



Key inclusion criteria for Part 1	Key exclusion criteria for Part 1 ^c
<ul style="list-style-type: none"> Genetic documentation of 5q SMA homozygous gene deletion, mutation, or compound heterozygote 	<ul style="list-style-type: none"> Ventilator use ≥ 16 hours/day for >21 days at screening
<ul style="list-style-type: none"> Onset of SMA symptoms at ≤ 6 months with 3 <i>SMN2</i> copies <p style="text-align: center;">OR</p> <ul style="list-style-type: none"> Onset of SMA symptoms at ≤ 6 months, >7 months at screening, and 2 <i>SMN2</i> copies <p style="text-align: center;">OR</p> <ul style="list-style-type: none"> Onset of SMA symptoms at >6 months, ≤ 18 months at screening, and 2 or 3 <i>SMN2</i> copies 	<ul style="list-style-type: none"> Hospitalization for surgery, pulmonary event, or nutritional support within 2 months prior to screening

Transition to SHINE (NCT02594124); open-label extension study of nusinersen every 4 months for up to 5 years