Research Grant Recipient:
Helen Cristina Miranda, Postdoctoral Researcher, La Spada Lab

Research Proposal:
Recent findings from our lab predict that muscle-directed therapies hold great promise as definitive treatments for SBMA motor neuron degeneration. Therefore, our goal in this proposal will be to use our well-established human SBMA induced pluripotent stem cell (iPSC) model system to generate a human-based muscle toxicity model and recapitulate the non-cell autonomous pathogenesis in SBMA. We will co-culture the iPSC-derived muscles with our iPSC-derived motor neurons cells to determine muscle-dependent toxicity by assaying motor neuron survival and mitochondrial membrane potential in the respective co-culture conditions. Our iPSC model for SBMA will allow us to use a stem cell approach to evaluate muscle-dependent non-cell autonomous motor neuron degeneration for the first time.