

Concept Clearance for a Notice of Funding Opportunity (NOFO) for Competing Revisions to Applications Awarded Under RFA-RM-22-015 to Allow Clinical Trials for the Somatic Cell Genome Editing ([SCGE](#)) Program

BACKGROUND INFORMATION

The Common Fund's [SCGE](#) program was launched in 2018 with the purpose of accelerating the translation of genome editing technologies into the clinic. After a successful first phase, where new genome editors, genome editing delivery systems, and assays to detect adverse effects of editing were developed, the second phase of [SCGE](#) is focused on therapy development and helping to define a regulatory path for genome editing drug development. A major component for achieving these goals was a cooperative agreement program initiative ([RFA-RM-22-015](#)) to accelerate further optimization and development of genome editing-based therapeutic leads toward IND (Investigational New Drug)-enabling studies. This initiative did not allow or anticipate clinical trials. Of the 12 applications submitted to this initiative 5 were funded, with the expectation that each program project will generate at least one IND approval for a genome editing drug product within the five-year project period.

EARLY PROGRAM LESSONS

Phase 2 of the [SCGE](#) is on track to meet these goals, as the program has been at the forefront of shaping regulatory guidance for the genome editing field through multiple interactions with the FDA, and with genome editing therapy development projects that are ahead of the program's scheduled timeline. An early lesson learned is that the [SCGE](#) funded investigators are advancing faster than anticipated, and some [SCGE](#) projects may complete IND-enabling studies ahead of schedule. If these projects continue to advance at the current pace, NIH [SCGE](#) program staff would like to allow [SCGE](#) investigators to expand the scope of their original awards, and to provide them the opportunity to move approved therapies into the clinic.

PROPOSED FUNDING OPPORTUNITY ANNOUNCEMENTS

We propose issuing a competing revision NOFO to permit [SCGE](#) investigators that were awarded under [RFA-RM-22-015](#) to expand the scope of their existing awards. The proposed NOFO would allow re-budgeting of existing awards and support small first-in-human genome editing clinical trials using therapeutic drug products developed through the investigators' cooperative agreement program project awards.

Planned Funding: \$0-6 million per year; anticipate 0-2 awards, with a 2-year project period.