Publication Number: 4441

Abstract Title : Patient-driven insights in sickle cell disease: Results from a global survey informing public health strategies

Category: 900s - Health Services, Quality Improvement, and Outcomes Research

Review Category: 904. Outcomes Research: Hemoglobinopathies

Authors

Mariangela Pellegrini[1], Baba Inusa[2], Raffaella Colombatti[3, 4], Issom David-Zacharie[5], Mariane de Montalembert[6, 7], Piel Fréderic B.[8], Béatrice Gulbis[9], Smith Lan-Lan [10], Maria Del Mar Mañú Pereira[11]

[1] Hôpital St Louis, Assistance Publique Hôpitaux de Paris, Paris, France, [2] Rare Blood Disorders and Advanced Therapies, Novo Nordisk, Bagsværd, Denmark, [3] Univerity of Padova, Padova, Italy, [4] Azienda Ospedale - Università di Padova, Padova, Italy, [5] European Sickle Cell Federation, Geneve, Switzerland, [6] Necker-Enfants Malades Hospital, Assistance Publique – Hôpitaux de Paris (AP-HP), Paris, France, [7] Université Paris-Cité, Paris, France, [8] School of Public Health, Imperial College London, London, United Kingdom, [9] Hôpital Universitaire de Bruxelles - Université Libre de Bruxelles, Brussels, Belgium, [10] Lancet Haematology, London, United Kingdom, [11] University Hospital Vall d'Hebron - Vall d'Hebron Research Institute, Barcelona, Spain

Abstract Body

Introduction

An effective public health strategy aimed at improving outcomes in Sickle Cell Disease (SCD) must be grounded in the lived experiences and needs of those directly affected. To ensure that these perspectives are recognised and integrated into community health plans, ERN-EuroBloodNet, the SCD Lancet Haematology Commission, and Academy for Sickle Cell and Thalassaemia (ASCAT) jointly launched a global initiative to collect patient-driven insights of people living with SCD. This initiative has the objectives of making the voices, priorities and needs of those living with the condition more visible in the definition of health policy objectives, public health and research agendas, thus guiding future actions to promote education, and consequentially achieving patient driven evidence to support advocacy efforts.

Methods

A two-round global survey was designed to assess through patient-driven insights the public health objectives preidentified. The survey targeted patients, as well as their parents, caregivers, partners, children, siblings and advocates. The first round (September 2023 – January 2024) was conducted in English. Based on feedback, the second round (June 2024 – February 2025) included a redesign of the structure and simplification of language, and it was as well translated into eight languages to ensure accessibility and reduce possible identified bias. This survey included a scoring-based prioritization of strategic actions for three topics: Research, Education and Policy. Respondents selected the priority of each element within each area from the most urgent to the less urgent. There were also open-ended questions to capture unmet needs potentially missing in the structured scoring part. The participation to the survey was anonymous. Responses were analysed quantitatively and qualitatively, indeed open-text entries were thematically coded by macro areas.

Results

The first and second rounds of the survey collected 196 and 199 responses, respectively. The final round received responses from 110 patients, 52 caregivers, 20 parents, and smaller numbers of siblings, partners, children, and patient advocates. Among non-patient respondents (parents, partners, caregivers, siblings, etc.) the majority were aged 40-65 (62 out of 84), with smaller numbers in the 25-40 (18) and 19-24 (2) age groups. Patient respondents were predominantly between 25-40 (55) and 40-65 (53), with very few under 24 or over 65.

The second round gathered answers from 30 countries, with the highest participation from the UK (41

responders), France (30), USA (16), Portugal and Belgium (15 each), the Netherlands (10) Italy (9), and Nigeria (7), Cameroon (6). Others included countries across Africa, Europe, North and South America, and Asia.

The top-ranked priorities were:

- Research: Development of new drugs and innovative therapies (confirmed in both rounds).
- Education: Training for health professionals (confirmed in both rounds).
- Policy: Implementation of neonatal and prenatal screening. (In round one, this followed closely behind the recognition of SCD in national disease classifications).

From 210 free-text responses, five key themes emerged: Equitable access to treatments and curative therapies globally; Recognition of SCD as a disability; Combatting racial bias in healthcare; Need for psychosocial and financial support; Creation of expert centres and support for low-resource settings.

Conclusions

A strong global consensus emerged around investment in innovative therapies, training of health professionals, and early screening. Respondents emphasize also the importance of innovation and data driven research. While topics like pregnancy risks and complementary therapies are viewed as supplementary, finding a cure, prevention and early detection emerge as top priorities. Accessibility to care and equitable treatment are also key concerns, alongside the need for formal recognition of SCD as a foundation for policy change. The survey confirms the critical need for equity goals in shaping healthcare responses to SCD. Indeed, free-text insights reveal persistent structural inequities, stigma, and the psychosocial burden experienced by individuals living with SCD. These findings should inform international, EU-wide and national strategies for supporting the inclusion of SCD communities as active stakeholders in policy-making, research, and service care planning.

Keywords: Quality of Care, Hemoglobinopathies, Real-World Evidence, Access, Representation, and Population Health, Quality Improvement, Diseases, Patient-Reported Outcomes, Health Disparities Research, Research, Sickle Cell Disease, Clinical Research, Value, Health Outcomes Research, Clinical Practice (Health Services And Quality)

Disclosure : Mariangela Pellegrini: None declared, Baba Inusa: Novo Nordisk, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: Yes, Current holder of stock options in a privately-held company: Yes, Current equity holder in private company: Yes, Honoraria: No, Raffaella Colombatti: Novo Nordisk, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Agios Pharmaceuticals, Inc., Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Novartis, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Vertex Pharmaceuticals, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Forma Therapeutics, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, European Haematology Association (EHA), Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Italian Association of Pediatric Hematology Oncology (AIEOP), Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Addmedica, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Global Blood Therapeutics, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, bluebird bio, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Issom David-Zacharie: Roche, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Vertex Pharmaceuticals, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Novo Nordisk, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Mariane de Montalembert: THERAVIA, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Piel Fréderic B.: None declared, Béatrice Gulbis: None declared, Smith Lan-Lan : None declared, Maria Del Mar Mañú Pereira: Novo Nordisk Health Care A/G, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Agios Pharmaceuticals, Inc., Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, BMS (Bristol-Myers Squibb) Company, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Novartis Pharma AG, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Pfizer, Inc., Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No