



# The Hidden Struggle: Confronting Sickle Cell Disease Health Inequalities

This manifesto aims to shed light on the long-neglected and life-threatening condition of Sickle Cell Disease (SCD) and the health inequalities faced by its patients at the EU and national levels.

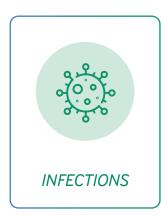
#### What is Sickle Cell Disease (SCD)

SCD is characterised by the premature death of sickle cells, leading to a persistent shortage of red blood cells, along with their tendency to obstruct small blood vessels during circulation. SCD, like most rare haematological diseases, causes chronic health problems and is a life-threatening disorder requiring numerous resources and multidisciplinary teams for correct diagnosis and management<sup>1</sup>.

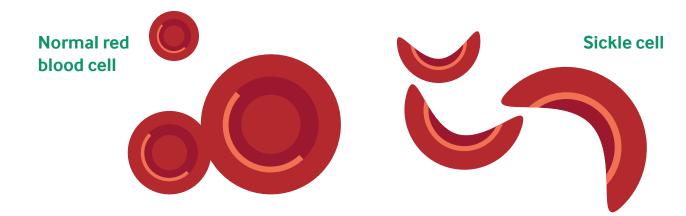
SCD complications often result in:











#### Treatment and accessibility for SCD

SCD currently lacks a cure beyond bone marrow transplant, which requires a compatible donor<sup>2</sup>. However, there are effective treatments available to manage symptoms and prevent complications. These include medications to prevent red blood cell sickling and reduce pain crises. They aim to prevent multiple complications, provide pain relief, administer antibiotics, and offer vaccinations for infection prevention, especially in children with the disease.





people in Europe<sup>3</sup> are affected, and it is therefore considered a rare disease in the region



SCD is particularly prevalent in Spain, Italy, Portugal, Germany, France and the UK. The cases in the EU are continually increasing due to global population movements and increasing life expectancy.

Red blood cell exchange (RBCX) is a valuable treatment for severe episodes, proving effective in managing symptoms, reducing costs, lowering hospitalization risks, and improving patients' quality of life<sup>4</sup>. Additionally, interventions like blood transfusions increase normal red blood cell count, reducing the risk of complications<sup>5</sup> such as stroke and acute chest syndrome. Intravenous fluids and oxygen therapy are crucial during crises.

Moreover, self-care practices and dietary supplements<sup>6</sup> are essential aspects of managing the overall condition. Adequate hydration, exercise, temperature control, and rest contribute significantly to patient well-being<sup>7</sup>. Early diagnosis through newborn screening remains crucial for timely intervention, ultimately leading to better outcomes. Treatment accessibility varies across the EU due to differences in healthcare resources. Urgent measures, including increased EU funding for education, awareness, and research, are essential to enhance diagnosis, treatment, and prevention strategies for sickle cell disease.

## Despite sickle cell disease affecting

# 2.6 in 10,000 PEOPLE

IN THE EU IN 2017°, MANY COUNTRIES LACK THE FACILITIES AND EXPERTISE TO DIAGNOSE AND MANAGE IT, AND PATIENT NUMBERS ARE UNKNOWN IN MOST MEMBER STATES.

## Unmet Needs Faced by SCD Patients in the EU

SCD patients' lives are profoundly impacted due to severe pain, fatigue, and reduced life expectancy. They often face additional challenges, including financial strain, limited knowledge among both healthcare professionals and patients, as well as disparities in care driven by racial and geographical factors.



#### **Limited Knowledge Among Healthcare Professionals**

Limited awareness of sickle cell disease (SCD) in healthcare, along with scarce treatment options and regional care disparities, undermines patient confidence in national healthcare systems, leading to subpar care. This often prompts SCD patients to avoid primary care visits, worsening their condition and contributing to a lack of diagnosis and treatment knowledge among healthcare providers.



#### **Health Equity for SCD Patients**

The healthcare system's inadequate response to SCD creates disparities, impacting patients through delayed diagnosis and limited treatment access. These issues contribute to an equity gap and worsen the overall disease burden. Additionally, it's essential to recognize that the prevalence of SCD in specific racial and ethnic groups stems from genetic diversity rather than inherent inequality. Acknowledging this is crucial to prevent misunderstandings linking SCD prevalence to social or racial biases.



#### **Financial Strain on Patients**

Dealing with SCD leads to significant financial strain due to medical costs and unplanned hospital stays. A 2021 survey found that 59% of people living with SCD reported insufficient funds to manage their condition, with 46% experiencing frequent financial distress<sup>10</sup>.



#### Lack of research and data on SCD

SCD, often unnoticed as a disability, makes it challenging to collect data on its financial, social, and mental impact. This contributes to ongoing healthcare inequality and delays the recognition of disability status for individuals with sickle cell disease at both national and EU levels<sup>11</sup>. Insufficient and fragmented data also hinder researchers from obtaining essential patient datasets for basic and clinical research.

# Key Policies for SCD Patients Needed in 2024-2029

While recognizing the EU's initiatives in Rare Diseases, TBCT acknowledges the ongoing need for action on conditions that are progressively impacting the European population. In the 2024-2029 mandate, it is recommended to:



#### Improve Access to Treatment and Address Health Inequities

Introduce an EU-wide action plan for rare diseases, prioritising health equity, and featuring a dedicated section to enhance the diagnosis, treatment, and care for SCD patients. The action plan should aim at ensuring equitable access to resources and treatment, engaging communities, educating stakeholders, and advancing research efforts. This would address increasing demands from EU institutions and civil society<sup>12</sup>.



#### **Invest in Training and Skill Development**

Allocate funding to train healthcare providers in SCD treatments, enhancing awareness and minimizing biases. The underfunded healthcare staffing requires increased investment, especially under EU4Health, Digital Europe, and Horizon Europe initiatives. Prioritizing this matter through proactive funding opportunities would incentivize essential training on the different existing SCD treatments.



#### **Encourage an EU-Wide SCD Registry**

Create a robust EU-wide SCD registry for efficient patient tracking, policymaking, and genotyping data collection. This registry would enable the systematic and wide collection of genotyping data, offering valuable insights into the genetic diversity of SCD across the European population. This initiative aligns with the goals of the European Health Data Space (EHDS), aiming to establish a secure infrastructure for sharing healthcare data across the European Union.



#### Newborn screening applied to EU 27 Member States

Early detection is crucial, and screening programs are the first essential step. However, the current decentralized approach in the EU leads to significant disparities among Member States, affecting individuals' access to timely diagnosis and treatment based on their location. Advocating for universal newborn screening, as successfully practised in Belgium, the Netherlands, Spain, and the UK, ensures equitable outcomes. To enhance effectiveness, screenings should be accompanied by referrals and follow-ups to support patients and their families, addressing barriers to care.



#### Strategic dialogue with relevant stakeholders

Collaboration with relevant stakeholders like ERN-EuroBloodNet is crucial to address SCD challenges. The EU should forge partnerships and open dialogue with healthcare professionals, patient organizations, research institutions, Member States, and industry for shared expertise, research support, advocacy, and a coordinated approach to enhance SCD management.

¹https://ehaweb.org/organization/newsroom/news-and-updates/tackling-sickle-cell-disease-the-need-for-a-european-approach/

<sup>&</sup>lt;sup>2</sup> https://www.thelancet.com/action/showPdf?pii=S2352-3026%2823%2900182-5

<sup>&</sup>lt;sup>3</sup> https://www.europarl.europa.eu/doceo/document/E-9-2022-000365\_EN.html

<sup>4</sup> https://www.sicklecellsociety.org/automated-red-blood-cell-exchange/

<sup>&</sup>lt;sup>5</sup>https://www.uclh.nhs.uk/our-services/find-service/cancer-services/blood-diseases-clinical-haematology/blood-diseases-types-and-services/red-cell-diseases/sickle-cell-anaemia/what-sickle-cell-disease

<sup>6</sup> https://www.ema.europa.eu/en/news/new-treatment-sickle-cell-disease

<sup>&</sup>lt;sup>7</sup> https://www.ema.europa.eu/en/news/new-treatment-sickle-cell-disease

<sup>8</sup> https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3171970

<sup>&</sup>lt;sup>9</sup> Juburi, G.A. et al (2012) Views of Patients about Sickle Cell Disease Management in Primary Care: A Questionnaire-Based Pilot Study. JRSM Short Rep., 11, 78.

<sup>10</sup> https://www.vrtx.com/stories/unraveling-hidden-impacts-sickle-cell-disease-and-beta-thalassemia/

<sup>&</sup>lt;sup>11</sup> https://www.thelancet.com/action/showPdf?pii=\$2352-3026%2823%2900182-5

<sup>&</sup>lt;sup>12</sup> In a recent conference in Bilbao, the European Economic and Social Committee EESC urgently called for an EU-wide strategy that ensures equitable access to resources and treatment, engaging with communities, educating stakeholders, and advancing research efforts.

### **MEPs Declaration**

In the next term and when shaping the new EU political agenda until 2027, we commit to prioritising SCD as a critical health issue and unmet need. We will collaborate with stakeholders, raise awareness, support affected individuals, improve patient access, and work toward innovative healthcare solutions for a better future for European citizens living with SCD.

Signatures of MEPs supporting the manifesto	

