RESEARCH PROTOCOL

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An Observational Outcomes Study for Autologous Cell Therapy Among Patients With COPD and Interstitial Lung Disease

Contents

1. Synopsis

Management of COPD has primarily been with inhaled corticosteroids, oral steroids, lung reduction surgery and lung transplant but none of these treatments have proven ability to slow the progression of COPD over time. Autologous, cellular therapy, using one's own platelet rich plasma, is a novel approach to possibly help slow disease progression and possibly improve the patient's quality of life. This study will descriptively explore the quality of life and lung function outcomes of participants at 3- and 6-months after cellular therapy.

2. Abbreviations and Acronyms

COPD= Chronic obstructive pulmonary disease

PRP=Platelet rich plasma

Background

Conventional treatment for COPD usually involves inhaled bronchodilators, steroids, oxygen therapy and lung reduction surgery for the management of symptoms, although none of these modalities thwarts long-term progression of the disease. Aside from lung transplantation, there is no cure for the disease, the availability of donor lungs is scarce, and the long-term need for anti-rejection medication and monitoring can be overwhelming. Recent advances in the field of regenerative medicine in lung disease have demonstrated that autologous cellular therapy may reduce inflammation and aid in the maintenance and repair of damaged lung tissue, As a result, some patients have elected to undergo autologous cellular therapy utilizing their body's own cells in an effort to control their chronic lung disease.

In the case of this study, cells were harvested from the patient by either the bone marrow or peripheral blood, isolated and concentrated using centrifugation, and then returned to the patient same-day via the peripheral circulation. As circulation occurs, the concentrated cells enter the right heart and are then disseminated into the lungs, becoming trapped in the lung's microcirculation. Here, the cells begin to produce bioactive factors such as cytokines and anti-inflammatory mediators. Several growth factors are released by activated platelets becoming honing cells for healing within the tissue. The exact long-term mechanism of action of stem cells and PRP in the lungs remains under investigation. But because of the minimal cell manipulation and the autologous nature of the cells, the risk of adverse events is low.

3. Objectives

The primary objective of this study is to descriptively examine the outcomes on lung function and perceived quality of life at 3 months and 6 months following autologous cellular therapy treatment.

4. Study Methodology

This is a descriptive, observational study only and all participants received cellular therapy. This is not an experimental design and there is no placebo/control group.

Study population

- *All persons undergoing treatment at the Lung Health Institute facility in Dallas, Texas from March 2016 through October 2020 will participate in this study.*

5. Study procedure

- *Potential participants will be presented with study information*
- *Each potential participant will have a consultation with a medical provider prior to enrollment to determine eligibility for treatment.*

  Participants will be presented with an informed consent by study staff.

  Baseline spirometry with a focus on FEV1 volume and FEV1% predicted will be collected on each participant. The CCQ (Clinical COPD Questionnaire) will be collected on each participant at baseline to assess quality of life.

  Participants will be followed at 3 months and 6 months. At both follow-up timepoints, each participant will complete the CCQ quality of life questionnaire. Participants will be asked to return after 3 months for follow-up spirometry. All data is documented in the participant's secure electronic medical record.

  The procedure for obtaining and processing the venous blood will be the same for all participants. Whole blood is drawn from the peripheral circulation, processed with specialized centrifugation using commercial equipment, and a final platelet rich plasma/platelet concentrate product prepared.

  Each participant will receive a reinfusion of platelet rich plasma/platelet concentrate on each of two, consecutive treatment days.

- *Study timelines: The study will close once 12-month post-treatment follow-up has been completed for each participant.*

6. Data Management

- *Data will be stored in a password-protected database and secure, encrypted electronic medical record.*
- *All patient identifiers will be removed for data analysis and dissemination of findings.*

- *Demographics, quality of life data and spirometry data will be summarized, and pre-and post- treatment quality of life and spirometry data will be compared in descriptive format. Tests of statistical significance will be performed on the difference.*

7. Adverse Event Reporting

- *Any events that are not anticipated are reported directly to the Principal Investigator and to the IRB within 24 hours of occurrence.*

8. Ethical Issues

There are no dual relationships, coercion or inducement to disclose
There are no non-negligible risks or burdens to participants anticipated

9. Finance and resource use

Participants will not be paid or reimbursed anything for their participation in the study. Their participation is voluntary, and they may withdraw their participation at any time.

10. Dissemination of Results and Publication policy

The study findings will be disseminated to any interested party as well as potentially prepared for publication. Other interested parties may include healthcare professionals and policymakers in the fields of primary care, pulmonology and regenerative medicine.