The Cost of Haemophilia in adults, a Socioeconomic Survey of People with Severe Haemophilia in Latin America: The 'CHESS LATAM' Study

RWD44

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Background

- Haemophilia is a recessive X-linked disorder characterised by a reduced ability to generate thrombin leading to prolonged bleeding events. Haemophilia A (HA) and haemophilia B (HB) are characterised by deficiency of clotting factor VIII (FVIII) or clotting factor IX (FIX), respectively[1].
- The prevalence of haemophilia varies across Latin and South America, with prevalence estimates in select South American countries ranging from 6.2 (Argentina, Brazil) to 9.4 (Chile) per 100,000 people[2].
- Though the comprehensive burden of haemophilia has been studied in Europe and North America, the impact of severe haemophilia in Latin and South America has not been well characterised. Robust data on the real-world burden and cost of haemophilia is needed to understand the impact and unmet needs of existing treatment patterns, and the potential improvements of emerging therapies.

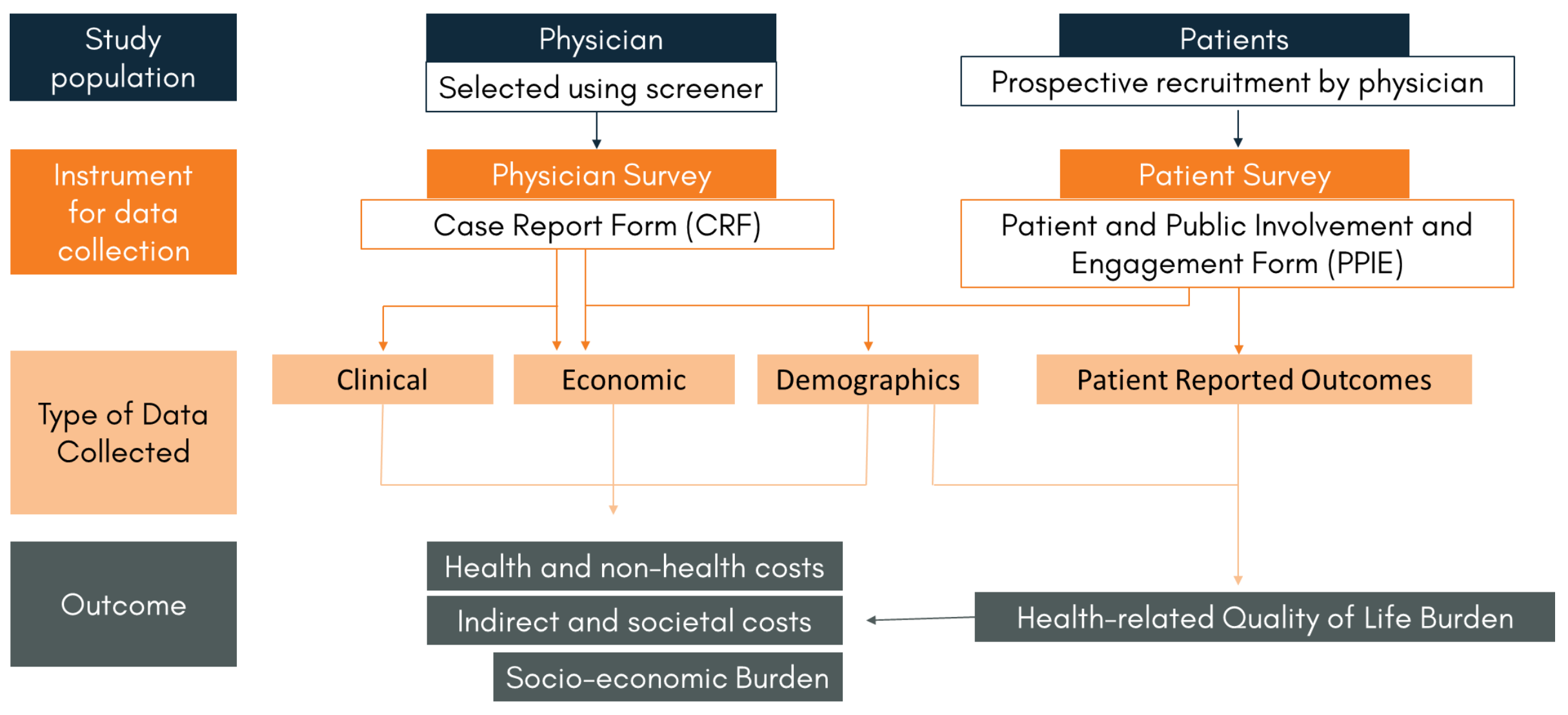
Aims and Objectives

- To quantify the socioeconomic burden of severe haemophilia across Latin America.
- To estimate the health-related quality of life of people with severe haemophilia across Latin America.

Methods

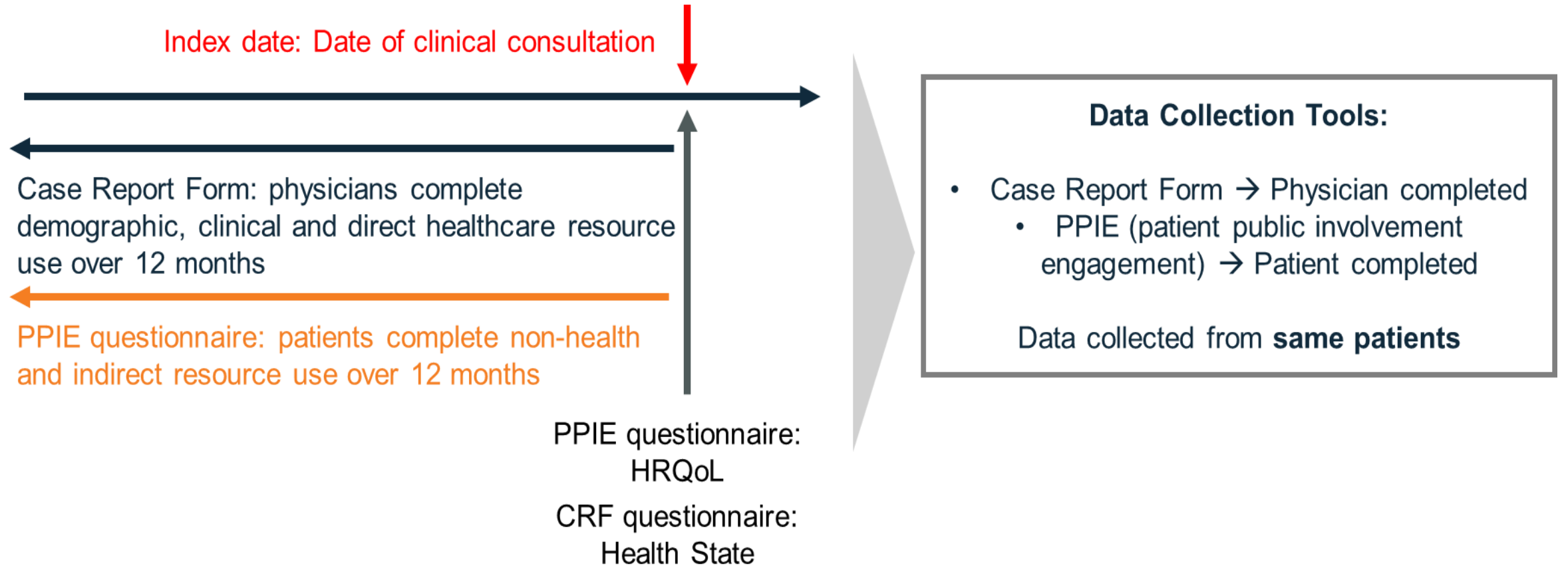
- The study was designed as a retrospective, cross-sectional, bottom-up prevalence-based burden of illness study.
- Haematologists were recruited using a panel-based approach, with patients recruited prospectively, with the index date defined as the date of clinical consultation, either in-person or via the phone.
- Case report forms (completed by haematologist) captured clinical, demographic, and direct medical cost data associated with haemophilia in each country.
- Linked patient-completed (voluntary) forms capturing direct non-medical and indirect costs, as well as patient reported outcomes including the EQ-5D-5L and the work productivity and Activity Impairment (WPAI).
- The study design for the CHESS LATAM study is presented in Figure 1.

Figure 1. CHESS LATAM study design


Study population
Physician
Selected using screener
Patients
Prospective recruitment by physician
Instrument for data collection
Physician Survey
Case Report Form (CRF)
Patient Survey
Patient and Public Involvement and Engagement Form (PPIE)
Type of Data Collected
Clinical
Economic
Demographics
Patient Reported Outcomes
Outcome
Health and non-health costs
Indirect and societal costs
Socio-economic Burden
Health-related Quality of Life Burden

- Data was collected between September 2020 and May 2021, with resource use collected over the 12 months prior to the index date. Health-related quality of life was captured at the index date, as shown in Figure 2.

Figure 2. CHESS LATAM data collection period


Index date: Date of clinical consultation
Case Report Form: physicians complete demographic, clinical and direct healthcare resource use over 12 months
PPIE questionnaire: patients complete non-health and indirect resource use over 12 months
PPIE questionnaire: HRQoL
CRF questionnaire: Health State

Data Collection Tools:
- Case Report Form → Physician completed
- PPIE (patient public involvement engagement) → Patient completed

Data collected from **same patients**

- Socioeconomic burden was calculated by capturing per-patient healthcare resource use and multiplying by country-specific unit costs. Some direct non-medical and indirect costs were captured directly from patients via the PPIE.
- Health state utility values were captured by applying the Uruguayan EQ-5D-5L preference set[3] to reported EQ-5D-5L data. The Uruguayan value set[3] was used as no country-specific value set was available for the four countries captured in the study at time of analysis.

Results

- The number of completed eCRFs and PPIEs captured during the CHESS LATAM study are reported in Table 1.

Table 1. CHESS LATAM population

Characteristic, n (%) unless noted otherwise	All Patients (N=830)	Argentina (n=259)	Brazil (n=262)	Chile (n=107)
Completed eCRFs (physicians)	830 (100)	259/830 (31)	262/830 (32)	107/830 (13)
Completed PPIEs (patients)	153/830 (18)	31/259 (12)	18/262 (7)	104/107 (97)
Haemophilia A	676 (81)	215 (83)	214 (82)	69 (64)
Haemophilia B	154 (19)	44 (17)	48 (18)	38 (36)

- A total of 105 haematologists completed 830 patient case report forms, with 157 associated patient-completed forms captured.
- Mean, per-patient, total costs across all four countries were US$117,414 and US$49,920 per year for Haemophilia A and Haemophilia B, respectively.
- EQ-5D-5L scores averaged at 0.85 for both Haemophilia A and B across countries.

Conclusions

- The 'CHESS LATAM' study is a novel and comprehensive dataset, encompassing the burden of severe haemophilia in four South American countries.
- This dataset allows for exploration of the socioeconomic burden across people with either haemophilia A or B, as well as identification of potential areas of unmet need in the population.
- Higher than expected average EQ-5D-5L scores across the countries could indicate the presence of a disability paradox, previously identified in haemophilia patients[4].

Acknowledgments

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- The study was approved by the Research Ethics Sub Committee of the Faculty of Health and Social Care within the University of Chester and conducted in correspondence with regional and relevant guidelines.

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