## Nonclinical Data for PGN-EDODM1 Demonstrated Mechanistic and Meaningful Activity for the Potential Treatment of DM1



• Increased levels of tissue delivery, enhanced mis-splicing correction and reversal of myotonia were observed with repeat dosing in HSALR mice.

- PGN-EDODM1 was well tolerated in NHP GLP repeat-dose toxicity studies at 60 mg/kg; repeat dosing did not exacerbate increases in serum creatinine.
- FREEDOM-DM1 Phase 1 randomized, double-blind, placebo-controlled single ascending dose study in people with DM1 is enrolling in Canada, the UK and the US. FREEDOM2 Phase 2 randomized, double-blind, placebo-controlled multiple ascending dose study in people with DM1 is enrolling in Canada and the UK.

Nonclinical data in DM1 cells, HSA<sup>LR</sup> mice and NHP support the development of PGN-EDODM1 and the FREEDOM1-DM1 Phase 1 and FREEDOM2-DM1 Phase 2 clinical studies.