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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,

Recently, interest has been placed on cell-based therapies, including stem cells for the treatment of idiopathic pulmonary fibrosis (IPF). We know very little about the impact of such therapies in humans, and rigorous clinical trials have not been conducted to test the role (or adverse effects) of stem cells in the management of IPF. Furthermore, few studies have been conducted in animals or humans for the treatment of any lung condition.

While the concepts of regenerating the scarred lung by stem cell “treatment” are pursued in clinical studies and trials, patients are understandably eager to receive such treatment now and are approaching physicians and care providers for access to such interventions. Patients need to be aware that the treatment regimen with stem cell/infusions and its safety and efficacy need to be determined in ongoing studies that are still in early phase 1 and 2 clinical trials (Patients can find more information about clinical trials on the PFF website.) At present, there are only a small number of approved clinical trials evaluating cell-based therapies in the United States, Europe, and other countries. (Refer to www.clinicaltrials.gov; www.clinicaltrialsregister.eu/ctr-search/search).

Despite limited investigations in humans, some have claimed significant benefits from cell-based therapies, and stem cells are being delivered to patients outside of approved clinical trials. Subsequent to this increase in medical tourism for stem cell therapy and its potential risks, the Pulmonary Fibrosis Foundation encourages patients to use caution.

Until the safety and effectiveness of such therapies have been rigorously documented, the Medical Advisory Board of the Pulmonary Fibrosis Foundation believes that patients should be skeptical about the usefulness of stem cell therapies and should engage only through participation in approved clinical trials, in which patients are closely monitored and the potential benefits and harm can be objectively assessed.
The IPF community should be proud of the advancements made in IPF research and patient care. These advancements have been possible through the careful implementation of rigorous scientific efforts, including randomized clinical trials. It was through this clinical trial process that two beneficial therapies for patients with IPF were identified and one therapy, that at the time was considered to be the “standard of care,” was later identified as clearly harmful to patients with IPF. To provide the best care for our patients with pulmonary fibrosis, or any illness for that matter, a rigorous evaluation of any therapy in controlled clinical trials is the only way to assess the benefits and potential harm. This includes rigorously evaluating cell-based therapies. We owe it to our patients.

The recent approval of two new drugs for the treatment of IPF has increased our hope and re-kindled our efforts for finding a cure. In essence, the approval of these drugs has opened the door to a new era; one that will likely bring further advancements in IPF research and the development of better treatments. Patients, relatives, advocacy experts, industry, physicians, researchers, and federal entities contributed to this effort. The entire IPF community is responsible for this progress and we should be proud. This work will continue, but is now better informed by observations made during carefully conducted clinical trials. As new studies are planned, we look to the future with hope as new targets for intervention continue to be identified.

On behalf of the Medical Advisory Board of the Pulmonary Fibrosis Foundation,

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