

Somatic Cell Genome Editing Program

Overarching Goal: to improve the efficacy and specificity of genome editing approaches to reduce the burden of disease



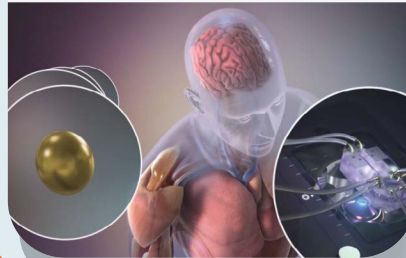
Develop Novel Technologies

Editing and delivery tools that target specific genes and tissues



Build a Foundation for Clinical Applications

Safety and efficacy testing of these tools via new methods, platforms, and animal models



Deliver Resources to Accelerate Treatments

Toolkit for therapeutic genome editing shared with the broad biomedical research community



The SCGE Toolkit will enable biomedical researchers to develop therapies for a variety of genetic disorders, like cystic fibrosis, neurodegenerative diseases, hearing loss, and muscular dystrophy

