

Low-dose Danazol for the Treatment of Telomere-Related Diseases



Researchers at the National Institutes of Health (NIH) are studying if the medication danazol can be used to treat people with short telomere disease who also have bone marrow failure, liver, or lung disease.

In recent studies, danazol, at high doses, showed a positive influence on telomere length. Additional research is needed to learn more about the role of danazol on telomere disease as well as its effects on secondary diseases.

Eligible participants:

- Are 3 and older (minors must have parent/guardian consent to participate);
- Are diagnosed with a short telomere disease;
- Also have abnormalities affecting the bone marrow such as aplastic anemia or low blood counts OR lung disease OR liver disease

Study procedures:

- Before starting danazol, you will have tests and evaluations including measurements of your telomere length.
- Depending on your secondary disease you may also have a bone marrow biopsy, lung function tests, or evaluation of your liver.
- You will then begin taking danazol either 100 mg twice a day, or 200 mg twice a day for up to a year.
- While taking danazol you will have outpatient visits to the NIH Clinical Center after 6 months, a year, and 6 months after stopping danazol.
- Your participation in this study will last about 2 years.

Study-related tests, procedures, and medications are provided at no cost. Travel to and from the NIH, within the United States may be reimbursed.

Location: The NIH Clinical Center, America's Research Hospital is located on the Metro red line (Medical Center stop) in Bethesda, Maryland.

For more information, call:
NIH Clinical Center
Office of Patient Recruitment
1-800-411-1222

TTY: 1-866-411-1010
<https://go.usa.gov/xnPYm>
NIH study 18-H-0004

