

1.

Our results suggest that metabolic changes precede muscle wasting in Pompe disease, which would promote increased cytoplasmic glycogen synthesis, resulting in aggravation of the disease phenotype. *This thesis.*

2.

In light of the failing muscle stem cell activation together with the absence of cell-intrinsic defects, we hypothesize that the muscle stem cell niche in Pompe disease suppresses or fails to generate signals that activate stem cells. This could suggest the niche as a novel therapeutic target in Pompe disease. *This thesis.*

3.

A deep understanding of the mechanisms that govern stem cell-mediated regeneration in skeletal muscle is paramount for their application for regenerative therapies. *This thesis*

4.

The main problem to date for the development of cell therapies for skeletal muscle is the requirement of regenerative cells that can be generated at sufficient numbers for therapeutic application, which so far remains elusive. *This thesis.*

5.

Reserve cells represent a valuable tool to decipher pathways mediating skeletal muscle regeneration, which would ultimately benefit the development of cell therapies for muscle disorders. *This thesis.*

6.

Questions of affordability and projected efficacy of cell therapy strategies should be considered against the backdrop of competing state-of-the-art advances, such as gene therapy or AONs, which

have also shown tremendous promise in correcting genetic defects in pre-clinical myopathy models and are entering the clinic. Judson and Rossi 2020

7.

It is critically necessary to show clear and significant clinical benefit in phase II studies because the heterogeneity of human disease in more extensive phase III and IV studies will often erode the significance of minor benefit apparent in early trials. Trounson and McDonald 2015.

8.

“We seem to arrive at the ridiculous conclusion that the clue to the understanding of life is that it is based on a pure mechanism”. Erwin Schrodinger, *What is life?*

9.

“Carbon is not a man, nor salt nor water nor calcium. He is all these, but he is much more, much more...” John Steinbeck, *The grapes of wrath*.

10.

“The highest goal that a man can achieve is amazement”. Johann Wolfgang von Goethe, *Theory of colours*

11.

Those who dance are considered mad by those who cannot hear the music. Unknown.