

Stem Cell/Cell-Based Therapies for Pulmonary Fibrosis Beyond the Context of Clinical Trials

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Cell-based therapies are treatments that involve the administration of living cells to treat disease. Stem cell therapies refer to cell-based therapies that involve stem cells, or precursor cells, that have the potential to mature into specialized cells with specific functions. While interest in the medical use of cell-based therapies continues to grow, the diseases for which these treatments have been proven safe and effective are currently limited to blood cancers and bone marrow failure.

Since the Pulmonary Fibrosis Foundation's initial position statement on this topic in 2015, there continues to be an interest in cell-based therapies, including stem cells, for the treatment of pulmonary fibrosis (PF). While this evolving field of research holds tremendous promise, at present, stem cell or cell-based therapies remain unproven and experimental for patients with pulmonary fibrosis. The senior medical leadership of the PFF recommends that, at this time, patients should only engage in stem cell and other cell-based therapies as part of an approved clinical trial.

Proving that a new therapy is safe and effective requires careful testing. To provide the best care for our patients with pulmonary fibrosis, or any illness for that matter, a meticulous evaluation of any therapy in controlled clinical trials is the only way to assess the benefits and potential harm. International guidelines have been developed to guide the efficient and appropriate assessment of new treatments. These guidelines require clinical trials to test the safety and benefit of the treatment, as well as review and approval by an appropriate regulatory agency.¹ Stem cell and other cell-based therapies must be evaluated according to these guidelines, similarly to any new therapy.

It is understandable that patients are eager to receive therapies that may prevent the devastating effects of their disease. Peer-reviewed research to date includes several small studies investigating a particular type of adult stem cell, called mesenchymal stem cells or multipotent mesenchymal stromal cells,² in patients with pulmonary fibrosis,³⁻⁷ COPD,⁸ acute respiratory distress syndrome,⁹ and lung dysfunction after lung transplant.¹⁰ These studies support conducting additional rigorous investigations to more definitively assess the safety and efficacy of stem cell and other cell-based therapies. It is essential to appropriately evaluate these potential therapies, which can only be done through approved clinical trials.

Direct-to-consumer clinics offering unproven stem cell “treatments” for PF have exaggerated claims of safety and efficacy, often with weak or absent scientific rationale.¹¹ Practitioners at these for-profit facilities operate with minimal oversight, and the treatments offered do not have a proven mechanism of action or set standards for cell preparation, cultivation, storage, and the treatment regimen. Cases of serious harmful events, including death, have been reported following administration of unproven stem cell “treatments”, and the harms experienced by patients remain under-reported due to a lack of oversight and reporting requirements.¹² Several cases of severe respiratory illness, occurring within a relatively short timeframe after individuals have received “stem cell” infusions from for-profit centers, have been identified and reported to the appropriate regulatory agencies.¹³ The Pulmonary Fibrosis Foundation encourages patients to use caution when considering stem cell and other cell-based therapies outside the context of a clinical trial and to be aware that an “experimental” treatment is not necessarily part of a legitimate research study or clinical trial. Warning signs that a stem cell “treatment” is unproven and not part of a clinical trial include claims that regulatory oversight is not needed, requests for payment for treatment, or claims of miraculous results based on testimonials.

The senior medical leadership of the PFF recommends that patients should only engage in stem cell and other cell-based therapies as part of an approved clinical trial (oversight by an institutional review board (IRB), participants give informed consent and are closely monitored, and the potential benefits and harm can be objectively assessed). Participants should never be asked to pay to take part in a clinical trial. This recommendation will persist until the safety and effectiveness of stem cell and other cell-based therapies have been determined by controlled clinical trials.

Additional recommended resources:

Pulmonary Fibrosis Foundation: Clinical Trials Education Center
<https://www.pulmonaryfibrosis.org/patients-caregivers/medical-and-support-resources/clinical-trials-education-center>

Pulmonary Fibrosis Foundation: Clinical Trial Finder
<https://trials.pulmonaryfibrosis.org/>

International Society for Stem Cell Research: The ISSCR Guide to Stem Cell Treatments
<https://www.isscr.org/treatment-guide>

International Society for Stem Cell Research: What should I be cautious about if I am considering an unproven stem cell “treatment”?
<https://www.aboutstemcells.org/info/what-should-i-be-cautious-about-if-i-am-considering-an-unproven-stem-cell-treatment>

International Society for Stem Cell Research: ISSCR Guidelines for Stem Cell Research and Clinical Translation: The 2021 update

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