

Phase 3, randomized, double-blind, placebo-controlled trial. Adult patients with active complex perianal CD and non or mildly active luminal CD were randomized to a single local injection of 120 million Cx601 cells or placebo added on to standard of care.

*Patients were refractory to antibiotics, immunosuppressants and/or antTNF.

Primary endpoint: Combined remission (closure of all treated external openings with no collections >2cm confirmed by MRI) and clinical remission (absence of draining fistulas at week 52)

Results:

- At week 52, 56.3% Cx601 vs 38.6% controls, $p=0.01$ achieved combined remission and clinical remission 59.2% vs 41.6% of controls, $p=0.013$
- No differences in % of adverse events between groups

Conclusion:

In a phase 3 trial of patients with Crohn's disease and treatment-refractory complex perianal fistulas, we found Cx601 to be safe and effective in closing external openings, compared with placebo, after 1 year.

Long-term Efficacy and Safety of Stem Cell Therapy (Cx601) for Complex Perianal Fistulas in Patients With Crohn's Disease

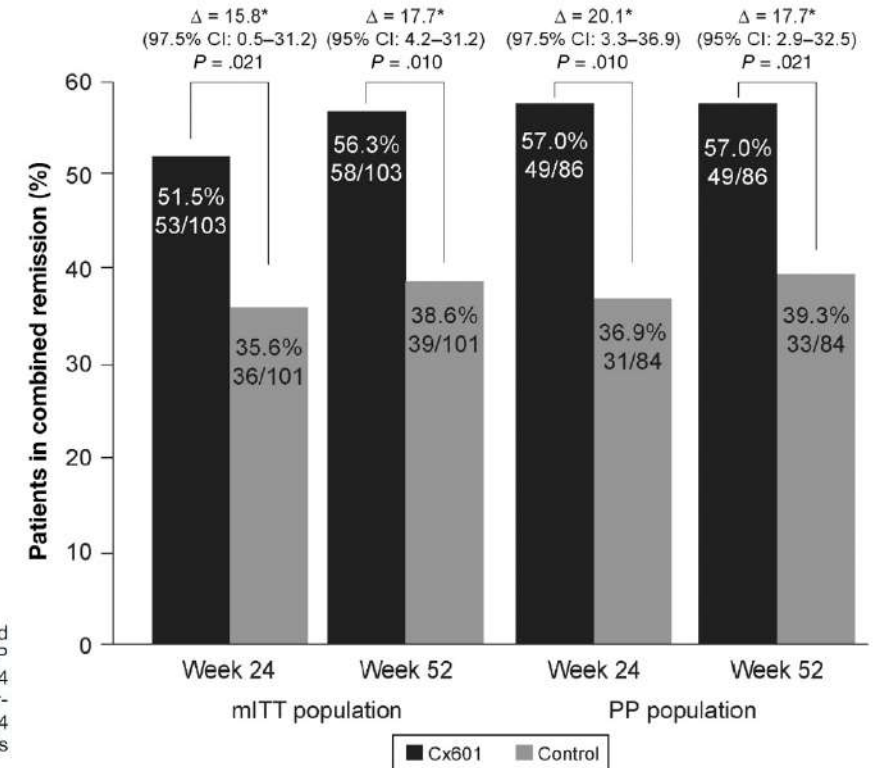


Figure 1. Combined remission in mITT and PP populations at weeks 24 and 52. *Difference in percentage points. Data at 24 weeks published in Panés et al.¹⁷