Autologous expanded adipose-derived stem cells for the treatment of complex cryptoglandular perianal fistulas: a phase III randomized clinical trial (FATT 1: fistula Advanced Therapy Trial 1) and long-term evaluation.

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Publication Date: 2012

Abstract:

BACKGROUND: Autologous adipose-derived stem cells may represent a novel approach for the management of complex fistula-in-ano. After successful phase I and II clinical trials, a phase III trial was performed to investigate the safety and efficacy.

DESIGN: In this multicenter, randomized, single-blind, add-on clinical trial, 200 adult patients from 19 centers were randomly assigned to receive 20 million stem cells (group A, 64 patients), 20 million adipose-derived stem cells plus fibrin glue (group B, 60 patients), or fibrin glue (group C, 59 patients) after closure of the internal opening. Fistula healing was defined as reepithelization of the external opening and absence of collection >2 cm by MRI. If the fistula had not healed at 12 weeks, a second dose (40 million stem cells in groups A and B) was administered. Patients were evaluated at 24 to 26 weeks (primary end point) and at 1 year (long-term follow-up).

RESULTS: All results are according to the "blinded evaluator" assessment. After 24 to 26 weeks, the healing rate was 39.1%, 43.3%, 37.3% in groups A, B, and C ($p = 0.79$). At 1 year, the healing rates were 57.1%, 52.4%, and 37.3 % ($p = 0.13$). On analysis of the subpopulation treated at the technique's pioneer center, healing rates were 54.55%, 83.33%, and 18.18%, at 24 to 26 weeks ($p <$

0.001). No SAEs were reported.

CONCLUSIONS: In treatment of complex fistula-in-ano, a dose of 20 or 60 million adipose-derived stem cells alone or in combination with fibrin glue was considered a safe treatment, achieving healing rates of approximately 40% at 6 months and of more than 50% at 1-year follow-up. It was equivalent to fibrin glue alone. No statistically significant differences were found when the 3 groups where compared.

CLINICAL TRIALS REGISTRATION: www.clinicaltrials.gov, identifier NCT00475410; Sponsor, Cellerix SA.