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Poster 15046

Adapting Patient Reported Outcomes' evaluation to COVID context – the case of ASCEND study

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ASCEND — Assessing physical, Social and Emotional Impact of Sickle Cell Disease in Portuguese Patients: an observational, multicenter study

KEY FINDINGS & CONCLUSIONS

- ASCEND methodology adaptation to digital data collection methods revealed to be very enriching to the study enrollment;
- Collaboration with Patient Association allowed a previous validation of the patient questionnaire and an increased number of recruited patients;
- Although digital data collection can be seen as a challenge for patient participation, the recruitment and completeness rate for this study showed the positive impact digital tools can have on study execution.

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INTRODUCTION

- Covid-19 pandemic imposed changes on disease management and follow-up namely with reduction on number of clinical appointments and use of telemedicine[1,2,3]
- Sickle cell disease (SCD) is a genetic blood disorder that is characterized by painful episodes caused by vaso-occlusion, with a significant impact on Quality of Life (QoL). This disease affects around 850 patients in Portugal[4]
- The primary objective of ASCEND study is to characterize the physical impact of SCD on patients, through collection of patient reported outcome (PROs). The study also aims to characterize SCD patients' demographics and clinical history, as well as to describe the social and emotional impact of SCD on patients
- This abstract aims to describe the adaptation of ASCEND study from a F2F methodology to a fully digital PRO study and its impact on Sickle Cell Disease (SCD) patients' recruitment, based on clinicians and patients' representatives' feedback

METHODS

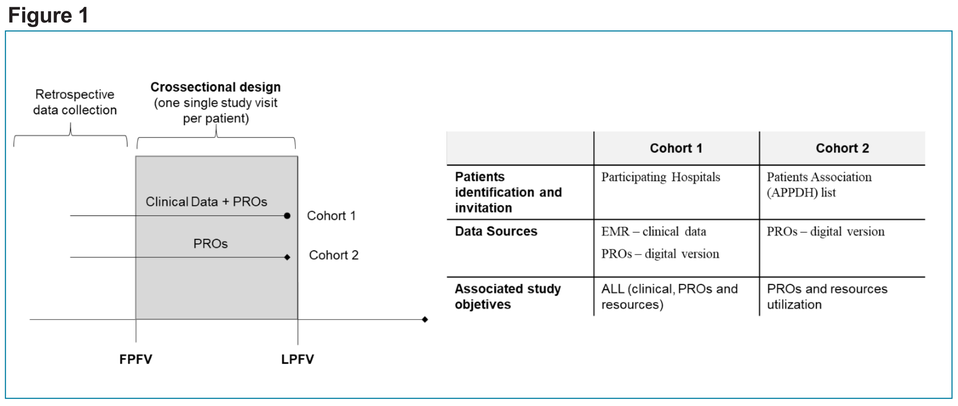
- ASCEND is non-interventional cross-sectional study of 2 cohorts of SCD adult patients (**Figure 1**);
- The planned study sample is 200 (recruited by hospitals) + 50 patients (recruited by patient association). Due to COVID pandemic, all study procedures (informed consent, clinical and PRO collection) were exclusively done using digital resources
- Digital resources allowed the patient to be included in-person (filled information using a tablet provided for the study) or remotely in a virtual appointment (investigator sent a link to the patient's device in order to fill information remotely)
- PRO questionnaires include both EQ-5D-5L and ASCQ-Me (Pain Episodes Frequency and Severity)
- Additional societal and emotional questions were added to PRO questionnaire
- All PRO questions went through a prior validation process done in collaboration with Portuguese patients and Social Studies investigators

RESULTS

Implementation

- "Assessing physical, Social and Emotional impact of sickle cell disease in Portuguese patients: an observational, multicenter study (ASCEND)" is a Novartis sponsored study implemented in mainland Portugal;
- Supported by epidemiological data, the majority of these patients in Portugal are concentrated in Lisbon metropolitan and centre/north of the country. Based on that, 6 of the recruitment sites were concentrated in Lisbon metropolitan area and 1 site in Coimbra;
- On top of these recruiting sites, the Portuguese Association of Parents and Patients with Hemoglobinopathies (APPDH) recruited patients not captured by cohort 1 (**Figure 1**)

Figure 1



	Cohort 1	Cohort 2
Patients identification and invitation	Participating Hospitals	Patients Association (APPDH) list
Data Sources	EMR – clinical data PROs – digital version	PROs – digital version
Associated study objetives	ALL (clinical, PROs and resources)	PROs and resources utilization

Figure 1 shows the study design and type of data collected in each cohort

- All study procedures were done using digital resources: from patient informed consent to both HCPs and Patient Reported Outcomes (PROs) data (**Figure 2);**
- During the study submission phase some sites were not convinced that the "digital only" strategy would be a good option;
- The two PROs used in this study were general (EQ-5D-5L) and disease specific questionnaires (ASCQ-Me);
- This digital implementation allowed the patient to be included in hospital and filling the information using a digital tool (e.g. tablet) but also to be included during a remote appointment. For that, the investigator triggered a temporary link to the patient's email or phone so that the patient could read and sign the inform consent, filling PROs data afterwards

Figure 2

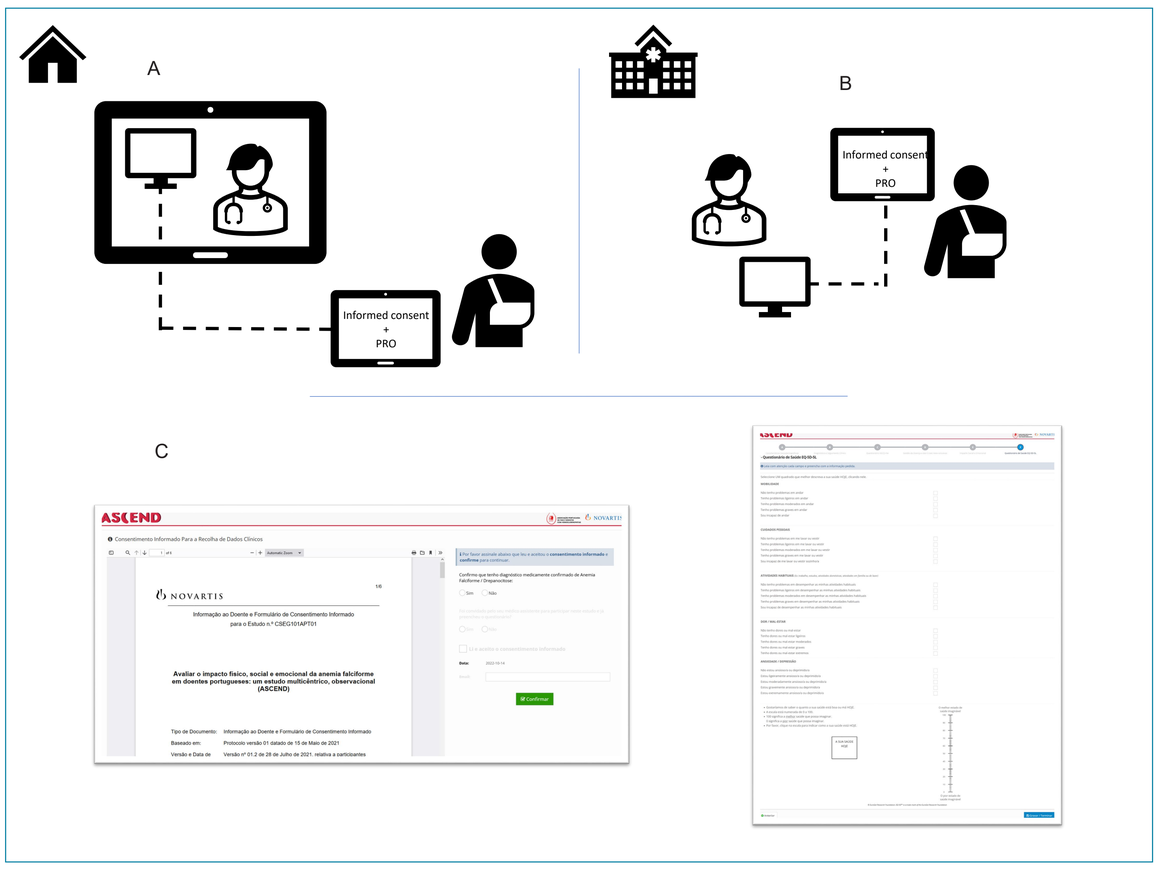


Figure 2 - Schematic representation of recruitment and information filling data both for remote (A) or face-to-face (B) inclusion. ASCQ-Me and EQ-5D-5L. Print screens of digital patient informed consent and EQ-5D-5L answering page (C)

Figure 3

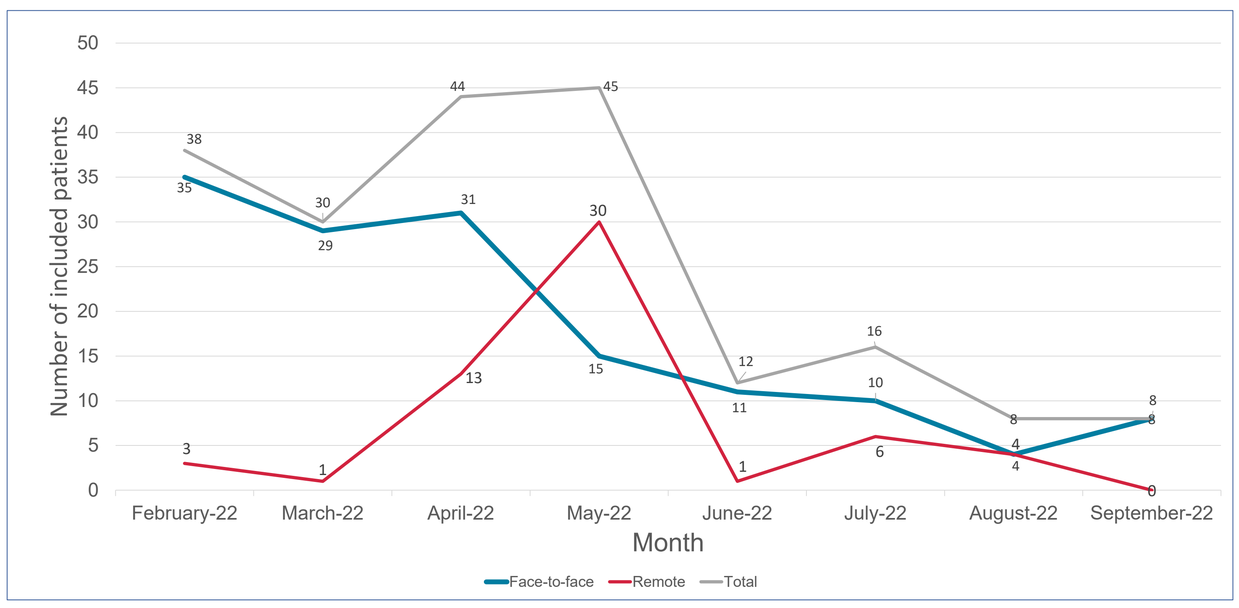


Figure 3 - Graphical representation of the recruitment evolution, comparing face-to-face (blue) and remote (red) patient inclusion. Total number of included patients is represented in grey

Outcomes

- For the current analyses, the first patient was recruited in February 2nd and the last patient in September, 29th 2022
- During the analysed period, 201 patients were included in the study. The included patients represent 24% of the total estimated number of patients living in Portugal with this disease[4];
- The average recruiment rate was 25 patients/month. However, for the first 4 months (February to May) this rate reached 40 patients/month. Majority of patients were included in the initial 3 months;
- This peak rate was significantly inlfuenced by the possibility to include patients remotely. In fact, the patients included remotely accounted for more than 30% of the total included patients in 4 months during the recruitment period, surpassing the face-to-face included patients in August and May (**Table 1**);
- 5% of the included patients entered the study with patients' association support - APPDH. These patients were not followed up in the recruiment sites;
- At the end of the anlysed period, remotely included patients accounted for 29% of the total;
- Except for one case, every included patient who signed digital informed consent filled 100% of PRO questionnaire

Table 1. Face-to-face vs remote recruitment rate per month

Month	Face-to-face	Remote	Number of recruited patients per month
February 2022	92%	8%	38
March 2022	97%	3%	30
April 2022	70%	30%	44
May 2022	33%	67%	45
June 2022	92%	8%	12
July 2022	63%	38%	16
August 2022	50%	50%	8
September 2022	100%	0%	8

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Disclosures

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The author Ines Moital is currently employee of Astrazeneca

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