

E4A03 Clinical Trial Results Summary

Lenalidomide and Dexamethasone With or Without Thalidomide in Treating Patients With Multiple Myeloma

What did this trial involve and who was it for?

E4A03 was a clinical trial for people with newly diagnosed multiple myeloma, a bone marrow cancer that affects white blood cells. Earlier studies showed that the combination of lenalidomide and dexamethasone was a highly effective treatment for myeloma, but the risk of serious side effects was a concern. Thus, the purpose of E4A03 was to find out if a lower dose of dexamethasone could work as well as a higher dose while causing fewer side effects.

E4A03 was open to enrollment from October 2004 to June 2007 to people who had not yet received treatment for their newly diagnosed myeloma. 445 participants were randomly assigned by a computer to one of two treatment groups:

1. Lenalidomide + high-dose dexamethasone (total of 12 doses per cycle)
2. Lenalidomide + low-dose dexamethasone (total of 4 doses per cycle)

Each group received four 28-day cycles of treatment. After the four cycles were completed, participants could choose to continue with study treatment or stop and receive other treatment, such as a stem cell transplant.

As part of this study, patients whose myeloma did not respond to treatment after the first four cycles were offered thalidomide, along with the dexamethasone, instead of lenalidomide. Patients were followed for response until progression and for survival for approximately 7 years.

What are the results?

After a planned analysis of results, the study was stopped early and patients in the high-dose group were immediately switched to the lower dose of dexamethasone, as it was found to be significantly more effective and generally safer:

- 96% of patients receiving the lower dose were alive after one year compared to 87% of patients receiving the higher dose.
- 32% of patients in the low-dose group experienced serious side effects (such as deep vein blood clots, infections, and fatigue) compared to 52% of patients in the high-dose group.

What do the results mean for patients?

- E4A03 was a groundbreaking, practice-changing trial: it is now standard practice for patients with myeloma to be given a lower dose of dexamethasone.
 - Lenalidomide and dexamethasone remain a common initial myeloma treatment.

For more information, go to:

- United States National Institutes of Health (NIH) Library of Medicine: <https://clinicaltrials.gov/study/NCT00098475>
 - *Lancet Oncology* (2010): <https://pmc.ncbi.nlm.nih.gov/articles/PMC3042271/>
 - *Blood* (2007): <https://doi.org/10.1182/blood.V110.11.74.74>
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About ECOG-ACRIN

This trial was led by the ECOG-ACRIN Cancer Research Group (ECOG-ACRIN). ECOG-ACRIN is a membership-based scientific organization that designs and conducts cancer research involving adults who have or are at risk of developing cancer. ECOG-ACRIN is a component of the National Cancer Institute's National Clinical Trials Network. Learn more at www.ecog-acrin.org.

To all the patients that participated in this trial, thank you. Your participation, and that of other patients like you, made this research possible.