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Efficacy and safety of auranofin for progressive desmoid-type fibromatosis: an open-label phase II trial

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ABSTRACT

Background and Objectives:
Because desmoid-type fibromatosis (DF) has a high recurrence rate after surgery, active surveillance is the mainstay of the treatment followed by medical therapy. However, there are few effective drugs with acceptable side effects. Among drugs that have been used for a long time and have a known safety profile, auranofin was found to be effective in suppressing DF using the drug repositioning method in our laboratory. This clinical study was designed to examine the efficacy and safety of auranofin, an already approved anti-rheumatic drug, in patients with progressive DF.

Designs and Methods:
This study is conducted as a single-center, single-arm, open-label study. Auranofin 3mg tablets will be administered twice daily to DF patients with progressive disease. The primary endpoint is progression-free survival at 26 weeks after starting treatment. Secondary endpoints include response rate, T2-weighted MRI evaluation, pain intensity, quality of life, and safety assessment.

Results and Conclusion:
This is the first clinical trial of auranofin in patients with aggressive DF. The study will allow an in-depth understanding of efficacy of auranofin for not only response rate, also changes in MRI findings, pain, and QOL in patients with aggressive DF.