**<Title>**

**Protocol Identifying Number: < Number>**

**Principal Investigator:** **< Principal investigator>**

**IND/IDE Sponsor: <Sponsor name, if applicable>**

**Funded by: < NIH Institute & Center (IC)>**

**Draft or Version Number: v.<x.x>**

**<Day Month Year>**

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|  |  |
| --- | --- |
| <Insert text> | <Insert text> |
|  |  |
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# STATEMENT OF COMPLIANCE

<Insert text>

# PROTOCOL SUMMARY

|  |  |
| --- | --- |
| **Title:** | <Insert text> |
| **Précis:** | <Insert text> |
| **Objectives:** | <Insert text> |
|  |  |
|  |  |
| **Endpoint** | <Insert text> |
| **Population:** | <Insert text> |
| **Phase:** | <Insert text> |
| **Number of Sites enrolling participants:** | <Insert text> |
| **Description of Study Agent :** | <Insert text> |
| **Study Duration:** | <Insert text> |
| **Participant Duration:** | <Insert text> |

# SCHEMATIC OF STUDY DESIGN

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# 1 KEY ROLES

<Insert text>

# 2 INTRODUCTION: BACKGROUND INFORMATION AND SCIENTIFIC RATIONALE

## 2.1 Background Information

*Include:*

<Insert text>

## 2.2 Rationale

<Insert text>

## 2.3 Potential Risks and Benefits

### 2.3.1 Known Potential Risks

<Insert text>

### 2.3.2 Known Potential Benefits

<Insert text>

# 3 OBJECTIVES AND PURPOSE

<Insert text>

# 4 STUDY DESIGN AND ENDPOINTS

## 4.1 Description of the Study Design

<Insert text>

### 4.2.1 Primary Endpoint

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### 4.2.2 Secondary Endpoints

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### 4.2.3 Exploratory Endpoints

<Insert text>

# 5 STUDY ENROLLMENT AND WITHDRAWAL

## 5.1 Participant Inclusion Criteria

<Insert text>

## 5.2 Participant Exclusion Criteria

<Insert text>

## 5.3 Strategies for Recruitment and Retention

<Insert text>

## 5.4 Participant Withdrawal or termination

5.4.1 Reasons for Withdrawal or Termination

<Insert text>

5.4.2 Handling of Participant Withdrawals or termination

<Insert text>

## 5.5 Premature Termination or Suspension of Study

<Insert text>

# 6 STUDY AGENT

## 6.1 Study Agent(s) and Control Description

### 6.1.1 Acquisition

<Insert text>

### 6.1.2 Formulation, Appearance, Packaging, and Labeling

<Insert text>

### 6.1.3 Product Storage and Stability

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### 6.1.4 Preparation

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### 6.1.5 Dosing and Administration

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### 6.1.6 Route of Administration

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### 6.1.7 Starting Dose and Dose Escalation Schedule

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### 6.1.8 Dose Adjustments/Modifications/Delays

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### 6.1.9 Duration of Therapy

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### 6.1.10 Tracking of Dose

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### 6.1.11 Device Specific Considerations

<Insert text>

## 6.2 Study agent Accountability Procedures

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# 7 STUDY PROCEDURES AND SCHEDULE

## 7.1 Study Procedures/Evaluations

### 7.1.1 Study specific procedures

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### 7.1.2 Standard of care study procedures

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## 7.2 Laboratory Procedures/Evaluations

### 7.2.1 Clinical Laboratory Evaluations

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### 7.2.2 Other Assays or Procedures

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### 7.2.3 Specimen Preparation, Handling, and Storage

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### 7.2.4 Specimen Shipment

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## 7.3 Study Schedule

### 7.3.1 Screening

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### 7.3.2 Enrollment/Baseline

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### 7.3.3 Follow-up

<Insert text>

### 7.3.4 Final Study Visit

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### 7.3.5 Early Termination Visit

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### 7.3.7 Schedule of Events Table

| **Procedures** | <Insert text> |  |  |  |  |  |  |  |  |  |  |  |  |  |
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## 7.4 Justification for Sensitive Procedures

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## 7.5 Concomitant Medications, Treatments, and Procedures

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### 7.5.1 Precautionary Medications, Treatments, and Procedures

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## 7.6 Prohibited Medications, Treatments, and Procedures

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## 7.7 Prophylactic Medications, Treatments, and Procedures

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## 7.8 Rescue Medications, Treatments, and Procedures

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## 7.9 Participant Access to Study Agent At Study Closure

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# 8 ASSESSMENT OF SAFETY

## 8.1 Specification of Safety Parameters

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### 8.1.1 Definition of Adverse Events (AE)

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### 8.1.2 Definition of Serious Adverse Events (SAE)

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### 8.1.3 Definition of Unanticipated Problems (UP)

<Insert text>

## 8.2 Classification of an Adverse Event

### 8.2.1 Severity of Event

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### 8.2.2 Relationship to Study Agent

<Insert text>

### 8.2.3 Expectedness

<Insert text>

## 8.3 Time Period and Frequency for Event Assessment and Follow-Up

<Insert text>

## 8.4 Reporting Procedures

### 8.4.1 Adverse Event Reporting

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### 8.4.2 Serious Adverse Event Reporting

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### 8.4.3 Unanticipated Problem Reporting

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### 8.4.4 Events of Special Interest

<Insert text>

### 8.4.5 Reporting of Pregnancy

<Insert text>

## 8.5 Study Halting Rules

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## 8.6 Safety Oversight

<Insert text>

## 8.7 Risks/Benefits Assessment

### 8.7.1 Foreseeable Risks

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### 8.7.2 Risk Minimization

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### 8.7.3 Potential Benefits

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# 9 CLINICAL MONITORING

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# 10 STATISTICAL CONSIDERATIONS

## 10.1 Statistical and Analytical Plans

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## 10.2 Statistical Hypotheses

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## 10.3 Analysis Datasets

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## 10.4 Description of Statistical Methods

### 10.4.1 General Approach

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### 10.4.2 Analysis of the Primary Efficacy Endpoint(s)

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### 10.4.3 Analysis of the Secondary Endpoint(s)

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### 10.4.4 Safety Analyses

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### 10.4.5 Adherence and Retention Analyses

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### 10.4.6 Baseline Descriptive Statistics

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### 10.4.7 Planned Interim Analyses

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#### 10.4.7.2 Efficacy Review

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#### 10.4.8 Additional Sub-Group Analyses

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### 10.4.9 Multiple Comparison/Multiplicity

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### 10.4.10 Tabulation of Individual Response Data

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### 10.4.11 Exploratory Analyses

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## 10.5 Sample Size

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## 10.6 Measures to Minimize Bias

### 10.6.1 Enrollment/ Randomization/ Masking Procedures

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### 10.6.2 Evaluation of Success of Blinding

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### 10.6.3 Breaking the Study Blind/Participant Code

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# 13 ETHICS/PROTECTION OF HUMAN SUBJECTS

## 13.1 Ethical Standard

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## 13.2 Institutional Review Board

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## 13.3 Informed Consent Process

### 13.3.1 Consent/assent and Other Informational Documents Provided to Participants

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### 13.3.2 Consent Procedures and Documentation

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## 13.4 Participant and data Confidentiality

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### 13.4.1 Research Use of Stored Human Samples,Specimens or Data

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## 13.5 Future Use of Stored Specimens

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# 14 DATA HANDLING AND RECORD KEEPING

## 14.1 Data Collection and Management Responsibilities

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## 14.2 Study Records Retention

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## 14.3 Protocol Deviations

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## 14.4 Publication and Data Sharing Policy

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# 15 STUDY ADMINISTRATION

## 15.1 Study Leadership

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# 16 CONFLICT OF INTEREST POLICY

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# 17 LITERATURE REFERENCES

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