Expedited IND Pilot Program

Sponsor Application for Pilot Participation

HHS Operation TrialBlazer

Part 1 of 3 — Sponsor Information

Section 1 Sponsor Identification

Sponsor Legal Name:

Sponsor DBA (if applicable):

Sponsor Address:

City, State, ZIP:

Sponsor Authorized Official Name & Title:

Sponsor Authorized Official Email:

Sponsor Authorized Official Phone:

Q1. Sponsor Organization Type *(select one)*

- ○ Academic institution / university
- ○ Non-profit research organization
- ○ Small / emerging biotech (fewer than 250 employees)
- ○ Mid-size pharmaceutical company (250–5,000 employees)
- ○ Large pharmaceutical company (more than 5,000 employees)
- ○ Other (please specify):

Q2. Prior US IND Experience *(select one)*

- ○ No prior IND submissions
- ○ 1–2 prior IND submissions
- ○ 3–10 prior IND submissions
- ○ More than 10 prior IND submissions

Q3. Prior FDA Engagement on this product *(select one)*

- ○ No prior FDA engagement on this product
- ○ Prior FDA engagement on this product *If selected, identify the type of engagement (e.g. INTERACT, pre-IND meeting, or WRO) and provide the date of the meeting or written response:*

Section 2 Sponsor IND Prioritization Characteristics

Sponsors should check all boxes that apply to the IND program under this pilot program application. Failure to confirm any item may result in exclusion from consideration in the pilot program.

The IND program identified in this application has the following characteristics:

- ☐ The product candidate is novel and will fall under the review jurisdiction of CDER or CBER.
- ☐ The IND is classified as a Commercial IND.
- ☐ The IND program targets a Phase 1 First-In-Human IND submission, and the investigational product does not have existing clinical experience.
- ☐ The intended IND submission is for a FIH Phase 1 clinical trial that will be run in the United States.
- ☐ The sponsor has sufficient nonclinical data at the time of application to the pilot program to support the FDA in evaluating the product's proposed development timeline and IND submission timeframe.
- ☐ The pilot will address the investigational product as defined in the application. Programs incorporating novel technologies or platforms are not excluded, but pilot support will be limited to the technology as incorporated into this specific IND, not to the platform or technology broadly.
- ☐ The sponsor commits to participating in the rolling IND submission model as defined by the Expedited IND Pilot Program.
- ☐ The sponsor commits to participating in post-pilot evaluation activities, including debriefs, surveys, and any structured data collection requested by FDA to assess pilot outcomes.

Section 3 Partnership Rationale

Describe the basis for the sponsor's selection of the named QRI and the value of this partnership for this specific IND program.

Q4. Sponsor Capability Gaps Addressed by the QRI *(select all that apply)*

- ☐ Nonclinical, including GLP toxicology operations, as applicable
- ☐ Nonclinical data standards and SEND dataset preparation, as applicable
- ☐ Phase 1 clinical site and IRB access
- ☐ CMC manufacturing and analytical methods
- ☐ IND regulatory strategy and authoring
- ☐ Integrated program management across disciplines
- ☐ FDA engagement and Pilot meeting support
- ☐ Electronic submission preparation and publishing (eCTD)
- ☐ Other (please specify):

Q5. Partnership Rationale Narrative *Required. Explain why this specific sponsor–QRI pairing represents a strong match and how the QRI's capabilities directly address the sponsor's needs for this IND program. Limit 1,500 characters.*

Part 2 of 3 — Investigational New Drug Program Information

Section 1 Program Overview

Investigational Drug Name or Code Name:

Proposed Therapeutic Indication:

Specific Disease or Condition: *e.g., ALS, glioblastoma, Duchenne muscular dystrophy*

Q1. Product Modality *(select one)*

- ○ Small molecule
- ○ Antibody or antibody constructs, including bispecific and monospecific products
- ○ Antibody-drug conjugate (ADC)
- ○ Protein therapeutic (non-antibody)
- ○ Peptide therapeutic (non-antibody)
- ○ Oligonucleotide (e.g., ASO, siRNA)
- ○ Autologous cell therapy (non-genetically modified)
- ○ Allogeneic cell therapy (non-genetically modified)
- ○ Gene therapy — viral vector (e.g., AAV, lentiviral, adenoviral)
- ○ Gene therapy — non-viral (e.g., mRNA/LNP, plasmid)
- ○ Gene therapy — ex vivo genetic modification of cells (e.g., CAR T, CAR NK, CD34+, genome edited)
- ○ Vaccine (prophylactic or therapeutic)
- ○ Blood product / blood-derived biologic
- ○ Other (please specify):

Q2. Mechanism of Action Classification *(select one)*

- ○ First-in-class / novel mechanism — e.g., no other known clinical programs directed at the intended molecular target(s)
- ○ Novel mechanism with prior investigational precedent (no approved pharmaceutical, but other clinical programs exist)
- ○ Well-characterized target and modality with at least one approved drug in the same class
- ○ Other (please specify):

Q3. FDA Designation Status *(select all that apply)*

- ☐ No FDA designation received or requested
- ☐ Orphan Drug Designation
- ☐ Rare Pediatric Disease Designation
- ☐ Other applicable FDA early development program (specify)

Attach copies of any received designation letters.

Section 2 Public Health Impact & Unmet Medical Need

Q4. Disease Severity *(select one)*

- ○ Cancer
- ○ Severely debilitating or life-threatening condition (SDLT) as defined under 21 CFR 312.81, other than cancer
- ○ Not cancer / not SDLT

Q5. U.S. Patient Population Size *(select one)*

- ○ Fewer than 100 patients in the U.S.
- ○ 100–1,000 patients in the U.S.
- ○ 1,001–10,000 patients in the U.S.
- ○ 10,001–100,000 patients in the U.S.
- ○ 100,001–200,000 patients in the U.SI.
- ○ More than 200,000 patients in the U.S.
- ○ Unknown / not yet estimated

Q6. Existing Treatment Landscape *(select one)*

- ○ No approved treatment, and no available therapy, for this indication
- ○ Limited approved options; significant unmet need remains
- ○ Multiple approved treatments exist, but meaningful gaps remain (e.g., resistance, tolerability, patient subgroup)
- ○ Adequate approved options exist; this program offers incremental improvement

Q7. Disease and Unmet Need Narrative *Required. Describe disease severity, chronicity, affected U.S. patient population, and the gap this program addresses. Reference any FDA designation(s) received. Limit 1,500 characters.*

Section 3 Drug Development Program Information

3A — Nonclinical & CMC Development Status

Not all nonclinical studies are conducted under GLP (Good Laboratory Practice, 21 CFR Part 58). GLP compliance is required for specific safety studies intended to support regulatory submissions. Exploratory, mechanistic, and characterization studies are typically conducted outside of GLP. Indicate completion status and compliance standard for each study type below.

Q8. Describe the status of your nonclinical development program, including key pharmacology, PK, and safety studies or assessments completed, in progress, and planned.

8A. Studies or Assessments in Process or Completed

8B. Planned Studies or Assessments

The questions below address the manufacturing and analytical development status of your investigational product. CGMP (Current Good Manufacturing Practice) standards govern the manufacturing, process and quality control of investigational drug products intended for use in clinical studies. Not all CGMP requirements otherwise applicable to drugs apply to early-investigational drugs used in Phase 1 clinical trials; however, any product intended for administration to human subjects must meet applicable GMP requirements prior to use. When responding to the questions below, indicate what standards you have applied in developing your manufacturing process and analytical controls.

Q9. Provide a brief description of the manufacturing process, including the expression system, cell line or production platform, and key process controls relevant to product safety.

Q10. Provide a brief overview of the proposed analytical control strategy and stability data you plan to have at submission.

3B — IND Status

Assess the overall status of the IND program across all three disciplines.

Q11. Development Timeline Narrative *Required. Summarize remaining development milestones and the credible basis for the target IND submission date. Note any attachment with a detailed milestone schedule. Limit 1,500 characters.*

3C —Draft General Investigational Plan

Responses in this section should reflect the sponsor's current development thinking and are understood to be subject to change as the program evolves. Reviewers will use these responses to assess program maturity, development rationale, and the scope of QRI support required across the full Year 1 program.

Q12. Scientific Rationale for the Drug and the Proposed Research *Required. Describe the scientific rationale for developing this drug for the proposed indication. Address the following: (1) the mechanism of action and its relationship to the disease pathophysiology; (2) the basis for believing the drug may be effective, including supporting nonclinical or clinical evidence; and (3) the early efficacy parameters or pharmacodynamic endpoints — informed by the mechanism of action — that will be used to assess biological activity or early signs of efficacy in the Phase 1 trial (e.g., target engagement biomarkers, PD readouts, surrogate endpoints). Limit 1,500 characters.*

Q13. Development Scope and Phase 1 Trial Population *Required. Briefly describe the overall development approach for this program, including: (1) the proposed indication(s) to be studied under this IND, including any planned expansion indications beyond the initial FIH cohort; (2) whether the program targets a single indication or multiple indications, and whether it is part of a broader platform program; and (3) the population to be enrolled in the Phase 1 trial (e.g., healthy adult volunteers, adult patients, pediatric patients, or a mixed population). If the trial spans multiple cohorts with different populations, describe the full scope. Limit 1,500 characters.*

Q14. Clinical Trials Planned in the First Year Following IND Submission *(select all that apply). Select all studies the sponsor anticipates initiating within the first 12 months following IND submission.*

- ☐ Phase 1 FIH study in healthy adult volunteers
- ☐ Phase 1 FIH study in patients with the target disease or condition
- ☐ Phase 1 FIH study – mixed cohorts (healthy volunteers and patients)
- ☐ Phase 1 food effect study
- ☐ Phase 1 drug-drug interaction (DDI) study
- ☐ Phase 1 special population study (e.g., renal or hepatic impairment)
- ☐ Phase 1 / 2 combined design
- ☐ Biomarker or pharmacodynamic substudy embedded within Phase 1
- ☐ No additional trials beyond the initial FIH study planned within the first year
- ☐ Other (please specify):

FIH Trial Design Summary *Required. Limit 1,500 characters. Provide a high-level description of the planned FIH trial design. Address the following: (1) whether the trial will enroll healthy volunteers, patients, or both, and the rationale for that choice; (2) the dose escalation approach (e.g., SAD only, SAD/MAD, accelerated titration, model-based Bayesian design such as BOIN or CRM); (3) whether the design is adaptive and, if so, the key decision rules; (4) primary safety and pharmacokinetic objectives; and (5) the anticipated number of cohorts, arms, and subjects. Note whether the trial will be conducted at a single center or multiple sites. Limit 1,500 characters.*

Part 3 of 3 — Authorized Signatures

By signing below, the authorized representatives of the Sponsor certify that: (1) all information contained in this application is accurate and complete to the best of their knowledge; and (2) the sponsor agrees to the terms of participation described in the Expedited IND Pilot Program webpage.

Sponsor Name:

Authorized Representative Name:

Title:

Signature:

Date: