

5597199 AGEING WITH SICKLE CELL ANEMIA AND HOW TO FACE IT

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Background: Sickle Cell Anemia, a hereditary monogenic disorder is caused by homozygous sickle mutation (Hb SS). Sickle erythrocytes promote vaso-occlusion and haemolysis, which are two major hallmarks of the disease.

Vaso-occlusive crisis results from the sickle red cells obstructing and reducing blood flow to the vital organs leading to ischemia, necrosis and pain(2). Data show that other blood cells i.e. leukocytes and thrombocytes are involved in this process as well, and the process starts from up regulation of P-selectin which contributes in cellular interaction and vaso-occlusion later on(3,4). All these cells stick together and can't easily move through the blood vessels. This situation can block small blood vessels and the movement of healthy, normal oxygen-carrying blood(5,6). Repeated episodes lead to bone and organ infarction and necrosis; organs damage occur over time. Hemochromatosis occurs as well, as a result of blood transfusions or/and persistent hemolysis. Patients suffering from this kind of anemia feel exhausted and need strictly monitoring for every health issues they represent, especially when they get older(8).

Because of this pathologic condition's complexity, the best method of treatment is a combined approach(1). Sickle Cell Disease is a very polymorphous disease and its severity varies largely in each patient, that's why the management has to be in a personalized model(7).

Aims: To give an overview and our considerations related to Sickle Cell Disease in Albania

Methods: To achieve the goal of treating and managing medically people living with Sickle Cell Disorder, we monitor every single sign and laboratory fluctuation happening to these patients in every clinical visit. F.A. is a female with Sickle Cell Disorder, 41 years old, diagnosed in early infancy and since then she has been treated with blood transfusions initially (in her hometown until 19 years old) and later on with pure red blood cells and iron chelation therapy in our clinic. This treatment was performed once per month. She was receiving Hydroxycarbamide as well. She started to be non compliant to ICT (iron chelation therapy) and as a consequence she has been presented with very high value of ferritin level (above 5000 ng/ml).

On 2013 she gave birth to a baby. A severe haemolysis was seen over time, she was put in transfusions twice per month. No changes in ultrasound or images. Two years ago she demonstrated a severe anaemia, arterial hypertension and proteinuria. She was diagnosed with Chronic Kidney Disease grade 3. Since then she had a personalised supportive therapy and this year was put in dialysis.

Results: According to our experience and our point of view Sickle Cell Disease seems to have a moderate burden in our country.

A new era of novel drugs seem to encourage and realize better achievement in SCD management. Sharing best experiences and our clinical cases between us, may affect positively the quality and management of these diseases.

Summary: A better understanding and a strictly monitoring of patients suffering from Sickle Cell Disease could help health professionals and patients themselves to cooperate and develop new modalities towards managing these polymorphous conditions.

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5610107 LIVE OUTCOMES AND BIOMETRIC MONITORING: POTENTIAL IMPLICATIONS FOR PATIENT QUALITY OF LIFE IN SICKLE CELL DISEASE

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Background & Aims: There remains limited understanding of factors such as patient quality of life on a day-to-day basis in Sickle Cell

Disease (SCD), with monitoring often limited to annual patient reviews and clinic attendances within a healthcare setting. This study aimed to explore an approach to assess quality of life in patients with SCD through a remote monitoring ecosystem accessible from patients' own homes, and with this, what potential impact such tracking may have upon patient wellbeing.

Methods: The patient ecosystem provided a clinically validated wearable for live remote monitoring of metrics (e.g., activity, sleep, and heart rate); a digital portal for input of patient-reported outcomes (PROs); and a digital platform of live and retrospective insights, healthcare timelines, and data entry – a 'digital patient wallet' – encapsulating their combined data, including medical records. Patients provided informed e-consent to participate and for analysis of their de-identified data, with no dropouts. Patients who had (1) participated for at least 5 months, (2) recorded a minimum of 5 EQ-5D scores across their first 30 days with the programme, and (3) recorded a minimum of 5 entries across the most recent 30 days were analysed (n=23). Percentage changes between the first and most recent 30-day average EQ-5D scores were calculated, to assess any potential changes since patients began utilising the ecosystem, as well as the statistical significance of any differences in EQ-5D between these timepoints.

Results: In comparison to their initial 30-day average, 78% (18/23) of patients showed an overall increase in average EQ-5D score by their most recent 30-day period, with 35% (8/23) showing a statistically significant change (p<0.05). In contrast, 22% (5/23) of patients showed an overall decrease in EQ-5D, however, only 4% (1/23) recorded a statistically significant change.

Early trends highlight a potential, albeit not statistically significant, emerging link between patient engagement rates with EQ-5D reporting (number of entries/days since first entry) and the percentage change in their EQ-5D score, with increasing engagement rates correlating with increasing percentage changes in EQ-5D score.



Conclusions & Future Steps: This work provides an early look at the potential impacts consistent monitoring of PROs may have on patient wellbeing. While not necessarily a causal relationship, it highlights early indications that this form of PRO monitoring may be beneficial to patient outcomes, potentially through heightened engagement with patient self-reporting and therefore self-monitoring to support disease management and behavioural changes.

However, such findings may also be influenced by factors including the wearable biometric monitoring component, health events patients may have experienced over this period, as well as differences in the time of year patients joined the programme – environmental factors and temperature known to impact patient wellbeing in SCD.

A key goal will be to increase the period of longitudinal data collection for more in-depth comparison within a larger volume of patients. Future steps will incorporate direct causal analysis, investigate whether these changes are impacted by each individual's starting point, and explore any associated changes in patients' physical health – building a stronger understanding of patient outcomes and quality of life in SCD, and the approaches that can be taken to improve this.

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5613386 CELEBRATING THE SICKLE CELL DISEASE WORLD DAY ON JUNE 19TH AFTER TWO YEARS OF PANDEMIC: MY LIFE WITH SCD, PATIENTS' PERSPECTIVE

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