Questions to Ask About a Drug Study or Clinical Trial

A patient-friendly guide for evaluating NF drug research and deciding what to ask before participating

How to use this guide

You do not need to ask every question at once. Mark the questions that matter most to your goals, safety, daily life, and other care options. Ask the study team to explain unfamiliar terms and provide important details in writing.

Consider discussing the study with your own NF clinician or another qualified healthcare professional who is not part of the research team.

01 Why this drug and this study?

1. What problem is this drug intended to treat, prevent, or better understand?
2. Why do researchers think this drug may work for my NF type, tumor type, or symptoms?
3. Does the drug target the underlying NF-related biology, the tumor, a symptom, or something else?
4. What laboratory, animal, or human evidence supports testing it now?
5. Has this drug been studied in people with NF, or mainly in people with sporadic tumors or another condition?

02 What is already known?

1. What phase is the study, and what is that phase designed to learn?

2. How many people have received this drug so far, including people outside this study?

3. What benefits, side effects, dose limits, or treatment failures have earlier studies found?

4. Is the drug approved for any condition, investigational for all uses, or being used differently in this study?

5. Are results available from earlier NF studies, and were those results peer reviewed?

03 Why might I qualify?

1. Which diagnosis, genetic result, tumor type, symptoms, age, or disease changes are required?

2. Why are the major inclusion and exclusion criteria necessary?

3. Could prior surgery, radiation, medication, or another trial affect eligibility?

4. Are measurable or growing tumors required, and how is growth defined?

5. What tests must confirm eligibility, and what happens if screening finds something unexpected?

04 How is the study designed?

1. Will everyone receive the study drug, or is there a placebo, observation, or comparison group?
2. Is treatment assignment randomized, and will I or the study team know what I receive?
3. Why were this comparison and study length chosen?
4. How many participants and study sites are planned?
5. What would make the study stop early, change doses, or expand to more participants?

05 What will participation require?

1. How is the drug taken, how often, and for how long?
2. How many clinic visits, scans, blood draws, biopsies, hearing tests, eye exams, heart tests, or questionnaires are required?
3. Which visits must be in person, and which can be completed locally or by telehealth?
4. How much time should I expect for screening, treatment, monitoring, and follow-up?
5. Can I keep working, attending school, traveling, or receiving routine care during the study?

06 What are the risks and unknowns?

1. What side effects are known, how common and serious are they, and when do they usually appear?
2. Which long-term or rare risks are still unknown?
3. What symptoms or test results require an urgent call, dose change, treatment pause, or permanent stop?
4. Could this drug interact with my medicines, supplements, surgeries, anesthesia, alcohol, foods, or other treatments?
5. Are pregnancy prevention, fertility, breastfeeding, or reproductive health precautions required?
6. Who provides care and pays if I am injured or become ill because of the study?

07 How will benefit be measured?

1. What is the main outcome, and why was it chosen?
2. How will researchers measure tumor size, pain, hearing, vision, movement, function, appearance, or quality of life?
3. What counts as improvement, stable disease, progression, or treatment failure?

4. How soon might a benefit appear, and how long would it need to last to be meaningful?

5. Could the study collect important information even if the drug does not help me personally?

6. Will I receive my own scan, laboratory, genetic, or other test results?

7. Will I be told about individual genetic findings, and can I choose whether to receive them?

08 What are my choices and rights?

1. What are my care options if I do not join this study?

2. Could joining delay or prevent surgery, another drug, another trial, or standard care later?

3. Can I leave at any time, and what follow-up would still be requested?

4. What happens to information or samples already collected if I leave the study?

5. Can the study team remove me from the study, and for what reasons?

6. Will I receive important new findings that could affect whether I want to continue?

7. Who can I contact about my rights as a research participant or about a concern I do not want to discuss with the study team?

8. Who can give an independent second opinion about whether participation fits my goals?

09 What happens after treatment ends, and how is my information used?

1. What happens if the drug helps me when the study treatment period ends?
2. Is continued access possible, and who would pay for the drug and monitoring?
3. What follow-up is required after I stop the drug?
4. How will I learn the overall study results, and when are results expected?
5. Who can access identifiable health or genetic information, and how will my privacy be protected?
6. May my data or samples be shared with other researchers or companies or used in future research?
7. Can I limit or decline future use, and would additional consent be required?

10 What will participation cost, and will I be paid?

1. Which drugs, tests, visits, scans, and procedures are paid for by the study?

2. What may be billed to me or my insurance?

3. Are travel, lodging, meals, parking, childcare, or lost work reimbursed?

4. Will I be paid for visits or time, when are payments issued, and what happens to earned payment if I leave early?

5. Could payment or reimbursement affect taxes or public benefits?

6. Who can provide the expected costs, payment, reimbursement, and financial-assistance details in writing?

Important reminder: Research participation is voluntary. You may ask questions, take time to decide, request a copy of the consent form, decline to join, or leave a study without penalty or loss of benefits to which you are otherwise entitled. Joining a study is not the same as receiving proven treatment, and personal benefit cannot be guaranteed.

Medical Disclaimer: This guide provides general educational and informational resources only. It is not a substitute for professional medical advice, diagnosis, or treatment. Always consult a qualified healthcare professional regarding medical concerns or treatment decisions.