骨髄は虚血性心不全治療に有効ではない

FOCUS Trial：骨髄を用いた虚血性心不全の治療はある心機能計測値を改善しなかった

FOCUS Trial: Treatment for chronic ischemic heart failure with bone marrow cells does not show improvement for certain heart function measures

患者の骨髄を用いた慢性虚血性心不全の治療は心機能計測値のほとんどを改善しなかったとのスタディ結果が2012年American College of Cardiology学会で発表され、同時にJAMAオンライン版に掲載された。FOCUSトライアルにおいて研究者らは、5つの米国内心、肺、および血液施設-スポンサーのついた心臓血管細胞療法研究 ネットワーク施設において、慢性虚血性心疾患および左室機能低下を伴う心不全かつ/または狭心症を有し最大限の薬物療法を受けている患者に対し、経心内膜自家骨髄単核球細胞（BMCs）投与の効果を評価するスタディを施行した。患者はBMCsまたはプラセボの経心内膜注入を受ける群に無作為に割り付けられた。データ解析の結果、左室収縮末期容積指数、最大酸素摂取量、および可逆的欠損（reversible defect）の変化から成るエンドポイントは両群間で統計学的な有意差がないことが示された。
パーセント心筋欠損、総欠損サイズ、固定性欠損サイズ、局所壁運動、および臨床上の改善などの二次エンドポイントのいずれにおいても差がなかった。左室駆出率を評価した検査分析では、62歳以下の患者においては統計学的に有意な治療効果が認められた。

Full Text

Use of a patient's bone marrow cells for treating chronic ischemic heart failure did not result in improvement on most measures of heart function, according to a study appearing in JAMA. The study is being published early online to coincide with its presentation at the American College of Cardiology's annual scientific sessions.

Cell therapy has emerged as an innovative approach for treating patients with advanced ischemic heart disease, including those with heart failure. "In patients with ischemic heart disease and heart failure, treatment with autologous bone marrow mononuclear cells (BMCs) has demonstrated safety and has suggested efficacy. None of the clinical trials performed to date, however, have been powered to evaluate specific efficacy measures," according to background information in the article.

Emerson C. Perin, M.D., Ph.D., of the Texas Heart Institute and St. Luke's Episcopal Hospital, Houston and colleagues conducted a study to examine the effect of transendocardial administration of BMCs to patients with chronic ischemic heart disease and left ventricular (LV) dysfunction with heart failure and/or angina. The patients in the phase 2 randomized trial were receiving maximal medical therapy at 5 National Heart, Lung, and Blood Institute–sponsored Cardiovascular Cell Therapy Research Network (CCTRN) sites between April 2009 and April 2011. Patients were randomized to receive transendocardial injection of BMCs or placebo. The primary outcomes measured for the study, assessed at 6 months, were changes in left ventricular end-systolic volume (LVESV) assessed by echocardiography, maximal oxygen consumption, and reversibility of perfusion (blood flow) defect on single-photon emission tomography (SPECT). Of 153 patients who provided consent, a total of 92 (82 men; average age: 63 years) were randomized (n = 61 in BMC group and n = 31 in placebo group).

Analysis of data indicated no statistically significant differences between the groups for the primary end points of changes in LVESV index, maximal oxygen consumption, and reversible defect. There were also no differences in any of the secondary outcomes, including percent myocardial defect, total defect size, fixed defect size, regional wall motion, and clinical improvement.

In an exploratory analysis, the researchers did find that when LVEF was assessed, patients age 62 years or younger showed a statistically significant effect of therapy. Patients in the BMC group demonstrated an average increase in LVEF of 3.1 percent from baseline to 6 months, whereas patients in the placebo group showed a decrease of −1.6 percent.

"In the largest study to date of autologous BMC therapy in patients with chronic ischemic heart disease and LV dysfunction, we found no effect of therapy on prespecified end points. Further exploratory analysis showed a significant improvement in LVEF associated with treatment. Our findings provide evidence for further studies to determine the relationship between the composition and function of bone marrow product and clinical end points. Understanding these relationships will improve the design and interpretation of future studies of cardiac cell therapy," the authors write.

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