



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

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

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Background: Patient engagement is becoming increasingly important for all facets of healthcare, from drug development and approval, to ensuring equitable access and the delivery of care. It is imperative to bring the voice of those impacted by the actual disease into these processes: this is particularly true for rare diseases, such as hereditary hemolytic anemias (HHAs), where the disease burden is often high and coupled with a poor quality of life and complex treatment requirements. The Red Cell Revolution™ (RCR) advisory council arose organically as part of an Agios Pharmaceuticals-sponsored discovery process, during which a range of stakeholders including healthcare providers (HCPs), advocates, patients, and company leaders offered perspectives on how to optimize engagement with the disease communities. Insights uncovered as part of this process demonstrated that there are unifying health concerns and needs among those impacted by pyruvate kinase (PK) deficiency, sickle cell disease (SCD), and thalassemia, and that there is an opportunity to explore new patient-advocacy research, supported by the creation of a unique multi-stakeholder council.

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

Methods: The RCR (supported by Agios Pharmaceuticals) was established across three allied disease areas, with representation from patients (N=5), caregivers (N=1), advocates (N=3), clinicians (N=8), and Agios representatives (N=5) impacted by HHA. The patient advocacy research method commenced with a survey shared with all RCR members, which curated both qualitative and quantitative insights from the group. These insights subsequently underwent cluster analysis to determine the shared concerns experienced by those impacted by HHA. The data were also subjected to linguistic analysis whereby terminology used to describe the experiences of participants was ranked according to frequency of use to reveal the most

prominent concerns. The results of these analyses were distilled into an agreed group vision by the RCR, and a specific research strategy was aligned on for the RCR to pursue.

Results: RCR members provided detailed answers about unmet needs across three categories: 1) impact of disease; 2) local and regional community needs; and 3) international community needs. When applied, the analysis uncovered 12 common concerns expressed by all participants across the three disease areas. These are outlined in Table 1.

These insights were distilled into four key topic areas: 1) emotional and physical fatigue, 2) timely care, 3) transition from pediatric to adult care, and 4) access disparities, which were then ranked according to four parameters, namely whether it was: 1) common across all three disease areas, 2) global in scope, 3) high potential for lasting impact, and 4) revolutionary (involving or causing a complete or dramatic change). This produced alignment on one key priority: emotional and physical fatigue.

Conclusion: The commonality analysis deployed here demonstrates the ability of a multi-stakeholder council to determine priority areas for research to address unmet needs. The RCR will conduct an in-depth evidence audit of the existing research in this field to identify key knowledge gaps, enabling the design of a study that will answer outstanding research questions. This study will generate patient experience data with the potential to inform both the clinical setting and drug development. As a next step, the RCR is developing a patient advocacy research study to collect the necessary evidence to better understand fatigue and its impact on psychosocial quality of life measures, such as feelings of guilt.

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Table 1: Commonalities in the unmet needs of those impacted by Thalassemia, Sickle Cell Disease, and PK Deficiency

Impact of Disease	Local and Regional Community Needs	International Community Needs
The impact of fatigue on professional and family life	Lack of medical support for those transitioning into adult care	Lack of medical support for those transitioning 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 HCPs
Isolation		Disparity in resources between regions

Table 1: Health Care Professionals (HCPs), PK (Pyruvate Kinase).

Figure 1

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