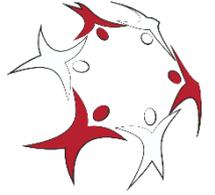


# **Haemophilia outcome measures: Industry's view**

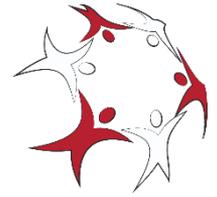
EHC Round Table of Stakeholders  
'Outcome measures in haemophilia'  
Brussels, Belgium  
November 28 2016

# ABOUT THE SPEAKER



- Jason Booth, MPH
- Associate Director, Global HEOR Haematology
- Shire
- US

# DISCLOSURES



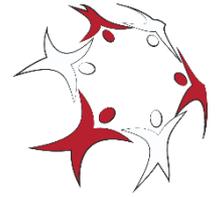
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**Conflict**

**Disclosure - if conflict of interest exists**

Employee	Shire
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# We need to better understand and use health outcome information in haemophilia

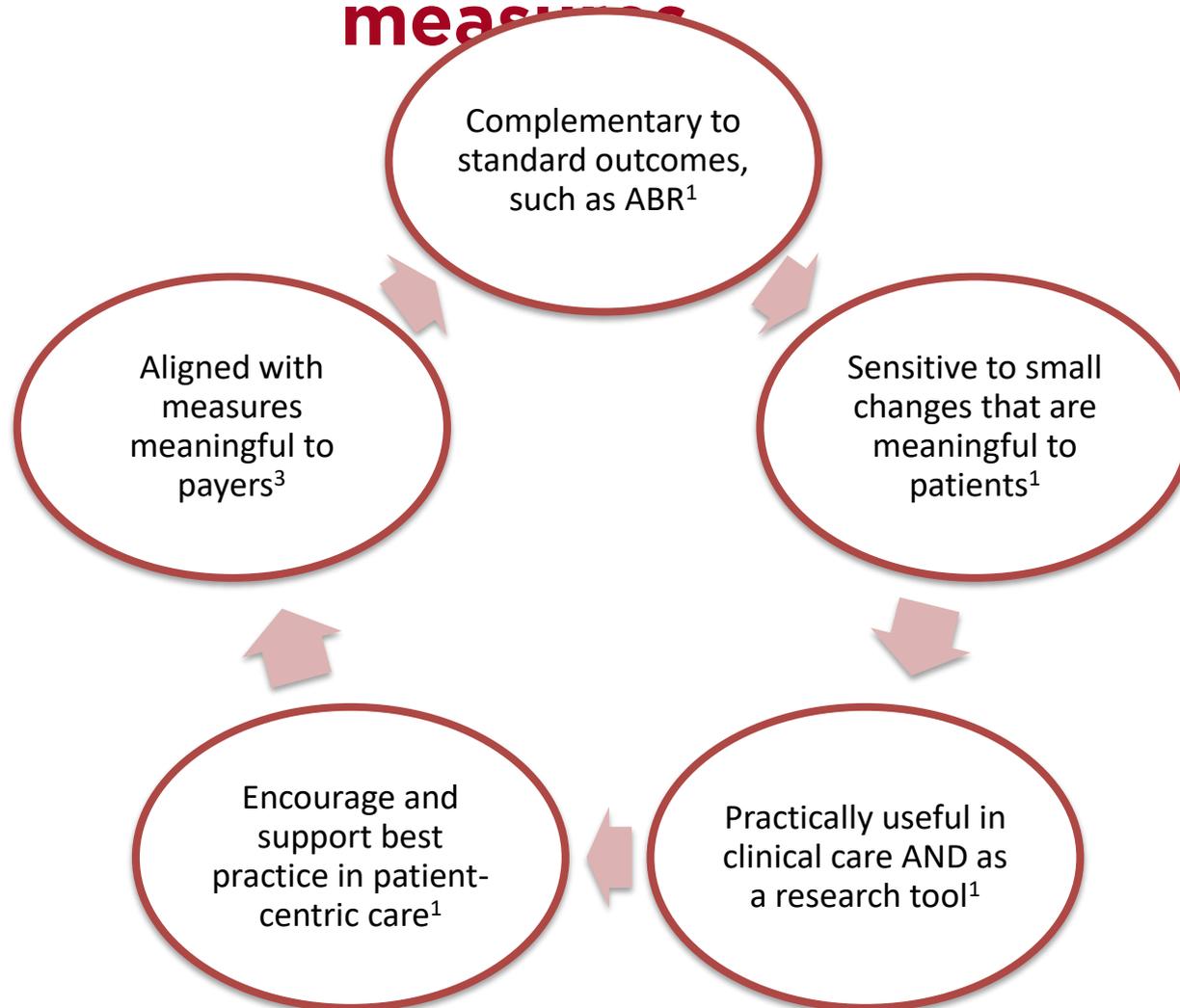
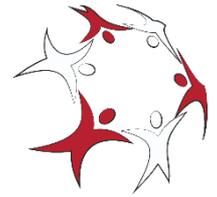


- Expectations of people with haemophilia are increasing all over the world and participation in social, work and physical activities is improving<sup>1</sup>
- Annualised Bleed Rate (ABR) is significantly reduced with prophylaxis compared to on demand treatment<sup>2</sup> however ABR doesn't reflect all disease impacts
- As a result ABR has limited utility in measuring further improved outcomes and current Health-Related Quality of Life measures are of limited use outside a research setting<sup>1</sup>
- With new therapies emerging it is important to develop an individualized view of the impact of therapy and, with support from payers, to use this information to improve standards of care

1.Recht, et al. Recognizing the need for personalization of haemophilia patient-reported outcomes in the prophylaxis era. *Haemophilia*. (2016), 1–8

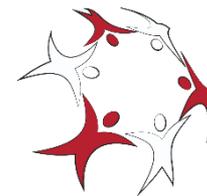
2. Ljung, et al. The current status of prophylactic replacement therapy in children and adults with haemophilia. *British Journal of Haematology* (2015) 169, 777–786

# New measures should be practical, sensitive and complement existing measures



1. Recht, et al. Recognizing the need for personalization of haemophilia patient-reported outcomes in the prophylaxis era. *Haemophilia*. (2016), 1–8  
3. Berger et al. Securing reimbursement for patient centered haemophilia care: major collaborative efforts are needed. *Haematologica* (2016); 101 (3)

# Goal Attainment Scaling (GAS) is an established approach to quantifying individual treatment goals<sup>1</sup>



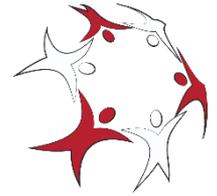
- Shared decision making in the process of setting goals and appropriate therapy is essential for effective patient-centric care<sup>5</sup>
- GAS - A *standardized* approach to *individualized* (patient-centered) goal monitoring, that is often done informally in clinical care<sup>1</sup>
- Engages the patient/caregiver in a dialogue about what is important to him/her
- Assesses extent to which a patient is able to meet/exceed their individual goals and also assess this consistently across a population<sup>4</sup>

1. Recht, et al. Recognizing the need for personalization of haemophilia patient-reported outcomes in the prophylaxis era. *Haemophilia*. (2016), 1–8

4. Turner-Stokes, et al. Goal attainment scaling (GAS) in rehabilitation: a practical guide. *Clinical Rehabilitation* 2009; 23: 362–370

5. Barry et al. Shared Decision Making — The Pinnacle of Patient-Centered Care. *N Engl J Med* (2012) 366;9: 780-781

# Standard Steps for GAS<sup>4</sup>



Define and select a patient identified goal



Define goal achievement (targeted / expected outcome)



Describe the baseline status

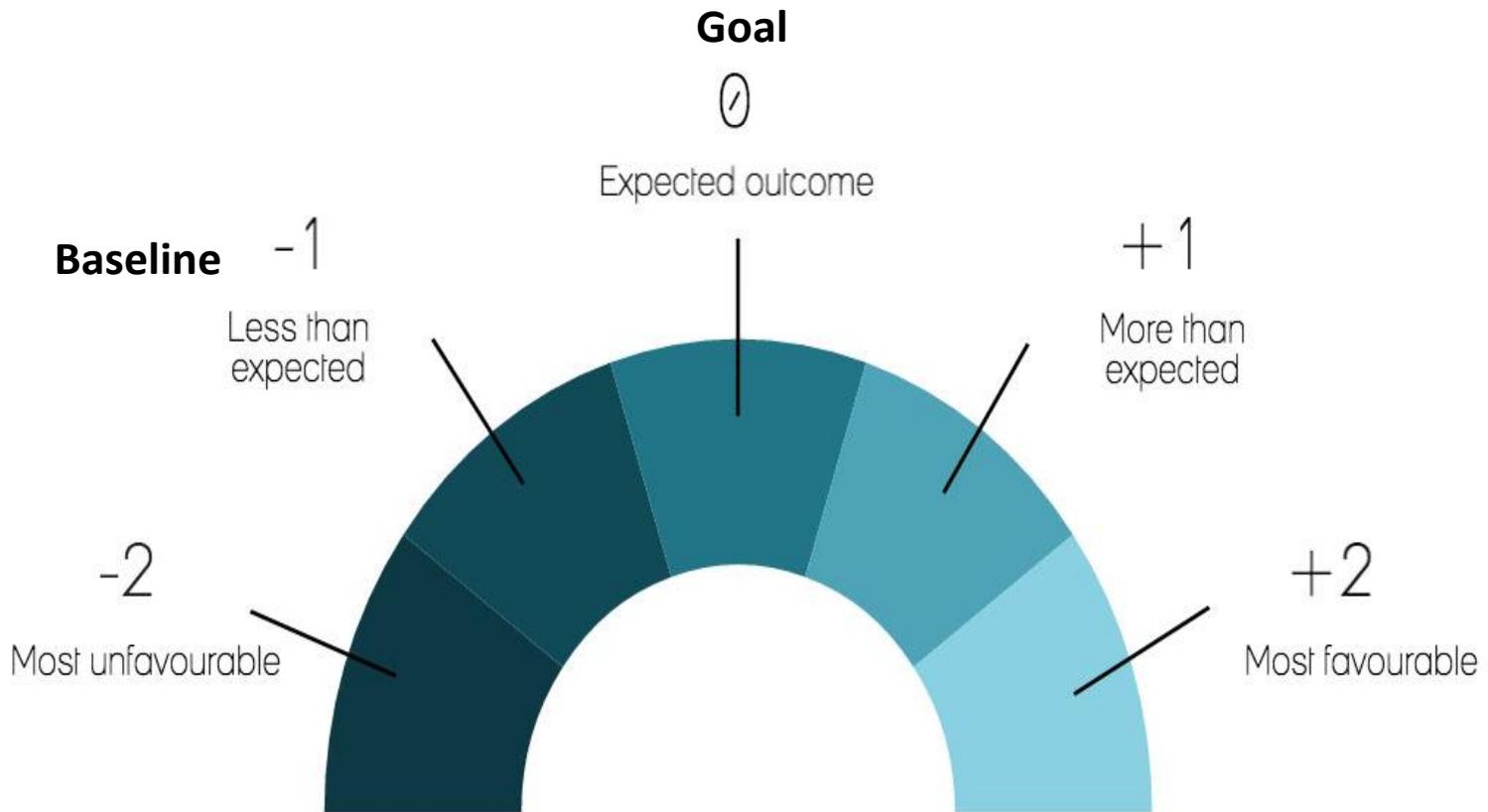
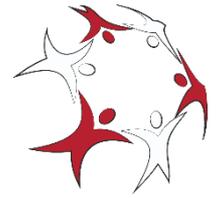


Define the better and worse outcomes to complete the scale



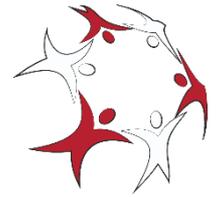
Measure goal attainment at designated follow-up interval(s) using the scale developed

# Standard Approach: 5-Point Scale<sup>6</sup>



6. Figure from <http://clik.dva.gov.au/rehabilitation-policy-library/15-goal-attainment-scaling/151-introduction-goal-attainment-scaling> Accessed Nov 2016

# A GAS for haemophilia is currently in development<sup>7</sup>



Goal Attainment Scaling for Life – Hemophilia (GOAL-Hem) will be a hemophilia-specific menu of patient-centered goal areas for use in clinical practice and research<sup>8</sup>

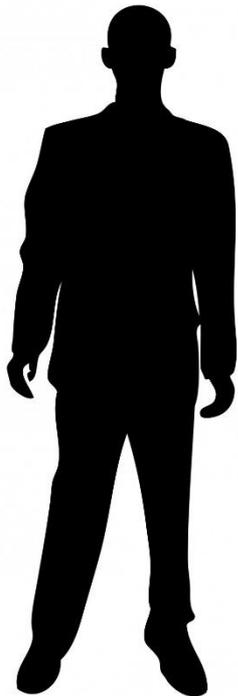
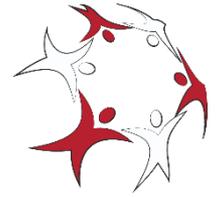
Table 1. Goal areas by category

Goal Area Category	Goal Areas	
Managing hemophilia	Being able to administer factor Medication adherence Procedure planning	Following treatment plan Weight, exercise, nutrition Hemophilia care planning
Hemophilia complications	Bleeds Muscle bleeds	Pain Joint problems
Impact on activities	Work attendance Attending school Career planning Engaging in sports Leisure activities General activities	Access to resources Daily personal care Use of assistive devices Negotiating health insurance coverage
Impact on emotions and relationships	Feelings of anger Feelings of sadness Narcotic use Substance misuse Depression	Relationship with significant other Relationships with friends Relationships with family Self-esteem

7. <https://clinicaltrials.gov/ct2/show/NCT02674997> Accessed November 2016

8. Recht, et al. Goal Attainment Scaling for Life – Hemophilia (GOAL-Hem): An Innovative Patient-Reported Outcome Measure. *Poster presented at WFH World Congress, Orlando, USA (2016)*

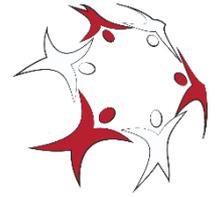
# Constructing goals with the GOAL-Hem - Example



Goal: Start exercise program to improve joint health

- (+2) I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 5x week* and able to stand on my feet *>30 min* without severe pain
- (+1) I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 3x week* and able to stand on my feet *>20 min* without severe pain
- **GOAL** (0): I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 2x week* and able to stand on my feet *>15 min* without severe pain
- **BASELINE** (-1): I have joint problems that bother me *constantly*: however, *it is difficult to manage* physical/emotional toll with *physiotherapy and/or pain management*
- (-2) I have joint problems that bother me *constantly*: however, I am *unable to manage* physical/emotional toll with *physiotherapy and/or pain management and have no desire to do so*

# GAS scoring standardizes individual goals across different patients



- Scores are effectively the sum of goal attainment X the relative goal weights transformed into a standardized (normally distributed) measure (T-score)<sup>4</sup>
- Overall scoring returns summary score of 50 when all goals are attained (individual goal attainment = 0)<sup>7</sup>

$$\text{Overall GAS} = 50 + \frac{10 \sum (W_i X_i)}{\sqrt{(0.7 \sum W_i^2 + 0.3 (\sum W_i^2))}} \quad 4$$

4. Turner-Stokes, et al. Goal attainment scaling (GAS) in rehabilitation: a practical guide. *Clinical Rehabilitation* 2009; 23: 362–370

7. <https://clinicaltrials.gov/ct2/show/NCT02674997> Accessed November 2016

# We need to better utilise existing and new outcome measures to drive improved care



- Health systems should provide... while utilising available resources
- If the right outcomes are measured and centred around these, improved efficiency (outcomes for resource used) could be achieved through personalized care<sup>9</sup>

“Cigna stressed that a key factor in this success was the alignment of payer and manufacturer incentives to support better outcomes.” *Neumann et al (2011)*<sup>10</sup>

## Example of procurement on adherence and outcomes performance in diabetes<sup>10</sup>



**Outcome measure**  
Glucose level reduction  
adherence



Discount if patients reduce glucose levels

Additional discount if patients are adherent

- If **outcomes are improved**, the manufacturer (Merck) provides a **discount to the payor** (Cigna) at the end of the year on their oral diabetes drug (Januvia and Janumet)
- Outcomes measured are **blood sugar levels** (through A1C lab tests) and **adherence** (through claims data)

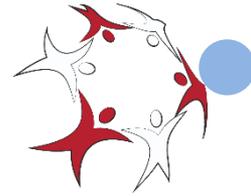
### Results after the first year

- **Blood sugar levels dropped by 5%**
- **Adherence increased by 87%**

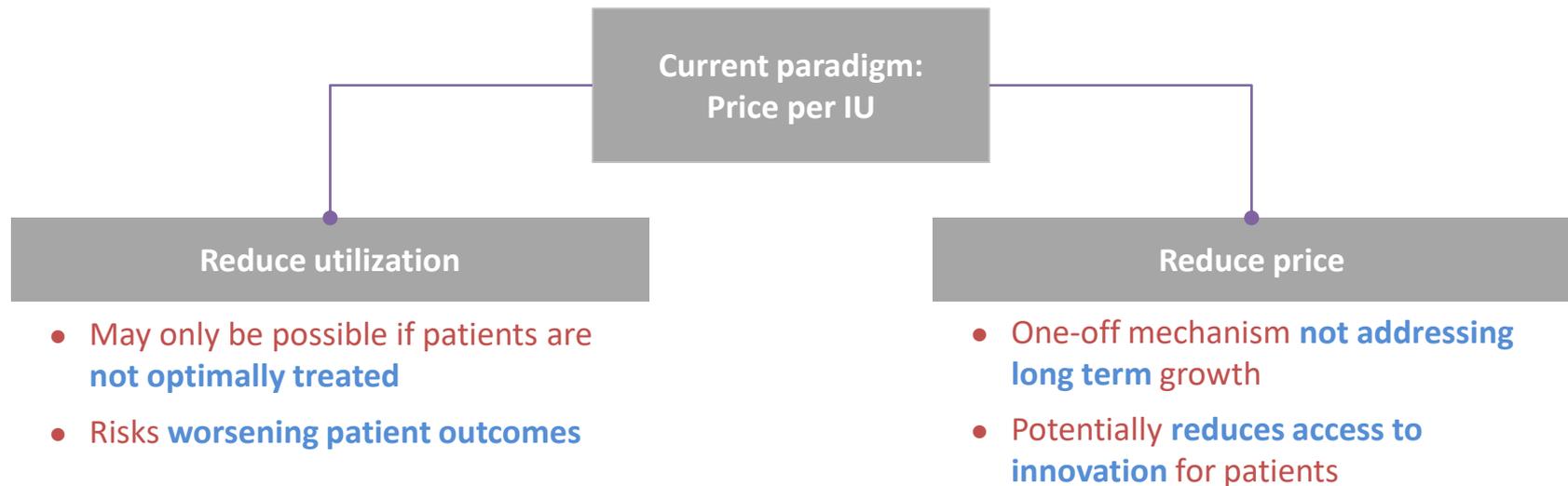
9. Gringeri, et al. An innovative outcome-based care and procurement model of hemophilia management. *Expert Review Pharmacoeconomics & Outcomes Research*, (2016) 16(3), 337–345

10. Neumann et al. Risk-Sharing Arrangements That Link Payment For Drugs To Health Outcomes Are Proving Hard To Implement. *Health Affairs* 30, no. 12 (2011): 2329–2337

# Procurement on price and volume does not create right incentives to improve care and value for money

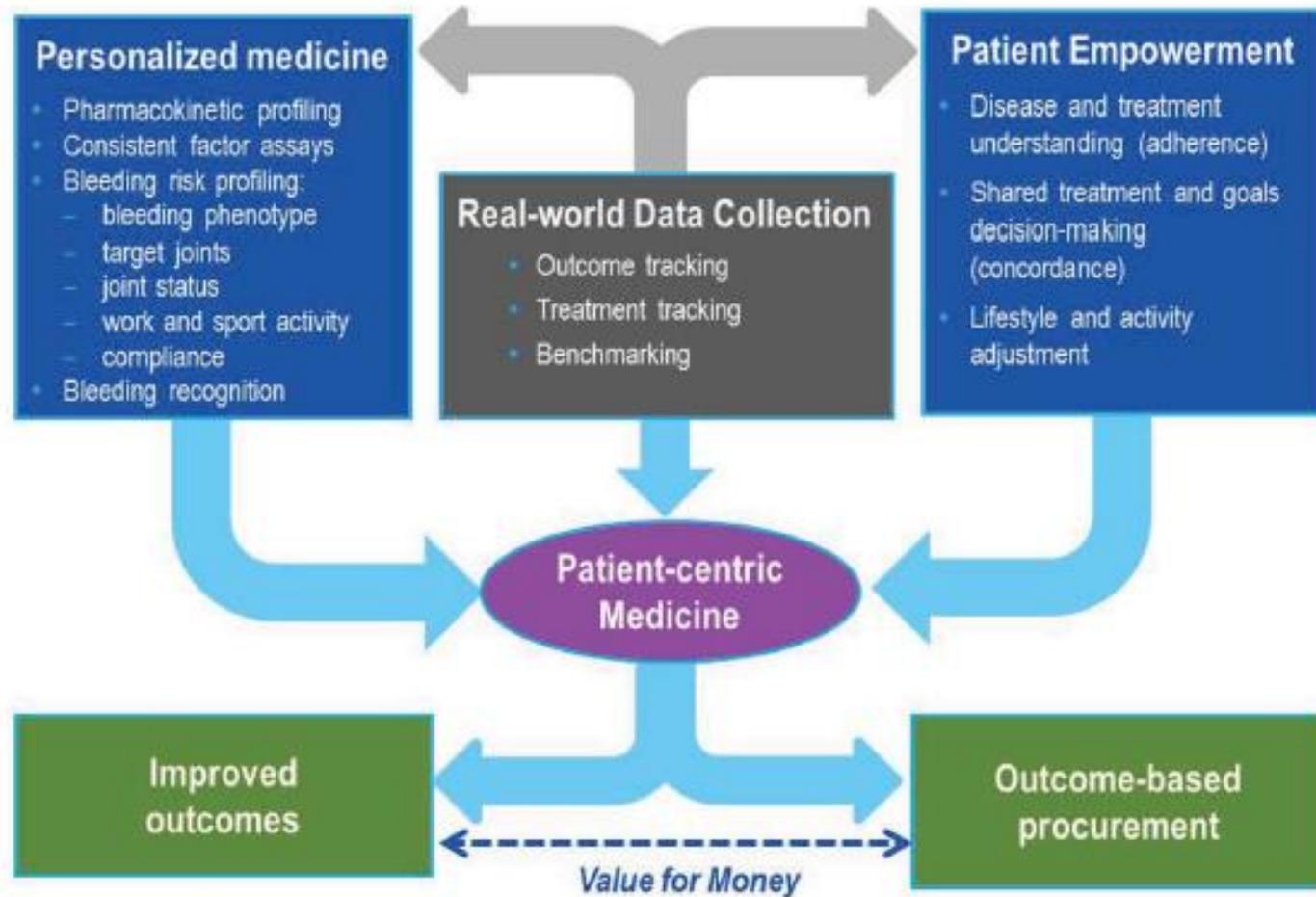
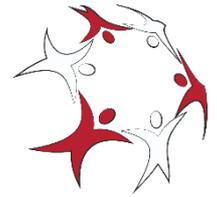


In the current procurement paradigm two levers exist to address efficiency and sustainability:

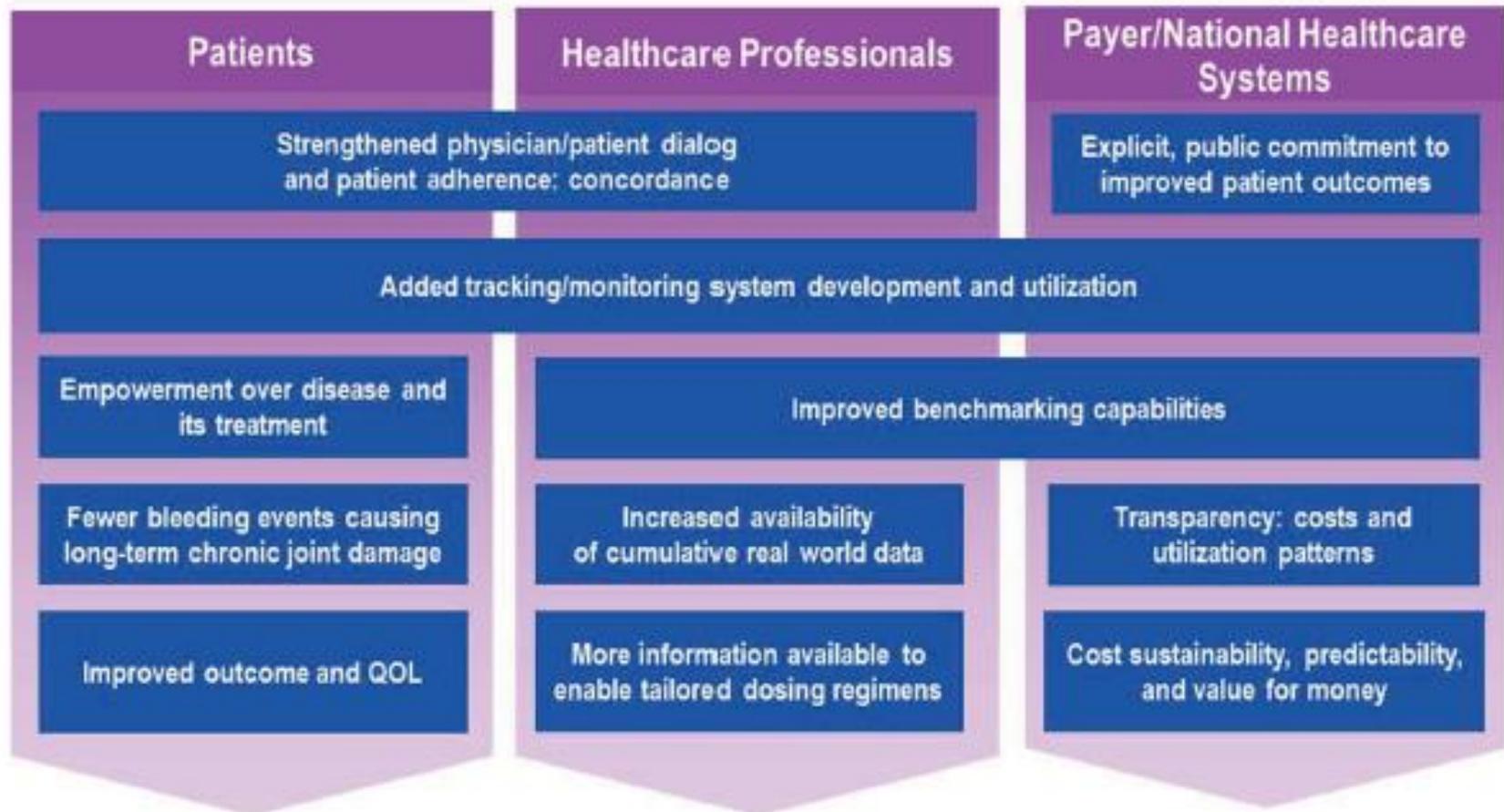
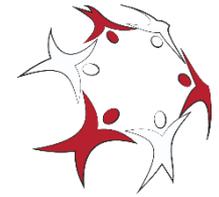


**Future efficiency and sustainability requires a new approach to procurement of haemophilia care**

# Outcomes data collection is critical to measuring and improving system efficiency



# Outcomes based procurement could better align incentives across all stakeholders



# Summary and Conclusions



- There is a need for more individualized outcome measures including patient treatment goals
- These should be complementary to existing clinical and patient reported outcomes, and useful in clinical practice and research
- New and existing outcomes could be used as a basis for procurement to incentivize optimal care in a financially sustainable way