GUEST EDITORIAL

National Science Open
2: 20230053, 2023
https://doi.org/10.1360/nso/20230053

Life Sciences and Medicine

Special Topic: Gene Editing towards Translation

Preface to the special topic on gene editing towards translation

Jinsong Li[1,*] & Anming Meng[2,*]

[1]*State Key Laboratory of Cell Biology, CAS Center for Excellence in Molecular Cell Science, Shanghai Institute of Biochemistry and Cell Biology, Chinese Academy of Sciences, Shanghai 200031, China;*
[2]*Laboratory of Molecular Developmental Biology, State Key Laboratory of Membrane Biology, Tsinghua-Peking Center for Life Sciences, School of Life Sciences, Tsinghua University, Beijing 100084, China*

*Corresponding authors (emails: jsli@sibcb.ac.cn (Jinsong Li); mengam@mail.tsinghua.edu.cn (Anming Meng))

Received 1 September 2023; Revised 1 September 2023; Accepted 1 September 2023; Published online 15 September 2023

The first draft of the human genome, published in 2001, has had a broad effect on biomedical studies and applications. In the past two decades, the human genome project (HGP) has dramatically promoted our understanding of how genomic sequences work during development, diseases and evolution. Meanwhile, genome-editing technologies have been rapidly developed to precisely and efficiently rewrite DNA sequences of different species, especially in 2012 as the development of a revolutionary genome editing method based on clustered regularly interspaced short palindromic repeat (CRISPR) and CRISPR-associated (Cas) proteins. The advent of CRISPR-Cas system, because of its diversity, modularity, and efficacy, in turn, further accelerates our pace of revealing the functions of the genome sequence during biological processes. In addition, CRISPR-Cas has been displaying its broad range of applications in clinical and agricultural fields.

Here, we present our readers a special topic on "Gene editing towards translation" with six high-quality, peer-reviewed articles, including four timely review articles, one insightful perspective, and one original research article. The review by Jun-Jie Gogo Liu and colleagues [1] discusses four nuclease-based gene-editing tools that can induce double-stranded DNA breaks, including meganucleases, zinc-finger nucleases (ZFN), transcription activator-like effector endonucleases (TALEN), and CRISPR-Cas; briefly introduces their structural mechanisms of targeted DNA recognition; and mainly describes CRISPR-Cas systems and their variants that have become a dominant strategy for gene editing in a broad range of applications from fundamental biology to biotechnology and biomedicine. The review by Sen Wu and co-authors [2] describes applications of gene editing in molecular breeding of farm animals by briefly introducing the development history of gene-edited farm animals, systematically discussing the progress of gene-edited farm animals with aspects of production performance improvement, disease resistance, bioreactor, animal welfare, and environmental friendliness, and providing their insights regarding the safety and supervision of gene-edited farm animals. The review by Liangxue Lai and colleagues [3] focuses on the history and recent advances in generation of pigs with multiple gene modifications to overcome the hurdle of immune rejection for xenotransplantation, which led to the most recent clinical trial of pig heart and kidney transplantation into

patients, showing promising clinical application prospects. The review by Yuxuan Wu and Haokun Zhang [4] describes the application of gene editing in infectious diseases, including antiviral therapy, such as human immunodeficiency virus, hepatitis B virus, severe acute respiratory syndrome coronavirus 2, and human papillomavirus, and non-viral infection diseases that involve bacteria, fungi and parasites. Mingyao Liu and colleagues [5] discuss in their perspective article the crucial role of gene editing in improving the safety, efficacy and accessibility of chimeric antigen receptor (CAR)-T cells in clinic. Finally, the research article by Caixia Gao and colleagues [6] establishes a new and rapid genome editing evaluation method in plants using *Agrobacterium* infiltration techniques to enable broad-spectrum, simplistic, and precise assessments of genome editing efficiencies, which may further expedite the development of genome-edited agricultural crops.

CRISPR-Cas-based applications in medicine and agriculture are already beginning and will certainly attract more and more attention from both academic and industry fields in next decades to accelerate their translation. We hope that this special topic will receive broad attention and contribute to the growth of gene editing fields in years to come. As the guest editors, we would like to express our sincere appreciation to all the authors, reviewers and also the editorial and production staff of *National Science Open* for their time and dedication that have made this special topic possible.

References

1 Li DY, Li LQ, Liu JJG. Nucleases in gene-editing technologies: Past and prologue. *Natl Sci Open* 2023; **2**: 20220067.

2 Gao F, Hou N, Du X, *et al.* Molecular breeding of farm animals through gene editing. *Natl Sci Open* 2023; **2**: 20220066.

3 Wu H, Lian M, Lai L. Multiple gene modifications of pigs for overcoming obstacles of xenotransplantation. *Natl Sci Open* 2023; **2**: 20230030.

4 Wu Y, Zhang H. Treatment of infectious diseases by *in vivo* gene editing. *Natl Sci Open* 2023; **2**: 20220061.

5 Du B, Zhang J, Liu M. Harnessing the power of gene-editing to develop the next generation of CAR-T cells. *Natl Sci Open* 2023; **2**: 20220070.

6 Xing S, Sun Y, Li B, *et al.* Anthocyanin-assisted *Agrobacterium* infiltration for the rapid evaluation of genome editing efficiencies across multiple plant species. *Natl Sci Open* 2023; **2**: 20220052.