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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 several 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 www.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 institutional 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” at www.senseaboutscience.org.uk, and it has a wealth of information. On the left menu at this site, the first tab is “I’ve Got Nothing to Lose.” If you select that, you will be taken to a page where you can download a booklet about “weighing up claims about cures and treatments for long-term 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 on the Sense about Science home page, about half way down on the left, is a link to a booklet called “I Don’t Know What to Believe...” 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.

<http://clinicalresearch.nih.gov/>

- How does clinical research work?
- Who participates in clinical research?
- What do I need to know if I am thinking about participating?
- Where can I find a clinical trial?
- What happens after a clinical trial is completed?
- Ethical issues in clinical research.

<http://clinicaltrials.gov/>

- Understanding clinical trials
- Search for clinical trials
- Glossary

<http://www.cancer.gov/clinicaltrials/learning/>

- 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

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