

# Towards a New, Patient-Centric, Model of Hemophilia Management

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## INTRODUCTION

- Each hemophilia subject requires a personalized approach to treatment, but self-management can be challenging for patients, leading to avoidable bleeds.
- In fact, more effective prevention of bleeds decreases the likelihood of arthropathy and associated chronic pain, missed time from school or work and progressive loss of mobility, and improves the quality of care for people with hemophilia efficiently and sustainably, reducing associated costs to society.

- However, low visibility on individual patient outcomes and utilization make it difficult to capture accurate data, and consequently help them personalize treatments to achieve zero bleeds.
- We propose a strategy to reduce or eliminate bleeds altogether through a holistic approach based on individual patient characteristics and targeted outcomes, linking procurement to patients' outcomes, adding incentives for all stakeholders to strive for optimum outcomes and ultimately a phenotypic correction.

## UNMET NEEDS IN THE HAEMOPHILIA COMMUNITY

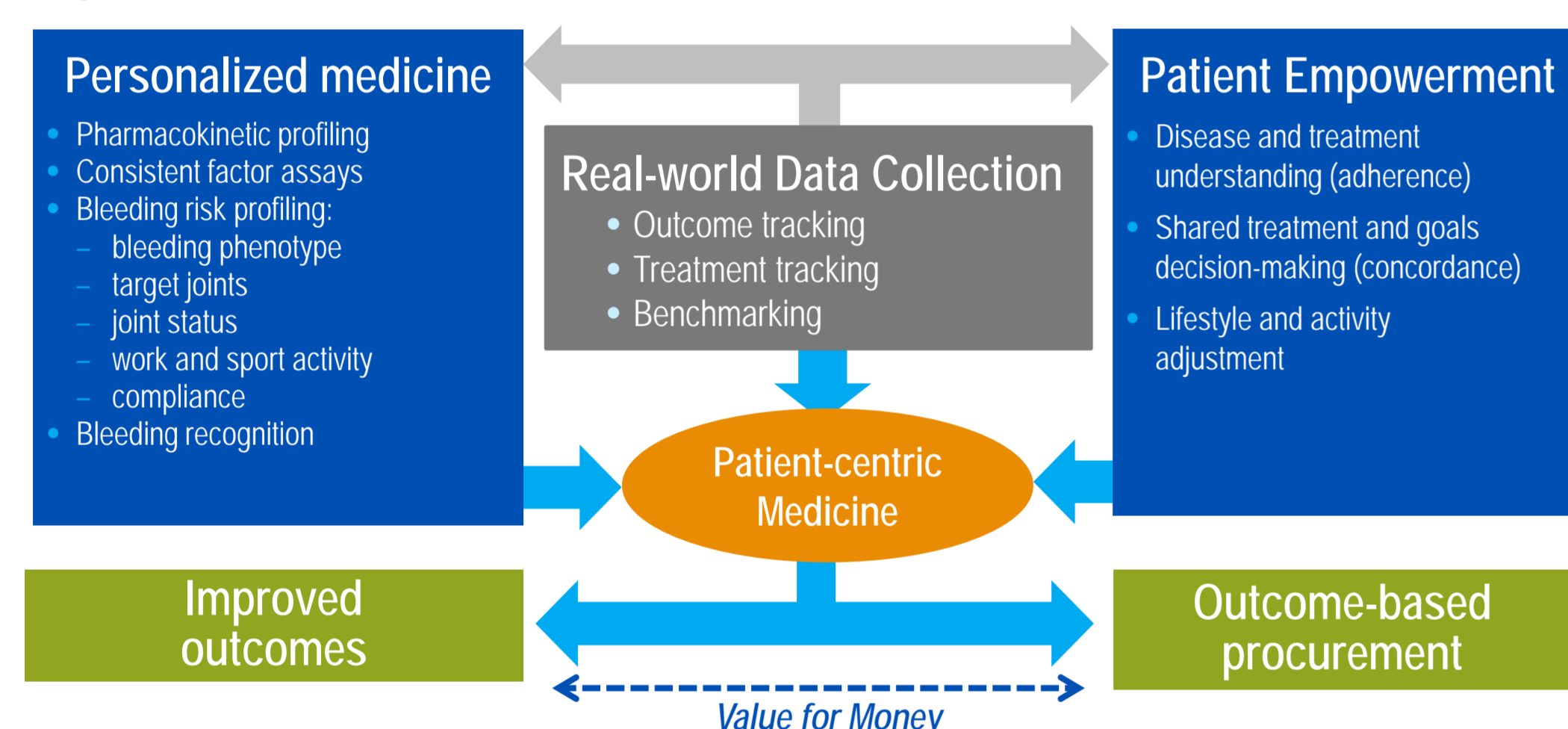
### NEED FOR SUSTAINABLE COST OF CARE

While up to 75% of hemophilia patients worldwide do not have access to adequate therapy,<sup>1</sup> the major obstacle to the goal of "zero bleeds for all patients" is represented by tremendous constraints on healthcare expenditure:

- Costs of care for a single hemophilia patient is one of the highest compared to other rare chronic diseases.
- Increasing lifespan and the introduction of newer, more expensive medications are multiplying the financial burden on all national health systems.

Patient-centric care will help to improve standards of care in a sustainable way (Fig. 1).

Figure 1: Ins and Outs of Patient Centric Care

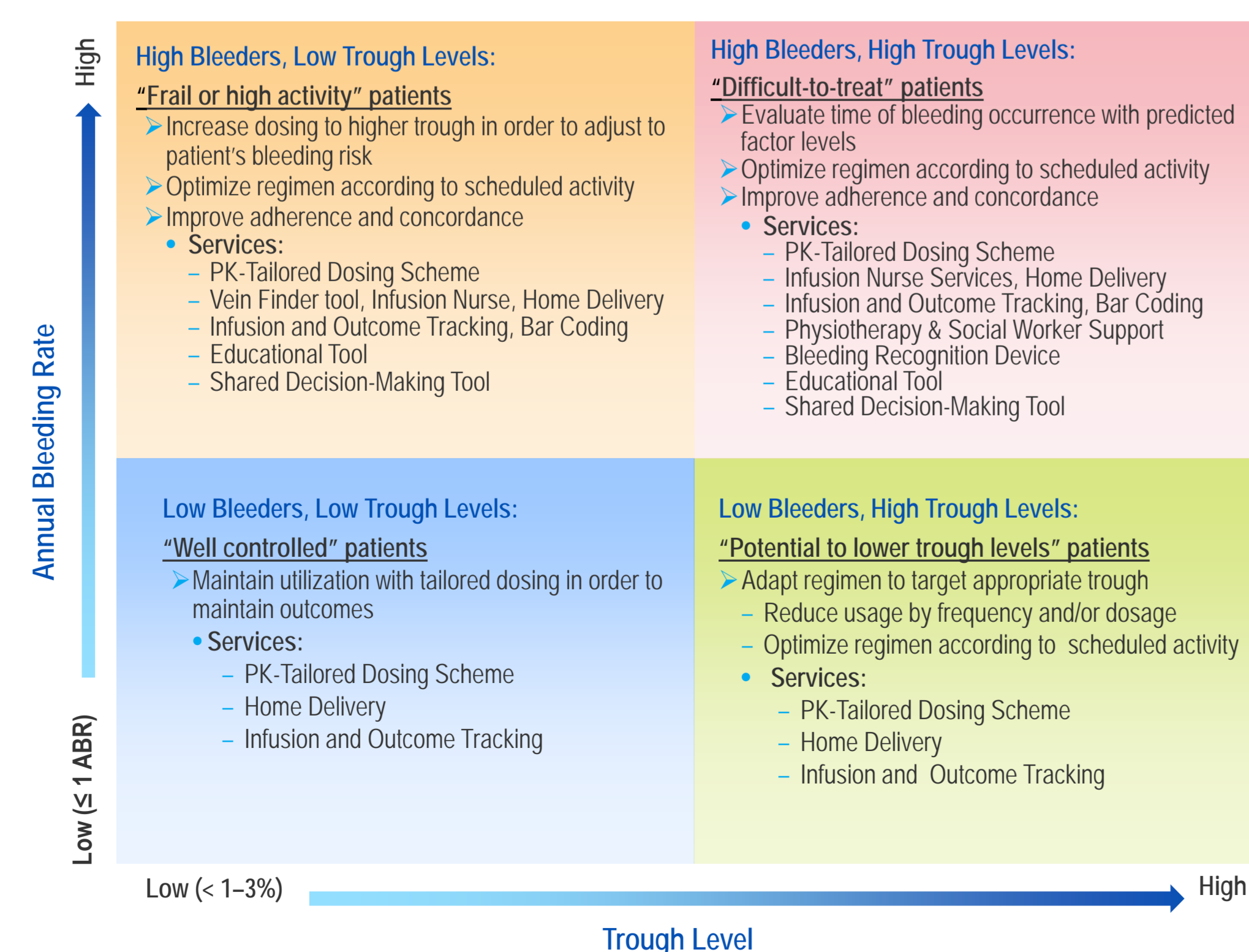


This is a call for an action to discuss possible paths that lead to optimal outcomes without increasing already high treatment costs.

### NEED FOR IMPROVED TRACKING AND BENCHMARKING

To optimize patient-centric care, improved tracking and benchmarking is needed. "High bleeders", patients bleeding frequently despite remaining adherent to the prescription plan, may be prescribed inadequate doses of CFC or at infusion intervals beyond the duration affording protection from bleeding. In contrast, "low bleeders", patients who rarely bleed, may receive doses of CFC that are greater than they might really require (Fig. 2).

Figure 2: Determining Treatment by Bleeding Rate and Trough Levels



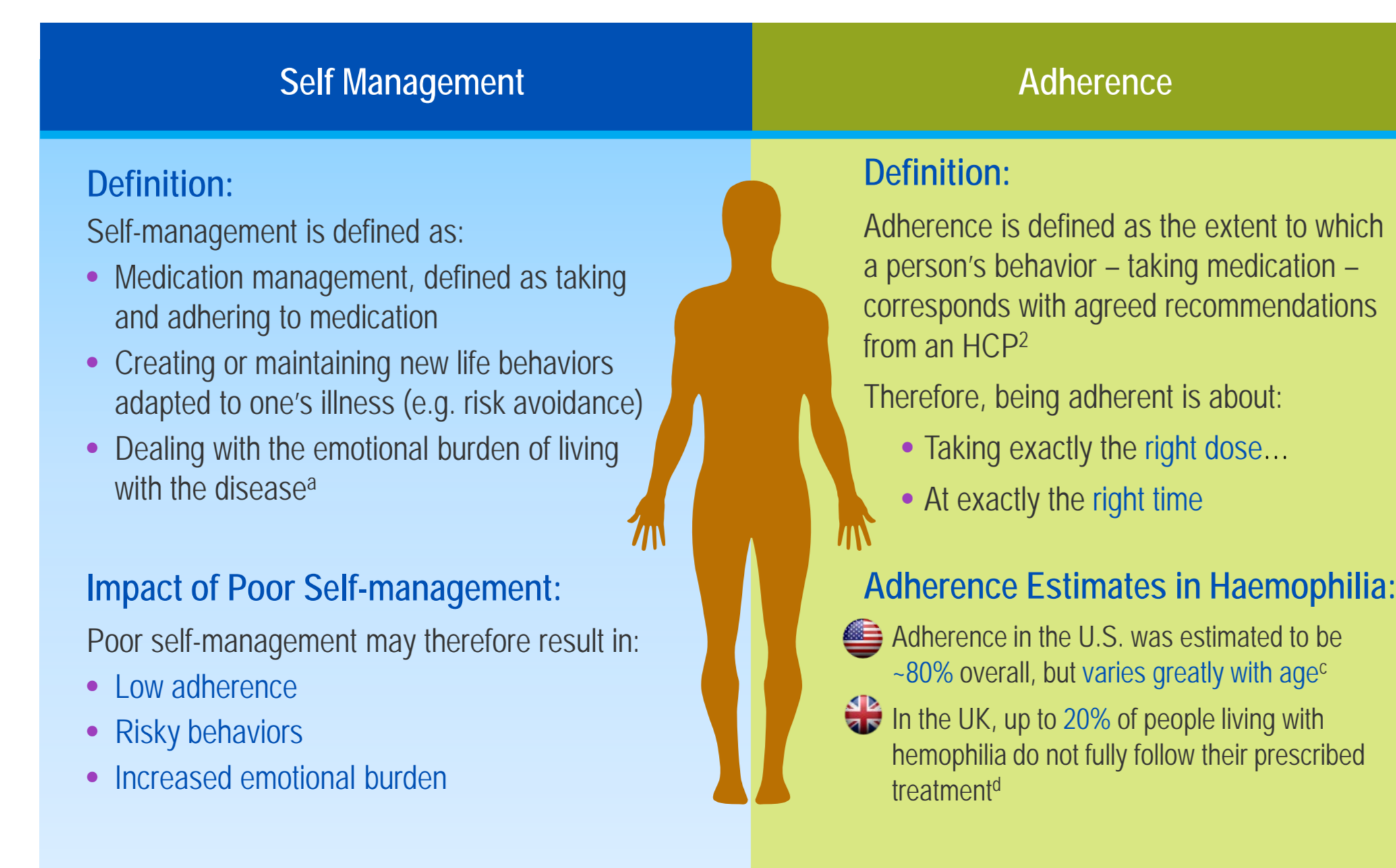
The right balance can only be determined through careful and transparent, real-time monitoring of patients, tracking bleeding events and infusions of CFC, together with accurate PK profile, driving dosage, intervals, and timing of infusions which can improve safety, effectiveness, and cost-effectiveness of hemophilia care.

### NEED FOR IMPROVED SELF-MANAGEMENT, ADHERENCE AND CONCORDANCE

Managing their own disease and treatment can be challenging for patients, especially for adolescents and young adults (Fig. 3).

- Compliance can be the result of understanding of the disease and the treatment (adherence). Adherence can influence participation in disease management and decision-making (concordance). Inadequate degrees of adherence and concordance can result in higher bleeding rates.
- 18-24 year olds have 1% lower adherence than 25-44 year olds, but have 14% more bleeds.<sup>2</sup>
- Poor adherence in pediatric patients is strongly associated with more days off school and worse health related quality of life (HRQoL) scores.
- In chronic diseases, non-adherence is estimated to cost \$100 billion per year in the US, or 4.5% of total healthcare spending, due to avoidable hospitalization costs only. Total cost of non-adherence is probably even greater.
- Patient-centric care aims to empower patients to better self-management to improve their own outcomes.

Figure 3: Self-Management and Adherence: Crucial for Optimized Outcomes



Sources: <sup>1</sup>Lorig and Holman, 2003; <sup>2</sup>Schrijvers, et al. 2013; <sup>3</sup>Zappa et al., 2012; <sup>4</sup>Clinical trial: "Nurse facilitated adherence therapy for haemophilia", 2014.

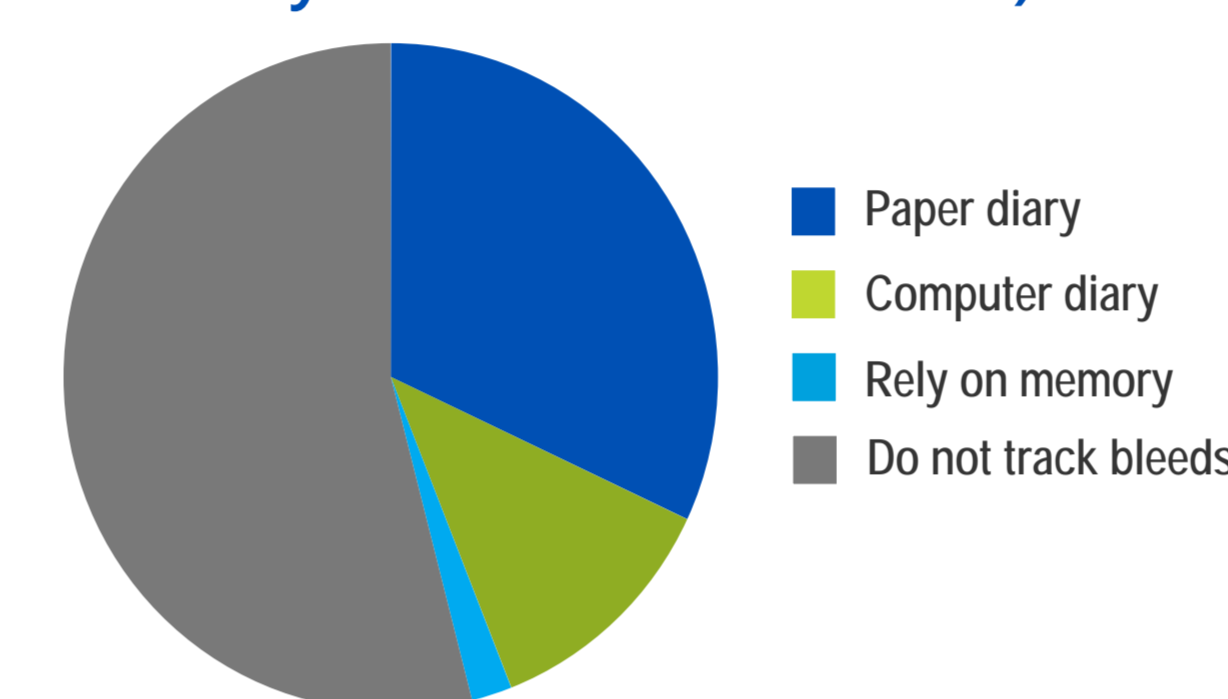
### NEED FOR ACCURATE DATA ON PATIENT OUTCOMES AND UTILIZATION

Accurate data on patients' individual outcomes are needed in order to assess treatment effectiveness and personalize treatments. However, a large proportion of patients do not keep records of their bleeds. Those who do mostly use paper diaries which are almost impossible to analyze by care-givers (Fig. 4).

## AIM

- The model is intended to be a comprehensive approach to purchase, provide and then assess care delivered, integrating precision medicine, recognizing the variability between each patient, and aiming to treat each individual differently in order to achieve an optimal outcome.

Figure 4: Bleed Record Keeping by Patients (164 Patients Surveyed Across 4 Countries)

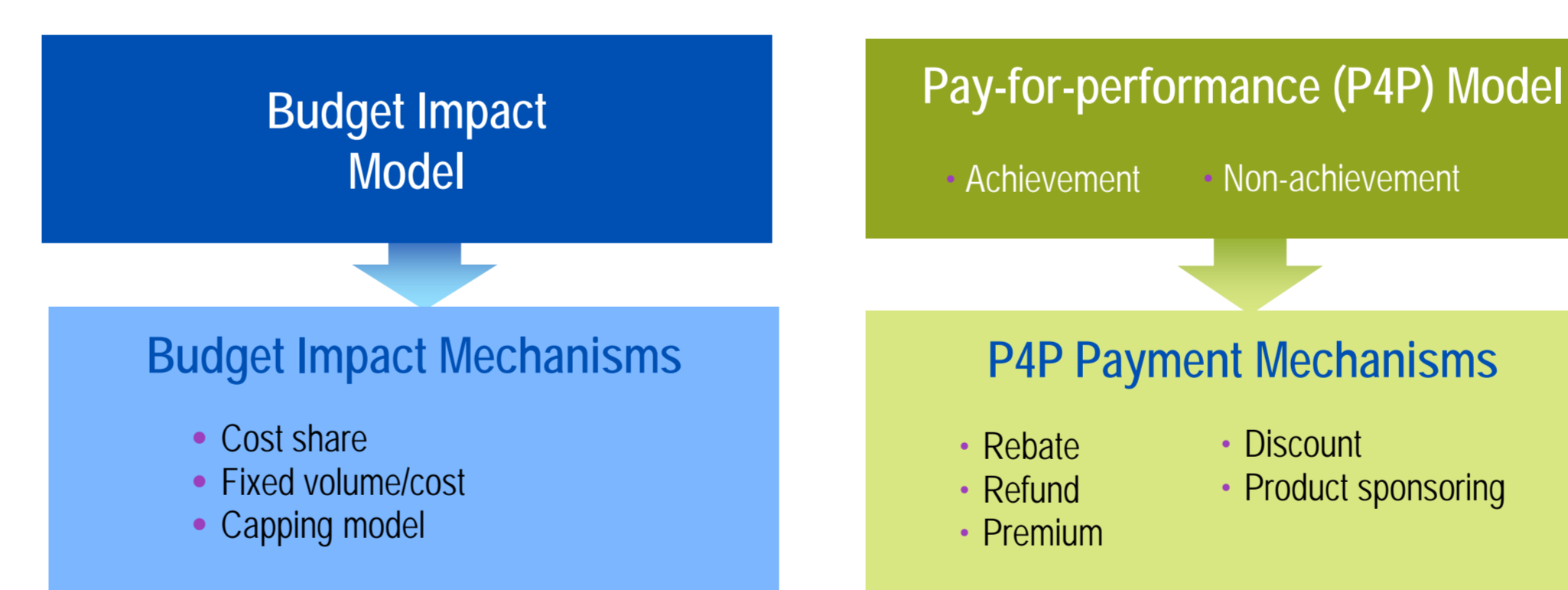


## POTENTIAL PROCUREMENT MODELS

### INNOVATIVE PATIENT-CENTRIC PROCUREMENT MODELS

- There are two potential innovative procurement models (Fig. 5):
- The budget impact model consists of three budget impact mechanisms allowing the payer to safely test the model without investment risk or demand limitation. This model could be used as "proof of concept" preceding a pay-for-performance-like model.
- The pay-for-performance model reflects achievement or non-achievement of outcome guarantees and their associated payment flow. Examples for indicators of outcome achievement are annual joint bleed rates, degree of adherence, health-related quality of life scores, pain scale, number of sick days, number of new target joints, joint status scale, and goal attainment scale.

Figure 5: Budget Impact and Pay-for-Performance Models



A combination of improved tracking systems, benchmarking, concordance, adherence, and patient-centered medicine should lead to improved outcomes among patients – to fewer or ideally no bleeding episode (Fig. 6).

Figure 6: Outlook with Patient-centric Care

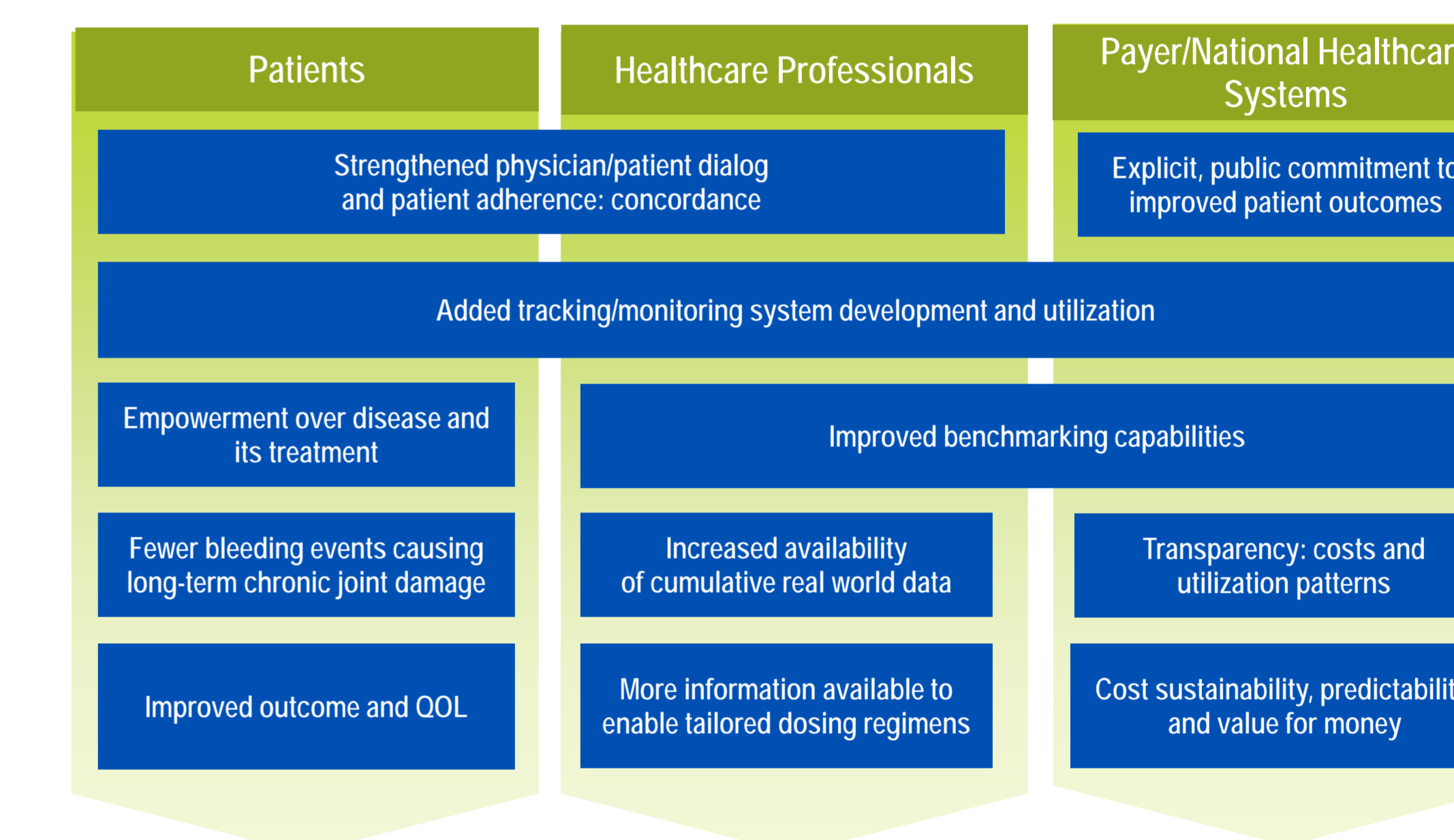
Procurement Journey	Short Term	Long Term
Principle	Start improving outcomes and collecting real world data to prove & quantify value	Pay-for-performance on outcomes delivered, based on study results and real-world data
Examples of What is Procured	<ul style="list-style-type: none"> <li>Capabilities to provide a solution to improve outcomes or efficiency</li> <li>Study results demonstrating the value and effectiveness of a novel solution</li> </ul>	<ul style="list-style-type: none"> <li>Actual outcomes (e.g. ABR, AJBR, adherence), validated by on-going collection of real world data</li> </ul>
	Proof-of-concept Phase	Pre-requisites for Pay for Performance: <ul style="list-style-type: none"> <li>Completion of pilot studies</li> <li>Data collection systems up &amp; running</li> </ul>

## SUMMARY

This initiative aims to procure on outcomes rather than volumes, and deliver better value for money.

- Every bleed matters and therefore should be prevented. Prophylaxis is the most effective way to prevent bleeds.
- The proposed, innovative model aimed to improve standards of care requires personalized, outcome-based, multidisciplinary care to provide better value for money by enabling procurement on outcomes.
- Every patient has unique characteristics, e.g. PK profile, clinical status, psychosocial conditions, personal needs and desires, thus requiring a personalized approach to prophylaxis.
- To improve outcomes, promotion of education and patients' empowerment is needed and requires appropriate tools and activities.
- The proposed model requires data collection tools to collect real-world data giving actionable insights, enabling tracking of outcome and health resources consumption, and enabling benchmarking to optimize care.

Figure 7: Benefits for All Stakeholders



## CONCLUSION

- To achieve lower bleeding rates in an environment of increasing budget constraints, we should start a journey from a procurement method based on price per volume of replacement therapy products to a performance-based procurement system where financial incentives are linked to a holistic care approach positioned to achieve agreed outcome targets.
- All stakeholders – patients, caregivers, HCPs, healthcare providers, payers, policy-makers and manufacturers – are called on to support this tectonic shift in the procurement paradigm and bring the treatment of people with hemophilia up to a new level of optimal care.
- This model represents a paradigm shift toward individualized care based on treatment outcomes rather than budget-centric price per unit, where all stakeholders cooperate to improve outcomes while containing costs.

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## DISCLOSURES

\*Author is an employee of Baxalta (Baxalta Innovations GmbH, Vienna, Austria, Baxalta US, Inc, Bannockburn IL, USA), now part of Shire.



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