Conclusions

- ✓ **GT-02287 rescued cultured rat dopaminergic neurons injured with α-synuclein PFFs, both with and without GCase activity lowering using CBE**
- ✓ **GT-02287 restored motor function in a mouse GBA1-PD model, even when treatment began several days after the initial toxic insult**
- ✓ **Rescue of locomotor impairment was reflected in decreased plasma levels of NfL suggesting a neuroprotective effect**
- ✓ **Animals in the most challenging treatment group (treatment beginning day 8 after toxic insult) showed a motor improvement from day 14 to day 27, suggesting progressive reversal of motor deficit associated with continued GT-02287 treatment duration**
- ✓ **These data support the potential of GT-02287 as a disease-modifying therapy for the treatment of PD that is already clinically established, as well as other diseases involving GCase and lysosomal dysfunction, such as Gaucher disease**