DRAFT

U.S. FOOD AND DRU[illegible]TRATIC

EXPEDITED IND PILOT PROGRAM

Application for Pilot Participation

HHS Operation Trialblazer

Submit completed application to: ExpeditedINDPilot@fda.hhs.gov

Applications must be substantially complete. Sponsor and QRI must apply jointly.

Select all options that apply unless the question specifies "select one." | Where "Other" is available, use the space provided to specify.

Where a question references supporting documentation, note the attachment title in your response.

PART 1 OF 4 — 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 Ex[illegible] *(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
- ☐ If pre-IND meeting(s) held or WRO was issued, provide meeting or WRO date:

_____

SECTION 2 MANDATORY ELIGIBILITY CERTIFICATIONS

All items below must be certified. Failure to certify any item will result in automatic exclusion from further review.

☐ The IND program identified in this application meets the following eligibility criteria:

- The product candidate is novel and will fall under the review jurisdiction of CDER or CBER.
- The IND is classified as a Commercial IND, i.e. the sponsor's primary goal is to determine if the product is reasonably safe for initial use in humans, and if the compound exhibits pharmacological activity that justifies commercial development.
- The IND program targets a Phase 1 FIH IND submission.
- The sponsor has sufficient nonclinical data at the time of application to support Pilot Onboarding discussions and reach alignment on an appropriate nonclinical program to support the eventual FIH protocol.
- The pilot will address the investigational product as defined in this 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.

☐ Both the sponsor and QRI affirm compliance with all applicable FDA statutes and applicable law.

☐ Both the sponsor and QRI commit to completing conflict-of-interest (COI) screening within 3 business days of acceptance into the pilot and to maintaining documentation of that screening. FDA reserves the right to request COI screening records at any point during or after the pilot period.

☐ Both the sponsor and QRI commit to participating in the rolling IND submission model as defined by the Expedited IND Pilot Program, including adherence to the Pilot meeting cadence, Pilot Onboarding interaction, and submission milestones specified by the program.

☐ Both the sponsor and QRI commit 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. Basis for QRI Selection *(select all that apply)*

- ☐ QRI has relevant modality expertise for this program
- ☐ QRI has relevant therapeutic area expertise for this program
- ☐ QRI has an established institutional relationship with the sponsor

☐ QRI offers integrated nonclinical, clinical (including clinical pharmacology), and CMC capability under one partnership

☐ QRI partnership is expected to enable a faster IND submission timeline than sponsor could achieve independently

☐ QRI partnership is operationally essential — sponsor lacks internal resources for one or more disciplines

☐ QRI offers efficiencies with IRB and site activation capability critical to FIH timeline

☐ Other (please specify): _____

Q5. Sponsor Capability Gaps Addressed by the QRI *(select all that apply)*

☐ Nonclinical, including GLP toxicology operations, as applicable

☐ Phase 1 clinical site and IRB access

☐ CMC manufacturing and analytical

☐ IND regulatory strategy and authoring

☐ Integrated program management across disciplines

☐ FDA engagement and Pilot meeting support

☐ Electronic submission preparation

☐ Other (please specify): _____

Q6. 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.*

PART 2 OF 4 — DRUG / IND PROGRAM INFORMATION

SECTION 1 PROGRAM OVERVIEW

Investigational Drug 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, mRNA)
- ☐ Autologous cell therapy (non-genetically modified)
- ☐ Allogeneic cell therapy (non-genetically modified)
- ☐ Gene therapy — viral vector (AAV, lentiviral, adenoviral)
- ☐ Gene therapy — non-viral
- ☐ Gene therapy – ex vivo genetic modification of cells (CAR T, CAR NK, CD34+ GT)
- ☐ 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 approved product 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:	☐ Granted	☐ Planned	☐ Under FDA Review
Rare Pediatric Disease Designation:	☐ Granted	☐ Planned	☐ Under FDA Review

- ☐ 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 1,000 patients in the U.S. (ultra-rare)
- ☐ 1,000–10,000 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 1,000 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.

Q9. Provide a list of pharmacology, PK, and safety studies or assessments conducted and planned *(please specify)*

9A. Studies or Assessments in Process or Completed

9B. Planned Studies or Assessments

The questions below address the manufacturing and analytical development status of your investigational product. GMP (Good Manufacturing Practice) standards govern the manufacturing, processing, and quality control of investigational drug products intended for use in clinical studies. For drug substances and drug products, GMP requirements are described in 21 CFR Parts 210 and 211. For biological products, additional requirements may apply under 21 CFR Parts 600–680. Not all early-phase investigational materials are required to meet full GMP standards; however, any product intended for administration to human subjects must meet applicable GMP requirements prior to use. When responding to the questions below, indicate whether your manufacturing process and analytical controls have been developed under or in alignment with GMP standards.

Q11. 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.

Q12. 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.

Q13. 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 1,000 characters.*

3C — General Investigational Plan

The General Investigational Plan (GIP) is a required component of the IND under 21 CFR 312.23(a)(3)(iv). 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.

Q14. 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 1,000 characters.*

Q15. 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.*

Q16. 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.*

PART 3 OF 4 — QUALIFIED RESEARCH INSTITUTION (QRI)

SECTION 1 QRI IDENTIFICATION

QRI Legal Name:

_____

Principal Business Address:

_____

City, State, ZIP:

_____

Primary Contact Name & Title:

_____

Primary Contact Email:

_____

Primary Contact Phone:

_____

Name of Sponsor Organization (as listed in Part 1):

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Name of IND Program / Investigational Drug (as listed in Part 3): _____

Q1. QRI Institution Type *(select one)*

- ☐ Academic Medical Center (AMC)
- ☐ Health Network (HN)
- ☐ Contract Research Organization (CRO)
- ☐ Non-profit research institute
- ☐ Other (please specify): _____

SECTION 2 MANDATORY ELIGIBILITY CERTIFICATIONS

All items below must be certified by the QRI. Failure to certify any item will result in automatic exclusion.

☐ The QRI is a U.S.-registered legal entity with a principal place of business in the United States. The QRI's core program management, regulatory affairs, and scientific leadership functions are U.S.-based.

☐ The QRI commits to providing integrated development support across nonclinical, CMC, and clinical disciplines for the IND program identified in this application. This includes vetting and guiding the sponsor's nonclinical program, providing CMC regulatory oversight or direct manufacturing support as applicable, and supporting Phase 1 FIH clinical trial execution in the United States through owned infrastructure or formally executed and documented partnership arrangements.

☐ The QRI affirms compliance with all applicable FDA statutes and applicable law.

☐ The QRI commits to completing conflict-of-interest (COI) screening within 3 business days of engagement notification.

☐ The QRI commits to participating in the rolling IND submission model as defined by the Expedited IND Pilot Program, including adherence to the pre-IND meeting cadence specified by the program.

SECTION 3 QRI QUALIFICATIONS

Complete all subsections. Attach supporting documentation and note attachment titles in relevant narrative fields.

3A — Nonclinical Leadership & Personnel

Q2. Nonclinical Leadership Credentials *(select all that apply)*

- ☐ Diplomate of the American Board of Toxicology (DABT)
- ☐ Ph.D. in pharmacology, toxicology, or a related discipline, or the equivalent demonstrated expertise through regulatory toxicology practice
- ☐ Prior pharmacology/toxicology experience as an FDA reviewer, contractor, or in industry
- ☐ ICH working group participant (e.g., S7A, S7B, M3, S9)
- ☐ Published expertise in FIH dose selection methodology
- ☐ Other (please specify) _____

Q3. Number of FIH INDs Previously Supported as a Partner to Sponsors *(select one)*

- ☐ None
- ☐ 1–5

- ☐ 6–15
- ☐ More than 15

Q4. Nonclinical Personnel Narrative *Required. Describe key nonclinical personnel, FIH IND experience, starting dose calculation expertise, and fit-for-purpose study design approach. Note attached CVs by name.*

3B — CMC Leadership & Personnel

Q5. CMC Leadership Credentials *(select all that apply)*

- ☐ Ph.D. in pharmaceutical sciences, analytical chemistry, or related discipline
- ☐ Prior industry CMC experience (pharmaceutical or biotech)
- ☐ Prior FDA CMC inspection experience
- ☐ CMC regulatory submission experience (IND CMC section authoring)
- ☐ Technology transfer and manufacturing scale-up expertise
- ☐ Process development and validation experience

Q6. CMC Personnel Narrative *Required. Describe key CMC personnel, regulatory submission experience, inspection history, and phase-appropriate GMP compliance expertise. Note attached CVs by name.*

3C — Clinical Leadership & Personnel

Q7. Clinical Leadership Credentials *(select all that apply)*

- ☐ Board-certified physician with subspecialty expertise in the relevant therapeutic area
- ☐ Extensive Phase 1 protocol design experience (dose escalation, safety monitoring)
- ☐ QTc / cardiac safety assessment expertise (ICH E14)
- ☐ Drug-drug interaction (DDI) study design experience
- ☐ Experience with adaptive trial designs (BOIN, mTPI, CRM)
- ☐ Active participation in relevant clinical research consortia or networks

☐ Prior FDA review or contractor experience

Q8. Number of FIH Trials as Principal Investigator or Clinical Lead *(select one)*

☐ None
☐ 1–5
☐ 6–15
☐ More than 15

Q9. Clinical Personnel Narrative *Required. Describe key clinical personnel, FIH experience, therapeutic area expertise, and clinical leadership qualifications. Note attached CVs by name.*

[Instruction to add CVs]

3D — Clinical Partnerships & Capabilities

Q10. Phase 1 Clinical Infrastructure *(select all that apply)*

☐ Active P1 CRU within parent organization
☐ Access to, or owned Phase 1 Clinical Research Unit (CRU) — inpatient beds
☐ Access to, or owned Phase 1 outpatient / ambulatory research clinic
☐ Phase 1 capability via documented site partnership
☐ Institutional IRB (owned)
☐ Reliance on external / central IRB
☐ Master Clinical Trial Agreement (MCTA) templates in place
☐ AI-enabled patient matching or EMR-to-EDC integration tools
☐ Other (please specify): _____

Attach executed site partnership agreements, if applicable.

Q11. Number of Phase 1 Trials Conducted or supported *(select one)*

☐ None
☐ 1–5
☐ 6–20
☐ More than 20

Q12. IRB First-Review Average Timeline: *e.g., 30 days from submission*

_____

Q13. Average Time-to-Contract (term sheet to executed agreement): *e.g., 45 days*

_____

Q14. Clinical Capabilities Narrative *Required. Describe Phase 1 CRU infrastructure, IRB capability, contracting mechanisms, and relevant clinical trial portfolio. Note any attached Phase 1 CRU description.*

3E — Nonclinical and CMC Capabilities & Infrastructure

Q22. Nonclinical and CMC Capabilities Narrative *Required. Describe the depth and breadth of nonclinical infrastructure, cGMP manufacturing infrastructure, analytical capability, QA/QC systems, and any partnership arrangements that the QRI plans to utilize on behalf of the sponsor's IND submission. Note any attached CMC facility description or inspection records.*

3F — Cross-Cutting Track Record & Integration

Q25. Modalities for Which the QRI Has Supported IND Submissions *(select all that apply)*

- ☐ 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, mRNA)
- ☐ Autologous cell therapy
- ☐ Allogeneic cell therapy
- ☐ Gene therapy — viral vector (AAV, lentiviral, adenoviral)
- ☐ Gene therapy — non-viral
- ☐ Vaccine (prophylactic or therapeutic)
- ☐ Blood product / blood-derived biologic
- ☐ None
- ☐ Other (please specify): _____

Q26. Total IND Submissions Supported Across All Modalities *(select one)*

- ☐ None
- ☐ 1–5
- ☐ 6–15
- ☐ 16–30
- ☐ More than 30

Q27a. Additional Sponsor Support Capabilities *Optional but encouraged. Describe any capabilities the QRI offers in support of sponsor IND development that are not captured in the sections above. This may include, but is not limited to, regulatory affairs expertise, biostatistics and clinical pharmacology support, patient advocacy engagement, health economics and outcomes research (HEOR), translational science, or any other service the QRI considers a meaningful differentiator in supporting FIH IND programs.*

If regulatory affairs expertise is described here, specify whether this capability is provided by dedicated in-house personnel, through a documented external partnership, or both.

Q28. Track Record & Integration Narrative *Required. Describe experience integrating nonclinical, clinical, and CMC development planning; highlight IND submission successes and FDA feedback incorporation examples. Note any attached program list or track record summary.*

PART 4 of 4 — AUTHORIZED SIGNATURES & ATTACHMENTS

By signing below, the authorized representatives of the Sponsor and the Qualified Research Institution jointly certify that: (1) all information contained in this application is accurate and complete to the best of their knowledge; (2) both organizations satisfy all mandatory eligibility requirements set forth in Section 2; and (3) both organizations commit to the terms of participation in the Expedited IND Pilot Program as described in applicable program guidance.

SPONSOR

_____

Authorized Representative Name:

QUALIFIED RESEARCH INSTITUTION (QRI)

_____

Authorized Representative Name:

DRAFT

Title:

Organization:

Signature:

Date:

Title:

Organization:

Signature:

Date:

ATTACHMENTS

List all documents attached to this application in the table below. Assign each attachment a short title and a sequential number (e.g., Attachment 1, Attachment 2). Where attachments provide additional detail in support of a specific question or narrative in this application, note the attachment title or number in that question's response. Reviewers will cross-reference attachments against the responses in which they are cited.

Only documents listed here and physically included with the submission will be considered during review. Do not reference documents that are not attached. There is no prescribed list of required attachments — include what is relevant and cited in your responses.

Attachment #	Title / Description	Cited in Question(s)
1		
2		
3		
4		
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Add rows as needed. Do not omit any document referenced in the application body.