



FOR YOUR INFORMATION

Thinking About Experimental Therapies

Should I consider trying an experimental treatment for my child/family member?

As new experimental treatments are emerging for rare diseases, you may be wondering if or when is the right time to think about trying a potential new therapy. You may have questions about the results of laboratory studies using the therapy in animals, and about whether such studies are relevant to use of the therapy in human patients.

This “FYI” is intended to give you a framework for thinking about these issues and for getting the information you need before making your decision about the experimental treatment. Please be aware that in some cases, there may be formal clinical trials for the experimental therapy in question, while in other cases, animal studies may suggest that the drug could have benefit to humans, but no formal studies have yet been done.

Some questions to ask about how animal research translates to use in humans:

Research Questions:

1. Has the treatment been studied in different animal models, and especially in one that is thought to mirror how it will act in humans?
2. Is the timing of administration of the drug developmentally similar in animals and humans? For example, if it is critical to deliver the drug early in the animal’s life in order to get results, is it possible to deliver the drug at a comparable age or time (from a developmental perspective) in humans.
3. Is it known exactly how the compound acts on the body, or do the researchers only see its effects (for example, changes in blood chemicals or in storage of materials) but not understand fully how these things happen?
4. Have the researchers determined the best dose of the treatment to use in humans – enough to have potentially beneficial effect, but not so much that it will cause problems like toxicity?
5. Have results of the research studies appeared in peer-reviewed journals? Has the work been successfully repeated by any other laboratories?

Efficacy/Effectiveness Questions:

1. Do researchers believe that the experimental treatment being tested may be effective in humans? Has it been tested before for another disease?
2. Does the drug cross the blood-brain barrier, and thus have a potential effect on the developing brain and central nervous system?
3. Does the drug in question act on all symptoms of the disease, or is it predicted to improve only some aspects of the disease?

4. Is the intervention expected to be a “cure,” i.e. is it expected to significantly improve all aspects of the disease, or is it a temporary intervention until another, better treatment can be found?

Risk Questions:

1. Are the risks of use of this drug known, or at least are the potential risks understood?
2. How do the possible risks, side effects, and benefits in the study compare with my current treatment?
3. Do the researchers think that the potential benefits warrant the risks involved in participating in the research? Do other independent experts in the field agree with their assessment of the risks and benefits?

Study Format:

1. Is the proposed treatment part of a formal study (clinical trial)? Check ClinicalTrials.gov to see if it is a formal clinical trial.
2. Does the effect of the experimental treatment last for a long time or a short time? Will multiple or frequent doses be needed to continue the effect of the drug?
3. Has a plan of follow up monitoring been defined for after the treatment?

Ethical Questions:

1. Do you understand that this experimental treatment could make things worse?
2. Who will pay for the experimental treatment?
3. Do you understand that using this experimental therapy may exclude you from other new treatments in formal clinical trials in the future?
4. Has the study been reviewed and approved by an Investigational Review Board charged with protecting human research subjects.
5. Who will pay for emergency medical care if something goes wrong?

When you or your children have a serious progressive disease for which there is only symptomatic relief but no sure or effective treatment, the temptation is to try almost anything, often on the slenderest of evidence, to prolong active life. It is important to remember that genuine medical discoveries are based on years of research performed at universities and pharmaceutical companies. There is a long process that shows first in laboratory studies and then in clinical research that something is safe and will work well.

There is an excellent publication on a website that originates in the United Kingdom. The site is called [Sense About Science](#) which has a wealth of information including a booklet titled [I've Got Nothing to Lose By Trying It – Weighing Up Claims About Cures and Treatments for Medical Conditions](#). The resources listed in the booklet are in the UK, but if you contact us, we can provide similar resources here in the US. We are also looking for a similar booklet published here. Also

available on the Sense about Science website is a booklet called [| Don't Know What to Believe... Making Sense of Science Stories](#) which is about how you can ask questions about the scientific information you read in the papers or hear on TV to help you understand if it is valid science.

There are also a number of US government-sponsored sites that can tell you more about **formally approved** clinical trials and many issues related to trial participation including questions to ask, what has lead up to the trial, how are participants protected, etc. Look to any of the following sites for additional information; each has many links to much about clinical trials.

[NIH Clinical Research Trials and You](#)

- The Basics
- Finding a Clinical Trial
- List of Registries
- Personal Stories
- For Parents and Children
- For Health Care Providers
- Resources for Trial Sites
- Educational Resources
- Glossary of Common Terms
- If You Have a Question
- In the News

[Learn About Clinical Studies](#)

- What Is a Clinical Study?
- Clinical Trials
- Observational Studies
- Who Conducts Clinical Studies?
- Where Are Clinical Studies Conducted?
- How Long Do Clinical Studies Last?
- Reasons for Conducting Clinical Studies
- Participating in Clinical Studies
- Who Can Participate in a Clinical Study?
- How Are Participants Protected?
- Relationship to Usual Health Care
- Considerations for Participation
- Questions to Ask

[Clinical Trials Information for Patients and Caregivers \(NIH National Cancer Institute\)](#)

Clinical trials: questions and answers
What is a clinical trial?
Should I take part in a clinical trial?
How do I take part in a clinical trial?
Participating in a trial: questions to ask your doctor
How is a clinical trial planned and carried out?
Protecting participants in clinical trials
Clinical trials education series
Protecting human research participants

[Should Your Child Be in a Clinical Trial?](#)

Why Clinical Trials in Children are Important
Potential Benefit
Informed Consent
Making a Decision
Withdrawing From a Clinical Trial
FDA Encourages Studies in Children

In addition to the information provided here, please don't hesitate to contact the NNPfD Family Services Coordinator if you need further assistance. We are always happy to help.

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