

SMA Clinical Trials Stanford University



Introduction

This pamphlet is intended strictly as an informational resource for patients interested in participating in clinical trials relevant to Spinal Muscular Atrophy (SMA). Reading this pamphlet or asking further questions about our research does not in any way oblige you to participate in the trials.

What is a clinical trial?

A clinical trial takes place after years of laboratory research investigating the cause and treatment of a disease.

After identifying the cause of a disease, researchers and physicians can design potential treatments. These treatments can be new drugs, or apply old drugs in new ways.

Clinical trials test treatments in human volunteers in order to check their safety and effectiveness before they can be approved for use in treating the general population.

What is the purpose of these SMA clinical trials at Stanford?

These clinical trials are aimed at obtaining data on the safety and effectiveness of some medications that may help the symptoms of SMA patients.

In addition, these trials hope to identify some reliable tools to measure the clinical effects of these treatments.

Who is eligible for the trial?

Two groups of patients with SMA will be recruited for these studies.

1. Type I SMA patients less than two years of age.
2. Type II SMA and severe Type III SMA patients between 2 and 8 years of age (those never achieve independent walking and those who were able to walk but need some form of assisted device before the age of 6 years).

What are my child's requirements for participation?

Full participation in either study will involve a time commitment of at least one month of baseline observation and six months of treatment.

Both studies will require periodic physical exams and blood draws from your child. These will be used to monitor possible side effects of the treatment as well as for analysis in our research lab.

There will also be tests performed to measure your child's muscle strength and breathing capacity.

There will be no cost for the family to participate in these studies. The investigators will pay for all procedures related to the trials (blood tests, physical therapy, etc.). However, you will be responsible for costs of routine care for your child.

Who is funding these projects?

This project is partially funded by

1. The Spinal Muscular Atrophy Foundation (SMAF)
2. The Muscular Dystrophy Association (MDA), USA

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