CLAIMS

1. A splice-switching oligonucleotide (SSO) that specifically binds to a site within a target region present on a pre-mRNA transcript of a variant of the *OTC* gene, the target region comprising the sequence of SEQ ID NO: 31, wherein binding of the SSO induces the exclusion of OTC-PE5 from a mature mRNA transcript of the variant of the *OTC* gene.

2. The SSO of claim 1, wherein OTC-PE5 comprises the sequence of SEQ ID NO: 33.

3. The SSO of claims 1 or 2, wherein the target region comprises the sequence selected from the group consisting of SEQ ID NOs 37 and 39.

4. The SSO of any one of claims 1 to 3, comprising a sequence selected from the group consisting of SEQ ID NOs 1 to 29 and 49 to 60.

5. The SSO of any one of claims 1 to 4, wherein the SSO is between 17 and 34 nucleotides in length.

6. The SSO of any one of claims 1 to 5, wherein at least one of the nucleotides of the SSO is chemically modified and wherein the chemical modification is 2’-O-methyl RNA modification, 2'-O-methoxyethyl RNA modification, locked nucleic acid substitution or phosphorothioate linkage.

7. The SSO of any one of claims 1 to 6, wherein each nucleotide of the SSO comprises either a 2’-O-methyl RNA modification, a 2'-O-methoxyethyl RNA modification or a locked nucleic acid substitution.

8. The SSO of any one of claims 1 to 7, wherein the SSO comprises phosphorothioate linkages between all nucleotides of the SSO.

9. An SSO of any one of claims 1 to 8 for use in treating an ornithine transcarbamylase deficiency.

10. The SSO for use according to claim 9, wherein the ornithine transcarbamylase deficiency is caused by a c.540+265G>A mutation in the OTC gene.

11. Use of an SSO of any one of claims 1 to 8 in the manufacture of a medicament for treating an ornithine transcarbamylase deficiency.

12. The use of claim 11, wherein the ornithine transcarbamylase deficiency is caused by a c.540+265G>A mutation in the OTC gene.

13. A pharmaceutical composition comprising (a) a therapeutically effective amount of an SSO according to any one of claims 1 to 8 and (b) one or more pharmaceutically acceptable carriers and/or diluents.

14. An ex vivo method of exon-skipping comprising providing an SSO that specifically binds to a site within a target region present on a pre-mRNA transcript of a variant of the *OTC* gene, wherein the target region comprises the sequence of SEQ ID NO: 31 and wherein binding of the SSO induces the exclusion of OTC-PE5 from a mature mRNA transcript of the variant of the *OTC* gene.

15. The method of claim 14, wherein the pre-mRNA transcript comprises a c.540+265G>A mutation.

16. The method of claims 14 or 15, wherein OTC-PE5 comprises the sequence of SEQ ID NO: 33.

17. The method of any one of claims 14 to 16, wherein the target region comprises the sequence selected from the group consisting of SEQ ID NOs 37 and 39.

18. The method of any one of claims 14 to 17, comprising providing an SSO having a sequence selected from the group consisting of SEQ ID NOs 1 to 29 and 49 to 60.

19. The method of any one of claims 14 to 18, comprising providing an SSO that is between 17 and 34 nucleotides in length.

20. The method of any one of claims 14 to 19, comprising providing an SSO with at least one of the nucleotides of the SSO being chemically modified and wherein the chemical modification is 2'-O-methyl RNA modification, 2'-O-methoxyethyl RNA modification, locked nucleic acid substitution or phosphorothioate linkage.

21. The method of any one of claims 14 to 20, comprising providing an SSO with each nucleotide having either a 2’-O-methyl RNA modification, a 2'-O-methoxyethyl RNA modification or a locked nucleic acid substitution.

22. The method of any one of claims 14 to 21, comprising providing an SSO comprising phosphorothioate linkages between all nucleotides of the SSO.