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A Patient-Centric Approach to Improve the Understanding of Sickle Cell Disease Using Real-World Data

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Disclosures

- **E Leila Jerome Clay** reports receiving consultancy fees from Novartis and GBT. **Dan Drozd** and **Kieran Mace** are employees of PicnicHealth. **Dan Drozd** also reports being a current equity holder in PicnicHealth. **Miranda Bailey**, **Jincy Paulose**, **Nicholas Ramscar** and **David Wormser** are employees of Novartis. **David Wormser** also reports being a current equity holder in Novartis

Introduction

- SCD is an inherited group of blood disorders associated with a range of clinical complications across multiple organ systems[1,2]

- These complications are driven primarily by vaso-occlusion and hemolytic anemia and can result in end-organ damage and early death[3,4]

- Painful VOCs are a characteristic feature of SCD and can require healthcare intervention

Despite advances in screening, management and treatment,
gaps remain in our understanding of SCD in the real-world setting

SCD, sickle cell disease; VOC, vaso-occlusive crises

1. Zhang D *et al. Blood* 2016;127:801–9; 2. Rees DC *et al. Lancet* 2010;376:2018–31; 3. Ballas SK, Lusardi M. *Am J Hematol* 2005;79:17–25; 4. Novelli EM, Gladwin MT. *Chest* 2016;149:1082–93

Introduction

- Real-world evidence (RWE) is often generated from information in payer databases (eg claims)

- Electronic medical records (EMRs) can provide in-depth clinical data that may not be available in payer databases, and therefore have potential as valuable real-world sources of clinical information. However, information from EMRs is difficult to analyze

EMRs for individual patients are not centralized in the USA

The information in EMRs typically contains unstructured data (eg text) and is often not available in a standardized format

- Harmonizing all available EMR data for an individual patient into a single, comprehensive data source could improve the accessibility of information and quality of real-world data

Aims

- This study will explore the value of collating EMRs for individual patients into a single, consistently structured format, with the aim of developing richer real-world data to complement existing data on SCD

- It is hypothesized that the resulting longitudinal overview of each patient's care will contribute to an improved understanding of SCD in the real-world setting by:

Better capturing how many VOCs patients with SCD experience over time, and how many of these are being managed at home compared with in a hospital or clinic setting

Obtaining greater insights into the prevalence and progression of end-organ damage and any association with VOCs

Highlighting the type of and site of care for SCD in the real world (eg medications prescribed, specialties of treating HCPs, and type of hospital or clinic visited)

HCP, healthcare professional

Enrollment

400 patients with SCD from the USA
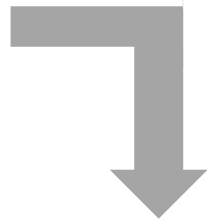
Recruitment

Direct	Indirect
Via social media and through working with patient ambassadors	Through a variety of partnerships, including with HCPs, patient advocacy groups and centers of excellence

Sign informed consent form allowing anonymized medical information to be shared with third-party organizations

Enrolled patients will gain access to their medical records via a dashboard

Enrollment

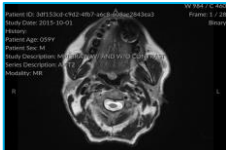| Key inclusion criteria	• Confirmed SCD diagnosis* • Aged ≥16 years at enrollment • ≥1 inpatient admission for a VOC in the 12 months prior to enrollment
Key exclusion criteria	• Absence of any medical records

*Irrespective of genotype

Data collection and interpretation

- Components of EMRs collected include providers' notes, laboratory and test results, clinical imaging and treatment records
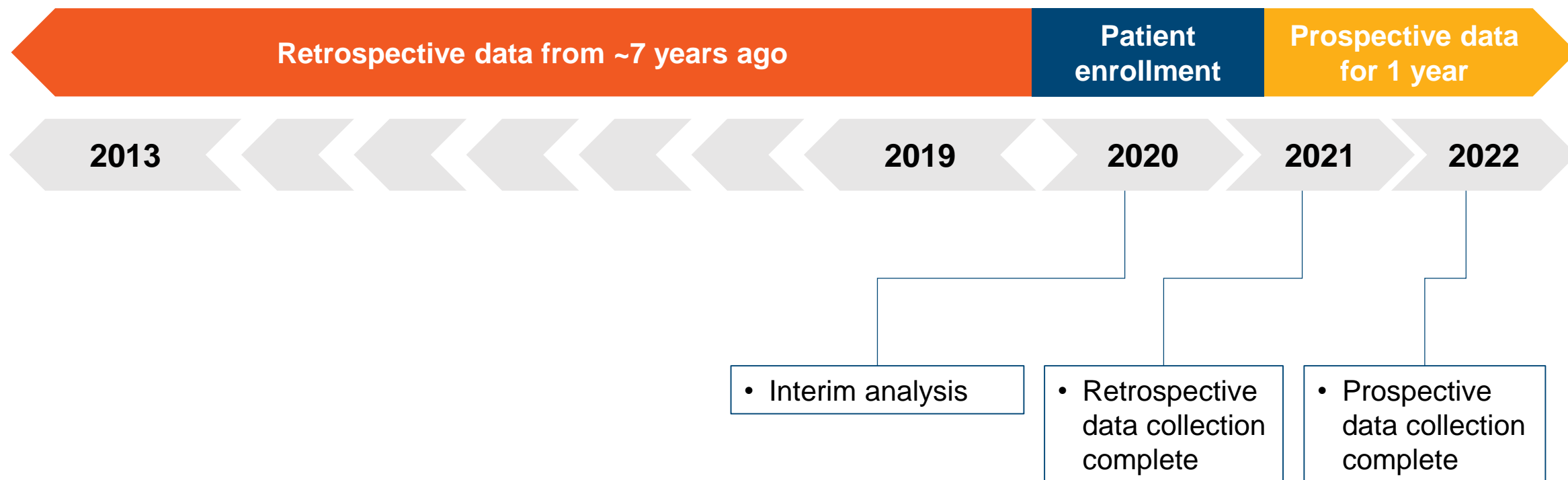
Illustrative medical record

Conditions	Labs
Drugs	Vitals
Procedures	Genetics
Narrative text (eg doctor's notes, image interpretation)	Imaging

- Human-curated natural language processing and machine learning is being used to extract, structure and code data from the structured sections and unstructured narrative text of the EMR

Study design

- All medical records from all visits will be collected

Retrospective data from ~7 years ago	Patient enrollment	Prospective data for 1 year

2013 ➤➤➤➤➤➤➤➤➤ **2019** **2020** **2021** **2022**

- Interim analysis

- Retrospective data collection complete

- Prospective data collection complete

Interim results

Patient demographics

- Between December 1, 2019 and October 23, 2020,* 103 patients with SCD (out of a target of 400) were enrolled

	Overall (N=103)
Median age at data cut-off, years (range)	34.0 (17.7–58.6)
Sex, n (%)	
Female	79 (76.6)
Male	24 (23.3)
Median (mean) number of years of retrospective data	6.6 (8.3)

*The data cut-off date for this interim analysis

This study aims to advance the understanding of **real-world practice** in the **management of SCD**

In particular, we will assess the **number of VOCs** patients experience, any association between **VOCs and end-organ-damage**, and **management of SCD** in the real world

Conclusions

Generating a **single, structured overview** of all EMRs for each patient will allow **richer insight generation** and a more comprehensive analysis of RWE versus existing approaches

The insights gained from this RWE may **inform future studies and clinical trials in SCD**, with the ultimate aim of improving the **quality of life** of patients

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