A Multi-Centre Open-Label Trial Evaluating the Efficacy, Safety and Tolerability of Prophylactic Administration of Concizumab in patients with Glanzmann thrombasthenia

PRESCOG

PRophylactic Efficacy and Safety of COncizumab in Glanzmann thrombasthenia

Sponsor:
Centre Hospitalier Universitaire de Bordeaux

Coordinating Investigator:
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CRA coordinator
Valerie Goin-Monsinjon

SCIENTIFIC COMMITTEE
Dr Sophie VOISIN, Dr Dominique DESPREZ, Dr Celine FALAISE, Dr Yoann HUGUENIN, Dr Roseline D'OIRON (clinicians), Pr Laura RICHER (methodologist and statistician), Dr Benjamin SOURISSEAU (pharmacist), Dr Magalie CASTOREO (Clinical research safety ans vigilant unit) and a representative from the sponsor

STUDY DESIGN

Inclusion **20 patients (≥ 12 y) over 24 months**

ABR: bleeding episodes (spontaneous which were treated or untreated ; treated traumatic bleed)
PBAC score
Blood transfusion
Change in Hb / Iron therapy
Quality of Life (QOL) scores

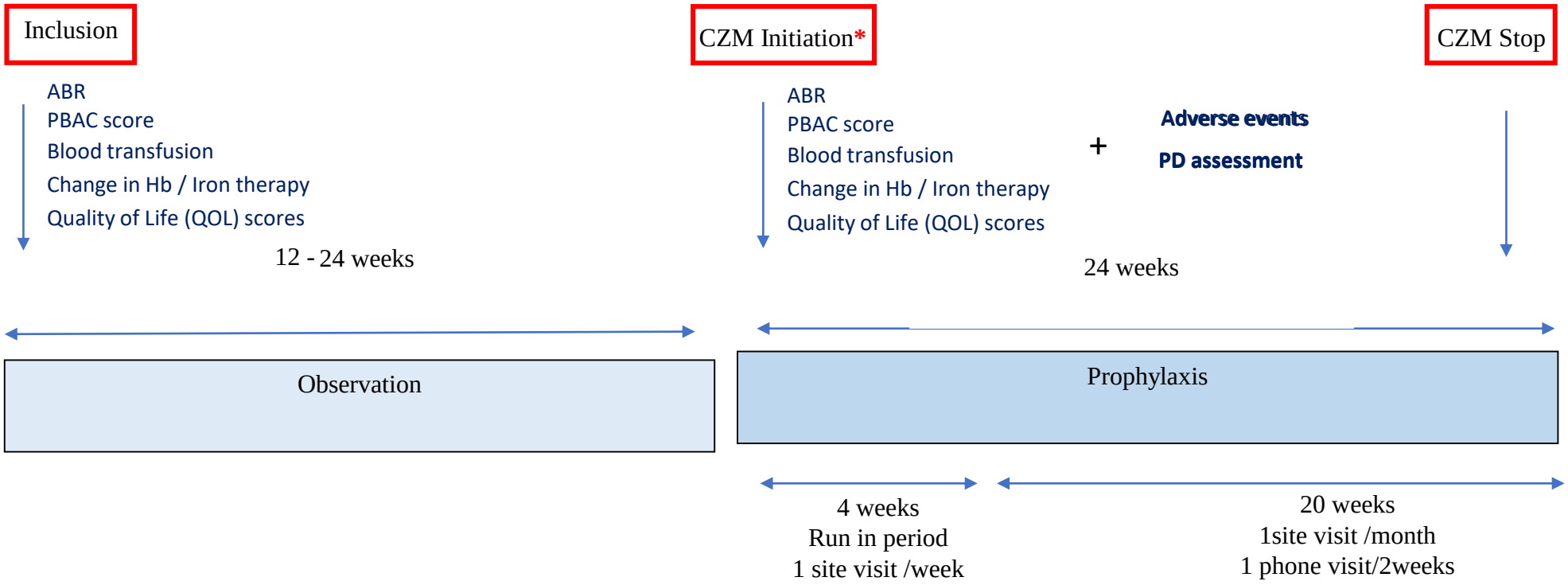
12 - 24 weeks

Observation

- **At least 3 bleeds of any type and severity** during a 6-month observation period, with the exception of :
  - bruising without painless and/or extensive that is smaller than the palm of the hand
  - menstrual bleeding defined by a PBAC score ≤100
  - traumatic bleeding requiring no haemostatic treatment

- **At least one spontaneous bleeding requiring hospital care** during a 3-month observation period

STUDY DESIGN



***Period treatment**

- Loading dose of **1 mg/kg** CZM
- A once-daily dose of **0.20 mg/kg** CZM administered s.c. once daily for a 6-month period

If at the end of the 4 weeks treatment at 0.20mg/kg and during the rest of CZM period, patient is still bleeding, dose can be increased to 0.25mg/kg.

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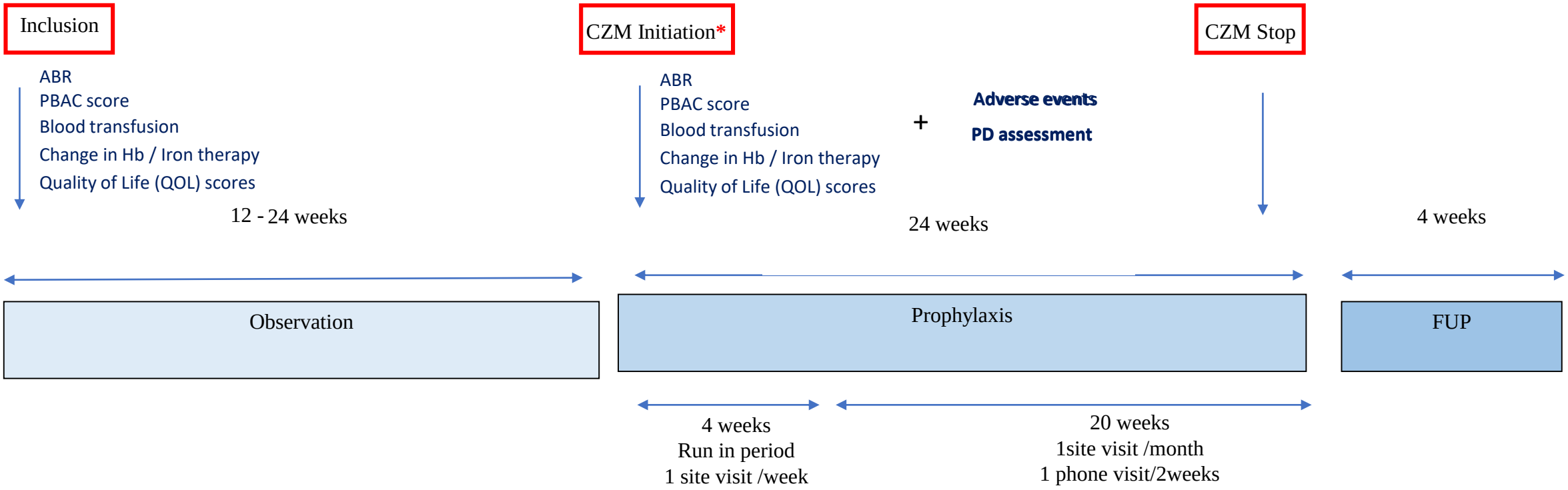

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Study milestones and timeline		
Milestone	**Description**	**Date**
1	**Activation:** Contract signature partenaires /CHU Bordeaux	En cours
2	**Promotion CHU Bordeaux:** Promotion agreement	
3	**Submission authorities**: ANSM, CPP, Clinical Trials	Mi-2025 ?
4	**Study initiation:** Site initiation visits	
5	**FPFV:** First Participant First Visit	Fin 2025 – début 2026 ?
6	**LPFV:** Last Participant First Visit	
7	**LPLV:** Last Participant Last Visit	
8	**Length of Study**	13 months
9	**Recruitment period**	24 months
10	**Data analysis**	
11	**Study report**/manuscript	