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AN UPDATE OF STEM CELL/CELL-BASED THERAPIES FOR PULMONARY FIBROSIS BEYOND THE CONTEXT OF CLINICAL TRIALS: A word of caution by the Medical Advisory Board of the Pulmonary Fibrosis Foundation (PFF).

To members of the Pulmonary Fibrosis Community,

Since our initial statement in 2015, there continues to be interest in cell-based therapies, including stem cells, for the treatment of idiopathic pulmonary fibrosis (IPF). The evolving field of regenerative medicine holds tremendous promise, but at present stem cell/cell-based therapies remain unproven, experimental, and may be detrimental for patients with pulmonary fibrosis. International guidelines have been developed to direct the efficient and appropriate assessment of innovative treatments that can be evaluated in clinical trials and approved only after appropriate regulatory review.¹ This contrasts sharply with the unproven interventions offered by practitioners operating with minimal oversight at for-profit stem cell centers that represent “the modern day equivalent of snake oil.”² The Pulmonary Fibrosis Foundation encourages patients to use caution when considering stem cell/cell-based therapies outside the purview of a clinical trial. Several cases of severe respiratory illness, temporally associated with “stem cell” infusions from for-profit centers, have been identified and reported to the appropriate regulatory agencies. Without a proven mechanism of action, no set standards for cell preparation, cultivation, storage and treatment regimen, the direct to consumer marketing of stem cell therapies have exaggerated claims of safety and efficacy, often with weak or absent scientific rationale.³

The Medical Advisory Board of the PFF recommends that patients should only engage in stem cell/cell-based therapies as part of an approved clinical trial, in which they are closely monitored and the potential benefits and harm can be objectively assessed. This recommendation will likely persist until the safety and effectiveness of stem cell/cell-based therapies have been rigorously evaluated.

It is understandable that patients are eager to receive therapies that may avert the devastating effects of their disease. Peer reviewed research to date includes several small studies investigating a particular type of adult stem cell, termed mesenchymal stem cells or multipotent mesenchymal stromal cells,⁴ suggesting acceptable safety profiles in Phase I clinical trials in patients with pulmonary fibrosis,⁵⁻⁹ COPD,¹⁰ acute respiratory distress syndrome,¹¹ and lung dysfunction after lung transplant.¹² Based on these initial studies, additional rigorous investigations to more definitively assess the safety and efficacy of stem cell/cell-based therapies are currently being organized. It is essential to rapidly but appropriately evaluate these potential therapies, which can only be done through the careful conduct of controlled clinical trials.

More detailed information about clinical trials can be obtained from the PFF website (pulmonaryfibrosis.org) and specific clinical trials evaluating innovative therapies can be identified using the PFF Clinical Trial Finder (<https://pulmonaryfibrosis-org.clinicaltrialconnect.com>).

The PF community should be heartened by the progress made in PF research and patient care, including the identification and drug agency approval of two therapies for patients with IPF. These advancements were made possible through the careful implementation of rigorous scientific efforts, including randomized clinical trials. 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. This includes prudently evaluating stem cell/cell-based therapies.

As new studies and clinical trials are planned, we look to the future with hope. Patients, relatives, advocacy experts, industry, physicians, researchers, and federal agencies contributed to the prior successes and are essential for further advances. The entire PF community is responsible for this continued progress and our work will continue as **we imagine a world without PF**.

On behalf of the Pulmonary Fibrosis Foundation Medical Advisory Board,



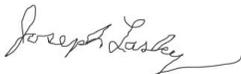
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