

The NIH Somatic Cell Genome Editing (SCGE) Program

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Colin Fletcher, Ph.D.

NHGRI



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

NIH Planning Workshop for the SCGE Program

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

Mission:

“The focus of the Somatic Cell Genome Editing program is to dramatically accelerate the translation of these technologies to the clinic for treatment of as many genetic diseases as possible.”

NIH Director Francis S. Collins, M.D., Ph.D

SCGE Program Goals

Lower the Barriers for New Gene Editing Therapies by:

Testing Gene Editing Reagents/Delivery Systems in 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 Program Structure

The SCGE program will utilize milestone-driven Cooperative Agreements and award ~\$196M over 5 years. Currently, applications are in review, and FOA reissues are expected in the near future.

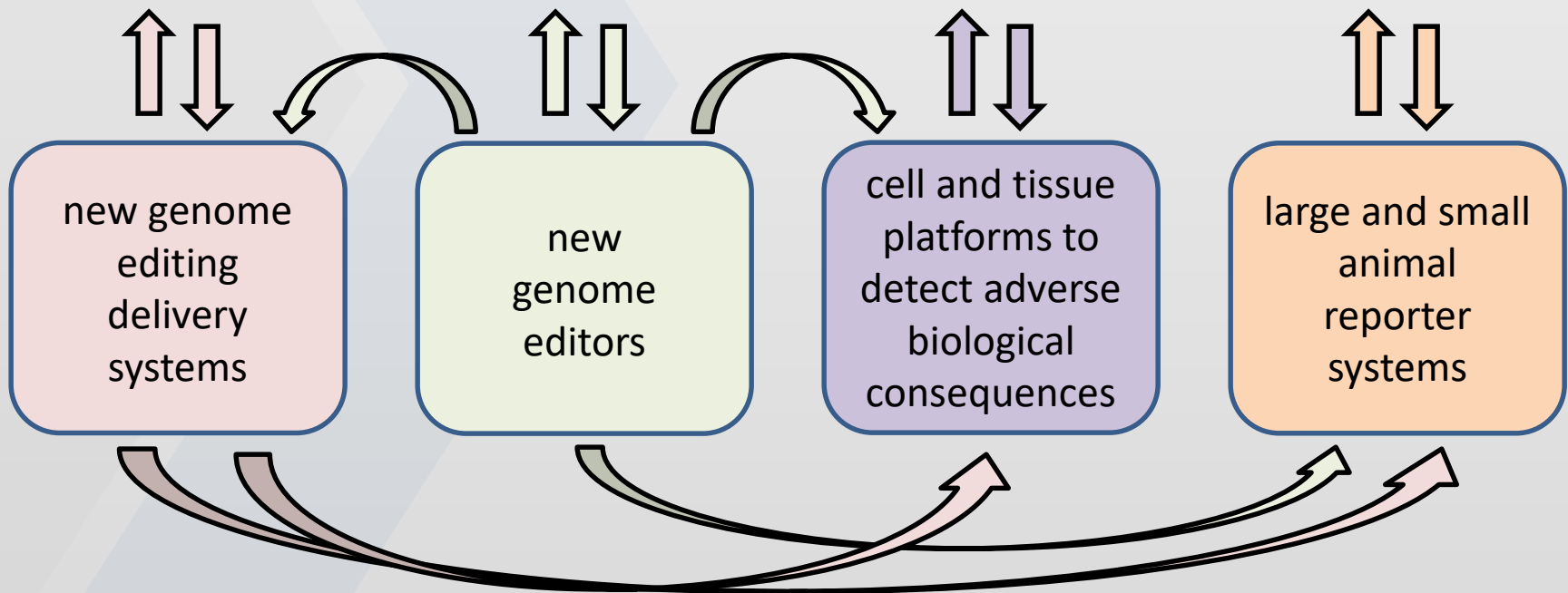
Awardees will be required to collaborate and share data, resources and information with other consortium members and the Dissemination and Coordinating Center.

Resources and information generated by the SCGE will be disseminated by the Dissemination and Coordinating Center to the broader research community through the SCGE Toolkit for Therapeutic Genome Editing (“SCGE Toolkit”)

SCGE Program Interactions

SCGE Dissemination & Coordinating Center

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



SCGE Program Focus

SCGE Dissemination & Coordinating Center (9)

new genome editing delivery systems
(85)

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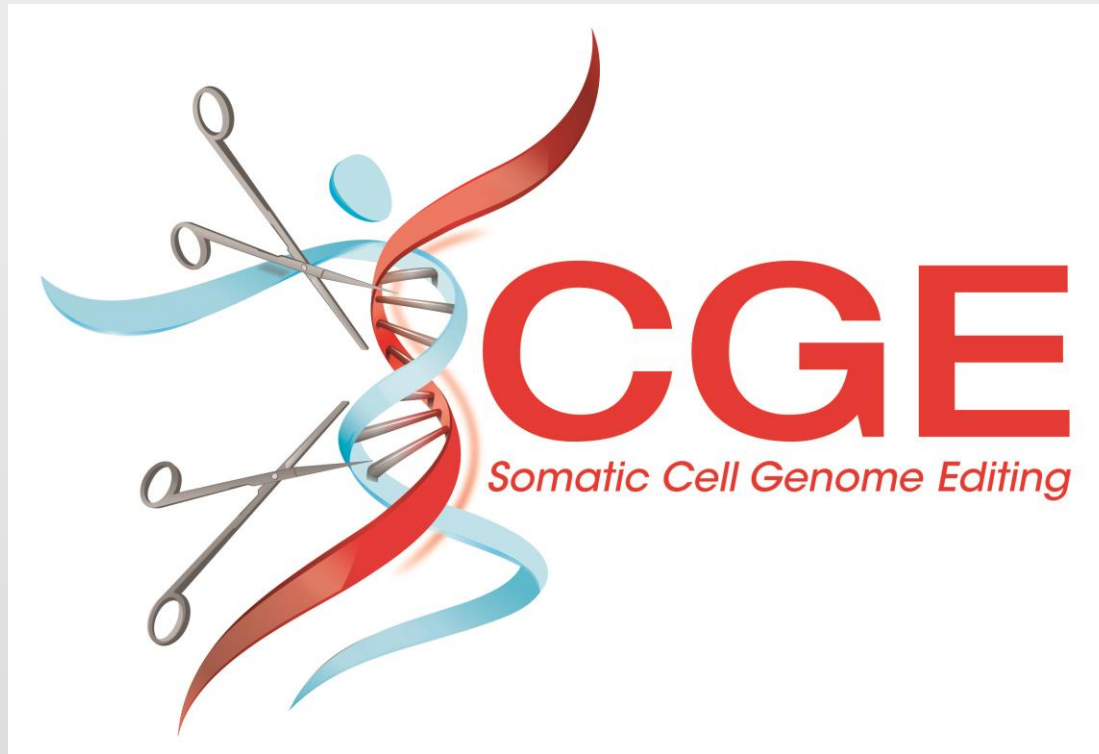
cell and tissue
platforms to
detect adverse
biological
consequences
(43)

large and
small
animal
reporter
systems
(30)



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Thank you!



Program Contacts

FOAs RM-18-012 and RM-18-013 (Animals):

Oleg Mirochnitchenko, Ph.D.
Office of Research Infrastructure Programs
Office of the Director
National Institutes of Health (NIH)
oleg.mirochnitchenko@nih.gov

FOA RM-18-017 (Editors):

Betty Poon, Ph.D.
Program Director
Targeted Interventions Branch
NIAID
poonb@niaid.nih.gov

Common Fund Program:

Mary Perry, Ph.D.
Program Leader
Office of Strategic Coordination
Office of the Director
National Institutes of Health (NIH)
perry@nih.gov

FOA RM-18-016 (Delivery Systems):

P.J. Brooks, Ph.D.
Program Coordinator
Division of Clinical Innovation
and Office of Rare Diseases Research
NCATS
pjbrooks@mail.nih.gov

FOA RM-18-015 (Biological Systems):

John Sheridan, Ph.D.
Program Director
Division of Lung Diseases
NHLBI
john.sheridan@nih.gov

FOA RM-19-018 (SCGE DCC):

Colin Fletcher, Ph.D.
Program Director
Division of Genome Sciences
NHGRI
colin.fletcher@nih.gov

NIH Common Fund SCGE Program Website:
<https://commonfund.nih.gov/editing>

SCGE “Ask Anything” Mailbox:

SCGEprogram@nih.gov