



Subcontract Executive Summary

***In Vitro* Biological Assay Support for SMA ¹**

CombinatoRx, Incorporated
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CombinatoRx will provide the SMA Project with *in vitro* assay implementation and compound screening capabilities to support iterative chemical optimization efforts to identify compounds with improved bioactivity, safety, and pharmacokinetic (ADME) characteristics for development of an SMA therapeutic. Planned activities include validation of assays, moderate and low throughput compound screening according to a routine schedule, and data entry and analysis. As the core SMA Project *in vitro* bioassay facility, CombinatoRx will also be responsible for entering biological data generated by other facilities into the SMA Project's centralized informatics system.

Additional capabilities that CombinatoRx may provide for SMA Project's virtual drug discovery and development enterprise are improvements to existing assays to increase the efficiency and reliability of compound screening, preparation of biological samples (e.g., plated cells), for assays to be performed at other SMA Project and/or collaborator facilities, development of new screening assays, and the processing and testing of biological specimens.

¹ A proposal to support this subcontract was submitted to the SMA Project's JL-31404-11, "An *In Vitro* Biological Assay Facility for Screening Compounds in Cellular Models of Spinal Muscular Atrophy." SAIC provides management support for The SMA Project to the NINDS through contract N01-NS-3-2356.