Sickle Cell Transitions
Policy Lab

# Bridging the Gap: Sickle Cell Disease as a Case Study of Paediatric to Adult Transition in Rare Disease

**European Parliament, Brussels**
**16 October 2025**
**Summary of the roundtable discussion**

The Policy Lab is funded by Novo Nordisk and Pfizer.

## Speakers

- Mario Ottiglio, World Coalition on Sickle Cell Disease (moderator)
- MEP Vytenis Povilas Andriukaitis (S&D, Lithuania)
- MEP Michalis Hadjipantela (EPP, Cyprus)
- Mimie Minsiemi Maboloko, European Sickle Cell Federation
- Mariangela Pellegrini, European Reference Network on Rare Haematological Diseases, ERN-EuroBloodNet
- Hélène Le Borgne, European Commission, Directorate General for Research and Innovation
- Marie-Claire Kofi, European Sickle Cell Federation

We would like to thank Mario Ottiglio for moderating the roundtable and our speakers and panellists for their contributions. We would also like to thank MEP Vytenis Povilas Andriukaitis for hosting the discussion and for his commitment to the rare disease community, as well as MEP Michalis Hadjipantela for his contributions and support.

Finally, we would like to thank everyone who attended the roundtable discussion for their commitment to the sickle cell – and rare disease – community.

## Executive summary

Following the successful launch of the *Charter for optimal care transitions from paediatric to adult care in Sickle Cell Disease*, on 16 October 2025, the Sickle Cell Transition Policy Lab Co-Chairs, MEP Vytenis Andriukaitis and MEP Michalis Hadjipantela hosted a roundtable discussion at the European Parliament, Brussels, to discuss the role of policy in supporting young people transitioning from paediatric to adult care across Europe. The discussion was attended by Members of the European Parliament (MEPs), representatives of the European Commission, healthcare professionals, patient advocates and representatives from European Reference Networks (ERNs).

A number of important points were raised during the discussion, including that:

- The transition from paediatric to adult care needs to be **recognised as a pivotal stage in care pathways for people living with sickle cell disease and other rare diseases.** Poorly managed transitions can lead to long-term health complications, reduced quality of life and increased hospitalisations and risk of mortality.
- **Experiences of transition vary across European countries**. While some countries have established protocols and specialised programmes, other lack formal plans and even where plans exist, implementation and quality are inconsistent. With the prevalence of sickle cell disease rising across Europe, formal plans need to be developed and implemented.
- **An optimal transition from paediatric to adult care needs to start early, be tailored to the individual and supported by multi-disciplinary teams**. The English 'Ready, Steady, Go' programme was highlighted as a model of best practice. An optimal transition should centre around four components: setting the right foundations, pre-transition preparation, holistic care and continuous support following the transfer from paediatric to adult care.
- **EU-level collaboration is key to scaling and improving transition care across Europe**. This includes incorporating transition into EU rare disease strategies and leveraging digital health infrastructure (e.g. European Health Data Space, ERNs). Notably, registries have a role to play in capturing epidemiological data, stratifying patients at higher risk and collecting indicators pertaining to quality of life.

- The roundtable concluded with a **call for collective advocacy and concrete action, including the development of an EU Action Plan on Rare Diseases** that prioritises transition, the sharing of best practices, and ongoing collaboration among all stakeholders to ensure equitable, patient-centred care for young people living with rare diseases.

## Overview of the discussion

***Welcome and opening words from MEP Vytenis Andriukaitis (S&D, LT)***

During his opening remarks, MEP Andriukaitis highlighted the scale of rare diseases in Europe, impacting an estimated 35 million people. He emphasised the critical nature of the transition from paediatric to adult care, identifying it as a key, and often vulnerable, moment in a young person's life that is often marked by increased hospitalisations and sub-optimal care. He called for a pan-European ecosystem that leverages ERNs as a cornerstone for Europe's rare disease policy, to facilitate innovation and address the challenges arising from fragmented care to ensure young people do not fall through the cracks in the system.

***Living with sickle cell disease – Mimie's story (Mimie Minsiemi Maboloko)***

Mimie delivered a powerful testimonial on her personal lived experience, stressing that transition is far more complex than a simple transfer of care – but rather, it is a process, with it taking time to learn about one's own condition and how to manage it while also adapting to a new care setting and healthcare team.

She underscored the need for time, coordination, compassion and person-centred transition to prepare physically and emotionally for the shift to adult care, and called for policies to recognise the importance of transition and start preparing early.

***Presenting the Charter for optimal care transitions between paediatric and adult care in sickle cell disease (Mariangela Pellegrini)***

Mariangela highlighted that sickle cell disease is the most common inherited blood condition in France and the UK and is becoming increasingly prevalent across Europe. Recent advances in newborn screening, preventative measures and disease modifying therapies have improved life expectancy, but in Europe, sickle cell disease care is often adult-focused due to immigration patterns, resulting in gaps in paediatric expertise and transition processes. She emphasised that paediatric care tends to be family-centred, while adult care is individual-centred and can be overwhelming, with young patients expressing that the 'adult world is hard and cold' and they 'don't want to move to adult care'.

Mariangela stated that the transition is a not just a medical gap, but a system gap, with many of the barriers young people face either systemic and structural. To address these gaps, the Sickle Cell Transitions Policy Lab has developed the *Charter for optimal care transitions*, to outline what an optimal transition looks like and how patients with sickle cell and other rare diseases can be supported during this pivotal part of their journey. She emphasised the robust methodology undertaken to develop the Charter and its recommendations, highlighting that it is a product co-created by clinicians, patient organisations, patients, public health representatives and industry from 11 European countries.

Mariangela concluded by calling for robust policies at EU and national levels to support an optimal transition by:

1. Recognising sickle cell disease transitions within wider rare or chronic disease policies ensuring that policies and guidelines are developed with input from young people and their families/carers.
2. Mandating early tailored and holistic transition planning, starting at least two years before the transfer to adult care, supported by dedicated funding and resources.

3. Embedding evidence-based best practice to support healthcare teams, ensuring clear, consistent and high-quality care across Europe.

***Panel Discussion and Q&A (Mario Ottiglio, Hélène Le Borgne, Mariangela Pellegrini and Marie-Claire Kofi)***

The panel discussion highlighted that the transition from paediatric to adult care for young people living with sickle cell disease – and by extension, most rare diseases – remains a critical and under-addressed challenge in Europe. There was broad agreement that transition is more than a simple transfer; it is a complex and vulnerable period affecting the medical, psychological and social dimensions of a young person's life.

Panellists stressed the importance of starting transition planning early, ideally in early adolescence, and doing so in a patient-centred, coordinated and multidisciplinary way. Examples of effective transition planning models, such as those developed in the UK, were cited, where preparation begins at ages 13-14 and the process is jointly managed by paediatric and adult care teams. However, it was noted that even in countries with established frameworks, gaps persist, implementation remains variable and more must be done to make adult care welcoming and accessible for young people.

There was a shared understanding that the lack of formal transition policies across most European countries is a challenge. Fragmented healthcare systems, insufficient education and support, cultural and social obstacles, and inadequate policy prioritisation were all highlighted as key barriers. As such, support for transition and its planning often depends on individual clinicians and hospitals recognising its importance and having the necessary skills to support young people effectively.

The panel also recognised the potential of ERNs, noting that digital health tools such as registries could better track transition, stratify patients at higher risk and collect indicators pertaining to quality of life. Infrastructure such as the European Health Data Space could further enable the collation of epidemiological data. ERNs also offer opportunities to connect research to care and have the ability to transform care and patient outcomes by sharing knowledge, best practice and guidelines across Europe.

The need for collective advocacy was a recurring theme, with calls for all stakeholders – patients, clinicians, policymakers and patient organisations – to work together for systemic change. Data collection must be inclusive and creative, reflecting the diversity of sickle cell disease and rare disease populations to ensure equitable care.

Finally, panellists emphasised that transition should be recognised as a priority in European and national rare disease strategies. This could be achieved through a range of actions: amplifying the voices of underrepresented communities, strengthening ERNs, fostering collaboration between patients and policymakers to better reflect lived experience, using available tools to support young people through transition and empowering clinicians to advocate for patients within hospital settings.

***Statement from Thalassemia International Federation (Read out by Mario Ottiglio)***

Mario read out a statement shared by Dr Androulla Eleftheriou, Executive Director of the Thalassaemia International Federation (TIF). In the statement, TIF highlighted that the period of transition is a complex, under-addressed phase in chronic and rare disease care that requires both clinical coordination and psychosocial, educational and policy-level support. Recognising the need to improve transition pathways, it called for structured, multidisciplinary and early transition planning, referencing UK and Canadian guidelines as examples. TIF stressed that improving transition care is both a scientific and political priority, requiring collaboration among all stakeholders, from policymakers to healthcare professionals to patients and patient organisations.

***Closing remarks: MEP Michalis Hadjipantela (EPP, CY)***

MEP Hadjipantela reiterated his commitment to advancing care for rare diseases, emphasising the importance of equitable, patient-centred pathways and investment in ERNs and digital health

initiatives. He called for ongoing collaboration, increased awareness and for the patient community's continued engagement.

## Key action items

The roundtable reinforced that the transition from paediatric to adult care is a critical, yet often overlooked aspect of rare disease management. However, there are tools and policy instruments that can alleviate this.

### 1. Develop and implement robust transition policies

Both EU and national governments are urged to **create evidence-based policies for the transition from paediatric to adult care in sickle cell disease and other rare diseases**. These policies should be co-designed with input from young people and their families.

A future **EU Action Plan on Rare Diseases** could provide healthcare systems with increased tools and resources to improve access to care and care standards for people living with rare diseases like sickle cell disease. Such a plan should include the transition from paediatric to adult care and be supported by patient-centred digital tools and resources that can track metrics related to transition.

To lay the groundwork for an EU Action Plan on Rare Diseases, a high-level event on 9-11 December 2025 will bring together key stakeholders including industry leaders, patient advocacy groups and EU policymakers for discussions on how to move forward.

### 2. Standardise and share best practices

**Identify best practice examples where optimal transitions are taking place, that can be replicated in care settings across Europe.** England's 'Ready, Steady Go' is a good case study that promotes early, patient-centred and multidisciplinary transition models anchored in policy at the national level.

Demonstrate how the process of transition for young people living with sickle cell disease can be used as a case study example for other rare diseases to support transitions for other patient populations. Similarly, the sickle cell disease community can learn from other people living with rare diseases through exchange of expertise and experiences.

### 3. Leverage EU-level collaboration and digital tools

Work to **integrate transition into EU rare disease strategies, utilise digital health infrastructure** (e.g. European Health Data Space, ERNs), **and develop registries** to track outcomes and quality of life.

Encourage **patients, clinicians, policymakers and patient organisations to work together** for systemic change, ensuring that transition is recognised as a priority in rare disease strategies.

These action items are designed to ensure that the transition from paediatric to adult care is recognised, prioritised, and improved across Europe, ultimately leading to better outcomes for young people living with sickle cell disease and other rare diseases.

*Novo Nordisk and Pfizer commissioned and funded the production of the Charter. The Charter has been developed by the members of the Sickle Cell Transitions Policy Lab and Lived Experience Council, with support from MHP Group acting as Secretariat. The companies provided no direction on the recommendations made by the authors within the Charter. The companies did not provide substantive input to the Charter language; however, both companies have reviewed the Charter for factual accuracy and to ensure compliance with all relevant industry codes of practice, including those of the EFPIA and ABPI.*