



WHAT SHOULD I BE THINKING ABOUT WHEN CONSIDERING PARTICIPATION?

- Diagnostic Journey:
 - Where are we on the journey?
 - Do we need additional clinical support in place?
 - At this time, will we be able to comfortably balance clinical care with research?
- Support:
 - Do we have our village?
 - Is there additional support I need to put in place for myself or my loved one to feel secure?
 - Who else should I be talking to about my potential participation (i.e. family, PCP, etc.)?
- Reasons for participating:
 - You should feel clear about why the research is being done and participating should align with you and your loved one's personal values.
- Patient burden:
 - Can me or my loved one tolerate participating in the core activities of the study at this time?
- Care partner/study partner obligations:
 - Am I able to fulfill the responsibilities of a study partner?
 - Do I understand what my obligations are for purposes of participating in this particular research study?
- Costs: Financial, time, effort, emotional, etc.
- Bandwidth for commitment:
 - Is the proposed study schedule realistic for us?
 - Can we reasonably commit to this?
 - Are we able to follow the participation guidelines?
- Benefit vs risk ratio:
 - Many studies offer no guaranteed personal benefit, so risks should be considered carefully.
- Personal health situation (for the participating individual):
 - Medications, concomitant health conditions, family planning, etc.

INFORMED CONSENT PROCESS AND WHAT TO CONSIDER

Listed below are types of questions to keep in mind while reviewing consent for a clinical trial you are considering participation in for yourself or your loved one. This is a non-exhaustive list, but a helpful jumping off point for individuals considering participation.

STUDY DESIGN & LOGISTICS

- What do researchers hope to learn from this study?
- What are the core activities I will be participating in?
- How long does the study last? How many visits are required? Are there follow-ups?
- Will I be compensated? Is there travel support available, and what does it cover?
- Will I be charged for any study activities or procedures?
- What happens if I am injured while taking part in the study?
- What is the drug, treatment, or device being tested?
- What documents do I need to have to participate in the study? (e.g. genetic test results)
- Is this a first-in-human evaluation? If not, what has been learned from other studies?
- Is there a placebo group? Is it randomized? Is the study blinded?
- After the study will there be open label or expanded access to the study drug?
- If I benefit from the intervention, will I be allowed to continue receiving it after the trial ends?
- If I am in the placebo group, will I have a chance to receive treatment after the trial ends?
- What are my expectations / goals for participating in the study (e.g. improve my symptoms, stop disease progression, contribute to the advancement of treatments even if I don't see a direct benefit)?
- If I participate in this study, will I still be eligible for future clinical trials or other research studies?
- For genetic therapies specifically:
 - What tissue or organ system is the genetic therapy designed to target?
 - Can this type of genetic therapy disrupt my DNA? If yes, did you see any off-target effects in prior studies?
 - How is the genetic therapy administered?
 - Are there any safety risks associated with the route of administration?
 - Can I ever get a 2nd genetic therapy? (e.g. Higher dose, different target tissues, if the effects of the 1 treatment wear off, etc.)?



COMMUNICATION, DATA PRIVACY, AND SHARING

- Who is my primary point of contact?
- How will communication occur between study visits?
- What is the procedure if I experience an emergency while in the study?
- What documentation will I be expected to maintain (e.g. Study logs)?
- How is my data privacy managed?
- What personal information will be collected?
- Will samples (blood, DNA, etc.) be stored for future research?
- Will your data be anonymous, coded, or identifiable?
- Who will have access to my data?
- Where might my data be shared and with whom?

PATIENT SAFETY AND RESULTS

- Are there any lifestyle or medication restrictions or changes I need to be aware of?
- What are the potential risks of participating?
- What are the known side effects?
- Will I be notified about safety concerns, even if I don't experience problems?
- Will I get any results from the research testing?
- Will results be available to my PCP or other physicians?
- What is stored in my electronic medical record?
- How do I withdraw my consent?

OTHER QUESTIONS TO CONSIDER

- Travel support details (can we bring an extra care partner, will the sponsor cover an extra night of accommodation after procedures, etc.)
- If you are participating in multiple studies, you may want to ask about differences/overlaps between those studies, data sharing, how patient burden is being minimized, etc.
- Is there flexibility with scheduling study activities?
- Are family conferences with physicians, genetic counselors, or other clinical or support staff available to us during participation?
- Is there any medication available to assist with discomfort during MRI or other procedures if I should need it?
- What results and information will be shared with me regarding the outcomes from the clinical trial and on what timeline?
- What information is collected about me as a study partner?

