# Cross-community Collaboration and Data Collection to Optimize Patient Care in Hemolytic Anemias

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## BACKGROUND

- Hereditary hemolytic anemias (HHAs) are rare, genetic diseases caused by mutations of one or more genes resulting in chronic disorders that adversely impact health-related quality of life (HRQoL) and require complex management regimens<sup>1–3</sup>
- Sickle cell disease (SCD), pyruvate kinase (PK) deficiency, and thalassemia are three such HHAs<sup>1–5</sup>
- Although each HHA has a distinct mechanism of disease, they have many shared effects, such as fatigue, chronicity, multi-organ complications, poor HRQoL, and psychosocial burden<sup>2-4,6,7</sup>
- It is imperative to bring the voice of those actually impacted by the disease into decision-making processes: this is particularly true for rare diseases, such as HHAs, where the disease burden is often high
- The Red Cell Revolution<sup>™</sup> (RCR) is an advocacy advisory council, supported by Agios Pharmaceuticals, and made up of members from the SCD, PK deficiency, and thalassemia communities, including patients, caregivers, advocates, Agios representatives, and specialist clinicians (**Figure 1**)

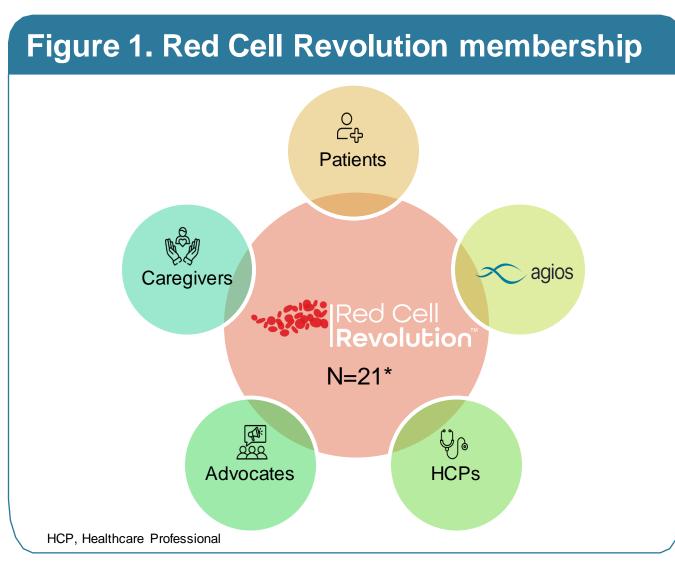
The RCR aims to raise a unified voice around issues in the HHA community and shape solutions with broad applicability



 To apply a multi-stakeholder, patient-advocacy research approach to understand the unmet needs of patients, caregivers, and HCPs for three HHAs: PK deficiency, SCD, and thalassemia

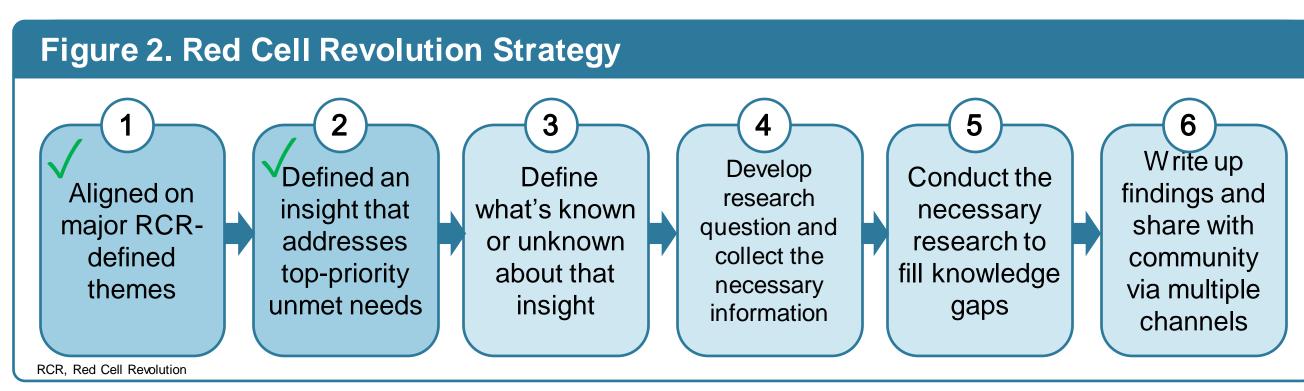
## METHODS

### **RCR Advisory Council**



### **RCR Research Strategy**

• The RCR strategy consists of a stepwise approach (**Figure 2**)

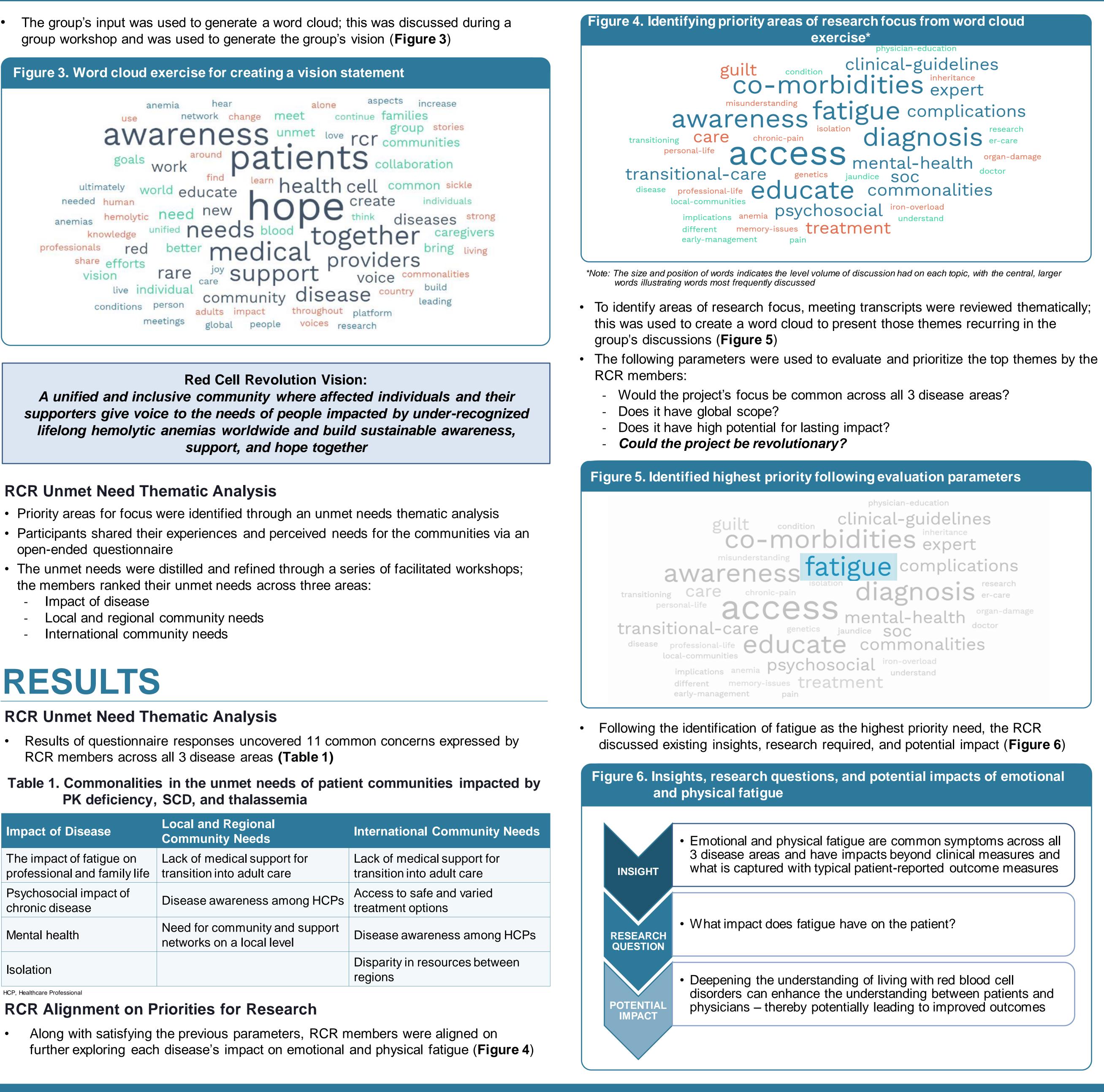


### **RCR Vision Statement**

- To develop a vision statement, RCR members were asked to share their thoughts on the following:
  - What is your vision for the RCR?

Revolution

- What do you hope the group can achieve in 2023?



### **RCR Unmet Need Thematic Analysis**

- open-ended questionnaire
- the members ranked their unmet needs across three areas:

### RESULTS

### **RCR Unmet Need Thematic Analysis**

Impact of Disease	Local and Regional Community Needs	International Community Nee
The impact of fatigue on professional and family life	Lack of medical support for transition into adult care	Lack of medical support for transition into adult care
Psychosocial impact of chronic disease	Disease awareness among HCPs	Access to safe and varied treatment options
Mental health	Need for community and support networks on a local level	Disease awareness among HCF
Isolation		Disparity in resources between regions

HCP, Healthcare Professional

#### **RCR Alignment on Priorities for Research**

• Along with satisfying the previous parameters, RCR members were aligned on

### Limitations – Thematic Analysis

- Commonly used as a qualitative analysis methodology, this was used to analyze the thoughts and opinions of the RCR members around topics of interest using survey input and meeting transcriptions as the dataset<sup>8</sup>
- Organic discussion from members was used as a dataset, requiring the use of an additional framework (coding) generated inductively by the reviewers (Agios)<sup>9</sup>
  - The initial results were presented to RCR members for feedback to ensure that final results were reflective of the group's discussion and qoals
- Insights elucidated by this thematic analysis may not be generalizable to the broader population of those living with rare disease

## CONCLUSIONS

### • The RCR collaboration between SCD, PK deficiency, and thalassemia communities aims to give voice to patients and patient advocates as a means to collectively promote sustainable awareness, support, and hope

- A thematic analysis identified emotional and physical fatigue as the highest priority area of unmet need
- The RCR plans to continue their research in this field to identify key knowledge gaps and better understand the personal impact of fatigue on patients' lives, as well as other priority topics identified by the group

Uniting the multi-stakeholder RCR advisory council offers hope of amplifying voices of those affected by HHAs and making an impact on issues faced by communities affected by SCD, PK deficiency, and thalassemia

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- Sarah Gheuens also serves on the Board of Directors for Viridian Therapeutics, Inc.
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