

CLAIMS

1. A method for targeted editing of target RNA in a cell based on LEAPER technology, wherein the target RNA is an RNA containing a G to A mutation in a USH2A gene transcript, comprising:

introducing a construct comprising an adenosine deaminase recruiting RNA (arRNA) for editing the target RNA or a construct encoding the arRNA into the cell, wherein the arRNA comprises a complementary RNA sequence that hybridizes to the target RNA, and wherein the arRNA is capable of recruiting adenosine deaminase acting on RNA (ADAR), thereby the target adenosine in the target RNA is deaminated.

2. The method according to claim 1, wherein the target RNA is a pre-mRNA.

3. The method according to claim 1 or 2, wherein the length of the arRNA is about 151-61 nt.

4. The method according to any one of claims 1-4, wherein the length from the targeting base to the 3' end in the arRNA is ≥ 7 nt, and/or, wherein the length from the targeting base to the 5' end in the arRNA is ≥ 25 nt.

5. The method according to any one of claims 1-4, wherein the target RNA is an RNA comprising a transcribed human USH2A gene NM_206933.2(USH2A)_c.11864 G>A (p.Trp3955Ter) mutation site.

6. The method according to any one of claims 1-5, wherein the arRNA is selected from the group consisting of: SEQ ID NO: 23, SEQ ID NO: 25, SEQ ID NO: 26, SEQ ID NO: 27, SEQ ID NO: 28, SEQ ID NO: 29, SEQ ID NO: 30, SEQ ID NO: 17, SEQ ID NO: 18, SEQ ID NO: 19, SEQ ID NO: 11, SEQ ID NO: 12, SEQ ID NO: 6, SEQ ID NO: 7, SEQ ID NO: 8, SEQ ID NO: 9, SEQ ID NO: 2, SEQ ID NO: 3, and SEQ ID NO: 4.

7. The method according to any one of claims 1-6, wherein the construct is a linear nucleic acid, a viral vector, or a plasmid, particularly, the viral vector is an adeno-associated virus (AAV) vector

or a lentiviral vector, more particularly, the AAV vector is introduced into the cell by infection after being packaged into an AAV2, AAV5 or AAV8 capsid.

8. The method according to any one of claims 1-7, wherein the cell is an optic nerve cell or auditory nerve cell of a mammalian.

9. An arRNA for targeted editing of the target RNA containing a G to A mutation in a USH2A gene transcript, comprising or consisting of a sequence selected from the group consisting of: SEQ ID NO: 23, SEQ ID NO: 25, SEQ ID NO: 26, SEQ ID NO: 27, SEQ ID NO: 28, SEQ ID NO: 29, SEQ ID NO: 30, SEQ ID NO: 17, SEQ ID NO: 18, SEQ ID NO: 19, SEQ ID NO: 11, SEQ ID NO: 12, SEQ ID NO: 6, SEQ ID NO: 7, SEQ ID NO: 8, SEQ ID NO: 9, SEQ ID NO: 2, SEQ ID NO: 3, and SEQ ID NO: 4.

10. A construct or delivery vector comprising the arRNA according to claim 9, particularly, the construct or delivery vector is a plasmid, virus, liposome, or lipid nanoparticle, more particularly, the construct or delivery vector is an adeno-associated virus (AAV) or a lentivirus, even more particularly, the adeno-associated virus (AAV) is AAV2, AAV5, or AAV8.

11. A cell obtained by the editing method according to any one of claims 1-8.

12. The arRNA according to claim 9 or the construct or delivery vector according to claim 10 for use in correcting a G to A mutation associated with Usher type II syndrome in a cell of an individual.

13. The use according to claim 12, wherein the arRNA according to claim 9 or the construct or delivery vector according to claim 10 is introduced into a subject.

14. The use according to claim 13, wherein the arRNA according to claim 9 or the construct or delivery vector according to claim 10 is introduced into the subretinal space of the subject.

15. Use of the arRNA according to claim 9 or the construct or delivery vector according to claim 10 in the preparation of a medicament for treating Usher type II syndrome.