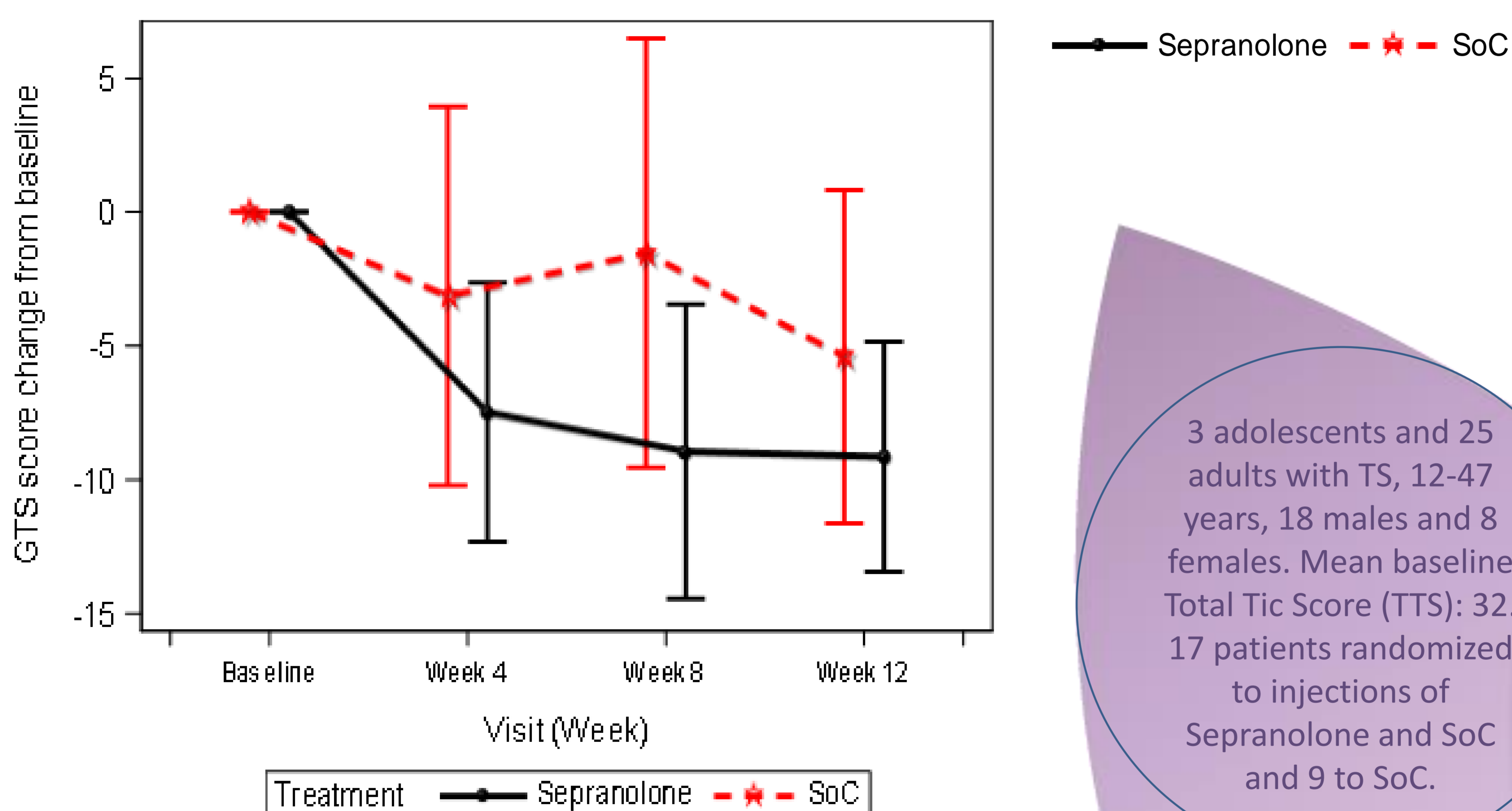
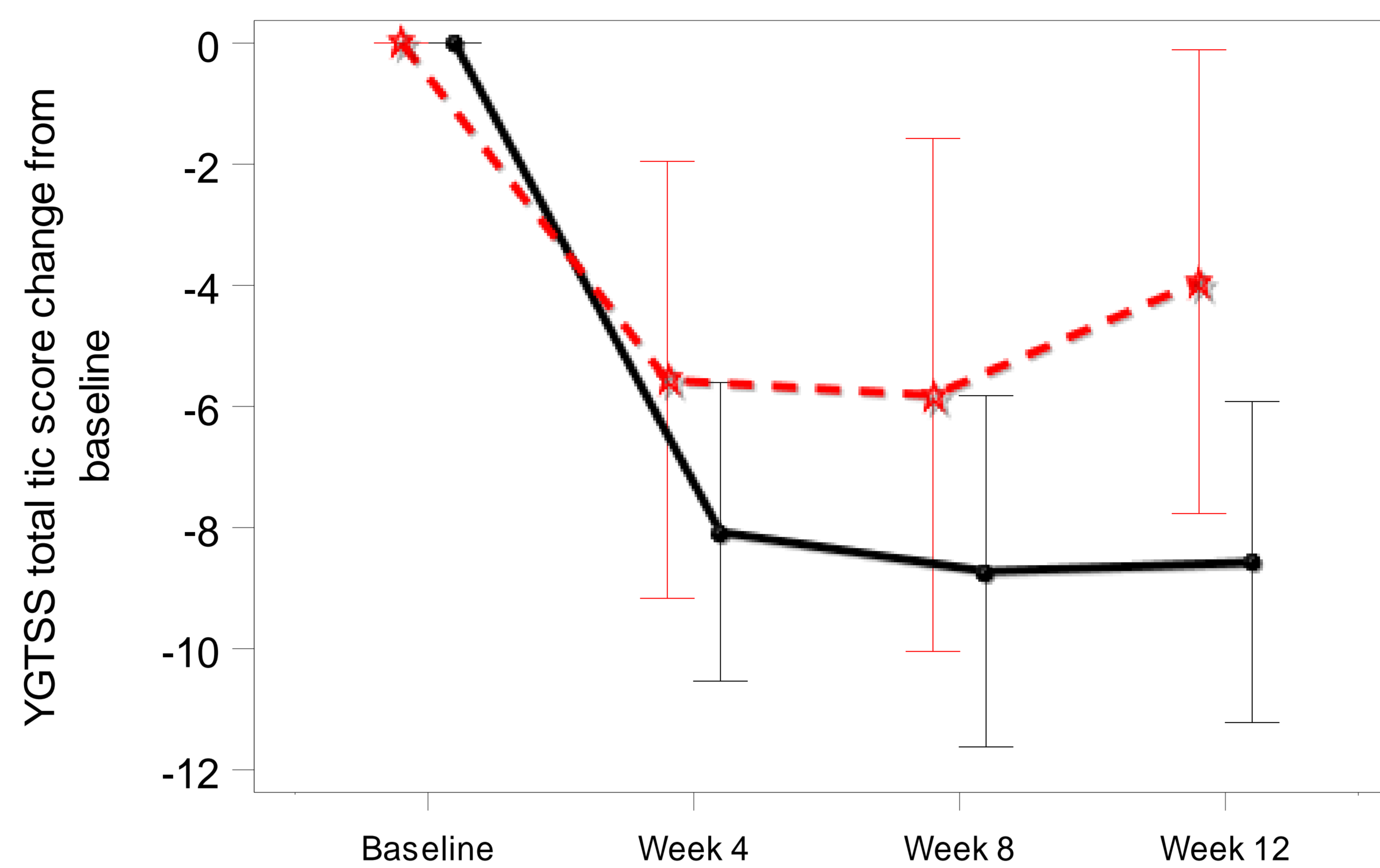


# Promising results on a clinical trial to explore the safety and efficacy of Sepranolone in pediatric and adult patients with Tourette syndrome

## TAKE HOME MESSAGE:

Treatment with Sepranolone might be a promising new treatment possibility for patients with TS.



3 adolescents and 25 adults with TS, 12-47 years, 18 males and 8 females. Mean baseline Total Tic Score (TTS): 32. 17 patients randomized to injections of Sepranolone and SoC and 9 to SoC.

Interventional, open-label, multi-center, randomized, parallel group study to explore the safety and efficacy of Sepranolone in patients with TS. Patients were randomized to either standard of care (SoC) or standard of care + injections of Sepranolone 10 mg twice weekly for 12 weeks.

Active group: TTS reduced by 8.6 points (28%)  
 Control group: TTS reduced by 3.9 points (12.6%) (p=0.051). Quality of Life: 69% greater reduction in active group. No systemic or CNS off target side effects. In 2% mild to moderate reversible skin reactions.

## Background

Several neurotransmitters, including GABA, have been proposed to play a role in the pathophysiology of Tourette Syndrome. Sepranolone is an endogenous neurosteroid that suppresses the effect of positive GABA-A receptor modulators, e.g. allopregnanolone (ALLO), and thus of potential interest for development of new treatment options for patients with TS. Blocking the production of ALLO with finasteride has previously shown a strong tic reducing effect and the present study seems to validate ALLO as a target for TS intervention.

