SMA Infant Biomarker Study – What, Why and How

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What is a Biomarker?

A Biomarker is any laboratory measurement that reflects the activity or stage of a disease process. For example, cholesterol levels reflect cardiovascular disease risk. Biomarkers are being used effectively for cancer, diabetes, and heart disease to help determine whether new drugs are helping patients, and to better understand the diseases.

What?

The goal of the SMA Biomarker Study is to identify laboratory measurements that can be used in SMA clinical trials.

Why are we studying infants?

Why?

Researchers have discovered that there is a “critical time period” when treatment is most effective in SMA mice. In mice, this time period is immediately after birth. The same therapy given to the mice at a later time period is much less effective. At this time, no one knows if such a critical time period exists in humans.

That’s why we need both SMA and healthy infants to *Show Us The Way*.

- To *Show Us The Way* to which tests will be the most informative for SMA clinical trials of the future.
- To *Show Us The Way* to how healthy infants develop and identify important biomarkers that will be useful in multiple infant studies of the future, including studies that do not involve SMA.

How can you get involved?

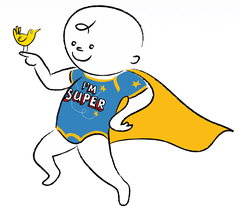
If you are a parent with an infant who is less than 6 months of age who has been diagnosed with SMA, or if you have an infant with no known medical condition, then your infant may be eligible to enroll in the SMA Infant Biomarker Study.

Please contact Amy Bartlett, CCRC at
1-855-762-2466 or 1-855-SMA-BIOM

YouTube

Please help us spread the word about this important study by sharing our informative YouTube™ video. Just type “SMA Biomarker Video” into your search engine.

How?

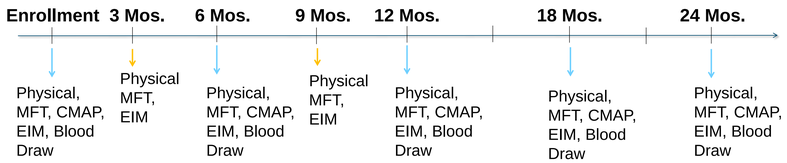


What procedures are involved for your child?

You and your child will come to a study site for regular study visits based on your child’s age. At these visits a variety of procedures will be preformed including:

1) motor function tests
2) noninvasive tests to measure the function of nerves and muscles
3) a blood draw that is performed on most of the study visits

Timeline of Study Procedures



*Motor Function Test (MFT), Compound Motor Action Potentials (CMAP), Electrical Impedance Myography (EIM)

Where are the SMA Infant Biomarker Enrolling Sites?

East:
- Boston Children’s Hospital, Boston, MA
- Children’s National Medical Center, Washington, DC
- Columbia University Medical Center, New York, NY
- State University of New York Upstate Medical Center (SUNY), Syracuse, NY

Midwest:
- Ann & Robert H Lurie Children’s Hospital of Chicago, Chicago, IL
- Nationwide Children’s Hospital, Columbus, OH
- Washington University in St. Louis School of Medicine, Saint Louis, MO

South:
- Children’s Healthcare of Atlanta, Atlanta, GA
- Children’s Medical Center, Dallas, TX
- Vanderbilt University, Nashville, TN

West:
- Children’s Hospital Colorado, Aurora, CO
- Doernbecher Children’s Hospital, Portland, OR
- University of California – Davis, Davis, CA
- University of California – Los Angeles, Los Angeles, CA
- University of Utah, Salt Lake City, UT

The SMA Infant Biomarker Study is the premier study of the NIH-Network for Excellence in Neuroscience Clinical Trials

NeuroNEXT

This study originates from The Ohio State University Wexner Medical Center and Nationwide Children’s Hospital in Columbus, OH

Wexner Medical Center

Nationwide Children's — When your child needs a hospital, everything matters.

This study is sponsored by the NINDS and Families of SMA and receives support from WryBaby.com

NIH National Institute of Neurological Disorders and Stroke

Families of SMA

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