Spotlight on gene therapy in China

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Building on decades of efforts from scientists, clinicians, and manufacturers, gene therapy is starting to realize its enormous potential to treat cancer, inherited genetic diseases, and even infectious and cardiometabolic diseases. China has a population of more than 1.4 billion, with estimated more than 57 million people with genetic diseases, and more than 4 million new cancer cases per year [1], posing an urgent demand for development of new therapeutics, in which gene therapies are becoming a critical component.

Being aware about the explosive development and broad prospect of the gene therapeutic field, the Chinese government, led by the National Health Commission (NHC) and the National Medical Products Administration, has released a series of progressive regulatory policies to gene and cell therapies between late 2017 and 2019. For example, in December 2017 and June 2018, China Food and Drug Administration (CFDA) granted a priority review path for drugs targeting rare or life-threatening diseases (data retrieved from CFDA, <http://www.cfda.com.cn/>); and in March 2019, NHC invited comments on use of biomedical techniques (including gene editing) in clinical trials regulation (data retrieved from NHC, <http://www.nhc.gov.cn/>). These regulatory policies have delivered a strong support from the Chinese government to the gene therapeutic field, especially after the “Wei Zexi incident” in 2016, which resulted an abrupt 19-month ban by the government on cell and gene therapy research and clinical use.

In virtue of the improving regulatory policies, as well as the technical and industry promotion, the gene therapeutic

field in China is now a booming field, with more than 45 local companies with Investigational New Drug-approved pipelines (data retrieved from GBI Health, <https://www.gbihealth.com/>), and more than 1000 clinical trials either conducted or underway granted by the Chinese government between 2017 and 2019 (data retrieved from ClinicalTrials.gov). Despite of this, many challenges remain, and joint efforts from the government, the scientists, the industry, and the clinicians need to be made to further advance the gene therapeutic field in China.

To recognize the efforts as well as challenges in this specific field in China, we were fortunate to have submissions invited from active scientists and clinicians in this field, and a special issue organized focusing on gene therapy in China. In this special issue, we overviewed the developmental history of gene therapeutics in China since the 20th century, with focuses on the main disease types in the gene therapeutic pipelines, technical advances including the development of various gene and base editing tools, as well as the evolution of policies [2, 3]. We also invited specialists involved in different aspects—target identification, discovery of new AAV vectors, setup of clinical trials—in gene therapy field, and discussed the recent advances as well as future perspectives focusing on a variety of diseases, including osteoarthritis [4], auditory diseases [5, 6], neuromuscular disorder [7], and cardiovascular diseases [8]. Besides, we are very happy to present three original articles, showcasing the efforts in establishing a new approach in which a natural polyphenol was applied to assist delivery of single-strand oligonucleotides [9], the use of AAV vectors in activating Cas9 expression and performing gene editing in neonatal and adult murine inner ear through micro-injection [10], and the application of gene therapy in treating Bietti crystalline dystrophy, a genetic disease with higher prevalence in Chinese and Japanese than other populations [11]. With these efforts, we hope this spotlight edition can provide a glance at the past and the present of gene therapy in China.

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Therapy as a generous and inclusive platform for introduction and communication among different regions around the world.

Compliance with ethical standards

Conflict of interest The author declare that she has no conflict of interest.

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