

CLAIMS

1. A method of predicting the prognosis of spinal muscular atrophy (SMA), comprising:
 - measuring a neurofilament level in a biological sample obtained from a human subject having mutations in both copies of the SMN1 gene that lead to functional SMN protein deficiency; and
 - comparing the neurofilament level measured in the biological sample to a control, wherein the neurofilament level measured in the biological sample, as compared to the control, is predictive of the severity or type of SMA that the subject will develop.
2. The method of claim 1, wherein the biological sample is obtained from the human subject before initiation of treatment with nusinersen or a nusinersen salt, and wherein the neurofilament level measured in the biological sample, as compared to the control, is predictive of the severity or type of SMA that the human subject will develop in the absence of treatment.
3. The method of claim 1, wherein the biological sample is obtained from the human subject after initiation of treatment with nusinersen or a nusinersen salt, and wherein the neurofilament level measured in the biological sample, as compared to the control, is predictive of the severity or type of SMA that the subject will develop while receiving the treatment.
4. The method of claim 3, wherein the biological sample is obtained from the human subject at least two weeks after initiation of the treatment.
5. The method of claim 3, wherein the biological sample is obtained from the human subject at least two months after initiation of the treatment.
6. The method of any one of claims 1 to 5, wherein the control is a pre-established neurofilament cut-off value.

7. The method of any one of claims 1 to 5, wherein the control is the neurofilament level in a biological sample or biological samples obtained from one or more human subjects that do not have SMA.
8. The method of any one of claims 1 to 7, wherein the neurofilament is a neurofilament heavy chain.
9. The method of any one of claims 1 to 7, wherein the neurofilament is a phosphorylated neurofilament heavy chain.
10. The method of any one of claims 1 to 7, wherein the neurofilament is a neurofilament medium/intermediate chain.
11. The method of any one of claims 1 to 7, wherein the neurofilament is a neurofilament light chain.
12. The method of any one of claims 1 to 11, wherein the biological sample is blood, serum, plasma, or cerebrospinal fluid.