

CLAIMS

1. A promoter comprising (i) a nucleotide sequence having at least 70% identity to SEQ ID NO: 4 or 17 and wherein the promoter has a length of about 1.1 kb or less.
2. A promoter which comprises or consists of a nucleotide sequence according to SEQ ID NO: 1 but wherein:
 - (i) position 1 to position n1 of SEQ ID NO: 1 is deleted, wherein n1 is an integer from 100 to 430; and/or
 - (ii) position n2 to position n3 of SEQ ID NO: 1 is deleted, wherein $n3 \geq n2$, n2 is an integer from 508 to 1061, and n3 is an integer from 508 to 1061;or a nucleotide sequence with at least 70% identity thereto.
3. The promoter according to claim 2, wherein:
 - (iii) the position corresponding to position 493 of SEQ ID NO: 1 is G, the position corresponding to position 1080 of SEQ ID NO: 1 is T, the position corresponding to position 1169 of SEQ ID NO: 1 is C, and the position corresponding to position 1249 of SEQ ID NO: 1 is G.
4. The promoter according to claim 2 or 3, wherein the promoter comprises (i) a nucleotide sequence having at least 70% identity to SEQ ID NO: 4 or 17 and/or wherein the promoter has a length of about 1.1 kb or less.
5. The promoter according to any preceding claim, wherein the promoter is a kidney-specific promoter.
6. The promoter according to any preceding claim, wherein the promoter further comprises (ii) a nucleotide sequence having at least 70% identity to SEQ ID NO: 5 or 18, a nucleotide sequence having at least 70% identity to SEQ ID NO: 6 or 19, and/or a nucleotide sequence having at least 70% identity to SEQ ID NO: 7 or 20.
7. The promoter according to any preceding claim, wherein the promoter further comprises (iii) a nucleotide sequence having at least 70% identity to SEQ ID NO: 8, or one or more fragments thereof.
8. The promoter according to any preceding claim, wherein the promoter comprises from 5' to 3': (i) a nucleotide sequence having at least 70% identity to SEQ ID NO: 4 or 17; (iii) optionally a nucleotide sequence having at least 70% identity to SEQ ID NO: 8, or one or more

fragments thereof; and (ii) a nucleotide sequence having at least 70% identity to SEQ ID NO: 5 or 18, a nucleotide sequence having at least 70% identity to SEQ ID NO: 6 or 19, and/or a nucleotide sequence having at least 70% identity to SEQ ID NO: 7 or 20.

9. The promoter according to any preceding claim, wherein the promoter has a length of about 1.0 kb or less, about 0.9 kb or less, about 0.8 kb or less, about 0.7 kb or less, about 0.6 kb or less, about 0.5 kb or less, about 0.4 kb or less, or about 0.3 kb or less.

10. The promoter according to any preceding claim, wherein the promoter has a length of from 0.265 kb to 1.0 kb, from 0.265 kb to 0.9 kb, from 0.265 kb to 0.8 kb, from 0.265 kb to 0.7 kb, from 0.265 kb to 0.6 kb, from 0.265 kb to 0.5 kb, from 0.265 kb to 0.4 kb, or from 0.265 kb to 0.3 kb.

11. The promoter according to any preceding claim, wherein the promoter comprises:

- (a) a retinoic acid receptor binding site;
- (b) a WT1 binding site;
- (c) an enhancer box;
- (d) a transcription factor binding region; and/or
- (e) a transcription initiation site.

12. The promoter according to claim 11 wherein the retinoic acid receptor binding site comprises or consists of a nucleotide sequence shown as SEQ ID NO: 10 or a nucleotide sequence having one or two substitutions, deletions, or insertions compared to SEQ ID NO: 10.

13. The promoter according to claim 11 or 12 wherein the WT1 binding site comprises or consists of a nucleotide sequence shown as SEQ ID NO: 11 or a nucleotide sequence having one, two or three substitutions, deletions, or insertions compared to SEQ ID NO: 11.

14. The promoter according to any one of claims 11 to 13 wherein the enhancer box comprises or consists of a nucleotide sequence shown as SEQ ID NO: 12 or a nucleotide sequence having one or two substitutions, deletions, or insertions compared to SEQ ID NO: 12.

15. The promoter according to any one of claims 1, or 4 to 14 wherein (i) the nucleotide sequence having at least 70% identity to SEQ ID NO: 4 or 17 comprises one or more of: (a) a retinoic acid receptor binding site; (b) a WT1 binding site; and (c) an enhancer box.

16. The promoter according to any one of claims 1, or 4 to 15 wherein one or more of the following nucleotide sequences is present in (i) the nucleotide sequence having at least 70% identity to SEQ ID NO: 4 or 17:

(a) GGGGTCA at a position corresponding to position 7 to position 13 of SEQ ID NO: 4 or 17;

(b) CGGAGGCTGGGGAGGCA at a position corresponding to position 14 to position 30 of SEQ ID NO: 4 or 17; and

(c) ATGTG at a position corresponding to position 49 to position 53 of SEQ ID NO: 4 or 17.

17. The promoter according to any one of claims 11 to 16 wherein the transcription factor binding region comprises or consists of a nucleotide sequence shown as SEQ ID NO: 13 or 43 or a nucleotide sequence having one, two, three, four or five substitutions, deletions, or insertions compared to SEQ ID NO: 13 or 43.

18. The promoter according to any one of claims 11 to 17, wherein the transcription initiation site comprises or consists of an "AG" dinucleotide.

19. The promoter according to any one of claims 11 to 18, wherein the transcription factor binding site is operably linked to the transcription initiation site, optionally wherein the transcription factor binding site is directly upstream of the transcription initiation site.

20. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 2 or SEQ ID NO: 3.

21. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 47.

22. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 90% identity to SEQ ID NO: 47.

23. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 95% identity to SEQ ID NO: 47.

24. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 98% identity to SEQ ID NO: 47.

25. The promoter according to any preceding claim, wherein the promoter comprises or consists of a nucleotide sequence having at least 99% identity to SEQ ID NO: 47.
26. A promoter which consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 2.
27. A promoter which consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 3.
28. A promoter which consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 15 or 59.
29. A promoter which consists of a nucleotide sequence having at least 70% identity to SEQ ID NO: 47.
30. The promoter according to claim 29, wherein the promoter consists of a nucleotide sequence having at least 90% identity to SEQ ID NO: 47.
31. The promoter according to claim 29 or 30, wherein the promoter consists of a nucleotide sequence having at least 95% identity to SEQ ID NO: 47.
32. The promoter according to any of claims 29-31, wherein the promoter consists of a nucleotide sequence having at least 98% identity to SEQ ID NO: 47.
33. The promoter according to any of claims 29-32, wherein the promoter consists of a nucleotide sequence having at least 99% identity to SEQ ID NO: 47.
34. The promoter according to any of claims 29-32, wherein the promoter consists of the nucleotide sequence of SEQ ID NO: 47.
35. A polynucleotide comprising the promoter according to any one of claims 1 to 34.
36. The polynucleotide according to claim 35, wherein the promoter is operably linked to a protein coding sequence.
37. The polynucleotide according to claim 35 or 36, wherein the promoter is operably linked to a protein coding sequence encoding NPHS2 or a fragment and/or variant thereof; a COL4A3, COL4A4 or COL4A5 polypeptide, or a fragment or derivative thereof; or CFI, CFH or FHL-1, or a fragment and/or variant thereof.
38. The polynucleotide according to any of claims 35-37, wherein the promoter is operably linked to a protein coding sequence encoding NPHS2, or a fragment and/or variant thereof,

preferably wherein the protein coding sequence encodes an amino acid sequence which is at least 70%, at least 80%, at least 90%, at least 95%, at least 98% or at least 99% identical to SEQ ID NO: 55 or a fragment thereof.

39. The polynucleotide according to any of claims 35-37, wherein the promoter is operably linked to a protein coding sequence encoding a COL4A3, COL4A4 or COL4A5 polypeptide, or a fragment or derivative thereof, preferably wherein the protein coding sequence encodes an amino acid sequence which is at least 70%, at least 80%, at least 90%, at least 95%, at least 98% or at least 99% identical to one of SEQ ID NOs: 21-23 or a fragment thereof.

40. The polynucleotide according to any of claims 35-37, wherein the promoter is operably linked to a protein coding sequence encoding CFI, CFH or FHL-1, or a fragment and/or variant thereof, preferably wherein the protein coding sequence encodes an amino acid sequence which is at least 70%, at least 80%, at least 90%, at least 95%, at least 98% or at least 99% identical to one of SEQ ID NOs: 49, 51 or 53, or a fragment thereof.

41. The polynucleotide according to any of claims 35-40, wherein the protein coding sequence is operably linked to one or more further regulatory elements, such as a post-transcriptional regulatory element and/or a polyadenylation sequence.

42. A vector comprising the polynucleotide according to any one of claims 35 to 41.

43. The vector according to claim 42, wherein the vector is capable of transducing podocytes, optionally wherein the vector is capable of specifically transducing podocytes.

44. The vector according to claim 42 or 43, wherein the vector is a viral vector, such as an adeno-associated virus (AAV) vector, a lentiviral vector, a retroviral vector, an adenoviral vector, an adeno-associated viral vector, a herpes simplex viral vector, an alphaviral vector, a flaviviral vector, a rhabdoviral vector, a measles viral vector, a Newcastle disease viral vector, a poxviral vector, and a picornaviral vector, preferably wherein the vector is an AAV vector.

45. The vector according to claim 44, wherein the viral vector is in the form of a viral vector particle, preferably wherein the viral vector is in the form of an AAV vector particle.

46. The vector according to any one of claims 42 to 45, wherein the vector is the form of an AAV vector particle encapsidated by AAV3B, LK03, or AAV9 capsid proteins.

47. A cell comprising the polynucleotide according to any one of claims 35 to 41, or the vector according to any one of claims 42 to 46.

48. A pharmaceutical composition comprising the polynucleotide according to any one of claims 35 to 41, the vector according to any one of claims 42 to 46, or the cell according to claim 47.

49. A polynucleotide according to any one of claims 35 to 41, vector according to any one of claims 42 to 46, or cell according to claim 47 for use as a medicament.

50. Use of the promoter according to any one of claims 1 to 34 to drive expression of a coding sequence.

51. The use according to claim 50, wherein the expression is kidney-specific.