CLAIMS

1. Use of a dose of golodirsen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for treating Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation of the DMD gene that is amenable to exon 53 skipping,.

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

3. The use according to claim 1, wherein the method increases dystrophin production in the patient.

4. The use according to claim 1, wherein the patient has a mutation of the DMD gene that is selected from the group consisting of: exons 3 to 52, 4 to 52, 5 to 52, 6 to 52, 9 to 52, 10 to 52, 11 to 52, 13 to 52, 14 to 52, 15 to 52, 16 to 52, 17 to 52, 19 to 52, 21 to 52, 23 to 52, 24 to 52, 25 to 52, 26 to 52, 27 to 52, 28 to 52, 29 to 52, 30 to 52, 31 to 52, 32 to 52, 33 to 52, 34 to 52, 35 to 52, 36 to 52, 37 to 52, 38 to 52, 39 to 52, 40 to 52, 41 to 52, 43 to 52, 42 to 52, 45 to 52, 47 to 52, 48 to 52, 49 to 52, 50 to 52, 54 to 58, 54 to 61, 54 to 63, 54 to 64, 54 to 66, 54 to 76, 54 to 77, and exon 52.

5. The use according to claim 1, wherein the patient is to be administered the medicament for at least 48 weeks.

6. The use according to claim 1, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament.

7. The use according to claim 1, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament and remains on corticosteroids during the administration of the medicament.

8. The use according to claim 1, wherein the medicament or a pharmaceutically acceptable salt thereof is formulated as a pharmaceutical composition.

9. Use of a dose of golodirsen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for restoring an mRNA reading frame to induce exon skipping in a patient with Duchenne muscular dystrophy (DMD) in need thereof who has a mutation of the DMD gene that is amenable to exon 53 skipping.

10. The use according to claim 9, wherein the dose is to be administered at a dosage of 30 mg/kg of body weight of the patient.

11. The use according to claim 9, wherein the patient has a mutation of the DMD gene that is selected from the group consisting of: exons 3 to 52, 4 to 52, 5 to 52, 6 to 52, 9 to 52, 10 to 52, 11 to 52, 13 to 52, 14 to 52, 15 to 52, 16 to 52, 17 to 52, 19 to 52, 21 to 52, 23 to 52, 24 to 52, 25 to 52, 26 to 52, 27 to 52, 28 to 52, 29 to 52, 30 to 52, 31 to 52, 32 to 52, 33 to 52, 34 to 52, 35 to 52, 36 to 52, 37 to 52, 38 to 52, 39 to 52, 40 to 52, 41 to 52, 43 to 52, 42 to 52, 45 to 52, 47 to 52, 48 to 52, 49 to 52, 50 to 52, 54 to 58, 54 to 61, 54 to 63, 54 to 64, 54 to 66, 54 to 76, 54 to 77, and exon 52.

12. The use according to claim 9, wherein the patient is to be administered the medicament for at least 48 weeks.

13. The use according to claim 9, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament.

14. The use according to claim 9, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament and remains on corticosteroids during the administration ofthe medicament.

15. The use according to claim 9, wherein exon skipping is measured by reverse transcription polymerase chain reaction (RT-PCR).

16. The use of claim 9, wherein the method increases dystrophin production in the patient.

17. The use according to claim 16, wherein the dystrophin production is measured by western blot analysis.

18. The use according to claim 16, wherein the dystrophin production is measured by immunohistochemistry (IHC).

19. Use of a dose of golodirsen or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for increasing dystrophin production in a patient with Duchenne muscular dystrophy (DMD) in need thereof who has a mutation of the DMD gene that is amenable to exon 53 skipping.

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

21. The use according to claim 19, wherein the patient has a mutation of the DMD gene that is selected from the group consisting of: exons 3 to 52, 4 to 52, 5 to 52, 6 to 52, 9 to 52, 10 to 52, 11 to 52, 13 to 52, 14 to 52, 15 to 52, 16 to 52, 17 to 52, 19 to 52, 21 to 52, 23 to 52, 24 to 52, 25 to 52, 26 to 52, 27 to 52, 28 to 52, 29 to 52, 30 to 52, 31 to 52, 32 to 52, 33 to 52, 34 to 52, 35 to 52, 36 to 52, 37 to 52, 38 to 52, 39 to 52, 40 to 52, 41 to 52, 43 to 52, 42 to 52, 45 to 52, 47 to 52, 48 to 52, 49 to 52, 50 to 52, 54 to 58, 54 to 61, 54 to 63, 54 to 64, 54 to 66, 54 to 76, 54 to 77, and exon 52.

22. The use according to claim 19, wherein the patient is to be administered the medicament for at least 48 weeks.

23. The use according to claim 19, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament.

24. The use according to claim 19, wherein the patient is on a stable dose of corticosteroids for at least 6 months prior to the administration of the medicament and remains on corticosteroids during the administration ofthe medicament.

25. The use according to claim 19, wherein the dystrophin production is measured by western blot analysis.

26. The use according to claim 19, wherein the dystrophin production is measured by immunohistochemistry (IHC).

27. A dose of golodirsen or a pharmaceutically acceptable salt thereof for use as a medicament.