



## SUBCONTRACT EXECUTIVE SUMMARY

### A 96-well Sandwich ELISA for SMN<sup>1</sup>

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Spinal muscular atrophy (SMA) is caused by the absence of functional survival motor neuron (SMN) protein produced from the *SMN1* gene. Survival of SMA patients is dependent on low levels of SMN produced by the *SMN2* gene. In general, the severity of SMA is closely related to the level of SMN protein produced by the *SMN2* gene. Therefore, drugs that elevate SMN levels could provide a way of reducing disability and prolonging life in SMA patients. A quantitative, sensitive immunoassay using antibodies targeted to SMN is needed for the measurement of SMN levels in SMA cell lines and animal models, and potentially in cells and tissues of SMA patients.

The overall goal of this project is to develop an enzyme-linked immunosorbent assay (ELISA)-based protein test kit for the reliable and quantitative detection of SMN protein.

The tasks/objectives and associated milestones of this project are:

- 1) Generate ELISA reagents, establish methods, and test pre-kits.  
*Milestone:* Feasibility demonstrated.
- 2) Generate ELISA kits for evaluation on fibroblasts.  
*Milestone:* Design approved by in vitro bioassay facility.

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<sup>1</sup> A proposal to support this subcontract was submitted to the SMA Project's JL-07604-1, "A Protein Detection Kit for Quantifying Survival Motor Neuron (SMN) Protein." SAIC provides management support for The SMA Project to the NINDS through contract N01-NS-3-2356.