UNIVERSITY OF CALIFORNIA, DAVIS

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Department of Physical Medicine
and Rehabilitation

Infants are needed...

for an observational research study to identify
Biomarkers in Spinal Muscular Atrophy

Researchers have discovered that there is a "critical time period" when treatment is effective in SMA mice. At this time, no one knows if such a critical time period exists in humans. Biomarker studies have been done in patients 2 years of age and older. In this study, we will focus on infants with SMA to determine whether this "critical time point" exists in babies. The goal of the SMA Biomarker study is to identify laboratory measurements that can be used in future SMA clinical trials.

We hope to learn how babies with SMA develop compared to their age matched peers.
We therefore need two different types of "Super Babies" younger than 6 months of age for this study:

1) Infants who have been diagnosed with Spinal Muscular Atrophy
2) Infants with no medical condition

We need both SMA and healthy infants to be "Super Babies for SMA" and *Show Us The Way* concerning:
• When treatments in SMA should be given
• What happens to SMN protein levels as children grow
• What tests will be required for SMA clinical trials of the future

<u>What will participants be asked to do?</u>

- Depending on age spend about 3.5 hours on seven occasions in the Exercise Physiology Lab in Lawrence J. Ellison Ambulatory Care Center located at 4860 "Y" St. Suite 1113
- Motor function tests
- Non-invasive tests to measure the function of nerves and muscles
- A blood draw that is performed on most, but not all, of the study visits. It is done after numbing cream is applied.

Participants will receive a stipend of $50 for each visit to offset travel expenses.

If you have any questions or are interested in this study, please contact:
Dr. Craig McDonald (Principal Investigator)
Study Coordinator: Randev Sandhu (916) 734-4303

Partners Human Research Committee
APPROVAL Effective Date
February 22, 2013

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