

Evaluating the Clinical Trial Option

QUESTIONS TO CONSIDER



INTRODUCTION

Clinical trial is a medical term for a controlled experiment designed to address questions about a condition or disease. The trial is designed according to the best data the researchers have from previous experiments with the treatment or therapy conducted on animal models (e.g., mice). ***Individuals in a clinical trial are voluntary participants in this experiment.***

It is important to have as much information as possible when considering whether to enroll your child in a clinical trial. Here are some questions to discuss with health care providers and/or study coordinators as part of the decision-making process.



Questions About the Study or Trial Design or Process

- What is this research about and why is it being done?
- What do researchers hope to learn?
- Why do the researchers think this new approach/treatment will be effective?
- What phase is the study in? (Different phases mean different stages of research including never tested in humans.)
- How long will the study last?
- What government agencies or individuals have reviewed and approved the study? Is this a Federal Drug Administration (FDA) or European Medicines Agency (EMA) approved study?
- What are the names and roles of the professionals involved in the study?
- What experience do the researchers, institution or sponsor involved in the study have?
- Who is funding the study?
- Where will the study take place? In a hospital? Is it inpatient (will my child be hospitalized)? Or is the study conducted on an outpatient basis and my child will be able to be at home?
- How and when will the results of the study be shared?

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- How will my child's privacy be protected during the study?
- Who do I contact if I have questions about the study?
- What happens if the researchers discontinue the study?
- What is the process if I decide to end my child's participation in the study?
- What is the phase of the study? (e.g. Phase I, Phase II, Phase III)
 - How many groups/arms/dose levels are there?
 - Will any group receive a placebo or an inactive treatment?
 - If yes, is there a Rescue Clause so that if my child gets progressively worse while on placebo they may move to the treatment arm of the study.
 - How are the participants in each group/arms/dose level determined?



Questions About Risks and Benefits

- What are the possible benefits/results and how likely are they to occur?
- How much is known about the potential risks of the intervention/treatment?
- What are the possible short-term risks or side effects and how likely are they to occur?
- How do the risks and benefits of this study compare with those of other approved or experimental treatment options?
- Are there any known prior results (efficacy and safety) of the treatment?
- If my child participates in this trial, will s/he be eligible for future studies/trials or other treatments?



After reading the Informed Consent documents, ask yourself if you understand the study and feel comfortable with your child participating. If not, ask more questions.



Questions About Financial Considerations

- Are there costs that I will have to pay to participate in the study?
 - Will travel costs, relocation services, childcare or any other study-related cost be covered?
 - Will I need to pay expenses out of pocket and be reimbursed, or is everything handled for me directly by the research team?
- Will my health insurance be utilized to cover any study-related tests or procedures?
- Who will cover the costs of treatment if my child has an adverse reaction or is harmed while participating in the study? Or while traveling to or from the study?
- When the study ends, how will continuation of the treatment (if needed) be facilitated and how will it be paid for?

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Questions About Care

- What kinds of treatments, medical tests or procedures will occur during the study?
How often will these be done?
- Who will monitor my child's care and safety during the study?
- Will the treatments, tests or procedures be painful? (Am I willing to see my child in pain?)
- Will the results of tests or procedures be shared with me?
- Will my child continue to see their current medical team, or will I need to switch all care to the center where the study is being conducted?
- For how long after the study ends will the medical team/researcher continue to follow my child?



Questions About Lifestyle Considerations

- What are the logistics of participating? (Will we need to live near the center where the study is taking place?)
- What kinds of supports (financial, logistical, emotional) will be available to our family if our child participates?
- If there are siblings, how will they be supported during the study?



Questions About Personal Values and Priorities

- Is this the right study for my child, given his/her current medical condition?
- What is my family's goal for participating in this study? (Is it to improve my child's short- or long-term prognosis? Is it to improve quality of life? Is it to contribute to the advancement of science?)
- Am I willing to accept the risks associated with the study on behalf of my child?
- What are the alternatives to participating in the study?
- Am I willing to be away from my spouse or other children if that is required?
- Do I understand that the possible benefits of the treatment/intervention are not guaranteed?

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- Am I willing to accept that my child's disease progression will likely be changed by the treatment/intervention in ways that no one can fully predict?
- How will I feel if the trial is unsuccessful?
- How will I feel if my child is not accepted into the study due to criteria?



CONCLUSION

Accurate information, and as much of it as you can gather, is your roadmap to making the best decision for your child and your family. Always feel free to ask questions and to follow up on anything you don't understand.