CLAIMS

1. Use of casimersen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation of the DMD gene that is amenable to exon 45 skipping, wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

2. The use according to claim 1, wherein the dose is to be administered at a dosage of about 30 mg/kg of body weight of the patient.

3. The use according to claims 1-2, wherein the dose is to be administered as a single dose.

4. The use according to claims 1-3, wherein the dose is to be administered once weekly.

5. The use according to any of the previous claims, wherein the patient has a mutation of the DMD gene that is selected from the group consisting of: exons 7 to 42, 12 to 42, 18 to 42, 44 to 46, 44 to 47, 44 to 48, 44 to 49, 44 to 51, 44 to 53, 44 to 55, 44 to 57, or 44 to 59, or exon 44.

6. The use according to any of the previous claims, wherein the patient is to be chronically administered casimersen.

7. The use according to any of the previous claims, wherein the patient is to be administered casimersen for at least 48 weeks.

8. The use according to any of the previous claims, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to administration of casimersen.

9. The use according to any of the previous claims, wherein casimersen or a pharmaceutically acceptable salt thereof is formulated as a pharmaceutical composition.

10. The use according to any of the previous claims, wherein casimersen or a pharmaceutically acceptable salt thereof is formulated as a pharmaceutical composition having a strength of about 50 mg/mL.

11. The use according to claim 10, wherein casimersen or a pharmaceutically acceptable salt thereof is formulated as a pharmaceutical composition having a strength of about 50 mg/mL and presented in a dosage form of about 100 mg/2 mL.

12. The use according to claim 11, wherein the dosage form is contained in a single-use vial.

13. The use according to claims 10-12, wherein casimersen or a pharmaceutically acceptable salt thereof is formulated as a pharmaceutical composition comprising casimersen or a pharmaceutically acceptable salt thereof and a pharmaceutically acceptable carrier.

14. The use according to claim 13, wherein the pharmaceutically acceptable carrier is a phosphate-buffered solution.

15. Use of casimersen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation of the DMD gene that is amenable to exon 45 skipping, wherein the medicament is for restoring an mRNA reading frame to induce exon skipping in the patient and wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

16. The use according to claim 15, wherein the dose is to be administered at a dosage of about 30 mg/kg of body weight of the patient.

17. The use according to claims 15-16, wherein the dose is to be administered once weekly.

18. The use according to claims 15-17, wherein the patient is to be administered casimersen for at least 48 weeks.

19. Use of casimersen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation of the DMD gene that is amenable to exon 45 skipping, wherein the medicament is for increasing dystrophin production in the patient and wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

20. The use according to claim 19, wherein the dose is to be administered at a dosage of about 30 mg/kg of body weight of the patient.

21. The use according to claims 19-20, wherein the dose is to be administered once weekly.

22. The use according to claims 19-21, wherein the patient is to be administered casimersen for at least 48 weeks.

23. The use of any of the previous claims, further comprising confirming that the patient has a mutation in the DMD gene that is amenable to exon 45 skipping prior to administering casimersen.

24. Casimersen or a pharmaceutically acceptable salt thereof for use in treating Duchenne muscular dystrophy (DMD) in a patient in need thereof, the patient having a mutation of the DMD gene that is amenable to exon 45 skipping, wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

25. Casimersen or a pharmaceutically acceptable salt thereof for use in restoring an mRNA reading frame to induce exon skipping in a patient with Duchenne muscular dystrophy (DMD) in need thereof, the patient having a mutation of the DMD gene that is amenable to exon 45 skipping, wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

26. Casimersen or a pharmaceutically acceptable salt thereof for use in increasing dystrophin production in a patient with Duchenne muscular dystrophy (DMD) in need thereof, the patient having a mutation of the DMD gene that is amenable to exon 45 skipping, wherein a dose of casimersen or a pharmaceutically acceptable salt thereof is to be administered to the patient.

27. Casimersen or a pharmaceutically acceptable salt thereof for use according to any one of claims 24 to 26, wherein the dose of casimersen or a pharmaceutically acceptable salt thereof to be administered to the patient is a single intravenous dose of casimersen of about 30 mg/kg once weekly.