

A. CLINICAL TRIAL SYNOPSIS

1. Background and rationale

2. Objectives

3. Study design

4. Population

5. Endpoints

6. Sample size and statistical justification

7. Description of the investigational product

8. Visit schedule/overall study duration

9. Safety monitoring plan

10. Outline of the statistical analysis plan

11. Preliminary study plan (including milestones) and timeline as diagram

B. BASIC READINESS

Comment on each topic:

1. Progress with drug supply
2. Progress with sites (number of planned countries and sites)
3. Outline of recruitment potential
4. Progress with other essential elements of the trial (diagnostics, assessments)
5. To what extent are eligibility criteria and outcome assessments ready for regulatory review?
6. Progress with dosing
7. Status of pivotal (GLP) nonclinical safety/ toxicology studies (completed or not)

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C. PRELIMINARY PLAN FOR PATIENT AND PUBLIC INVOLVEMENT AND ENGAGEMENT (PPIE)

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D. INITIAL REGULATORY DEVELOPMENT PLAN

Comment on each topic

1. Orphan Drug Designation (ODD) status
2. Regulatory pathway envisaged
3. Anticipated CTA/CTIS submission
4. Paediatric-specific element (if applicable)
5. Repurposed drug/biologic-specific element (if applicable)

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