Drugs to treat rare and ultra-rare diseases: Beyond the price and headlines

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What is the thought process to manage medications for rare diseases?

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Know Each New Specialty Medication Early

- Surveillance of the drug development pipeline ≥ 24 months
- Products projected to be approved by the FDA in the next 12-18 months
- Quarterly review and updates

Clinical Pipeline Report

Drug Information Team uses this information to develop clinical and financial formulary therapeutic class strategy

It is all About Determining “Value”, But…

Member

Look for the best medication possible and access to all

Commercial health plan

Healthy Population

PBM

Clinical efficacy / cost compared to other options

Value

Self-funded employer

Good outcomes but tight budgets

Prescribers

Drugs that work and no hassles

PhRMA

Good utilization of products

Things to consider with rare diseases…

What else is available to treat patients with this orphan disease?

What is the natural progression of the disease? What will happen to the patient without this new therapy?

Is there credible evidence the new medication will improve life or provide a positive outcome?

Are we communicating well with all parties?

What is the public missing beyond the cost and headlines?

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Example of Poor Evidence

- The Food and Drug Administration on Monday approved a controversial drug to treat a rare disease that confines boys to wheelchairs and condemns them to an early death.
- The decision came after months of protracted debate about whether the drug maker had provided enough evidence to demonstrate that its medication had a meaningful impact on patients. The drug would be priced at about $300,000 a year in the US.
- In reaching its decision, the agency overruled its own medical staffers, who earlier this year questioned the effectiveness of the drug, which was tested in a small clinical trial. The wrangling raised still larger questions about standards for approving a drug, especially when it's intended for patients with a rare and deadly disease and no other treatment options.
- Many health plans have refused to cover the medication because there is not sufficient evidence of efficacy and by the FDA's conclusions find the drug to still be investigational.

Good Example of Sensationalizing Cost Only

New Hemophilia Drug can Cost $500,000 to $750,000 per year for each patient!!!

What may be missing is…

Clinical **Cost Offsets** **QOL**

Example - What is typically missing?

Drug X

FDA Approval: November 2017

Indication:

Indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors

Place in therapy

1st FDA approved the medication for hemophilia A patients with factor VIII inhibitors

Favorable efficacy and safety profile

Desirable delivery route (sub-Q vs infusion) and administration

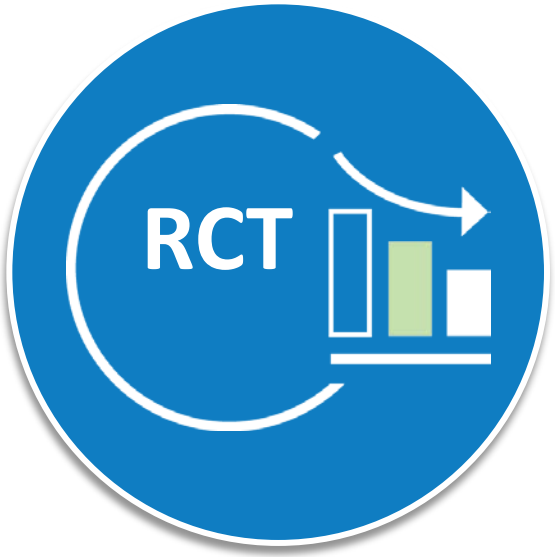
Clinical Trial Data – Haven 1 Trial Results

Primary Endpoint: Difference in bleeding rates (annualize bleeding rates) between Arm A and B

Endpoint	New Drug Prophylaxis (Arm A; n=35)	No Prophylaxis (Arm B; n=18)
Treated Bleeds		
ABR (95% CI)	2.9 (1.7, 5.0)	23.3 (12.3, 43.9)
% reduction (95% CI), p-value	87% (72.3%, 94.3%), < 0.0001	
% patients with zero bleeds (95% CI)	62.9 (44.9, 78.5)	5.6 (0.1, 27.3)
All Bleeds		
ABR (95% CI)	5.5 (3.6, 8.6)	28.3 (16.8, 47.8)
% reduction (95% CI), p-value	80% (62.5%, 89.8%), < 0.0001	

ABR = annualized bleed rate; CI = confidence interval; IQR = interquartile range, 25th percentile to 75th percentile

What is Missing with Orphan Disease Agents?



Evidence and Data prior to launch with low numbers (ie. N = 20)

Evidence and Data post launch

Real Life Experiences

Why should we think about this differently?

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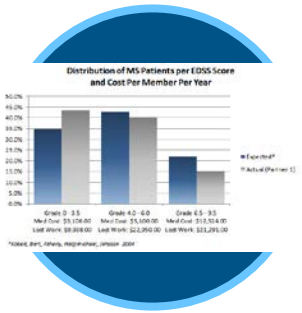
Things to consider with rare diseases…



Are there any other treatments for this rare disease? How effective are they?

What is the prognosis for patients with this rare disease (i.e. disability, death) Will this new agent change the prognosis of the disease?

If not, is there credible evidence the new medication will improve life?

Are there genetic tests that could help us understand if the new medication will work in each patient?

Possible solutions? Next steps?

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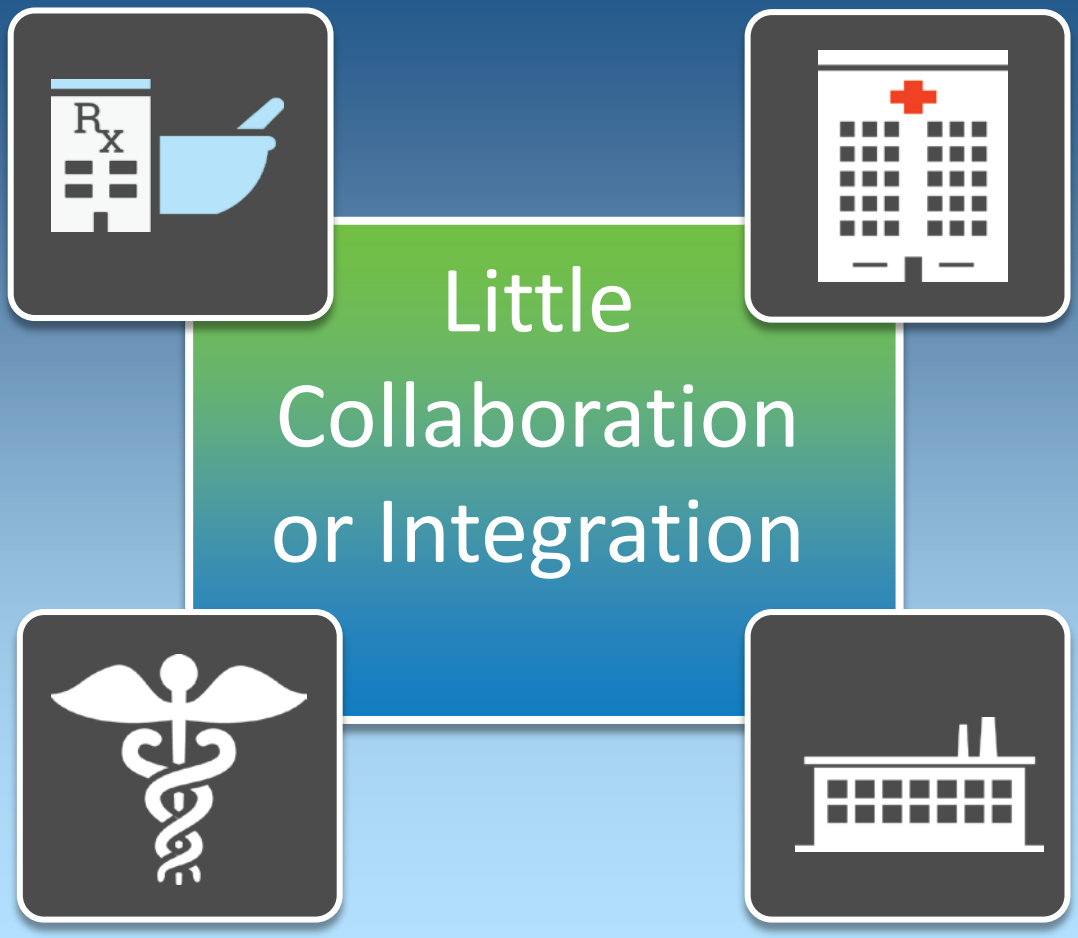
Possible Solutions…

- Provide better information to the payers…
- Showcase examples of outcomes and benefits
- Provide real world data on cost offsets
- Move toward better integration

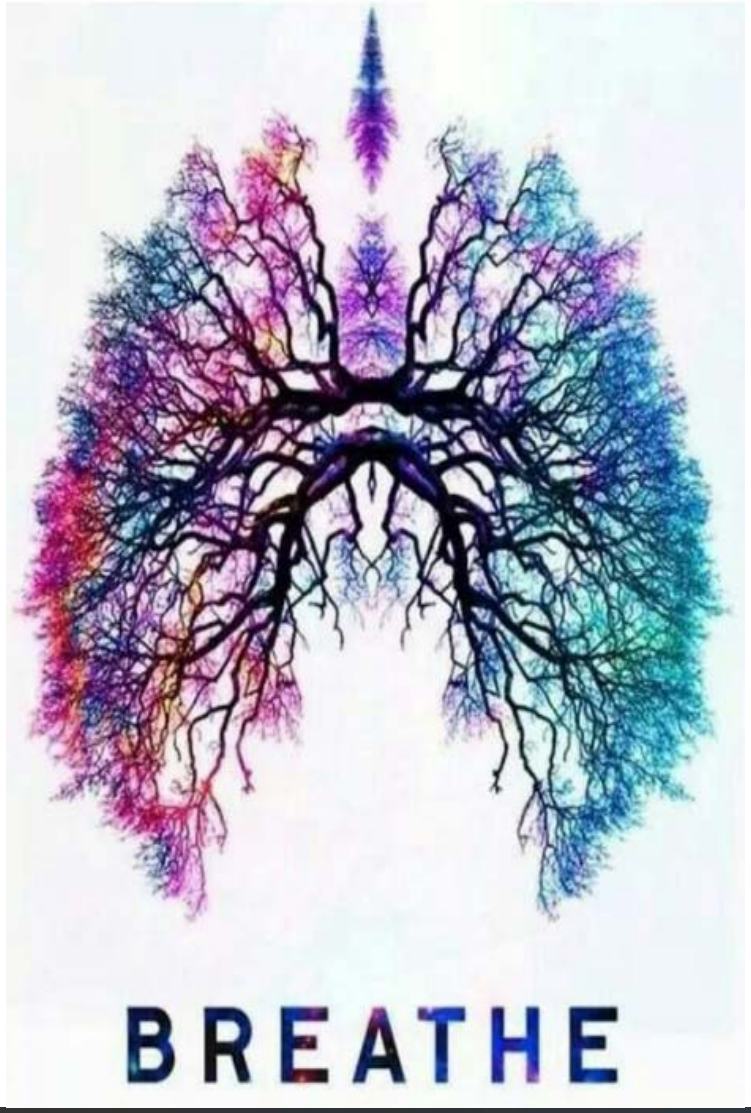
The US Health Care System is Fragmented



Example of Non-Cost Related Solutions

- **Cystic Fibrosis**
  - **Considered the challenges for payers**
  - **Considered the issues with the disease**
    - **Determine the "value" of the high cost pharmaceuticals**
    - **Total organ disease causes multiple co-morbidities**
    - **How do we prevent multiple lung infections?**
    - **Can we prevent hospitalizations?**
  - **What solutions could we provide?**
    - **Provide experts that can manage the total patient**
    - **Ensure the most appropriate agents are being utilized**
    - **Work with patients and families to prevent infections**
    - **Ensure monitoring total patient care including non-drug therapies**
    - **Provide more comprehensive reporting**

Cystic Fibrosis Clinical Support and Management



New Patient/Family Medication Counseling

- Prior to first medication shipment
- Performed by CF clinical specialist
- Focused on prescribed medication education and coaching

Example of Data Collection in Cystic Fibrosis

CATEGORY	ADDITIONAL INFORMATION REQUIRED
Patient Name	Patient first and last name
Current age of patient	For reference
Current height and weight of patient	For reference
Name of Pharmacy	For reference
Name of Physician	For reference
List of all CF-related medications	List all drugs with dosages
FEV1 level	Trend FEV1 levels over time
Chloride sweat test	Obtain levels when patient is tested
List of medications for co-morbidities	List drugs related to other disease states
Cardiopulmonary difficulties	Number and types of difficulties
Any changes in CF therapy	Change in drug or dosage
Other non-drug therapies	Respiratory care, etc.
ER or Hospitalization (Y/N)	Any ER or hospitalizations in the past month
Any specific exacerbations?	Description of any exacerbations
Last visit to a CF Center	Date of last appointment
Survey Outreach Date	Quarterly?
Chart Notes	
Any concerns that should be escalated	Escalate to Medimpact or Payer

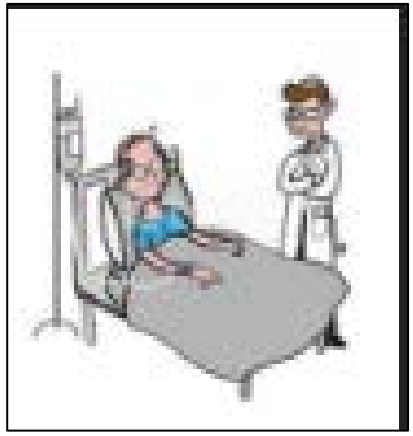
Solutions Are Coming

Medical Data

MERGE

Pharmacy Data

Total Patient Experience with Related Outcomes



Looking Forward to the Discussion

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