

Questions to Ask Your Doctor Before Deciding to Take Gene Therapy

Am I a good candidate?

- Am I eligible to receive gene therapy?
- Can my child receive gene therapy?
- Is there a difference between gene therapy for Hemophilia A and Hemophilia B?
- Which gene therapy options are currently available to me in clinical trials?
- Which gene therapy options are available to me as an approved treatment?
- Which gene therapy treatment(s) or clinical trial(s) would you recommend for me?
- Can you help me find more information to help me understand my options?
- Can I talk with people who have received gene therapy for hemophilia?

What are the potential benefits and risks of gene therapy?

- How effective is gene therapy for hemophilia?
- Will I still need to use factor after gene therapy?
- What can I expect my factor level to be at after gene therapy?
- How long will the effect on my factor levels last?
- Is it possible that gene therapy will not work for me?
- Can I remove the gene therapy from my body if I no longer want it?
- If gene therapy does not work for me, can I try another gene therapy in the future?
- How might my daily life change after gene therapy?
- What are the potential short-term risks of gene therapy? How often do they occur?
- What are the potential long-term risks of gene therapy? How often do they occur?
- What steps will be taken to monitor my safety?
- Am I at risk of getting an inhibitor after gene therapy?
- Will taking gene therapy affect my ability to have children?
- After receiving gene therapy, can my future children still have hemophilia or be hemophilia carriers?

