WHAT YOU SHOULD KNOW ABOUT PARTICIPATING IN CLINICAL TRIALS
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What does the process of developing a new medical treatment entail?
The medical research process begins with a basic investigation of how a therapeutic intervention (drug or other treatment) might work for a particular disease. Early on, the intervention may be tested in human cells and/or animals. When studies in cells and animals provide sufficient evidence that a particular intervention is likely to be a safe and effective treatment for a disease, the company or organization developing the therapeutic intervention files an application with the Food and Drug Administration (FDA) to obtain permission to begin testing in human subjects through a clinical trial.

What is a clinical trial?
Clinical trials are studies conducted using human participants and designed to explore and answer questions related to a disease and to examine potential new therapies. Clinical trials that examine potential new therapies are generally categorized into a series of carefully designed phases:

- **Phase I trials** – initial studies to determine the safety and pharmacokinetics of drugs in humans, and the side effects associated with increasing doses; in some cases can also be used to gain early evidence of effectiveness. Healthy participants and/or patients may be recruited for this first phase of clinical trials.

- **Phase II trials** – clinical studies conducted to evaluate the effectiveness of a drug for a particular disease or signs or symptoms in patients with the disease or condition under study, and to determine the common short-term side effects and risks associated with dosing.

- **Phase III trials** – expanded trials which take place after review of preliminary safety and efficacy data from Phases I and II; the intent of Phase III trials is to gather additional information to evaluate the overall benefit-risk relationship of the drug. After Phase III is completed, a New Drug Application (NDA) or Biologic License Application (BLA) can be submitted to the FDA for approval of the drug for marketing.

- **Phase IV trials** – post-marketing (i.e. post-approval) studies designed to provide additional information on the drug which may include the drug’s risks, benefits, and optimal use. These studies may be voluntary or required by regulatory agencies as part of their approval for marketing.

In clinical trials testing therapeutics developed to treat a disease that primarily affects children, extra measures are taken to protect pediatric patients and their growing bodies. Phase I in the trial may involve healthy, adult volunteers rather than children. If the drug is well tolerated in adults, Phase II may begin with a small number of pediatric patients. If the drug can demonstrate safety and efficacy in this group of patients, additional pediatric patients may participate in the Phase III trial.
How can I find out more about pediatric clinical trials?
For general information about pediatric clinical trials, the National Institutes of Health has created a family-friendly website, Children & Clinical Studies. The site presents interviews with children, parents and physicians who have worked together on clinical trials. Questions addressed include: Will my child benefit from the study? What if I want to say no? What happens when the study ends?

Why are clinical trials important?
Carefully conducted clinical trials are the most efficient and safest way to determine whether a treatment works in patients.

Why participate in a clinical trial?
Patients who participate in clinical trials have an active role in their medical care and a chance accessing potential treatments. Patients enrolled in clinical trials also make an important contribution to the field of medicine, since information learned from clinical trial participants can be used to help others.

How can patients participate in clinical trials?
As a patient or a parent, the decision to participate in a clinical trial should be thoroughly evaluated. For more information about clinical trials, including a more complete explanation of what they are, questions to consider and ask before participating in a trial, and information about studies involving a specific disease, please visit clinicaltrials.gov and ciscrp.org. Clinicaltrials.gov was developed by the National Library of Medicine and is a service of the National Institute of Health (NIH). The Center for Information & Study on Clinical Research Participation (CISCRP) is a non-profit organization focused on educating and informing the public about clinical research participation.

What clinical trials are available or approaching for SMA?
Please visit the clinicaltrials.gov and search for “spinal muscular atrophy” in the “Search for Clinical Trials” search engine to find more information about enrolling or ongoing clinical trials in SMA.