in reimbursement methodology will have a profound impact on the care of these vulnerable populations



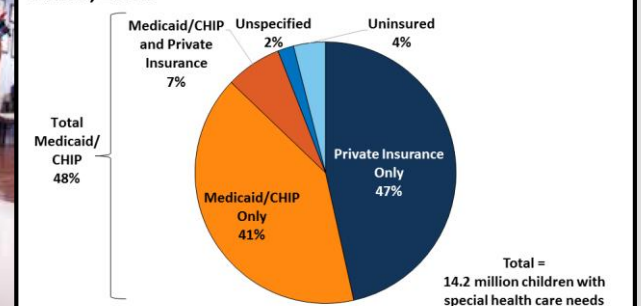
## POLITICS

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**Three red states vote to expand Medicaid during an election where health care was the top issue**



Figure 1  
Health insurance status of children with special health care needs, 2016.



NOTES: Includes non-institutionalized children ages 0-17. Totals may not sum to 100% due to rounding.  
SOURCE: Kaiser Family Foundation analysis of the 2016 National Survey of Children's Health, Topical File.

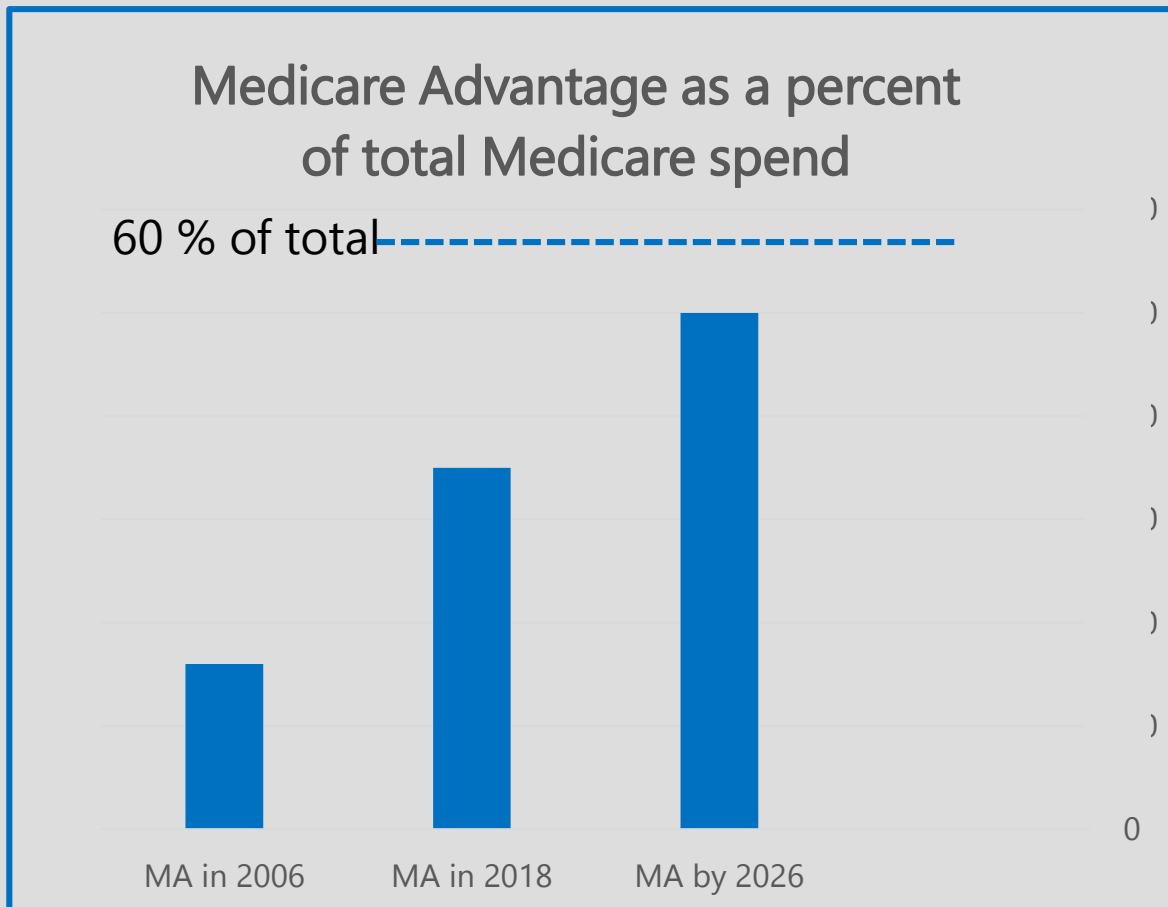
# Navigating a transforming healthcare world:

*Increasingly constrained resources and the transition to value based reimbursement, will result in a competition for finite resources*

*What steps can Pediatric Orthopedics take to ensure that the treatment of rare conditions can continue to succeed and innovate*



# Prepare for the steady shift of control in Medicare & Medicaid:



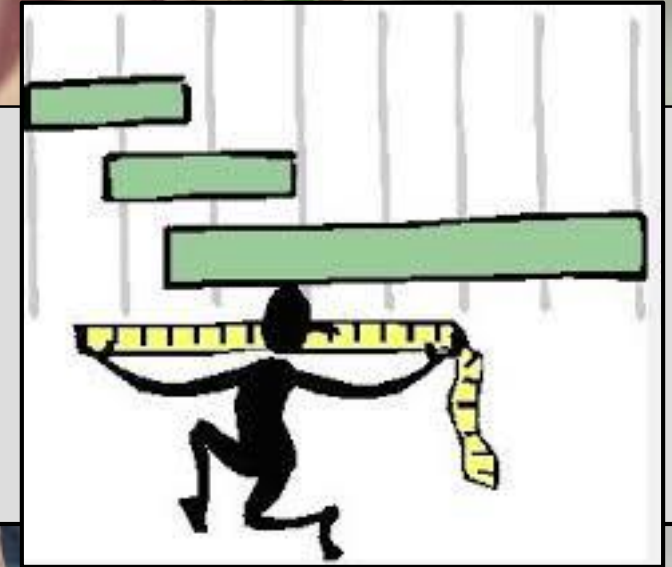
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- As in Medicare Advantage, Managed Medicaid will increasingly be delegated to private payer by CMS and state Medicaid offices
- Major Managed Medicare & Medicaid players;
  - United - 26%*
  - Humana - 18%*
  - Aetna - 9%*
  - Kaiser - 8%*
  - Anthem - 5%*
  - Others - 35%*
- Private payers will increasingly decide;
  - who is in network
  - what will be covered
  - how much to pay providers
  - what will be the reimbursement model
- Government will do requirements & oversight

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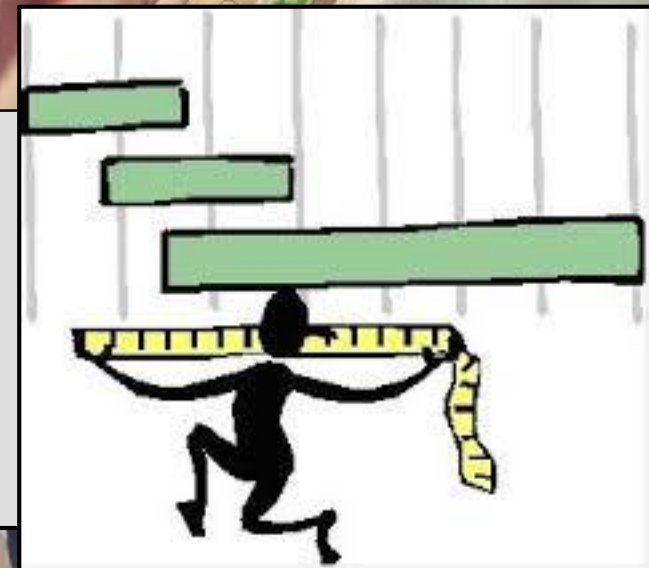
# Strategies for success in the treatment of rare condition in a changing world:

- Registries are an essential tool to combine and aggregate information in rare conditions and should be a major purpose and function of professional societies and organizations
- When building a registry;
  - Identify partners with shared interests to co-fund
  - Build to minimize implementation & maintenance
  - Focus on high level ontology leaving taxonomy
  - Should use data reconciliation tools and not rigid formats to take data from multiple sources and
  - incorporate technology that will minimize the need for human data extraction
  - Must meet today's business, care & research needs, but be extensible to future needs and requirements



# Strategies for success in the treatment of rare condition in a changing world:

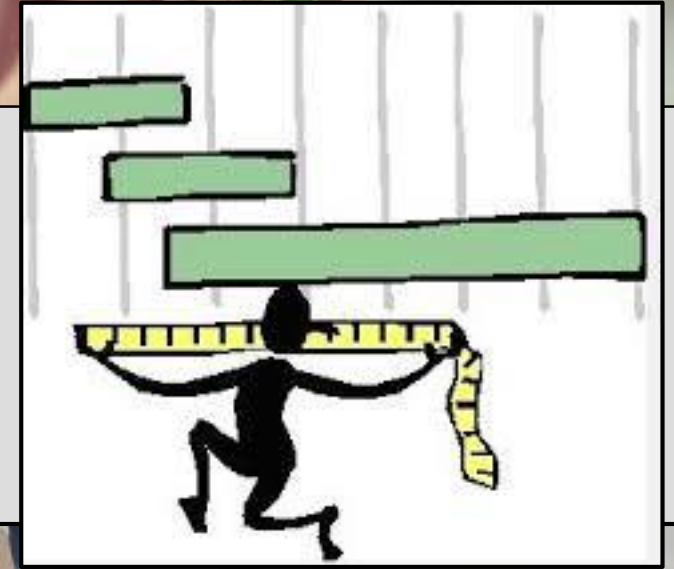
- In rare health conditions, traditional approaches to outcome measure development, such as RCT, are relative barriers to the approval of new treatments and the successful participation in value based reimbursement
- Create innovative approaches and paths for assessing outcomes for pediatrics and rare condition;
  - Use registry data to fuel "Big Data" and artificial intelligence, to derive outcome measures
  - Explore Bayesian statistical methodology
  - Patient Reported Outcome Measure use and efficacy
  - Be open to and explore other novel approaches





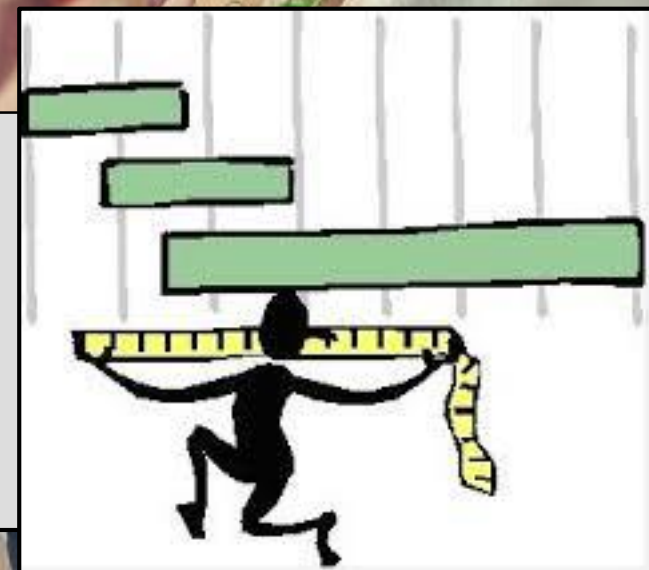
# Strategies for success in the treatment of rare condition in a changing world:

- Participation in value based reimbursement will remain difficult in pediatrics and especially for rare conditions for the foreseeable so consider interim steps;
  - Seek center of excellence contracts and reimbursement strategies as a pathway to develop the clinical and data infrastructures and the experience required for success in value based reimbursement for both payer & provider
  - Develop and implement care paths and data collection approaches that meet
    - current business needs
    - near term business concerns (*ex. --MACRA implementation*)
    - planned extensibility to the still evolving VBR models



# Strategies for success in the treatment of rare condition in a changing world:

- Continue the work with regulatory agencies and private payers to develop and establish pediatric specific paths for outcome measures that ease new treatment approval and participation in value based reimbursement models
- Policy advocacy and public opinion are essential tools that
  - ~~must be leveraged, planned & coordinated;~~ high level goals and approaches should be determined nationally but flexibly implemented on a state & local level
  - Must be coordinated across specialties whenever possible
  - Specialty specific advocacy must not loss sight of and work with, not at crossed purposes with the bigger picture
  - ***Be engaged, lean forward, be visible and be vocal***



# The transition to value based reimbursement models will be bumpy and erratic in course but steady in direction:

- Pediatric Orthopedics will need to take a different road to higher value for rare conditions than the traditional approaches being used in other conditions
- This will require openness to innovative solutions, approaches to care & thoughtful investment strategies
- Early adopters and innovators in determining value for rare condition treatment will be advantaged when a FFS to FFV tipping point is reached and the transition accelerates
- *None of this will happen without bold, forward leaning, collaborative and courageous leadership from professional organizations*



***“Of all the forms of inequality,  
injustice in health care is  
the most shocking  
and inhumane.”***

***- Dr. Martin Luther King, Jr.***