

First Patient Enrolled in Pilot Phase of the Economics and Patient Outcomes in China in Haemophilia (EPOCH) Study

Acceptance
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OBJECTIVES

EPOCH is a global collaboration initiating a patient register to observe current standards and costs of care, quality of life (QoL), and unmet needs for people with haemophilia (PWH) in China. Health economic and outcomes research (HEOR) data collected includes direct and indirect costs and supporting clinical and patient reported outcomes (PRO) to measure the impact of different treatment modalities and varying levels of treatment access. EPOCH establishes a centralized data collection/analysis program based in China.

METHODS

EPOCH is designed with two phases (Figure 1): the pilot phase (one year), will assess the feasibility of the project, and the implementation phase (two years), will collect data to understand the treatment landscape in China. A cooperative network of haemophilia treatment centres across China will enrol participants. Data is collected on a web-based platform that links provider-patient pairs. Providers extract information from medical records; patients complete a questionnaire with QoL measures synthesizing elements from currently-available studies with validated instrument: the Patient Reported Outcomes, Burdens, and Experiences (PROBE) Study, a haemophilia-specific QoL questionnaire (Table). Socioeconomic insight will be derived from the register to form comparison with CHES (an ongoing survey in Europe/Americas quantifying humanistic and economic burden of haemophilia) and PROBE surveys conducted in other parts of the world.

FIGURE 2. Example Page from Web-Based Platform

Epoch CRF: EpochCRF

Eligibility Criteria

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Sociodemographic Characteristics

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Disease History

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Consultation History

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Hemophilia Treatment History

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History Of Inhibitors

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Hospitalization History

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Patient Adherence

0 / 2

What is the patient's baseline FVIII/FIX level (in the absence of treatment) *(Required)*

☐ Mild hemophilia - Baseline FVIII/FIX level from 5.01% to 40% (% or IU/dL)

☒ Moderate hemophilia - Baseline FVIII/FIX level from 1% to 5% (% or IU/dL)

☐ Severe hemophilia - Baseline FVIII/FIX level less than 1%(% or IU/dL)

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FIGURE 1. EPOCH Study Timeline and Outcome Collection Overview

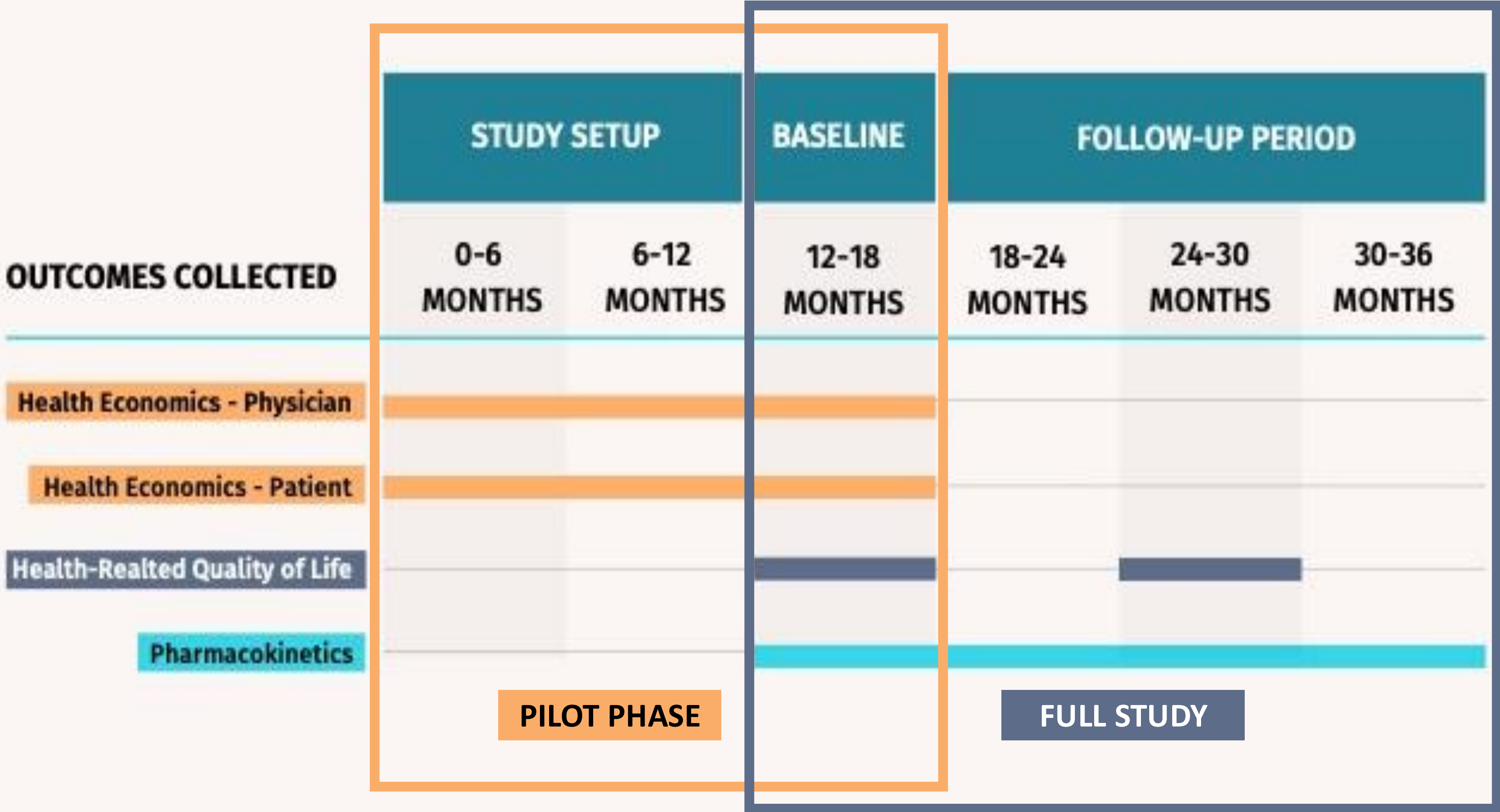


TABLE. Patient Reported Outcomes

Outcome Category	Outcomes Collected
Quality of Life	PROBE (pain, independence, education, employment, family life, mobility) and EQ-5D-5L with VAS (current health status)
Direct Health – Related Costs	Medical/surgical procedures, visits/consultations, hospitalizations, coagulation factor, tests and examinations, traditional Chinese medicine, other self medication (over the counter), devices and person aids, professional caregivers
Direct Non-Health Related Costs	Home alterations, travel costs, non-professional caregivers, transfer payments, alternative therapies
Indirect/Societal Costs	Absenteeism, work productivity impact

RESULTS

The web-based platform has been developed and deployed for hosting on a server based in China (Figure 2). Ethical approval has been obtained and the first patient is expected imminently. EPOCH expects to enroll 500 and 1500 PWH in the pilot and implementation phases, respectively.

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CONCLUSIONS

The EPOCH platform is designed to capture real-world data and observe individual patient journeys as they access treatment. Information collected will provide evidence about the experience of living with and treating haemophilia in China that can be used to advocate for improved care and more equitable access to treatment.

