The Somatic Cell Genome Editing (SCGE) Program Phase 2 pre-application webinar

May 4, 2022
2:00-3:30PM ET

National Institutes of Health
Office of Strategic Coordination–The Common Fund

Webinar Housekeeping

- All participants will be muted
- This webinar will be recorded for internal use. A recording of the webinar will not be available; however, slides will be posted after the webinar at https://commonfund.nih.gov/editing
- Ask questions during the webinar in the Q&A box or by emailing scgeprogram@od.nih.gov
- A list of frequently asked questions will be posted and periodically updated at https://commonfund.nih.gov/editing

Agenda

- Introduction to the NIH Common Fund
- Somatic Cell Genome Editing Program Overview: Phase 1 & Phase 2
- Overview of Funding Opportunity Announcements
  - Technologies and Assays for Therapeutic Genome Editing INDs (RFA-RM-22-014)
  - IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)
  - Platform Clinical Trials of Genome Editors in Multiple Diseases (RFA-RM-22-016)
  - Somatic Cell Genome Editing Program Translational Coordination and Dissemination Center (TCDC) (RFA-RM-22-017)
- Cooperative Agreement Overview
- Frequently Asked Questions
- Q&A session

The Common Fund Moves the NIH Mission Forward – Faster

- Supports **bold, five-year, goal-driven** scientific programs that **catalyze** biomedical research
- **Complements the missions** of the NIH Institutes and Centers
- Addresses **emerging opportunities and challenges** that **no single IC** can address on its own
- Is supported by the Office of the NIH Director and managed in **partnership with the Institutes and Centers**

SCGE Program Overview Phase 1 & 2

Timothy LaVaute, Ph.D.

Program Director

Division of Neuroscience

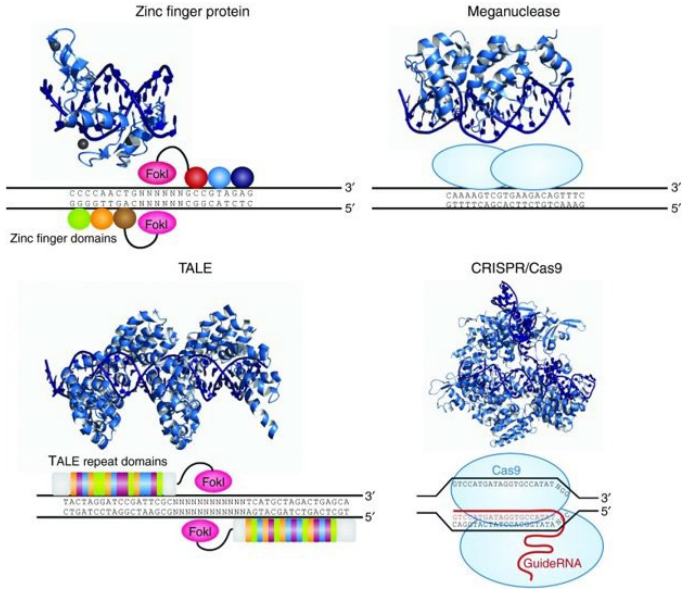
National Institute of Neurological Disorders and Stroke (NINDS)

National Institutes of Health

Office of Strategic Coordination–The Common Fund

SCGE Program Background

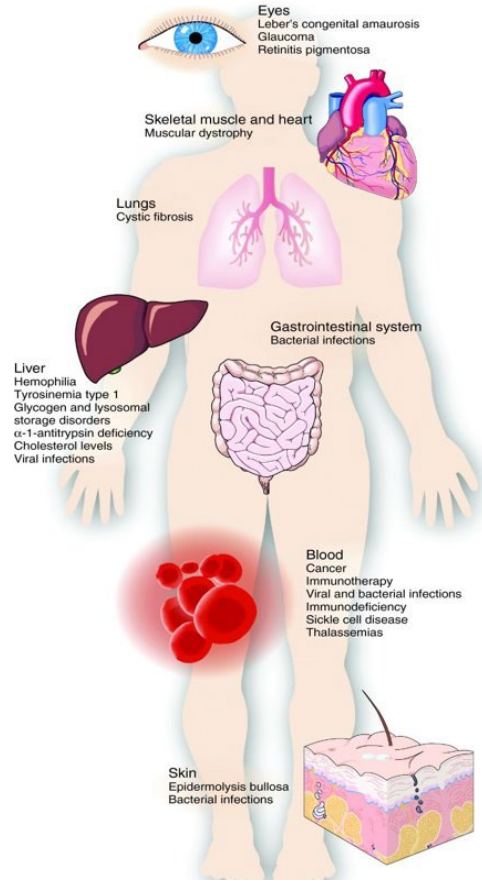
- Genome editing allows precise corrections to be made in patients' DNA and RNA
- CRISPR-cas9 catalyzed development of experimental genome editing therapeutics
- Thousands of genetic diseases are amenable to targeted *in vivo* genome editing approaches



Gaps and Opportunities from 2017 Common Fund Planning Workshop

- **Improved animal models to detect editing**
- **Human cell systems for measuring adverse events**
- **Delivery systems for *in vivo* targeting**
- **Methods to track edited cells *in vivo***
- **Safer and more effective editors**

SCGE Phase 1 addressed these gaps



SCGE Phase 2 - Planning Activities

Workshop with:
20 subject matter experts from academic, government and industry - April 20, 2021
https://commonfund.nih.gov/editing/meetings

- Session 1: Clinical Trial and Regulatory Innovation
- Session 2: Prospects for Advancing *In Utero* SCGE
- Session 3: Gaps and Opportunities in Basic Development and Discovery
- Session 4: IND-Enabling Preclinical Tools
- Session 5: Issues in Immunogenicity

Consultations with:

- SCGE Phase 1 Program Consultants
- FDA Center for Biologics Evaluation and Research staff
- NIH Leaders of translational programs for genome-based therapies (NINDS's CREATE-Bio and URGenT; NHLBI's Catalyze)
- DARPA Program Manager for PREPARE

Environmental scan of:

- *In vivo* genome editing therapeutics in clinical trials
- Industry genome editing pipelines
- NIH genome editing therapeutics portfolio

SCGE Phase 2 - Translating *in vivo* Genome Editing Therapies into the Clinic More Broadly & Efficiently

Objective: To accelerate the development of genome-editing therapeutic agents by facilitating IND-enabling studies, establishing pathways to regulatory approval, and disseminating successful strategies for initiating first in human clinical trials.

Phase 2 Initiatives:

1. Development of technology/assays that support IND-submissions
2. Optimizing genome editing-based therapeutic leads for safety and efficacy
3. Platform clinical trials initiative using genome editing therapeutics for more than one disease
4. Coordination and Dissemination Center to foster collaboration and share new technologies and protocols with the public and research community

Program Duration: 5 years, FY23-27

Estimated Total Budget: $227M

SCGE Phase 2 – Funding Opportunity Announcements

- Technologies and Assays for Therapeutic Genome Editing INDs (RFA-RM-22-014)
- IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)
- Platform Clinical Trials for Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)
- Somatic Cell Genome Editing Translational Coordination and Dissemination Center (RFA-RM-22-017)

Key Dates:

Letter of Intent	Application Due Dates	Review and Award Cycles		
New	New	Scientific Merit Review	Advisory Council Review	Earliest Start Date
June 17, 2022	July 19, 2022	November 2022	January 2023	April 2023

SCGE Phase 2 - Request for Information

Request for Information (RFI):

Inviting Comments and Suggestions on the Potential Development of a Challenge Prize for Transformative Genome Editor Delivery Technologies (NOT-RM-22-013)

https://grants.nih.gov/grants/guide/notice-files/NOT-RM-22-013.html

Response Date: June 17, 2022

Send responses to: SCGEprogram@od.nih.gov

Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Betty Poon, Ph.D.

Program Officer

Division of AIDS

National Institute of Allergy and Infectious Diseases (NIAID)

National Institutes of Health
Office of Strategic Coordination–The Common Fund

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Research Objectives:

To support the optimization of IND-enabling technologies and assays to help accelerate the clinical development and evaluation of novel genome editing therapeutics to treat a broad array of rare and common diseases

- Examples of technologies and assays that would be included under this initiative are those for chemistry, manufacturing and controls (CMC), potency, pharmacology/toxicity, detection and measurement of on/off-target effects, immune responses, and cell tracking studies.
- Applicants should have an IND-enabling technology or assay to be optimized, with supportive preliminary data, at the time of submission.
- Projects should focus on further development and rigorous characterization of the technology and/or assay for utilization and adoption in regulatory submissions.
- This FOA is intended to bring assays to the point where they could be integrated with future clinical trials/studies.

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Research Scope

- In INDs submitted to the FDA, sufficient CMC information should be provided to assure safety, identity, quality, purity, and strength (including potency) of the investigational product entering clinical trials.
- CMC activities include the establishment of manufacturing processes and product characteristics, as well as defining product testing methods to ensure that the product is safe, effective, and consistent between batches. To guide the CMC development plan, it is important to establish the Critical Quality Attributes (CQAs), a set of criteria to which a drug product should conform to be considered acceptable for its intended use.
- This RFA will support the optimization, refinement, and establishment of acceptability criteria of technologies and assays that will provide data on the efficacy and safety of genome editing technologies and delivery systems in future regulatory submissions.
- Technologies that can be broadly applicable to more than one genome editing therapeutic product and/or indication are encouraged

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Research Scope (cont'd)

Examples of product and process characterization assays supported by this FOA include, but are not limited to:

- Technologies that enable more informative assessment of patient adaptive and/or innate immune (immunogenicity) responses to genome editors and vectors during clinical trials, including whether those responses change over time or in response to redosing
- In vitro and in vivo assays for clinically relevant evaluation of the pharmacokinetic and pharmacodynamic properties of a genome delivery or editing reagent, including bioavailability, bioactivity, cell/tissue specificity, and/or dose-prediction in clinical trials
- Toxicological assays applicable for genome editing safety, for example assessment of undesired off-target effects including cytotoxicity, genotoxicity, mutagenicity and tumorigenicity potential
- Potency assays to assess specificity and sensitivity measurements of the functionality and efficiency of genome editing product, including vector infectivity and identity, editor activity, and other parameters as appropriate
- Process development technologies for scale-up and cGMP manufacturing of genome editing products
- Bioanalytical methods for final product identity and potential contamination
- Technologies for tracking and monitoring of genome editing therapies in vivo, which may include amongst others, in utero therapeutic products

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Research Scope (cont'd)

Applications addressing the following topics will be deemed non-responsive and will not be reviewed:

- Exploratory research for new technology development that lack supporting, unpublished, and/or preliminary data
- Assays that are not applicable to genome editing INDs
- Discovery or development of new genome editing therapeutic products
- Projects proposing clinical trials

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Section IV: FOA-specific instructions to be included in the Research Plan:

- Provide a rationale for the choice of methodology and/or assay, including how the technology will be used to support IND applications of genome therapeutic products, and which CMC requirement(s) the improved technology is intended to fulfill.
- Describe the overall strategy and analytical approaches for optimization and refinement of the methodology and/or assay, and provide quantitative benchmarks to support the proposed approaches.
- Discuss how the outcome will inform the establishment of CQAs to meet quality requirements for a genome editing therapeutic product.
- Provide a plan for assay replication and validation, using samples from relevant animal experiments or from human clinical studies within and without the project, as appropriate.

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Application Review Information Specific to this FOA

- Is the choice of methodology and/or assay well-reasoned, and how strong is the explanation for the use of the technology to support IND applications of genome therapeutic products? Are the overall strategy and analytical approaches adequately developed, and appropriate to lead to an outcome that will inform the establishment of CQAs to meet quality requirements for genome editing therapeutic products? Are the plans for assay replication and validation adequate and sufficient to establish the utility of the platform(s)?

SCGE Phase 2 – Technologies and Assays for Therapeutic Genome Editing Investigational New Drugs (INDs) (RFA-RM-22-014)

Anticipated Budget and Timelines

Milestones: A Gantt chart with milestones is required for all studies. Milestones should be scientifically justified, well defined for each year of the project, and based on the proposed specific aims. The milestones section should describe how the applicants will achieve the below objectives during the period of the award

- Complete studies to establish an assay profile and performance criteria of sufficient quality to support IND-submission of genome editing therapeutic products
- Perform assay optimization and further define analytical parameters using relevant samples, including samples from other consortium members as scientifically appropriate.
- Encouraged to collaborate with other consortium members in evaluating assay performance

Anticipated awards**:** four 3-year U01 Cooperative Agreements

Estimated funds available: $2,000,000 per year (FY23-25).

IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)

Chris Boshoff, Ph.D.

Scientific Program Manager

Division of Translational Research

National Institute of Neurological Disorders and Stroke (NINDS)

National Institutes of Health

Office of Strategic Coordination–The Common Fund

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) Multi-Component U19 Program

Research Objectives:

- To facilitate further characterization, optimization, and development of genome editing-based therapeutic lead(s) that show promise for therapeutic development as evidenced by relevant, rigorous, convincing preliminary in vitro and/or in vivo data.
- To support activities such as lead selection and optimization, manufacturability, biodistribution, in vivo efficacy, optimal dosing combined with other properties consistent with the intended clinical application.

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) Multi-Component U19 Program

Research Scope:

- Identification and optimization of genome editing therapeutic lead(s):
  - Improve in vivo editing efficiency, pharmacokinetic/pharmacodynamic (PK/PD) relationship, biodistribution, dose range, and safety profiles such as off-target effects and/or immune responses for the intended route of administration.
- Optimization of delivery system(s), process development, Chemistry, Manufacturing, and Control (CMC) related activities:
  - CMC analytical development, final formulation development, scale-up manufacturing or cGMP manufacturing) intended for IND-enabling safety/toxicology studies.
- Development of a regulatory strategy:
  - Stage-appropriate interactions (INTERACT and/or pre-IND meetings) followed by IND submissions to FDA.

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) Multi-Component U19 Program

Distinguishing Character of a U19 Program

- Multi-component Structure
  - Overall Component
  - Administrative Core
  - Resource Core(s)
  - Research Projects
- Integrated contribution of Research Projects and Cores to the overall aims of the U19 Program
  - Interdisciplinary nature and synergy facilitated across Research Projects and Cores

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) Multi-Component U19 Program

Elements of a Successful U19 Program:

- Clear set of overall goals that are aligned with the SCGE objectives and outcomes
- Coordination with other U19 Programs and the Translational Coordination and Dissemination Center (TCDC) (U24) in this SCGE Consortium
- Multidisciplinary team science: Evidence it has collective expertise AND can work together

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) Individual U19 Program Components

Component	Component Type for Submission	Page Limit	Required/ Optional	Minimum	Maximum
Overall	Overall	12	Required	1	1
Administrative Core	Admin Core	6	Required	1	1
Resource Core(s)	Core	6	Required	1	3
Research Projects	Project	12	Required	3	5

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) U19 Application: Overall Component

Establish the central scientific, data, and resource production goals of the U19 Program

- Outlines the integration and interrelated nature of research components

Rationale and brief research approach/plan for individual research projects and core(s)

- Interdisciplinary nature, synergy and integration across Research Projects and Core(s)
- Unique contribution of Research Projects and Core(s) to the overall aims of the U19 Program

Multidisciplinary Team

- Integration of expertise for proposed approaches and fostering team science

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)
U19 Application: Administrative Core

The Administrative Core is expected to have effective administrative and organizational capabilities to support multidisciplinary research to foster synergy and support planning and evaluation activities.

Contribution of the Administrative Core to the U19 Program:

- Database infrastructure, information management & monitoring, complex multimodality data management, statistical analysis, data integration
- Bioinformatics expertise, data integration & analysis support, study design & statistical support/services

Data Management and Coordination with U24 Center

- Support and coordinate data for Research Projects and Cores in the U19 Program
- Coordinate with other U19 Programs, and with the Translational Coordination and Dissemination Center (TCDC)

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) U19 Application: Resource Core(s)

The resource core(s) will be a point of contact for dissemination of technology, expertise, and/or materials to at least two U19 Projects

Contribution to Research Projects of the U19 Program

- Broad activities and services of the proposed Core
- Should not duplicate resources already available at the institution

Facilities, services and/or types of resources provided and managed

- How they will meet the specific needs of each Research Project
- Rationale for centralizing activities in Resource Core rather than including in Research Projects

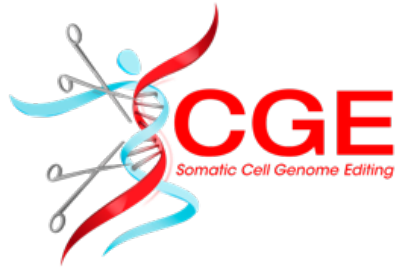
Core objectives - to improve technologies, tools, and/or reagents

- Overcome limitations and gaps of the current technologies and tools in throughput, sensitivity, selectivity, scalability, etc.

Team

- Expertise for proposed approaches, facilities and services to be provided
- Resource Core coordination and management plan

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015) U19 Application: Research Projects

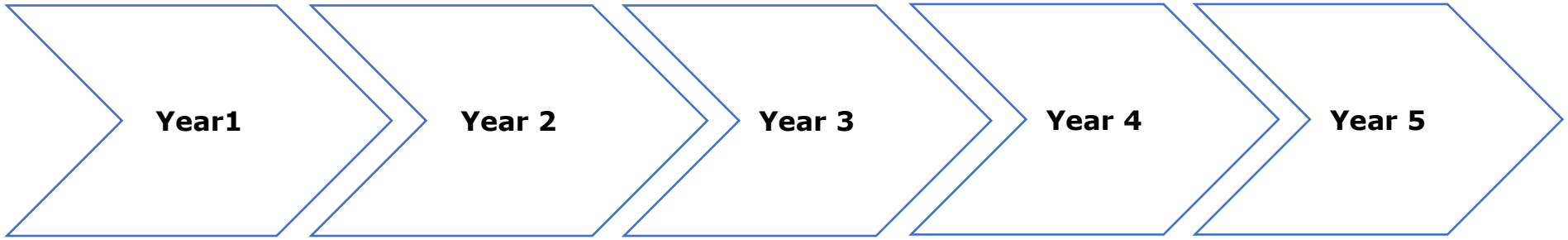


- Each U19 application must propose a minimum of 3 and maximum of 5 distinct Projects.
- All proposed Projects should target the same tissue or cell type and collectively represent an overarching strategy to establish a synergistic *in vivo* genome editing therapeutic approach.
- At least one Project should be identified as the lead or "trailblazer" Project that is poised to advance a therapeutic clinical candidate to an IND package submission within five years.
- The other proposed "follower" Projects may enter at earlier stages of development, e.g., lead selection, but all Projects should be on a clear path proposing progressive activities expected to culminate in identifying a clinical candidate within the five years of funding support.
- Clear contribution of research Projects to the goals of U19 Program and goals of the SCGE Consortium
- Provide rationale and detailed research approach/plan and budget for each individual research Project

SCGE Phase 2 – IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)

Anticipated Budget and Timelines

- All specific aims should include quantifiable milestones for each Project year
- Milestones are a basis for Go/No-Go Criteria to advance to subsequent years
- Each Project proposes a Target Product Profile (TPP)
- Research strategy should include the entire scope of each proposed Project



Anticipated awards: five 5-year Cooperative Agreements

Estimated funds: $25,000,000 per year (FY23-27)

Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

P.J. Brooks, Ph.D.

Program Coordinator
Office of Rare Diseases Research
National Center for Advancing Translational Sciences (NCATS)

National Institutes of Health
Office of Strategic Coordination–The Common Fund

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Research Objectives:

- **Goal**: to support novel genome editing clinical trials that include at least two different diseases, using the same genome editor, route of administration, and delivery system.
  - Two-phase, milestone-driven projects consisting of an IND enabling studies phase (UG3) and a pragmatic or implementation trial execution phase (UH3). Milestones are required for both phases in the application.
  - Obtaining regulatory approval from the FDA to proceed with the clinical trial of more than one disease is the main criterion for transitioning between phases

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Research Scope:

- **UG3 Phase:** This phase, which can be up to 3 years, is intended to support IND-enabling studies, including translational bench/in vitro, and animal studies as necessary, to support the preparation and submission of the IND. Regulatory meetings with the FDA will be required as milestones during the UG3 phase.
  - The proposed clinical trials must utilize the same genome editor, route of administration, and in vivo delivery system for both diseases.
  - Any genome editor, including but not limited to those that create a double-strand break, base editors, prime editors, RNA editors, or epigenome editors, can be chosen for use.
  - Collaborations with commercial entities that are developing genome editing therapeutics are encouraged.
  - Applications should include plans to proactively confront potential delays or risks in meeting the milestones .
  - Applications must include a milestone plan for both the UG3 and UH3 phases.

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Research Scope Cont'd:

- **UH3 Phase:** If the transition is made, the UH3 phase of the cooperative agreement will also support the subsequent small clinical trial(s), which must involve at least two different diseases.
  - Sufficient detail should be provided to allow for effective review of the proposed activities to be performed during the UH3 phase.
  - Finalized clinical trial protocols, registration of the clinical trial in ClinicalTrials.gov, completion of regulatory approvals, information on enrollment of the first subjects, and contingency plans for continued clinical observation of patients beyond the UH3 phase, will be required with the submission of the UH3 phase non-competing application to NIH.
  - Milestones and timelines for the UH3 phase are required at the time of application but may be revised at the time of the UG3/UH3 transition. Less than satisfactory progress in the UH3 phase may lead to phasing out the award.
  - In preparing applications to this FOA, investigators should be aware of and plan to address all requirements for gene editing clinical trials as identified in the relevant FDA Guidance documents (see https://www.fda.gov/vaccines-blood-biologics/biologics-guidances/cellular-gene-therapy-guidances).

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Application Review Information Specific to this FOA

Investigator(s):

- Does the Clinical Trial Experience attachment demonstrate strong expertise of the personnel to conduct the proposed trial?
- Are the roles/responsibilities of the Project Manager and other key personnel adequate to monitor study progress and identify/manage risks?
- Is the project management expertise strongly represented among the key personnel?
- With regard to the proposed leadership for the project, do the PD/PI(s) and key personnel have the expertise, experience, and ability to organize, manage and implement the proposed clinical trial and meet milestones and timelines? Do they have appropriate expertise in study coordination, data management and statistics? For a multicenter trial, is the organizational structure appropriate and does the application identify a core of potential center investigators and staffing for a coordinating center?

Approach:

- Is the timeline feasible to complete all requirements for obtaining an IND(s) for the proposed clinical trial(s) by the end of the UG3 phase (3-year maximum), as described in the milestone plan?

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Application Review Information Specific to this FOA cont'd

Approach:

Does the application adequately address the following, if applicable

Study Design

- Is the study design justified and appropriate to address primary and secondary outcome variable(s)/endpoints that will be clear, informative and relevant to the hypothesis being tested? Is the scientific rationale/premise of the study based on previously well-designed preclinical and/or clinical research? Given the methods used to assign participants and deliver interventions, is the study design adequately powered to answer the research question(s), test the proposed hypothesis/hypotheses, and provide interpretable results? Is the trial appropriately designed to conduct the research efficiently? Are the study populations (size, gender, age, demographic group), proposed intervention arms/dose, and duration of the trial, appropriate and well justified?
- Are potential ethical issues adequately addressed? Is the process for obtaining informed consent or assent appropriate? Is the eligible population available? Are the plans for recruitment outreach, enrollment, retention, handling dropouts, missed visits, and losses to follow-up appropriate to ensure robust data collection? Are the planned recruitment timelines feasible and is the plan to monitor accrual adequate? Has the need for randomization (or not), masking (if appropriate), controls, and inclusion/exclusion criteria been addressed? Are differences addressed, if applicable, in the intervention effect due to sex/gender and race/ethnicity?

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Application Review Information Specific to this FOA cont'd

Approach:

- Are the plans to standardize, assure quality of, and monitor adherence to, the trial protocol and data collection or distribution guidelines appropriate? Is there a plan to obtain required study agent(s)? Does the application propose to use existing available resources, as applicable?

Data Management and Statistical Analysis

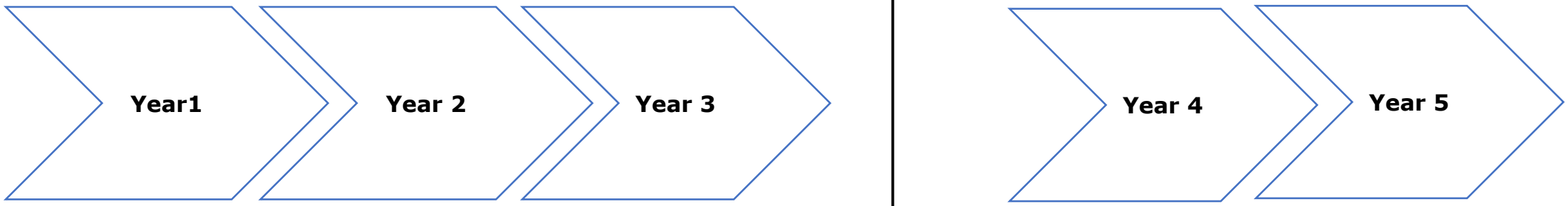
- Are planned analyses and statistical approach appropriate for the proposed study design and methods used to assign participants and deliver interventions? Are the procedures for data management and quality control of data adequate at clinical site(s) or at center laboratories, as applicable? Have the methods for standardization of procedures for data management to assess the effect of the intervention and quality control been addressed? Is there a plan to complete data analysis within the proposed period of the award?

SCGE Phase 2 – Platform Clinical Trials of Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)

Anticipated Budget and Timelines

- INTERACT and Pre-IND meetings with FDA
- IND-enabling studies
- IND approval by the FDA

- Small clinical trials involving at least two diseases



UG3 Phase

UH3 Phase

Anticipated awards: two cooperative agreements totaling 5 years; Preclinical UG3 phase of up to 3 years, followed by clinical UH3 phase

Estimated Budget: $10M in FY23, $20M in FY24-25, and $12M in FY26-27

Translational Coordination and Dissemination Center (RFA-RM-22-017)

Marrah Lachowicz-Scroggins, Ph.D.

Program Director
Division of Lung Diseases
National Heart, Lung, and Blood Institute (NHLBI)

National Institutes of Health
Office of Strategic Coordination–The Common Fund

SCGE Phase 2 – Translational Coordination and Dissemination Center (RFA-RM-22-017)

Research Objectives:

- **Goals**:
  - (1) Lead consortium-wide activities that facilitate intra-consortium collaborations and support broad dissemination strategies for regulatory submission;
  - (2) Develop a publicly available online platform for data collection and dissemination of consortium-wide activities including data generated by SCGE in Phase I

In partnership with the SCGE Consortium, the Translational Coordination and Dissemination Center will:

- Provide national leadership and technical expertise in all aspects of therapeutic genome editing.
- Lead the design, development and execution of a high impact online platform that will facilitate data sharing and best practices for translation of genome editing therapeutics.
- Make available data, tools, technologies, and protocols from projects across the Consortium to accelerate the use of genome editing therapeutics in the clinic.
- Manage consortium-wide education and outreach activities.

SCGE Phase 2 – Translational Coordination and Dissemination Center (RFA-RM-22-017)

Research Scope:

Specific roles and activities of the TCDC include, but are not limited to:

1. Consortium Coordination:
2. Oversight of the SCGE Program Steering Committee, including subcommittees and working groups
    - Maintain consortium-wide communication/dissemination channels including supporting policies and procedures for best practices for activities across the Program
    - Support emerging education and training needs of the Consortium, including hosting a "Meet the Expert" series covering topics such as regulatory, IND development, clinical trials, etc.
3. Design, curation and dissemination of the SCGE Phase II Platform, including stewardship of the Phase I data.
    - Serve as the main resource for data acquisition, presentation and preservation expertise for the SCGE Program
    - Developing platform provides clear and easy management and retrieval of data, tools and protocols from the SCGE Program to disseminate findings to broader audiences in a manner that is accessible to the general scientific community across a variety of platforms
4. Collaboration support
    - Coordinating efforts across and between SCGE components
    - Facilitating multi-directional interactions between the Consortium and appropriate external research and/or community organizations

SCGE Phase 2 – Translational Coordination and Dissemination Center (RFA-RM-22-017)

Application Review Information Specific to this FOA

Significance

- How strong are the plans for the TCDC proposed activities to meet those needs? What is the likelihood that successful the completion of the proposed aims will help the SCGE Consortium advance genome editing technologies into clinical practice?

Investigator(s)

- Will the team be able to contribute unique expertise and perspectives to the overall SCGE Program goals? Is the expertise well described for successful coordination, data management and facilitation of Program collaborations? Do study personnel have experience with complex data presentation in meaningful ways, experience in web-interface design and experience in collaborative research with a variety of stakeholders?

Innovation:

- Does the application demonstrate that the proposed activities will challenge and seek to shift current research or clinical practice paradigms, national policies and practices to advance genome editing technologies by utilizing novel theoretical concepts, approaches, methodologies, interventions, or tools? Will the proposed activities advance and seek to impact current data management and research implementation strategies by utilizing novel theoretical concepts, approaches or methodologies, or tools? Are the plans adequate for leveraging novel collaboration and communication strategies? Does the proposed application indicate creativity and flexibility to innovate on an ongoing basis?

SCGE Phase 2 – Translational Coordination and Dissemination Center (RFA-RM-22-017)

Application Review Information Specific to this FOA Cont'd

Innovation:

- Will the proposed design of the SCGE Phase II Platform contain innovative and novel strategies for integrating and curating critical resources developed by the SCGE Consortium? Are novel dissemination strategies proposed?

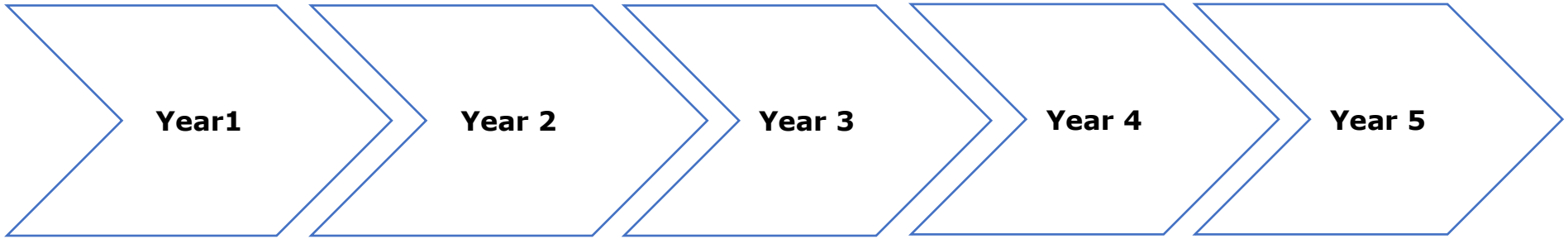
Approach:

- Are the plans well described for the proposed design of the SCGE Phase II Platform? Are the plans feasible for sustaining the SCGE Phase I and Phase II data beyond the SCGE Program funding period? How effective are the dissemination strategies proposed? Is the proposed timeline feasible for launch and completion of the SCGE Phase II Platform?

SCGE Phase 2 – Translational Coordination and Dissemination Center (RFA-RM-22-017)

Anticipated Budget, Timeline and Milestones

- Define operating procedures and policies
- Coordinate consortium activities, including educational activities
- Outreach and dissemination to broader biomedical research community
- Development of the data sharing platform



Protype of Phase II platform

Complete usability testing and begin launch of Phase II platform

Complete QC, finalize data curation for Phase II platform

Anticipated award: one 5-year Cooperative Agreement

Estimated Funds: $2,000,000 per year (FY23-27)

Cooperative Agreement Overview

Felicia Qashu, Ph.D.

Program Leader
Office of Strategic Coordination
Division of Program Coordination, Planning, and Strategic Initiatives
Office of the Director
National Institutes of Health (NIH)

SCGE Phase 2 – Important details

Awards funded via Cooperative Agreement → greater involvement from NIH Program Staff

- Final milestone plan will be negotiated and agreed to before award
- NIH Program staff will periodically assess progress toward achieving the milestones

SCGE program components will form a consortium governed by a Steering Committee of principal investigators and NIH staff

- Develop consensus policies and procedures for Consortium-wide activities such as data and resource sharing
- Coordinate the activities being conducted by the program

Frequently Asked Questions

National Institutes of Health
Office of Strategic Coordination–The Common Fund

SCGE Phase 2 – Frequently Asked Questions

Will a recording of the applicant webinar be posted online?
No. However, webinar slides will be available. A list of common FAQs will be updated to reflect questions asked during the webinar. Applicants are encouraged to send in additional questions to SCGEprogram@nih.gov.

When is the application deadline?
Applications to RFA-RM-22-014, RFA-RM-22-015, RFA-RM-22-016, and RFA-RM-22-017 are due July 19, 2022.

When are the letters of intent due?
Letters of intent for RFA-RM-22-014, RFA-RM-22-015, RFA-RM-22-016, and RFA-RM-22-017 are due June 17, 2022.

SCGE Phase 2 – Frequently Asked Questions

Are letters of intent required?
No, letters of intent are recommended but not required.

Are for-profit organizations eligible to apply?
All SCGE Phase 2 FOAs allow applications from for-profit institutions. RFA-RM-22-016 encourages collaborations with commercial entities developing genome editing therapeutics.

Please contact the Grants Management Officer listed on the FOA for additional information.

Are multi-PI applications allowed?
Yes.

SCGE Phase 2 – Frequently Asked Questions

Can I be a PI on an application for one FOA (for example, technologies and assays development) and also be a PI or multi-PI on an application submitted to another FOA (for example IND-enabling studies)?
These are separate FOAs. An investigator can be a PI on one application and a PI or multi-PI on a second separate application.

What would be the eligibility rules regarding foreign researchers and institutions?
Foreign institutions are allowed to apply to RFA-RM-22-014. RFA-RM-22-015, RFA-RM-22-016, and RFA-RM-22-017 **do not** accept foreign applicants but allow for foreign components. For additional information please see **Section III.1 Eligible Applicants** in each FOA.

SCGE Phase 2 – Frequently Asked Questions

Can NIH intramural investigators apply to any of the funding announcements?
Intramural investigators are eligible to apply to RFA-RM-22-014, RFA-RM-22-015, and RFA-RM-22-016, **but not** to RFA-RM-22-017.

Do I have to abide by the timeline outlined in the FOA?
Milestones must be completed within the timeframe outlined in the FOA. However, milestones may be completed early.

Can I submit just the UG3 portion to RFA-RM-22-016?
No, only applications for the combined mechanism (UG3/UH3) will be deemed responsive to this FOA.

SCGE Phase 2 – Frequently Asked Questions

What are "U" grants and how are they different from R01s?
The SCGE FOAs use the cooperative agreement funding mechanism. A cooperative agreement supports discrete, specified, circumscribed projects to be performed by investigators in an area representing their specific interest and competencies, and is used when substantial NIH programmatic involvement is anticipated.

What info should be included in Letters of Intent?
See section IV of FOA; Provide PI name(s) and telephone number(s), names of other key personnel, title of project, participating institution, and name and number of FOA.

Will I receive feedback on a Letter of Intent?
No. The Letter of Intent will help NIH staff plan the review.

Q&A Session

Please send your questions in the Q&A box or by emailing scgeprogram@od.nih.gov

SCGE Funding Opportunity	Contacts
Technologies and Assays for Therapeutic Genome Editing INDs (RFA-RM-22-014)	Betty Poon poonb@mail.nih.gov
IND-enabling Studies of Somatic Genome Editing Therapeutic Leads (RFA-RM-22-015)	Chris Boshoff chris.boshoff@nih.gov
Platform Clinical Trials for Somatic Genome Editing for Multiple Diseases (RFA-RM-22-016)	P.J. Brooks pj.brooks@mail.nih.gov
Somatic Cell Genome Editing Translational Coordination and Dissemination Center (RFA-RM-22-017)	Marrah Lachowicz-Scroggins marrah.lachowicz-scroggins@nih.gov

Contact us: SCGEprogram@od.nih.gov

Join our listserv: SCGE@LIST.NIH.GOV

Program website:
https://commonfund.nih.gov/editing

commonfund.nih.gov

@NIHCommonFund

@NIH_CommonFund

National Institutes of Health
Office of Strategic Coordination–The Common Fund