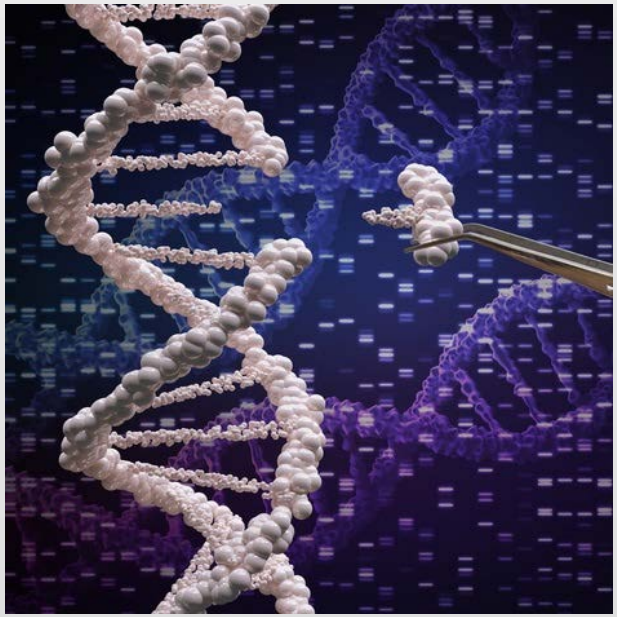
# NIH-Somatic Cell Genome Editing Applicant Webinar

February 16, 2018



*We will start @ 9:05 AM EST*

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

# Webinar Logistics

WebEx

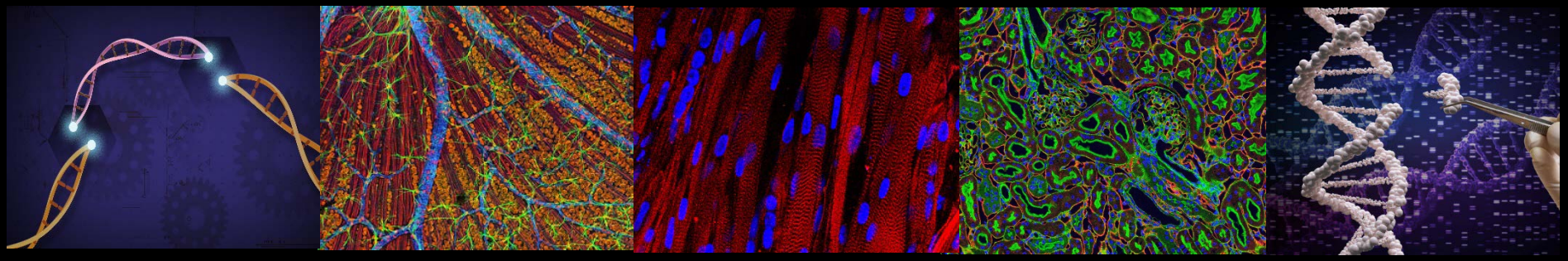
- We will not be recording and posting this webinar after, however the slide decks will be made available on the Common Fund Website: https://commonfund.nih.gov/editing

- All participants are muted until the Q&A session
  - These questions are intended for us to answer the group at large
  - If you have a specific question for a specific person, please reach out to them directly.
  - You can also submit questions during the talks to the SCGE mailbox, they will be answered during the Q&A: SCGEprogram@nih.gov

- A list of commonly asked FAQs are posted and will be updated on the Common Fund Website: https://commonfund.nih.gov/editing/faq

# Agenda

- **Introduction & WebEx overview** – 3 min; Kayla Valdes, NCATS
- **Program overview** – 5 min; Mary Perry, Common Fund, OD/OSC
- **Funding announcement overviews** – 5 min each; Oleg Mirochnitchenko, ORIP; John Sheridan, NHLBI; PJ Brooks, NCATS; Betty Poon, NIAID; Colin Fletcher, NHGRI
- **Review procedure** – 5 min; Elena Smirnova, CSR
- **Cooperative agreement overview & application deadlines** – 5 min; Ki-Cha Flash, NCATS
- **Q&A session** – 60 min



# SCGE Program Overview


Mary Ellen Perry, Ph.D.

Program Leader

Office of Strategic Coordination

Division of Program Coordination, Planning, and Strategic Initiatives

Office of the Director, NIH

One Hundred Ninth Congress
of the
United States of America

AT THE SECOND SESSION

*Begun and held at the City of Washington on Tuesday, the third day of January, two thousand and six*

An Act

To amend title IV of the Public Health Service Act to revise and extend the authorities of the National Institutes of Health, and for other purposes.

*Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,*

SECTION 1. SHORT TITLE.

This Act may be cited as the "National Institutes of Health Reform Act of 2006".

TITLE I—NIH REFORM

# Origins of the Common Fund

2004: NIH Roadmap is launched

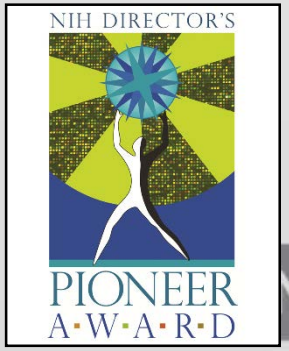
2006: Congress unanimously reauthorizes NIH

Establishes the Division of Program Coordination, Planning, and Strategic Initiatives (DPCPSI) within Office of the Director and the NIH Common Fund to provide a dedicated source of funding to enable *trans*-NIH research

# Criteria for Common Fund Programs

- **Transformative:** Must have high potential to dramatically affect biomedical and/or behavioral research over the next decade
- **Catalytic**: Must achieve a defined set of high impact goals within 5-10 years
- **Synergistic:** Outcomes must synergistically promote and advance individual missions of NIH Institutes and Centers to benefit health
- **Cross-cutting:** Program areas must cut across missions of multiple NIH Institutes and Centers, be relevant to multiple diseases or conditions, and be sufficiently complex to require a coordinated, trans-NIH approach
- **Unique:** Must be something no other entity is likely or able to do






NIH Collaboratory *Rethinking Clinical Trials®*
Health Care Systems Research Collaboratory

NIH National Institutes of Health
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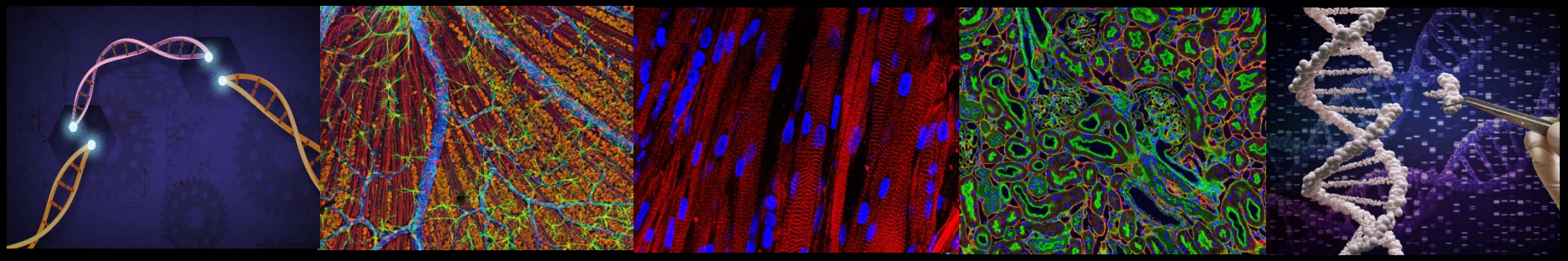

# NIH SCGE Working Group

Olivier Blondel, NIDDK
Pj Brooks, NCATS
Tom Cheever, NIAMS
Colin Fletcher, NHGRI
Maria Giovanni, NIAID
Linda M. Griffith, NIAID
Min He, NCI
Keith Hoots, NHLBI
Chamelli Jhappan, NCI
Tim LaVaute, NINDS
Jerry Li, NCI
Nicole Lockhart, NHGRI
Aron Marquitz, OSC/OD
Oleg Mirochnitchenko, ORIP/OD
Nasrin Nabavi, NIAID
David Panchision, NIMH
Mary Perry, OSC/OD
Betty Poon, NIAID
David Rampulla, NIBIB
John Sheridan, NHLBI
Jeff Struewing, NHGRI
Kayla Valdes, NCATS
Wendy Wang, NCI

DEPARTMENT OF HEALTH & HUMAN SERVICES · USA

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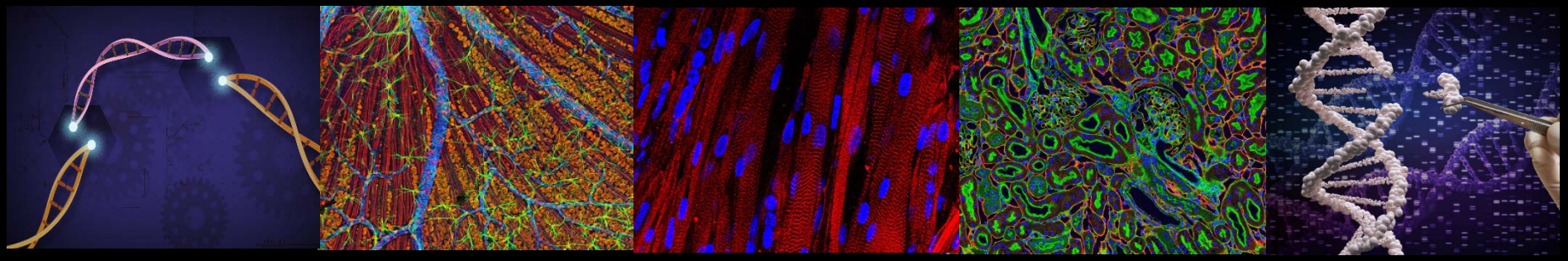

# The NIH SCGE Planning Workshop

July 24, 2017

## Major Gaps Identified:

- Relevant human and animal models systems for pre-clinical testing
- Cell- and tissue-specific delivery systems
- Error-free editing machinery (nuclease alternatives)
- Standardized assays for measuring genetic off-target effects
- Long-term cell tracking assays

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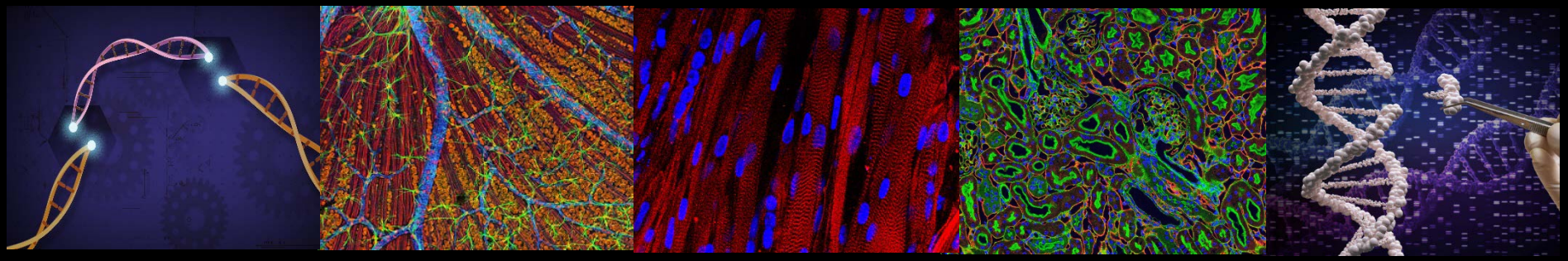
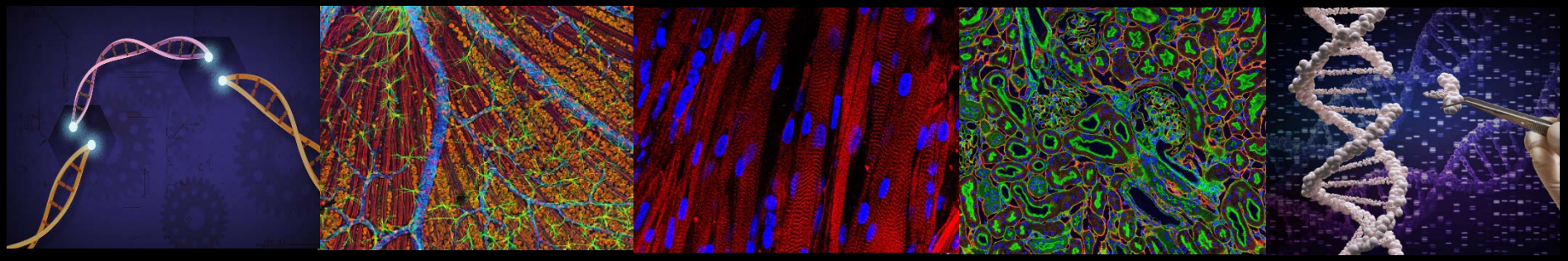


# SCGE Program Goals

Lower the Barriers for New Genome Editing Therapies by:

- Testing Genome Editing Reagents and Delivery Systems in Better Animal Models
- Assessing Unintended Biological Effects
- Improving *In Vivo* Delivery of Genome Editing Machinery
- Expanding the Human Genome Engineering Toolkit
- Coordinating Partnerships and Disseminating Information



## SCGE Dissemination & Coordinating Center (RM18-018)

- Facilitate interactions and communication between consortium components
- Disseminate a SCGE Toolkit to the research community

large and small animal testing systems (RM18-012 & 013)

cell and tissue platforms to detect adverse biological consequences (RM18-015)

new delivery systems (RM18-016)

expanding the repertoire of genome editors (RM18-017)

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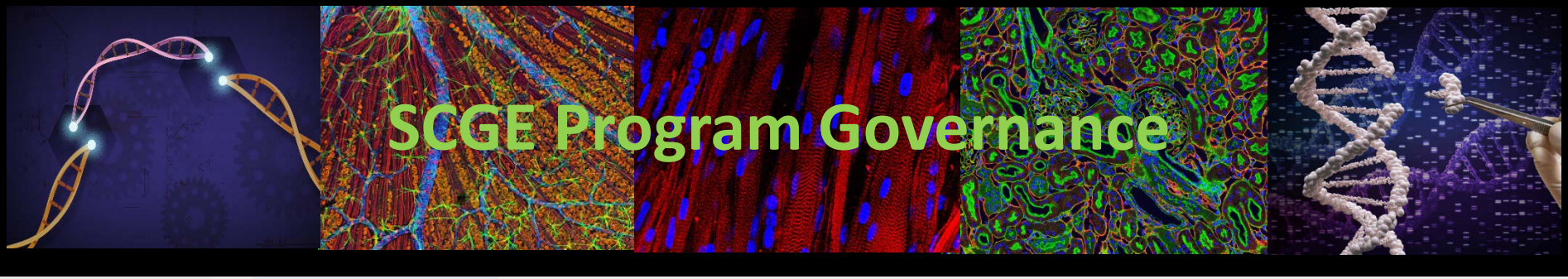


# SCGE Program Governance

Directors, OSC & DPCPSI

External Scientific Advisory Panel

NIH SCGE Working Group of Program Staff (Chair, NCATS Director)

Sub-committee on publications ?

Steering Committee of SCGE PIs

Additional sub-committees as needed

Sub-committee on data sharing ?

SCGE Consortium of Awardees

Sub-committee on common protocols ?

# Rodent Testing Centers for Development of Reporter Systems and Evaluation of Somatic Cell Genome Editing Tools (U42 Clinical Trial Not Allowed)

**RFA-RM-18-012**

Oleg Mirochnitchenko, ORIP on behalf of the NIH Common Fund

(oleg.mirochnitchenko@nih.gov)



National Institutes of Health
*Office of Strategic Coordination - The Common Fund*

**RFA-RM-18-012** Due date April 3, 2018

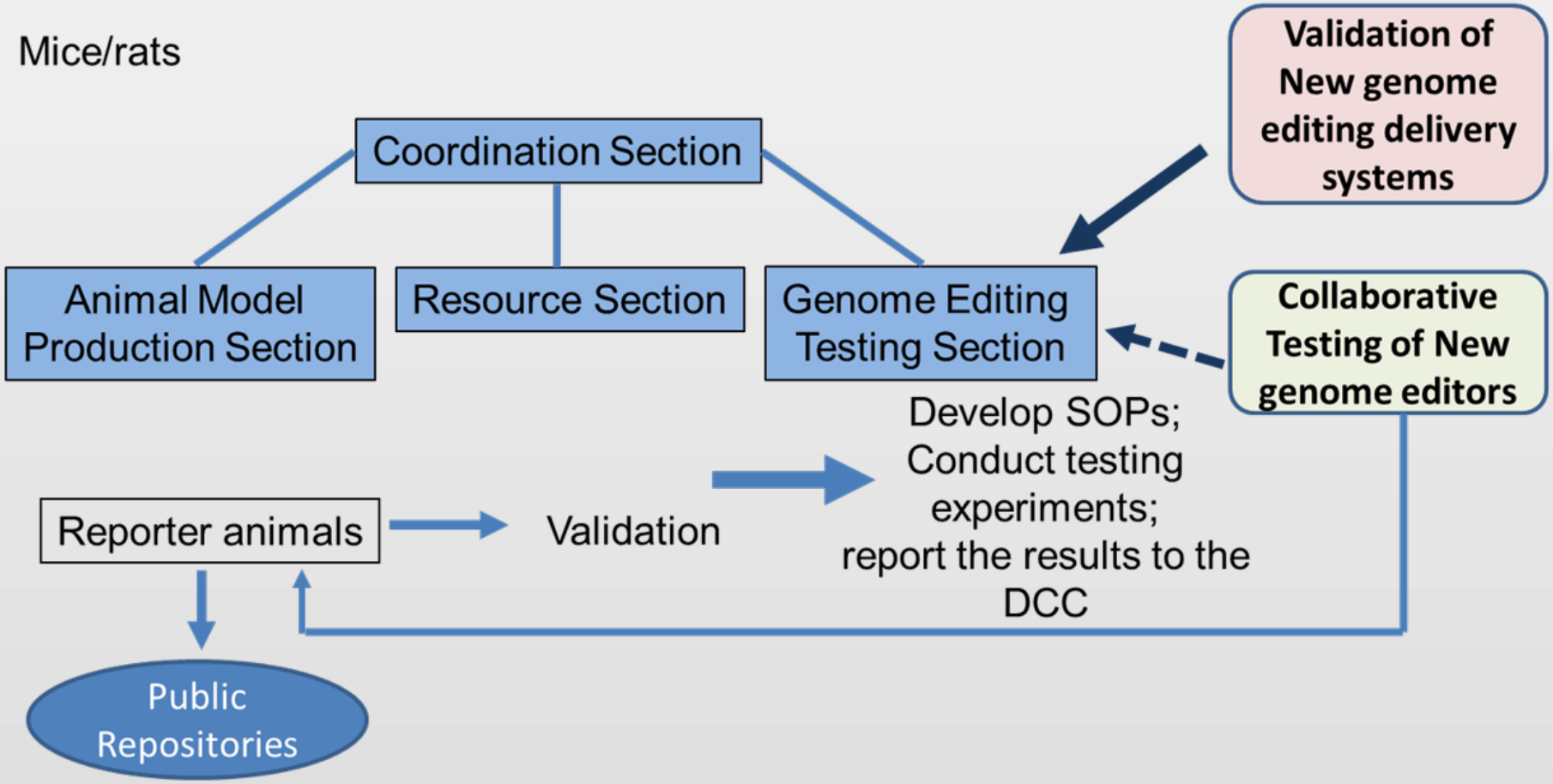
U42 Animal (Mammalian and Nonmammalian) Model, and Animal and Biological Materials Resource Cooperative Agreements

# Program Objective

- Develop reporter mice and rats to detect on-target and off-target genome editing in all cell types
- Distribute strains to national repositories
- Validate delivery systems developed under RFA-RM-18-016
- Collaborative studies to test new editors developed under RFA-RM-18-017

**Reporter animals:**

- animal models allowing quantitative evaluation of targeted genome editing, as well as off-target events, in individual cell types.
- Must allow detection in all cells (including germ cells)
- Testing of different types of editing activities (on-target and off-target in the same cells)


Mice/rats
Coordination Section
Animal Model Production Section
Resource Section
Genome Editing Testing Section
Validation of New genome editing delivery systems
Collaborative Testing of New genome editors
Develop SOPs; Conduct testing experiments; report the results to the DCC
Reporter animals
Validation
Public Repositories

## Review Criteria Specific to RFA-RM-18-012 (Section V):

- **Investigators** - Are the Center PD/PI's plans to support the team science environment sufficient to complete the proposed transdisciplinary work? Is the Center PD/PI (contact PD/PI for applications with multiple PDs/PIs) a scientist with extensive rodent animal model creation and use experience?
- **Approach** - Does the design and operating plan provide an opportunity for collaboration, integration, and interaction within the SCGE program (e.g., communication and collaboration with the SCGE Delivery Technologies, SCGE Editing Systems and the SCGE DCC)? Have the applicants described an approach for developing new reporter strains as part of collaborative studies? Does the overall strategy for validation, prior to collaborative studies, lead to the generation of reporter animals that will be able to detect and differentiate on-target and off-target genome editing in all cells with single-cell resolution? Are the methodology and SOPs for the use of the reporter animals, to detect genome editing in individual cells described in detail? Does the project demonstrate plans for ongoing communication and sharing of data and resources within the Center and the whole program? Do applicants described how they will work with the SCGE DCC as well as with SCGE Program Steering Committee to define the testing service priorities and mode of operation that best matches the needs of the whole Consortium within broad guidelines of parity, openness, cost-effectiveness, timeliness, as defined by the SCGE Program Steering Committee, the NIH and its advisors?
- **Additional Review Criteria**
  - Coordination Section
  - Genome Editing Section
  - Resource Section
  - Animal Model Production Section

# Program Timeline and Budget

Generating Resources

- Creating genome-editing reporter animals
- Verifying the reporter animal performance
- Developing assays and SOPs for detecting genome editing in vivo
- Expanding colony of reporter animals;
- Submitting new strains to NIH-supported national repositories for archiving and distribution

Collaboration & Validation

- Validating new delivery technologies developed by SCGE Delivery FOA
- Producing and testing additional reporter animals for testing new editors


Milestones: quantitative milestones should be proposed that address creation and delivery of the reporter animals and collaboration with SCGE grantees.

Award Budget: $225K (DC) FY18; $450K (DC) FY19-22

# Development of Large Animal Reporter Systems for Testing Somatic Cell Genome Editing Tools (U24 Clinical Trial Not Allowed)

**RFA-RM-18-013**

Oleg Mirochnitchenko, ORIP on behalf of the NIH Common Fund

(oleg.mirochnitchenko@nih.gov)


NIH National Institutes of Health
Office of Strategic Coordination - The Common Fund

**RFA-RM-18-013** Due date April 3, 2018

U24 Resource-Related Research Projects – Cooperative Agreements

## Program Objective

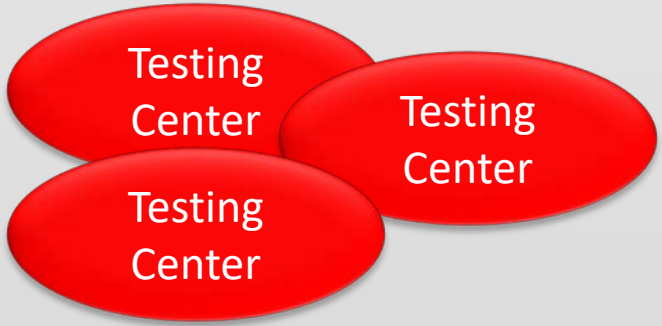
Pigs and NHPs (marmosets or rhesus macaques)

- Create large animal reporter models allowing quantitative evaluation of targeted genome editing, as well as off-target events, in individual cell types.

- Reporter animals should be designed to allow detection of the different types of editing activities.

- Validate and submit animals to the planned SCGE Large Animal Testing Centers for archiving, distribution and use for testing delivery systems and genome editors.

Reporter animals → Validation

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# Overall Review Criteria for RFA-RM-18-013 (section V):

• **Significance:** Does the proposed large animal production Center address the needs of the SCGE Consortium that it will serve? Is the scope of activities proposed for the Center appropriate to meet those needs? Will successful completion of the aims bring unique advantages or capabilities to the SCGE Consortium?

• **Investigator(s):** Are the PD(s)/PI(s) and other personnel well suited to their roles in the Center? Do they have appropriate experience and training, and have they demonstrated experience and an ongoing record of accomplishments in managing complex research? Do the investigators demonstrate significant experience with coordinating collaborative basic research? If the Center is multi-PD/PI, do the investigators have complementary and integrated expertise and skills; are their leadership approach, governance, plans for conflict resolution, and organizational structure appropriate for the Center? Does the applicant have experience overseeing selection and management of subawards, if needed?Does the applicant have a background, expertise and demonstrated track record of creating genetically modified large animal species using state-of-the-art technologies?

• **Innovation:** Does the application propose novel management strategies in coordinating with the SCGE Consortium the Center will serve? Are the concepts, strategies, or instrumentation novel to one type of research program or applicable in a broad sense? Is a refinement, improvement, or new application of management strategies proposed? Is the conceptual design of the reporter animals creative, innovative and likely to significantly improve currently available approaches for evaluation of the delivery systems and genome editing tools? Does the project achieve an optimal balance in proposing state-of-the-art technologies, approaches and models that are also proven, reliable, consistent and validated to meet the throughput and turnaround expectations of the program?

# Overall Review Criteria for RFA-RM-18-013 (Section V) (cont'd):

• **Approach**: Are the overall strategy, operational plan, and organizational structure well-reasoned and appropriate to accomplish the goals of the SCGE program the Centerwill serve? Will the investigators promote strategies to ensure a robust and unbiased scientific approach across the[program, as appropriate for the work proposed? Are potential problems, alternative strategies, and benchmarks for success presented? If the program is in the early stages of operation, does the proposed strategy adequately establish feasibility and manage the risks associated with the activities of the project? Are both an appropriate plan for work-flow and a well-established timeline proposed? Have the investigators presented adequate plans to ensure consideration of relevant biological variables, such as sex, for studies of vertebrate animals or human subjects? Is preliminary or published data, that demonstrates prior experience using the proposed approaches or animal model strategies, provided? Is how the model will be created, including methods for generating, breeding, validating and testing for each proposed reporter animal model described in detail? Do applicants describe the methodology for production of reporter animals, along with a strategy for validation prior to submission to the Large Animal Testing Centers? Will created reporter animals be able to detect and differentiate on-target and off-target genome editing in all cells, with single-cell resolution? Are the technologies, approaches and models proposed state-of-the-art, and do they also have a proven track record for producing reliable, consistent and validated results within the throughput and turnaround expectations of the program? Is the rationale for the assays proposed to evaluate the function of the reporter animals described, including the strengths and limitations of each assay, plans to validate the results, and alternative strategies if not successful? When appropriate for the model, are the plans to increase the rigor and reproducibility of the outcomes acceptable? Is the plan to coordinate with the SCGE DCC well described and adequate? Does the plan adequately address evaluation activities, including progress reports, site visits, and additional communication and materials to the NIH as needed? Does the applicant provide a timeline and preliminary set of milestones for: 1) creating reporter animals for specific applications; 2) verifying the reporter animal performance using standard delivery vehicles and genome editing tools; 3) submitting new created strains for the archiving and distribution to the Large Animal Testing Centers.

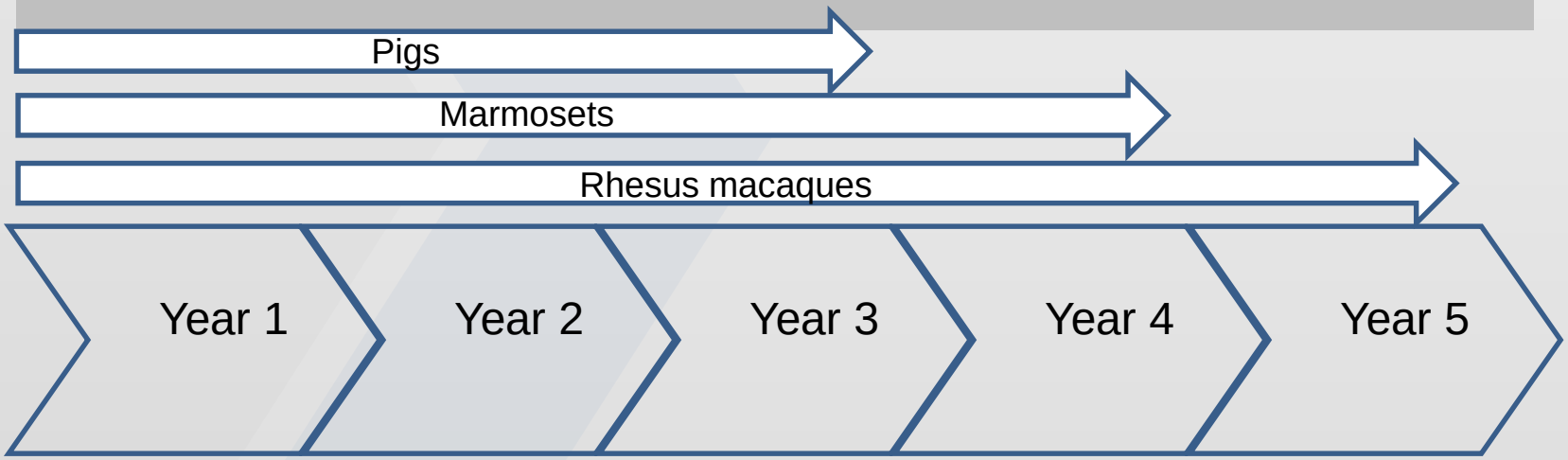
• **Environment:** Will the institutional environment in which the Center will operate contribute to the probability of success in facilitating the research SCGE Consortium it serves? Are the institutional support, equipment and other physical resources available to the investigators adequate for the Center proposed? Will the Center benefit from unique features of the institutional environment, infrastructure, or personnel?

Are resources available within the scientific environment to support electronic information handling?

# Program Timeline

Generating Resources

- Creating reporter animals
- Verifying the reporter animal performance using standard delivery vehicles and genome editing tools;
- Submitting newly created strains to the Large Animal Testing Centers
- Adjusting the pipeline for new type of reporter animals




Milestones: quantitative milestones should be proposed that address creation and delivery of the reporter animals and collaboration with SCGE grantees.

Award Budget: $500K (DC) FY18-22

# Development of Cell and Tissue Platforms to Detect Adverse Consequences of Somatic Cell Genome Editing (U01 Clinical Trial Not Allowed)

**RFA-RM-18-015**

John Sheridan, NHLBI on behalf of the NIH Common Fund

(john.sheridan@nih.gov)

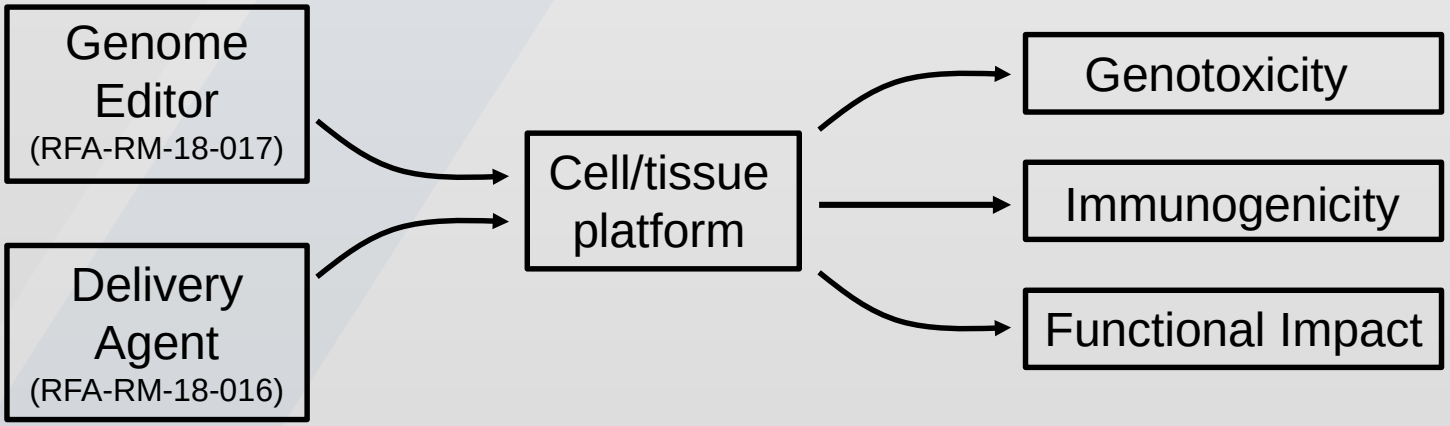


National Institutes of Health
*Office of Strategic Coordination - The Common Fund*

## Program Objective

Goal: To support the development, validation, and testing of new and existing human tissue- and cell-based platforms that can provide information on the safety of genome editing technologies and delivery systems.

Does exposure to the editor or delivery agent have any adverse biological consequences in specific target cells ?



## Research Scope

There are several highlighted areas of interest applicable to this FOA:

- Replicate critical aspects of normal human physiology
- Amenable to sequencing-based approaches to monitor off-target editing
  - DNA sequencing, RNA-seq, other “omics”-based approaches
- Development of assays to monitor other unintended biological effects
  - Biological function, genotoxicity, immunogenicity, etc.
- Bioinformatics and computational techniques should be implemented in combination with the cell/tissue platform

Examples of activities that will not be considered responsive:

- Platforms that use non-human cells/tissues or immortalized human cell lines
- Development of new sequencing-based approaches for the detection of off-target genome editing alone, without biological system

## Tissue Types of Interest

- Cell types of interest include but are not limited to disease-relevant cells, especially endogenous stem cells and cell types of origin for cancer, in the following organs or systems:
  - Liver (cell types other than hepatocytes)
  - Nervous system
  - Cardiovascular system, including hematopoietic and immune cells
  - Lung
  - Sensory organs
  - Kidney
  - Muscle
  - Bone
  - Pancreas

***Applications focused on other cell types should include a justification of disease relevance

## Applications Instructions Specific to RFA-RM-18-015 (Section IV):

The complete application instructions can be found in section IV of the FOA

Important instructions to keep in mind:

Describe novel concepts, approaches, or methodologies to be developed or used to establish and/or validate the platform. Explain refinements, improvements, adaptations, or new applications of existing technologies or platforms to be implemented in the project.

Describe the overall strategy and methodology to be used to establish feasibility and for making the platform sufficiently selective and sensitive to evaluate adverse biological consequences of genome editing. Provide a plan for validating the platform and describe in detail the potential functional outputs of the associated assays.

A timeline (Gantt chart) that includes milestones is required for all studies

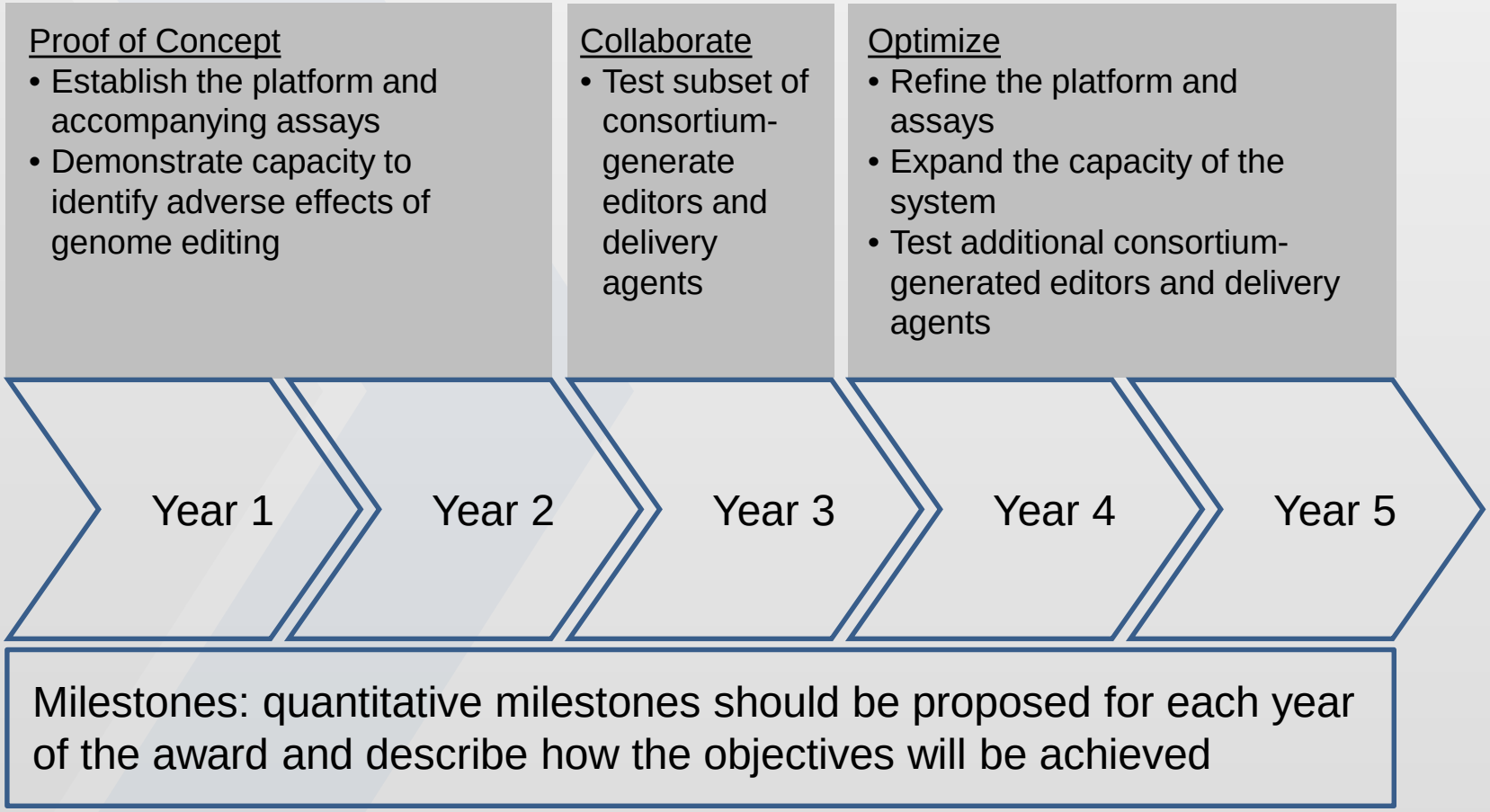
## Review Criteria Specific to RFA-RM-18-015 (Section V):

Investigator: Does the application provide sufficient evidence that the PD/PI(s) and key personnel have the necessary expertise to design and validate a platform to evaluate adverse effects of genome editing?

Innovation: How appropriate are the proposed concepts, approaches, or methodologies to establish and/or validate the cell- or tissue-based platform to evaluate adverse effects of genome editing?

Approach: Are the overall strategy, methodology, and analytical approaches adequately developed, well-integrated, and appropriate to establish feasibility of the platform? How strong is the evidence that the platform will be sufficiently selective, sensitive, or otherwise appropriate to evaluate adverse biological consequences of genome editing? Is the validation of the platform adequate and appropriate and are the outputs sufficiently described in detail?

# Program Timeline and Budget




Award Budget: $390K direct costs per year in FY18-22

# Innovative Technologies to Deliver Genome Editing Machinery to Disease-Relevant Cells and Tissues (UG3/UH3 Clinical Trial Not Allowed)

**RFA-RM-18-016**

PJ Brooks, NCATS on behalf of the
NIH Common Fund
(pj.brooks@nih.gov)



National Institutes of Health
*Office of Strategic Coordination - The Common Fund*

## Program Objective

Goal: To support the development and evaluation of innovative approaches to deliver genome editing machinery into somatic cells, with the ultimate goal of accelerating the development of genome editing therapeutics to treat human disease.


https://www.wired.com/story/what-good-is-crispr-if-it-cant-get-where-it-needs-to-go/

# Research Scope

- Projects can focus on a single cell type, or multiple cell types.
  - Same list of cell types of interest applies here

- Emphasis on organs and cell types that have no effective delivery technologies currently available; or
- For organs and cell types currently accessible to genome editing; emphasis on substantial qualitative improvements in clinical application.
  - Slight modifications or incremental advances will not be considered responsive to this FOA.

- Examples of qualitative improvements that would substantially impact clinical utility include but are not limited to:
  - Greater capacity and versatility in size or type of genome editing machinery delivered
  - Support of transient or regulatable expression of genome editors
  - Reduced immunogenicity
  - Avoidance of pre-existing immunity
  - Improved or expanded cell-type targeting
  - Simplification or increased scalability of production
  - Less invasive mode of administration

## Research Scope (cont'd)

- Non-viral technologies could include but are not limited to:
  - Nanoparticles
  - Liposomes
  - Extracellular vesicles
  - Physical delivery methods (devices)
  - Prokaryotic systems
  - Human cells modified to deliver genome editing machinery in vivo
  - ??????
- Screening approaches welcome
- Deliver genome editors as :
  - DNA
  - mRNA
  - Protein

- Viral technologies
  - Applications proposing to incorporate genome editors into currently known AAV serotypes not responsive

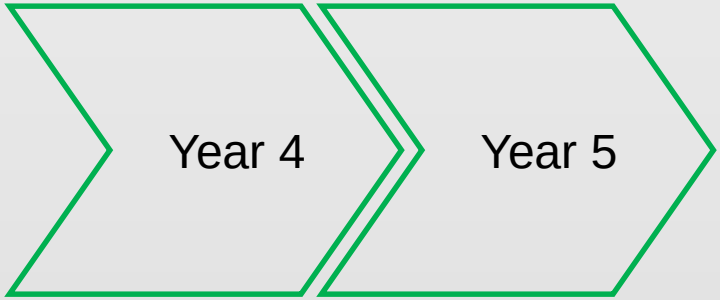
# Program Timeline and Budget : UG3 / UH3 Mechanism

Proof of Concept
- Establish and optimize the delivery technology
- Demonstrate genome editing in vivo in target cell types (by end of year 2).

Validation
- Send delivery reagents to SCGE testing center (RM-18-12) for independent validation
- Collaboration with other SCGE Awardees

- Scale-up and optimization for use in large animals
- Collaboration with other SCGE Awardees


UG3: $500K direct costs per year




UH3: Up to $1,000,000 / year (new Award)

UG3/UH3 transition:
- Programmatic review by NIH
- Based on negotiated milestones
  - e.g. % of target cells edited *in vivo*
- ***Independent validation essential***

## Application instructions specific to RFA-RM-18-016 (Section IV):

### *Overall*

Both the UG3 and UH3 phases should be described in the Research Strategy section,
**12 pages total**

A timeline (Gantt chart) that includes milestones is required. Milestones must include clear and quantitative criteria (e.g. % of target cells edited).

### *Added Review Criteria*

If the investigators have chosen a cellular target not included on the list of cell types and tissues of interest under Section I, Research Scope, have they provided a convincing therapeutic rationale for that choice?

Do the investigators have sufficient knowledge of the scientific domains of both delivery technology and genome editing biology to successfully direct the project?

If the investigators are proposing a project involving a currently available viral vector, have they provided a convincing explanation of how the approach would substantially impact clinical utility?
Have they included plans to assess whether the proposed delivery technologies will function in target human cells, including the use of human cell systems *in vitro* if available?

# Expanding the Human Genome Editing Repertoire (U01 Clinical Trial Not Allowed)

**RFA RM-18-017**

Betty Poon, NIAID on behalf of the NIH Common Fund

(poonb@niaid.nih.gov)



National Institutes of Health
*Office of Strategic Coordination - The Common Fund*

# RFA RM-18-017 Expanding the Human Genome Editing Repertoire

**Program Objective**

- The objective of this FOA is to support research aiming to develop innovative genome editing systems with improved specificity, efficiency, or functionality over currently available systems.
- Genome editing technology supported by the SCGE Consortium is defined broadly, extending beyond nuclease-dependent activities for manipulating DNA. Thus the platforms may also include, but not be limited to, nuclease-independent targeted editing, epigenetic modifiers, transcriptional repression and activation approaches, and RNA editors.
- Furthermore, the scope includes more than a single editor type (e.g., CRISPR-Cas).

# RFA RM-18-017 Expanding the Human Genome Editing Repertoire

**Research Scope:**

Design and optimization strategies to enhance genome editing capabilities in human cells can include but is not limited to:

•Rational screening, directed evolution, and selection methodologies to guide the identification of complexes with novel enzymatic activities and substrate specificities.

•Epigenome reprogramming technologies to alter the epigenetic composition of the genome at any given genomic location.

•Strategies for manipulation of normally inaccessible genomes or genomic regions that are relevant to the treatment of certain diseases.

•Approaches to increase the frequency of HDR within target regions, while reducing non-specific events.

•In silico computational design, including software tools, to improve or alter the activity of existing genome editing platforms. Modeling and simulation results should complement experimental studies that quantify the genome editing system efficacy and characterize both on-target and off-target system activities.

•Modifications of existing nucleases may be proposed, but should represent a significant enhancement compared to present nucleases, such as improved delivery or packaging in viral or non-viral vectors, short-term or regulatable expression or activity, higher fidelity with reduced off-target activity, and decreased immunogenicity.

# RFA RM-18-017 Expanding the Human Genome Editing Repertoire

**Application Instructions specific for this FOA (section IV):**

Under Research Strategy: All projects should include plans for comprehensive evaluation of efficiency and accuracy of the proposed genome editing platforms.

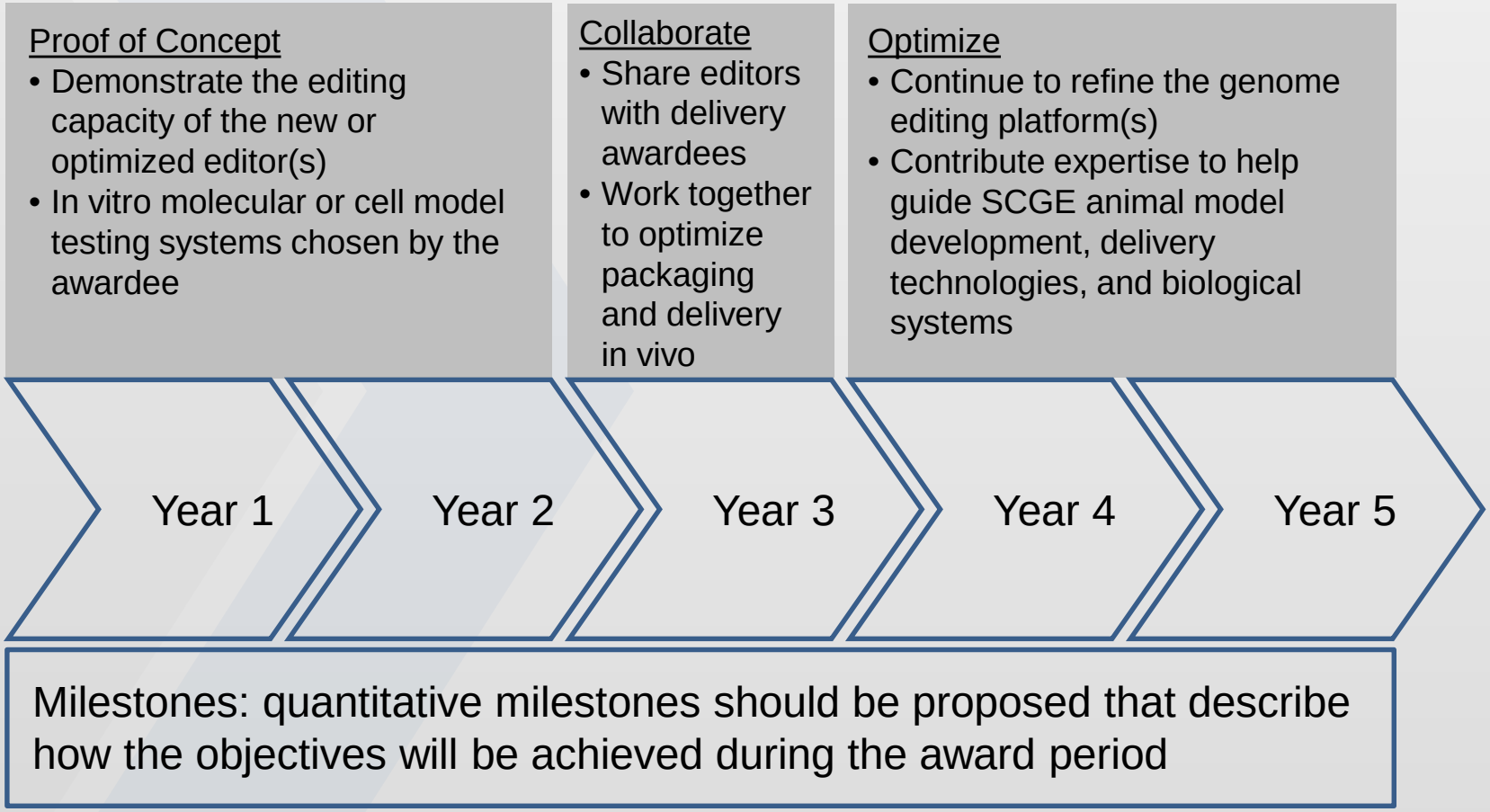
If modifications of existing nucleases are proposed, applicants should clearly articulate the quantitative performance metrics they aim to achieve that represent a significant improvement over the state of the art

A timeline (Gantt chart) that includes milestones is required for all studies

**Specific Review Criteria for RFA-RM-18-017 (section V)**:

Under Approach: Are the plans for evaluating the efficiency and accuracy of the proposed genome editing platforms well described, comprehensive, and appropriate to establish the feasibility of the platform? If modifications of existing nucleases are proposed, are the performance metrics quantitative and do the modifications provide significant improvements over current genome nuclease-mediated editing strategies?

# Program Timeline and Budget




Award Budget: $250K direct costs per year in FY18-22

# Somatic Cell Genome Editing Dissemination and Coordinating Center (U24 Clinical Trial Not Allowed)

**RFA RM-18-018**

Colin Fletcher, NHGRI on behalf of the NIH Common Fund

(colin.fletcher@nih.gov)



National Institutes of Health
*Office of Strategic Coordination - The Common Fund*

# RFA RM-18-018 Somatic Cell Genome Editing Dissemination and Coordinating Center

**Program Objective:**

- The purpose of this FOA is to support the establishment of a Dissemination and Coordinating Center (DCC) for the NIH Somatic Cell Genome Editing (SCGE) Consortium.
- Close interaction and efficient lines of communication between the DCC and the research teams of other Consortium components will be essential due to the DCC's role in: 1) the scientific and logistic coordination of intra-Consortium collaborations; 2) collection of complex data from multiple research teams for storage, sharing and dissemination; and 3) management of the Collaboration Opportunity Fund.
- The deliverables of the SCGE program will be a collection of tools, methods, data, and best practices that will accelerate development and testing of new treatments for many diseases, i.e. the SCGE Toolkit for Therapeutic Genome Editing or SCGE Toolkit.

# RFA RM-18-018 Somatic Cell Genome Editing Dissemination and Coordinating Center

**Research Scope:**

Robust and effective strategies to enhance project coordination and data dissemination:

1. Consortium Coordination activities could include:

   Provide logistical and administrative assistance,

   Develop and implement a comprehensive Consortium-wide Manual of Operations,

   Develop an internal communication platform to facilitate information exchange and discussion

   Developing an internal Consortium communication platform to facilitate Consortium-wide information exchange and discussion

2. Assembly and Dissemination of the SCGE Toolkit

   The SCGE Toolkit should present resources generated from the Consortium in an intuitive and readily accessible online interface. Resources should be organized and presented at a high level, while also allowing researchers to access more detailed information associated with each tool or approach.

3. Collaboration support

   A single Collaboration Opportunity Fund (COF) of up to $3,000,000 total costs per year in fiscal years 2020-2022 will be established under this FOA to support new and pilot research projects led by investigators within the SCGE program. The COF is meant to promote the exchange, cross-testing and evaluation of the improved technologies within the Consortium.

# RFA RM-18-018 Somatic Cell Genome Editing Dissemination and Coordinating Center

**Application Instructions specific for this FOA (section IV):**

Under Research Strategy: Provide a brief overview of the proposed DCC and its role in the NIH SCGE Consortium indicating how the DCC can facilitate and enhance the work of the entire NIH SCGE Consortium.

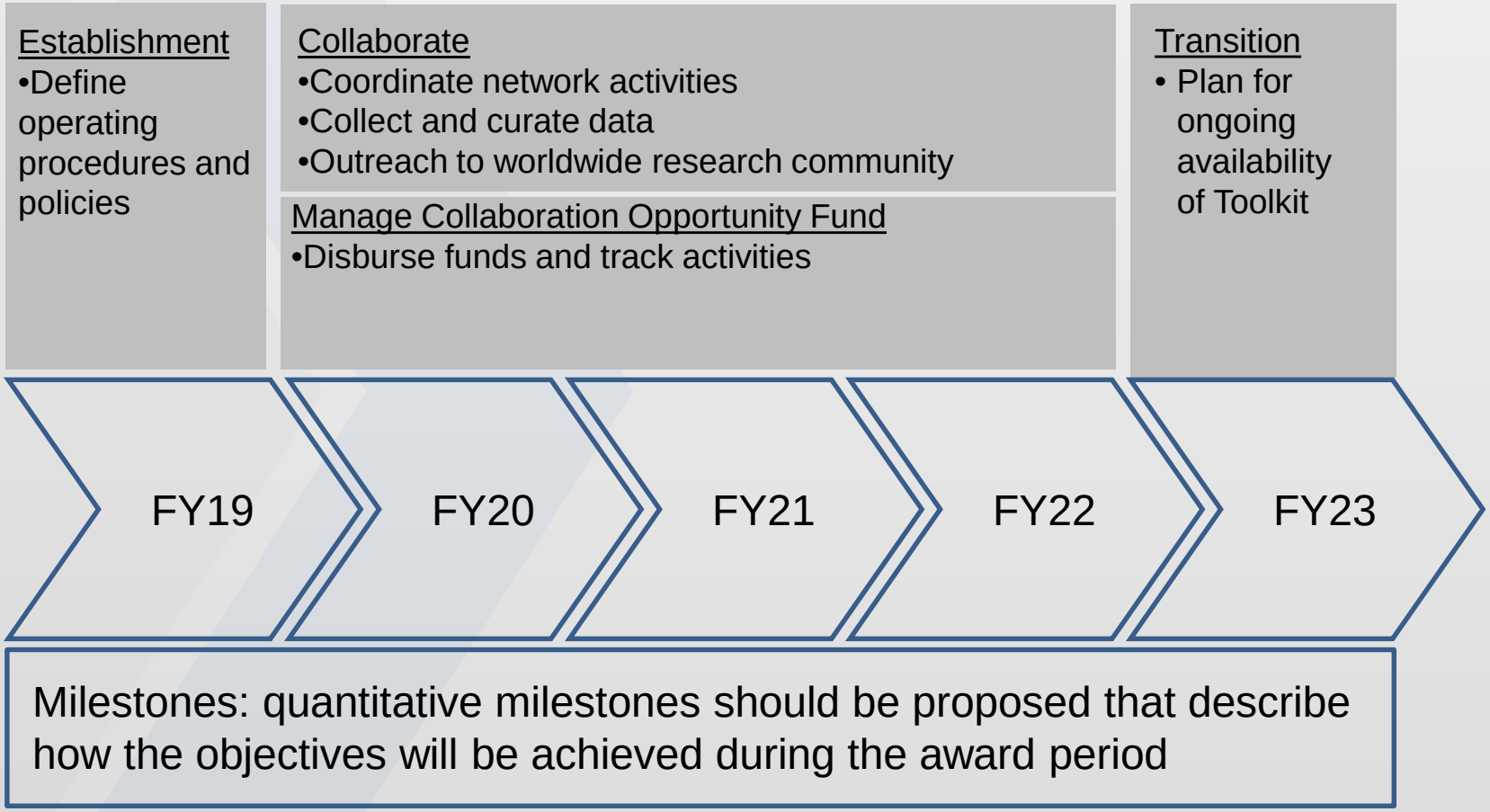
Applicants should describe how the resources developed by the SCGE will be organized into an intuitive and accessible online platform to enable researchers to determine the best approach for their intended therapeutic development program.

Describe a management plan for the collaboration opportunity fund (COF) and plans for interacting with the institutions that will receive COF funds.

**Specific Review Criteria for this FOA (section V)**:

Under Approach: Is the proposed design of the SCGE Toolkit robust and rigorous? Are effective dissemination strategies proposed?

# Program Timeline and Budget




Milestones: quantitative milestones should be proposed that describe how the objectives will be achieved during the award period

Award Budget: variable, $1.5-2M direct costs per year excluding COF

U.S. Department of Health & Human Services

NIH Center for Scientific Review

# Overview of Scientific Review Process

Elena Smirnova, Ph.D.
Acting Chief, Genes, Genomes and Genetics IRG

# Review – who will review my application?

- Reviewed in Center for Scientific Review (CSR)
- Special Emphasis Panels (SEP) – no need to look up and request a standing study section. One-time panels held to review applications on special topics.
- Include only temporary members
- Meeting rosters will be posted online 30 days before the review meeting - https://public.csr.nih.gov/StudySections/SpecialEmphasis/

# Review Information

- Refer to Section V of the FOA – “Application Review Information”
- Read Criteria.
- Pay special attention and address “Specific to this FOA” review questions.

# Overall Impact and Review Criteria

- **Overall Impact**: The reviewers will assess the likelihood for the project to exert a sustained, powerful influence on the research field(s) involved, in consideration of the following review criteria (as applicable for the project proposed).
- Five Scored Review criteria: Significance, Investigator(s), Innovation, Approach, Environment
- Additional Review Criteria: Protections for Human Subjects, Vertebrate Animals, Biohazards

# Grants Management

Ki-Cha Flash, MS, MBA
Grants Management Specialist


NIH National Institutes of Health
Office of Strategic Coordination - The Common Fund

# NIH Common Fund Somatic Cell Genome Editing (SCGE) Consortium FOAs

- ORIP RFA (U42 mechanism): RFA-RM-18-012
- ORIP RFA (U24 mechanism): RFA-RM-18-013
- NHLBI RFA (U01 mechanism): RFA-RM-18-015
- NCATS RFA (UG3/UH3 mechanism): RFA-RM-18-016
- NIAID RFA (U01 mechanism): RFA-RM-18-017
- NHGRI RFA (U24 mechanism): RFA-RM-18-018
- **All are Cooperative Agreements (U activity code)**

# Cooperative Agreements: U Mechanism

- Used when **substantial programmatic involvement is anticipated between the Federal agency and the recipient** during performance of the assisted activity.
- Supports and stimulates the recipients' activities by involvement in and working jointly with the award recipients in a **partnership role**; it is not to assume direction, prime responsibility, or a dominant role in the activities. The dominant role and prime responsibility reside with the awardees of the project as a whole.
- The Cooperative Agreement Terms and Conditions of Award in each FOA clearly outlines the roles and expectations of the PD/PI and NIH Program Staff.
- This information will also be in the Notice of Award (NoA)

# Remember

- Refer to your specific FOA for:
  - specific information associated with the award mechanism
  - names of individuals who may be contacted for additional or clarifying information prior to application submission
  - Specifics of eligibility criteria

- Contact Awarding Component:
  - Consult with the NIH Scientific/Research contact of the appropriate awarding component prior to submitting an application, as eligibility criteria, support levels, and availability of awards may vary among NIH Institutes or Centers and for any other questions

# Resources

- NIH Grants Policy Statement: https://grants.nih.gov/grants/policy/nihgps/HTML5/introduction.htm
- SF424 (R&R) General Instructions for NIH and other PHS Agencies: https://grants.nih.gov/grants/how-to-apply-application-guide/forms-d/general-forms-d.pdf
- Funding Opportunity Announcements:
  - ORIP RFA *(U42 mechanism):* RFA-RM-18-012
  - ORIP RFA *(U24 mechanism):* RFA-RM-18-013
  - NHLBI RFA (*U01 mechanism): RFA-RM-18-015*
  - NCATS RFA *(UG3/UH3 mechanism):* RFA-RM-18-016
  - NIAID RFA *(U01 mechanism):* RFA-RM-18-017
  - NHGRI RFA (*U24 mechanism*): RFA-RM-18-018

# Important Contacts

## Scientific/Research Contact(s)

- **RFA-RM-18-012 & RFA-RM-18-013**
  - Oleg Mirochnitchenko, Ph.D.; ORIP
  - Tel.: 301-435-0748
  - Email: oleg.mirochnitchenko@nih.gov
- **RFA-RM-18-015**
  - John Sheridan, Ph.D.; NHLBI
  - Tel.: 301-435-0202
  - Email: john.sheridan@nih.gov
- **RFA-RM-18-016**
  - Phillip J. ("Pj") Brooks, Ph.D., NCATS
  - Tel: 301-443-0513
  - Email: PJ.Brooks@nih.gov
- **RFA-RM-18-017**
  - Betty Poon, Ph.D., NIAID
  - Tel: 240-669-5024
  - Email: poonb@niaid.nih.gov
- **RFA-RM-18-018**
  - Colin Fletcher, Ph.D.; NHGRI
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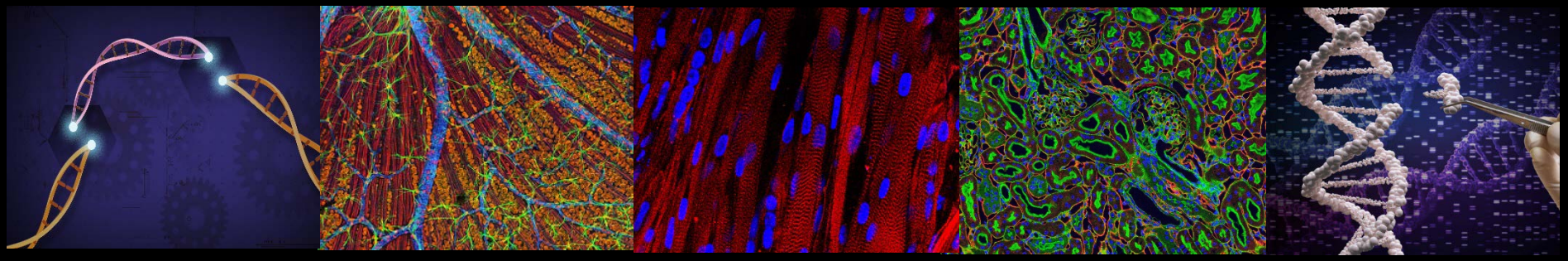
  - Email: fletcherc2@mail.nih.gov

- **Peer Review Contact**
  - Elena Smirnova, Ph.D.; CSR
  - Tel: 301-357-9112
  - Email: smirnove@csr.nih.gov
- **Grants Management****
  - Ki-Cha Flash, MBA; NCATS
  - Tel: 301-435-0846
  - E-mail: flashk@mail.nih.gov
- **Common Fund**
  - Mary Perry, Ph.D., OD/OSC
  - Tel: 301-435-5082
  - E-mail: perryma@mail.nih.gov

**Please check FOA for specific GM contact

# Questions?

- Please submit using the **chat box**
- All questions submitted to the SCGE email during the webinar will be answered in the order they were received.



# Instructions for NIH IRP Applicants

Instructions for IRP applicants are in the Common Fund Handbook (NIH access only):
https://osc.cf.cit.nih.gov/CommonFundHandbook/Pages/Chapter_6_Section_C_Intramural_Involvement_in_Common_Fund_Programs.aspx

Some highlights:
IC Scientific Director needs to sign application
IRP can request funds only for project activities – not to support federal staff
If acting as a collaborator, the IRP PI gives a budget request to the ERP PI that gets submitted with the application
The IRP funds go directly from the CF to the IRP as an interagency agreement, not from the CF to the ERP and then to the IRP.