

Accepted Manuscript

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PII: S0142-9612(18)30147-9

DOI: [10.1016/j.biomaterials.2018.02.047](https://doi.org/10.1016/j.biomaterials.2018.02.047)

Reference: JBMT 18520

To appear in: *Biomaterials*

Received Date: 6 December 2017

Revised Date: 20 February 2018

Accepted Date: 24 February 2018

Please cite this article as: Santhanam N, Kumanchik L, Guo X, Sommerhage F, Cai Y, Jackson M, Martin C, Saad G, McAleer CW, Wang Y, Lavado A, Long CJ, Hickman JJ, Stem cell derived phenotypic human neuromuscular junction model for dose response evaluation of therapeutics, *Biomaterials* (2018), doi: 10.1016/j.biomaterials.2018.02.047.

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Stem cell derived phenotypic human neuromuscular junction model for dose response evaluation of therapeutics

Running Title: Human NMJ systems for therapeutic dose response evaluation

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Abstract:

There are currently no functional neuromuscular junction (hNMJ) systems composed of human cells that could be used for drug evaluations or toxicity testing in vitro. These systems are needed to evaluate NMJs for diseases such as amyotrophic lateral sclerosis, spinal muscular atrophy or other neurodegenerative diseases or injury states. There are certainly no model systems, animal or human, that allows for isolated treatment of motoneurons or muscle capable of generating dose response curves to evaluate pharmacological activity of these highly specialized functional units. A system was developed in which human myotubes and motoneurons derived from stem cells were cultured in a serum-free medium in a BioMEMS construct. The system is composed of two chambers linked by microtunnels to enable axonal

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