

The Value of Shared Decision-Making in Paroxysmal Nocturnal Hemoglobinuria: Insights from a Patient Advocate Council

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Introduction

- Paroxysmal nocturnal hemoglobinuria (PNH) is an ultra-rare, life-threatening disease caused by uncontrolled complement activation and associated with chronic intravascular hemolysis, and a high risk of thrombosis.^{1,2}
- As advances in treatment continue to improve the prognosis for individuals with PNH, the importance of shared-decision making (SDM) in selecting therapy options is increasing.²
- SDM is a collaborative approach in which individuals and clinicians work together to evaluate treatment options and develop a tailored care plan, considering benefits, risks, and individual preferences.³
- While SDM has demonstrated positive outcomes in other chronic conditions, its role in PNH remains unexplored.

Objectives

- Evaluate existing best practices in SDM and its potential impact for improving treatment outcomes in PNH.
- Gain an understanding of the current role of SDM in PNH treatment decisions.

Methods

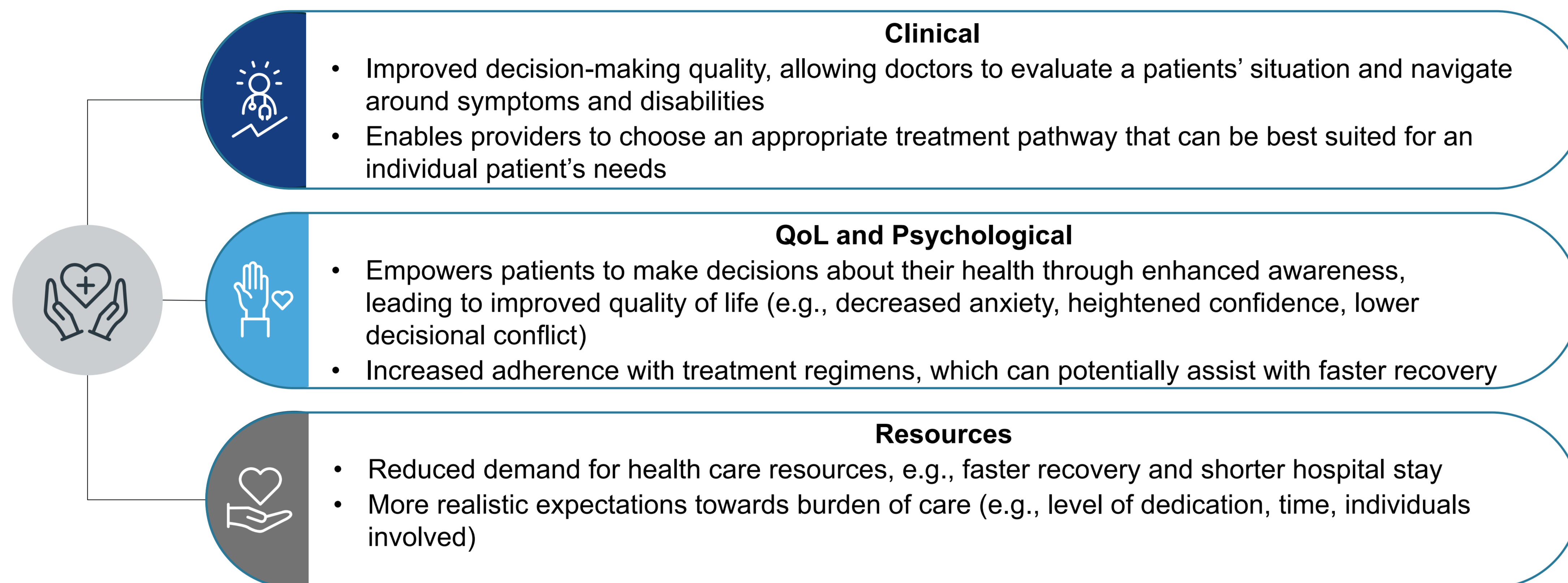
- A comprehensive desktop analysis was conducted to identify best practices, tools, and frameworks in SDM.
- The analysis reviewed over 50 publications across therapy areas and publication types, including:
 - Medical journals
 - Resource guides
 - Conference materials
 - Accredited healthcare provider (HCP) education
- Findings from the analysis were assessed by an advisory PNH Patient Advocate Council, comprising 8 people identified via patient advocacy groups:
 - 5 people living with PNH
 - 3 caregivers
- The aim of the Council meeting was to explore the current state of patient-provider dialogue, including how treatment decisions are discussed and made, and to investigate key inputs and strategies to design, develop, and deploy SDM to advance individualized care plans in PNH.

Results

Why is SDM needed?

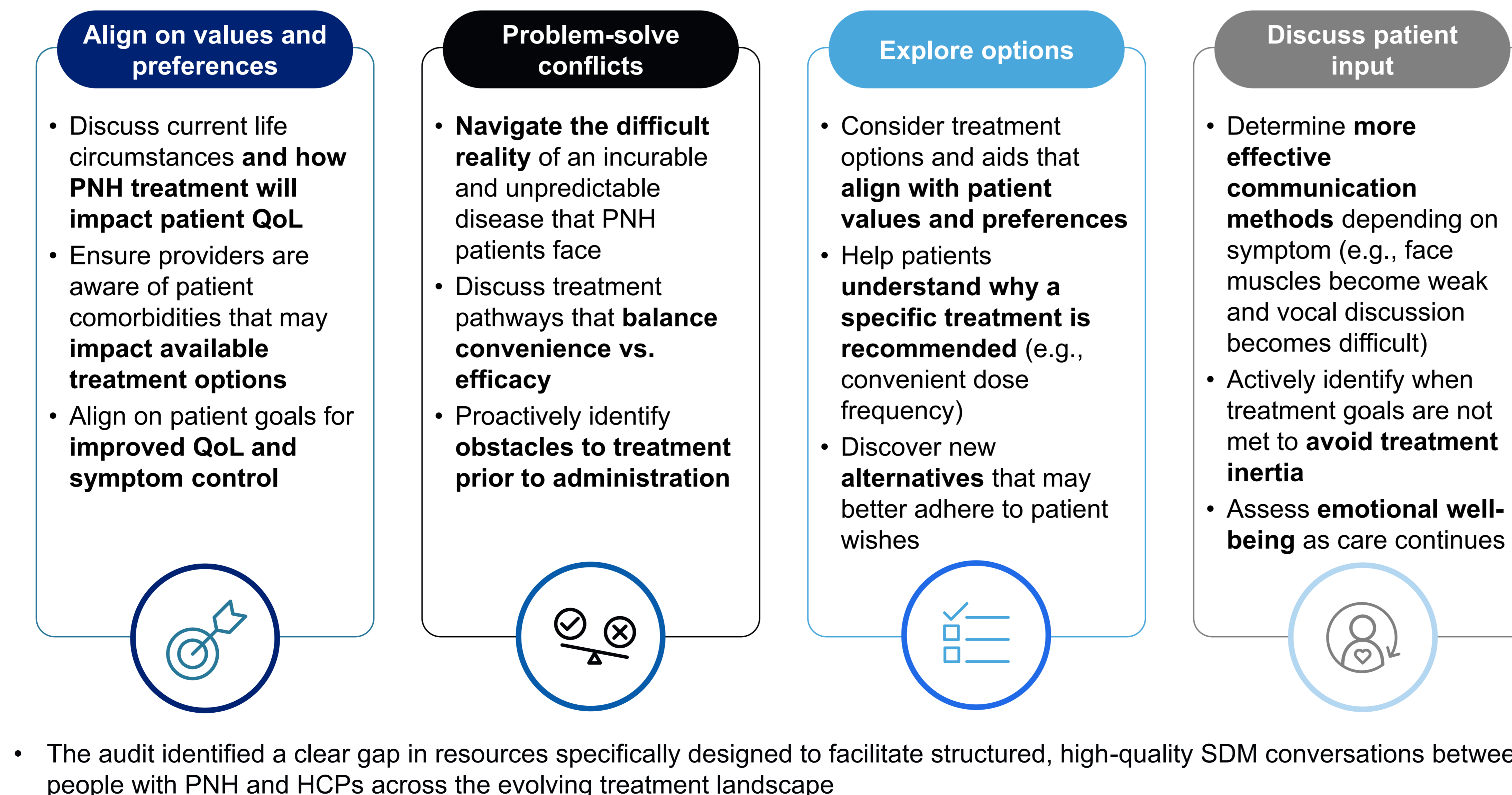
Our findings indicate that SDM leads to improved satisfaction, adherence, and clinical outcomes across multiple disease areas

What are the benefits of SDM?



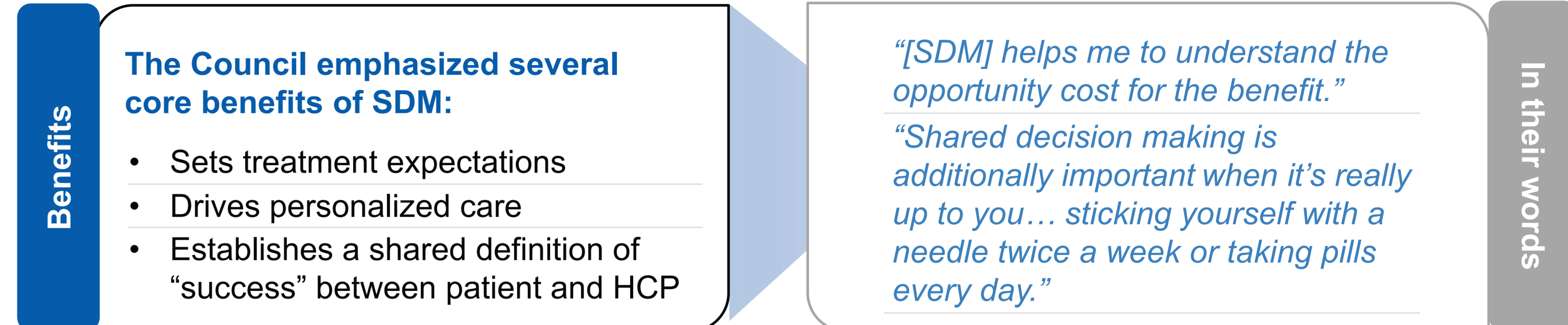
SDM is applicable to individuals with PNH

PNH involves complex healthcare conversations, numerous treatment options, and a long-term duration of treatment; therefore, SDM is likely to see successful implementation in PNH, allowing providers and patients to:



Patient Advocate Council Insights

Key benefits of SDM for people with PNH, caregivers, and providers center on care personalization and alignment on outcomes



Tailored SDM resources could help individuals with PNH identify their preferences, integrate product information, and advance effective conversations with their healthcare providers

- Council members provided feedback on the utility of 3 types of SDM tools for people living with PNH



Description: Provides an overview of different treatment options highlighting key product characteristics.

Key attribute identified by council: simple way to share important information

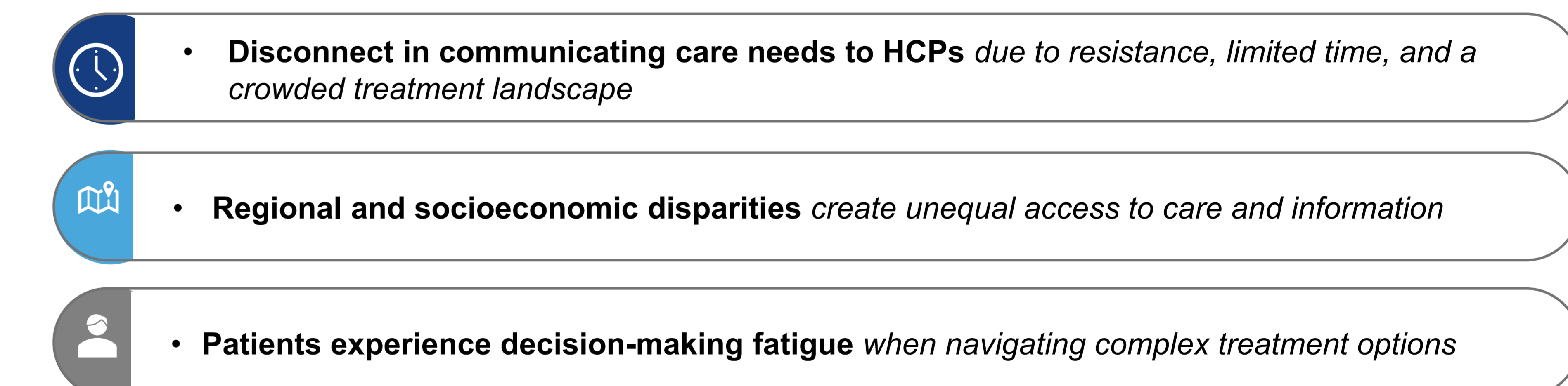
Description: Provides a list of questions for individuals and families to ask their physicians about treatment.

Key attribute identified by council: boosts confidence for HCP discussions; some tools are currently available but primarily targeted at newly diagnosed

Description: Provides exercises or questionnaires to help individuals, and their families identify and prioritize what matters most to them related to treatment.

Key attribute identified by council: clarifies an individual's treatment goals

Key barriers to effective SDM identified by the Council



Conclusions

- The implementation and benefits of SDM could be applicable to PNH, given the lifelong symptoms that people with PNH and their clinicians must manage.
- Increasing awareness and advocacy for the use of SDM in the PNH community could enable people with PNH to navigate the complex disease landscape, and could enable them to align with their HCPs on values and preferences, problem-solve conflicts, and explore options regarding treatment choices.

References

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Acknowledgements

The authors would like to thank the participants who made this study possible. This study was funded by Regeneron Pharmaceuticals, Inc. Editorial support was provided by Prime, (Knutsford, UK) under the direction of the authors, and was funded by Regeneron Pharmaceuticals, Inc.

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