ILD Collaborative

A Patient-Physician Collaborative for the Understanding, Management, and Treatment of Interstitial Lung Diseases

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New Directions for IPF Treatments: Updates on Clinical Trials

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What are clinical trials?

- Clinical trials are at the heart of medical advances
- Clinical trials look at every aspect of patient care
  - new ways to prevent, detect, monitor or treat diseases
- Clinical trials determine if a new test or treatment
  - is safe
  - Is effective

Clinical trials are conducted in “phases”

- Each phase has a different purpose and helps researchers answer different questions
- **Pre-clinical studies:** Researchers first test new therapies or procedures in the laboratory and in animal studies – the most promising experimental treatments are moved into clinical trials
- **Phase I trials:** Researchers test an experimental drug or treatment in a small group of people for the first time. The purpose is to evaluate its safety
- **Phase II trials:** The experimental drug or treatment is administered to a larger group of people to determine its effectiveness
- **Phase III trials:** The experimental drug or treatment is administered to large groups of people to compare it with standard or equivalent treatments

Inclusion / Exclusion Criteria

- All clinical trials have guidelines about who can participate, called **Inclusion / Exclusion Criteria**
- Factors that allow someone to participate in a clinical trial are "inclusion criteria“
- Factors that exclude or not allow participation are "exclusion criteria"
- These criteria are based on factors such as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions

Randomization

- Many or most Phase II and III clinical trials are **randomized**
- **Randomization** is the process by which two or more alternative treatments are assigned to volunteers by chance
- **Randomization** is done to avoid any bias with investigators assigning volunteers to one group or another
- The results of each treatment are compared at specific points during a trial, which may last for months or years
- When one treatment is found superior, the trial is stopped so that the fewest volunteers receive the less beneficial treatment

What is a placebo?

- In some studies, participants may be randomized to receive a **placebo** (an inactive product that resembles the test product, but without its treatment value)

- In diseases which have no effective therapies, new products or therapies are compared with placebos in order to determine whether the new therapies have therapeutic effectiveness

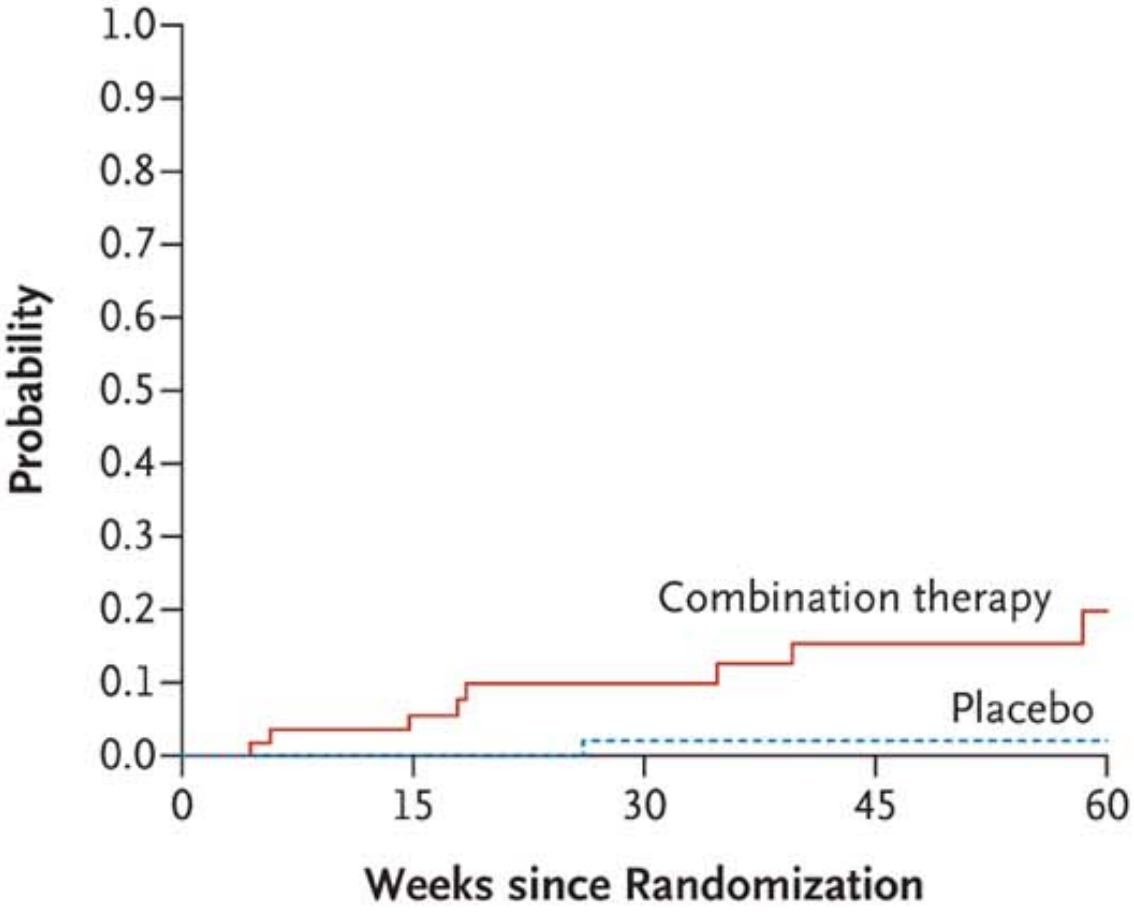
- In diseases which have effective therapies, clinical trials compare a new product or therapy with another that already exists, to determine if the new one is as successful as, or better than, the existing one

What is Blinding?

- Many or most randomized trials are ***double-blind studies***
- In a double-blind study, only the study pharmacist knows what is being administered; neither the patients nor the members of the research team are told which patients are getting which medication. (If medically necessary, however, it is always possible to find out what the patient is taking)
- Studies are “blinded" in order to prevent members of the research team or study participants from biasing the results. This allows scientifically accurate conclusions

Why do we do clinical trials?

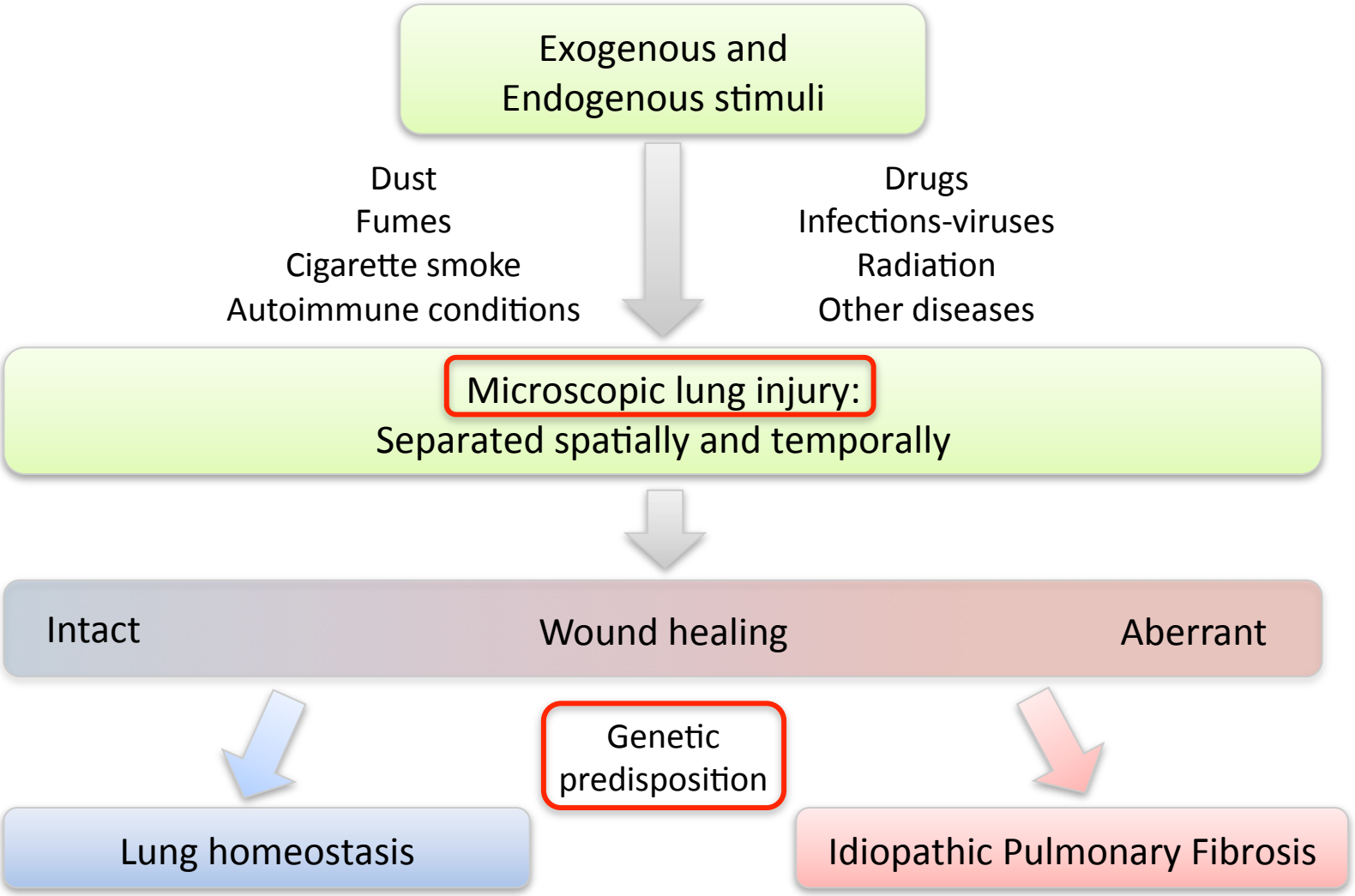
- Treatments that become standard care without clinical trial evidence may not be effective, or even safe



Why do people participate?

- Participation in clinical trials is strictly **VOLUNTARY!**
- People participate in clinical trials for a variety of reasons
- Participants often express the desire to help others with their disease and to contribute to moving medical treatments forward
- Participants may also want the opportunity to possibly receive the newest treatments
- Participants may find the additional care and attention from the clinical trial staff helpful
- Clinical trials offer hope for many people and an opportunity to help researchers find better treatments for others in the future

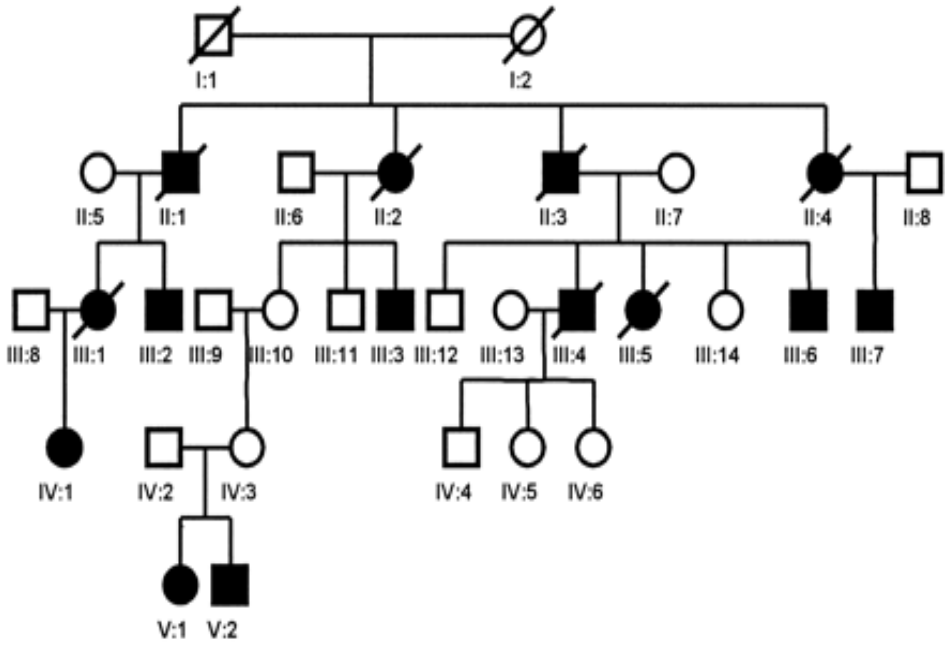
Genes and environment both appear to contribute to IPF



Selman M, King TE, Pardo A, *Ann Intern Med* 2001
Steele MP, Schwartz DA. *Annu Rev Med.* 2013;64:265-276.

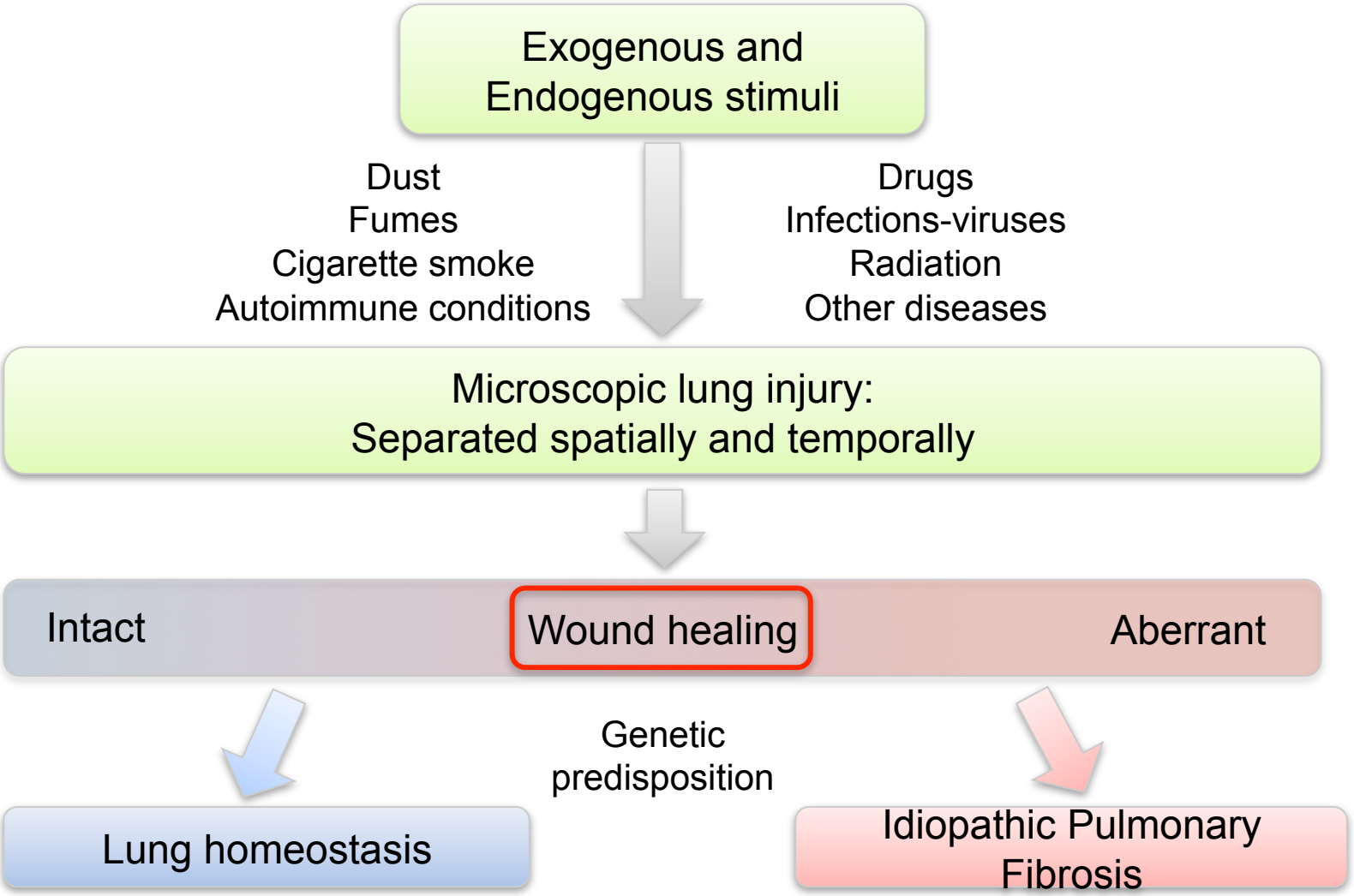
Genetic Causes of Pulmonary Fibrosis

- Familial Interstitial Pneumonia
  - Families with ≥ 2 cases among first-degree family members



- Due to gene mutations that can cause fibrosis by themselves

Genes and environment both appear to contribute to IPF



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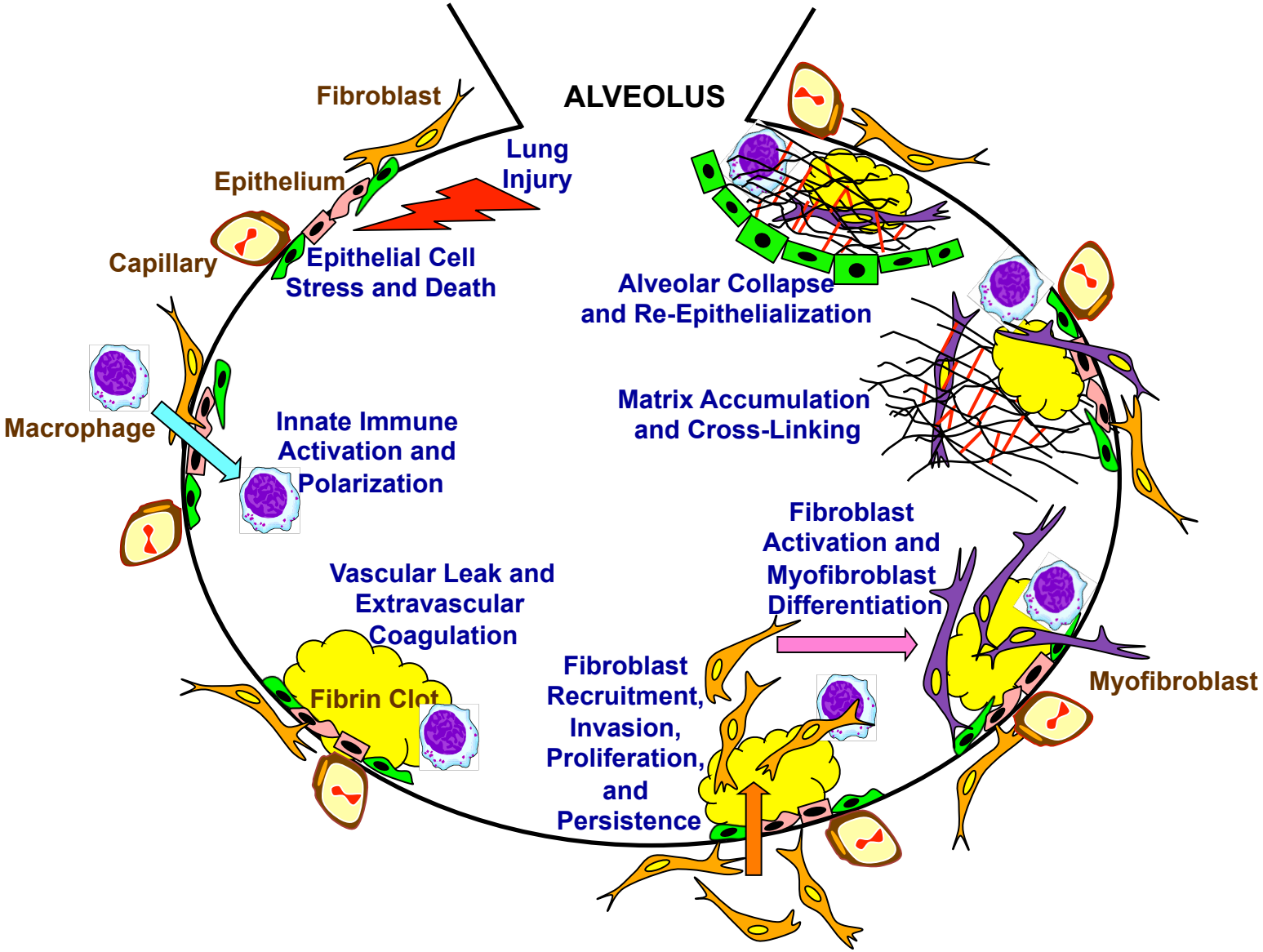
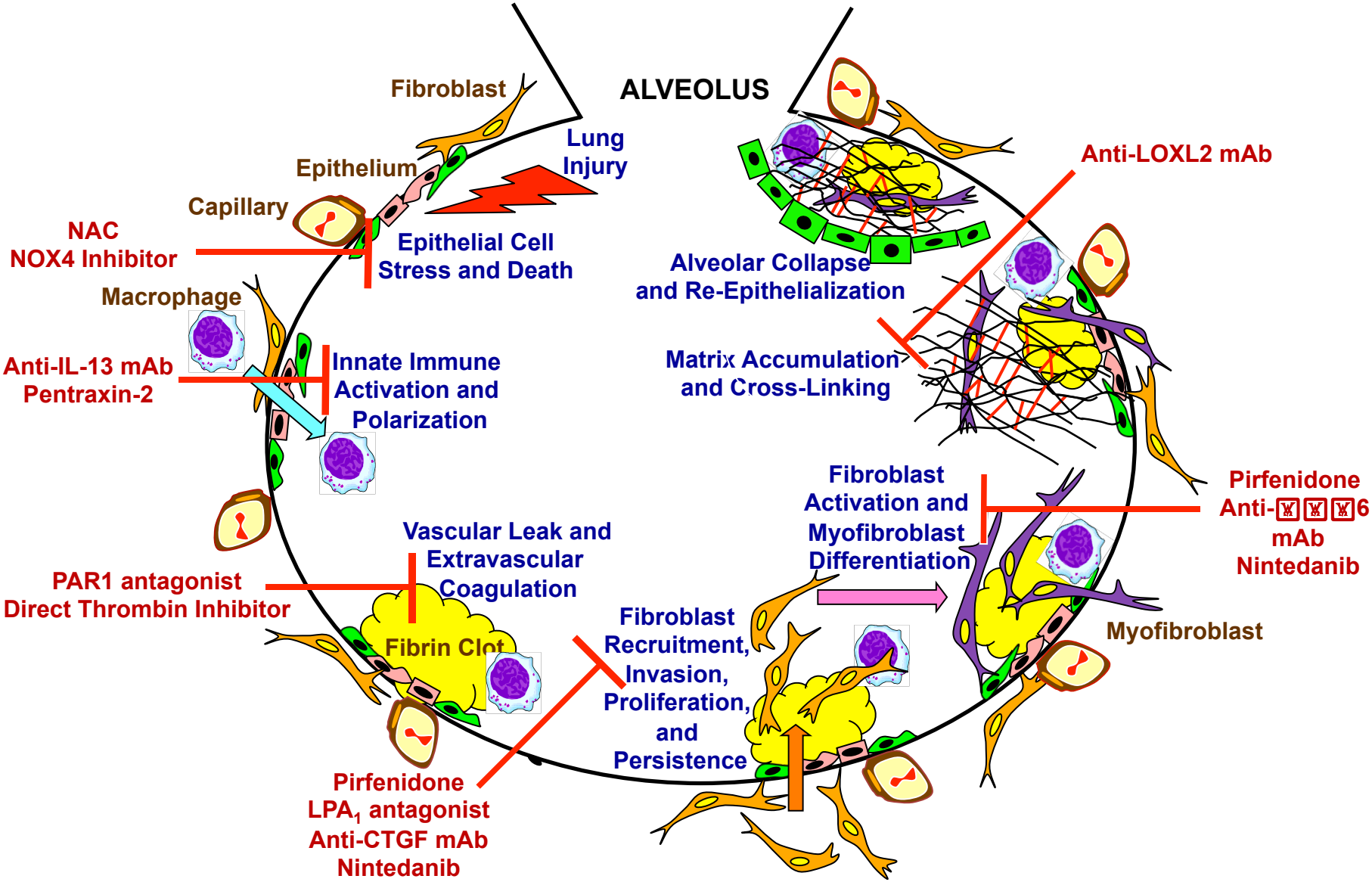
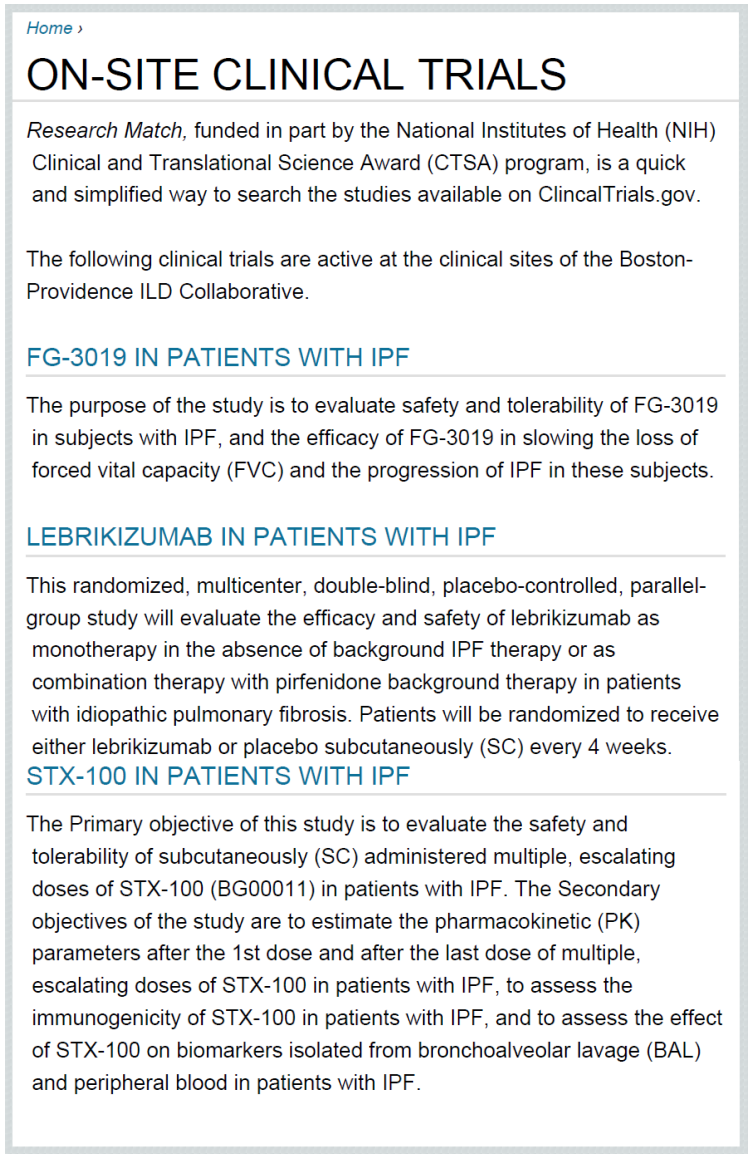
Wound-healing responses that drive fibrosis



New therapeutic strategies targeting wound-healing responses in IPF



Information about available IPF clincal trials is on the ILD Collaborative website



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ON-SITE CLINICAL TRIALS

Research Match, funded in part by the National Institutes of Health (NIH) Clinical and Translational Science Award (CTSA) program, is a quick and simplified way to search the studies available on ClincalTrials.gov.

The following clinical trials are active at the clinical sites of the Boston-Providence ILD Collaborative.

FG-3019 IN PATIENTS WITH IPF

The purpose of the study is to evaluate safety and tolerability of FG-3019 in subjects with IPF, and the efficacy of FG-3019 in slowing the loss of forced vital capacity (FVC) and the progression of IPF in these subjects.

LEBRIKIZUMAB IN PATIENTS WITH IPF

This randomized, multicenter, double-blind, placebo-controlled, parallel-group study will evaluate the efficacy and safety of lebrikizumab as monotherapy in the absence of background IPF therapy or as combination therapy with pirfenidone background therapy in patients with idiopathic pulmonary fibrosis. Patients will be randomized to receive either lebrikizumab or placebo subcutaneously (SC) every 4 weeks.

STX-100 IN PATIENTS WITH IPF

The Primary objective of this study is to evaluate the safety and tolerability of subcutaneously (SC) administered multiple, escalating doses of STX-100 (BG00011) in patients with IPF. The Secondary objectives of the study are to estimate the pharmacokinetic (PK) parameters after the 1st dose and after the last dose of multiple, escalating doses of STX-100 in patients with IPF, to assess the immunogenicity of STX-100 in patients with IPF, and to assess the effect of STX-100 on biomarkers isolated from bronchoalveolar lavage (BAL) and peripheral blood in patients with IPF.

THANK YOU!

QUESTIONS?