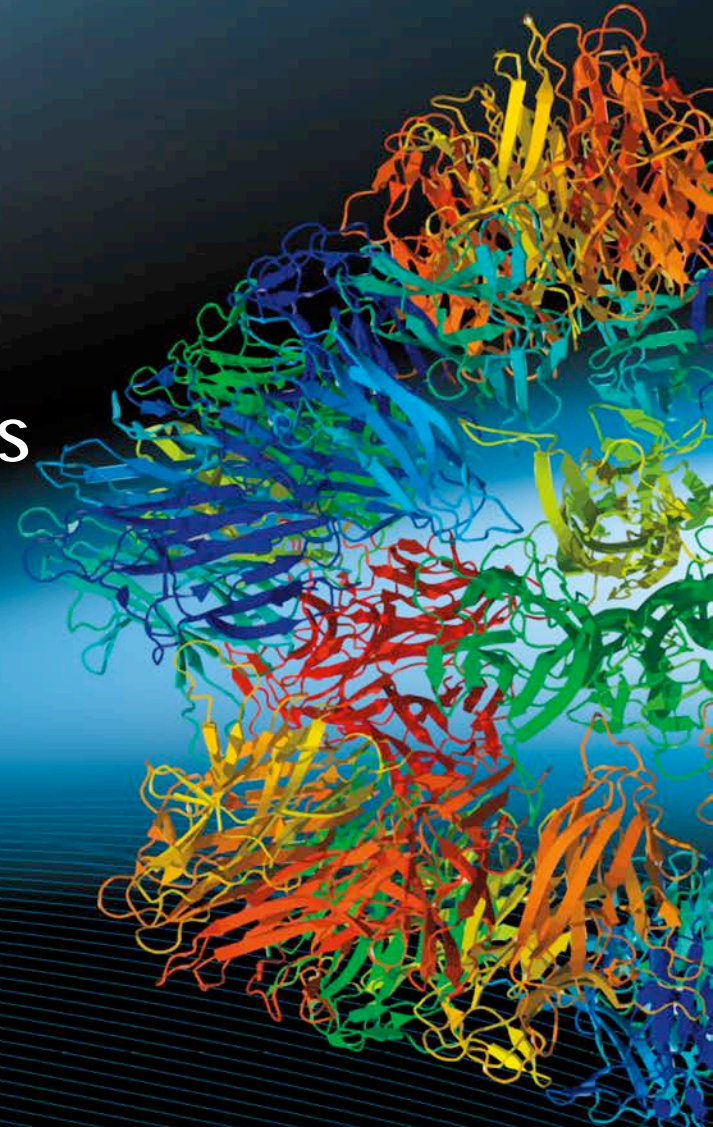




National Jewish Health®

Science Transforming Life®

Leveraging an Academic-Industry Partnership for Commercial Success





For 115 Years, the Nation's Leading Respiratory Hospital





Respiratory Heritage:

115 Years of Respiratory Research and Care



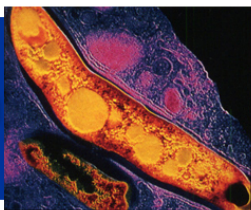
Allergy, Asthma, COPD,
Cystic Fibrosis,
Interstitial Lung Disease,
Lung Cancer, Infectious
Disease, Immunologic
Diseases, Heart Disease



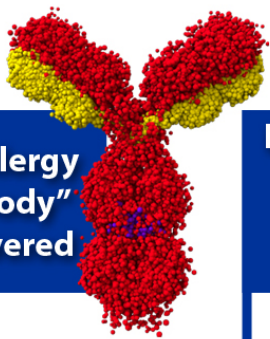
Research Discoveries

- Heritage of Scientific Innovation

First combined chemotherapy treatment for tuberculosis



IgE "Allergy Antibody" discovered



Low-cost culture medium for tuberculosis developed



1940s

1950s

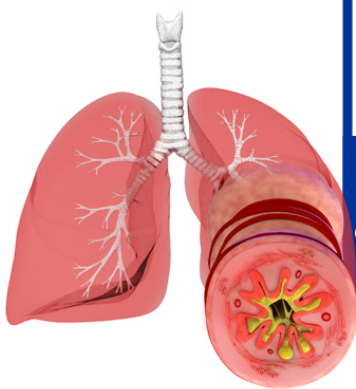
1960s

1970s

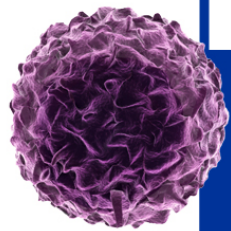
1980s

1990s

2000s



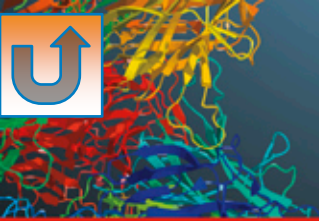
First method of diagnosing asthma



T cell gene identified



Genetic roots of pulmonary fibrosis discovered



Patient Population

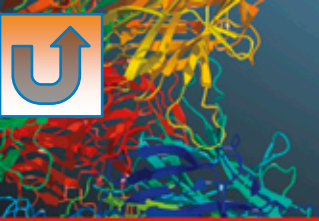
More than 172,000 patient visits in 2013





Asthma

- More than 28,000 patient visits in 2013
- AsthmaNET clinical research network study site
- Clinical program enriched by basic and translational research utilizing patient and biological samples
- Biorepository with blood & tissue specimens
- Asthma models, including mouse models of allergen challenges (house dust mite, *Aspergillus fumigates*, ovabumin), viral (rhinovirus and RSV) and bacterial (mycoplasma, *Pseudomonas*, *Haemophilus*) infections.



COPD

- More than 9,900 COPD patient visits in 2013
- COPDGene® study site - one of the largest studies ever to investigate the underlying genetic factors of COPD
- Clinical program enriched by basic and translational research utilizing patient and biological samples
- Biorepository, including fresh frozen plasma, serum, FFP, leukocytes, PBMC RNA and DNA
- COPD models, including mouse models of COPD (tobacco smoke exposure) and E cigarette exposure and in vitro exposure models to whole smoke and cigarette smoke extract



Cystic Fibrosis

- Largest adult CF Program and clinical research site in the United States
- Over 25 ongoing clinical research studies
- More than 1,250 CF patient visits in 2013
- Clinical program enriched by basic and translational research utilizing patient and biological samples
- Biorepository with sputum & blood specimens
- CF models, including mouse models of chronic infection, cultured primary epithelial cells and biofilms



ILD & Pulmonary Fibrosis

- Largest Interstitial Lung Disease clinic in the United States
- More than 5,200 ILD patient visits in 2013
- Clinical program enriched by basic and translational research utilizing patient and biological samples
- Biorepository, including lung tissue in multiple formats, BAL and blood with fully annotated samples
- ILD animal models and lung fibroblasts

Notable Academic-Industry Partnerships

Pharmaceutical Companies

Academic Centers

Bristol-Myers Squibb	↔	Vanderbilt University
Elan	↔	Cambridge University
Johnson & Johnson	↔	Queensland University
Novartis	↔	University of Pennsylvania
Roche	↔	Cook Medical, Biomet
Sanofi	↔	UCSF
Sanofi	↔	Brigham & Women's Hospital, Harvard Medical School

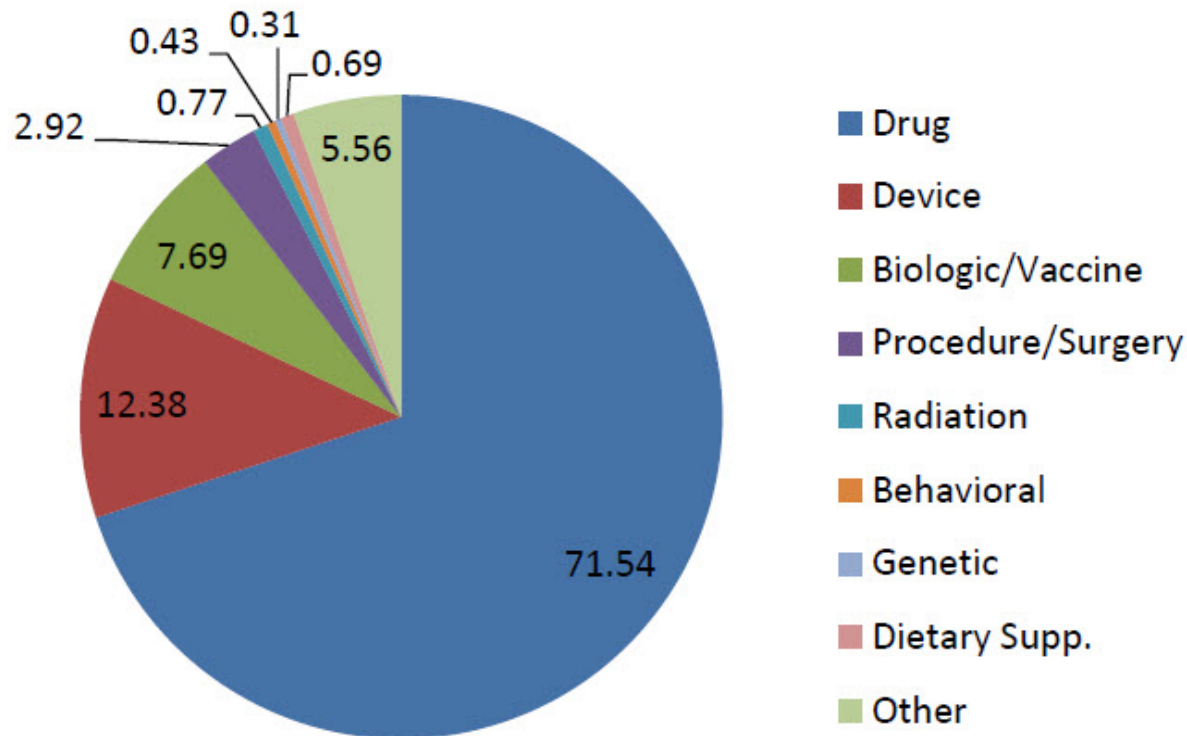


Leveraging Academic Partnerships

- Academic centers are synonymous with translational medicine environments
- Pharmaceutical and medical device manufacturers can successfully navigate the changing landscape of their industries using academic partnerships
- Presentation features case studies highlighting successful pharmaceutical, *In Vitro* Diagnostics (IVD), CLIA Lab for Hire and Imaging partnerships

Leveraging Academic Partnerships

Trials by type of intervention in selected states 2008-2010 (by percent)



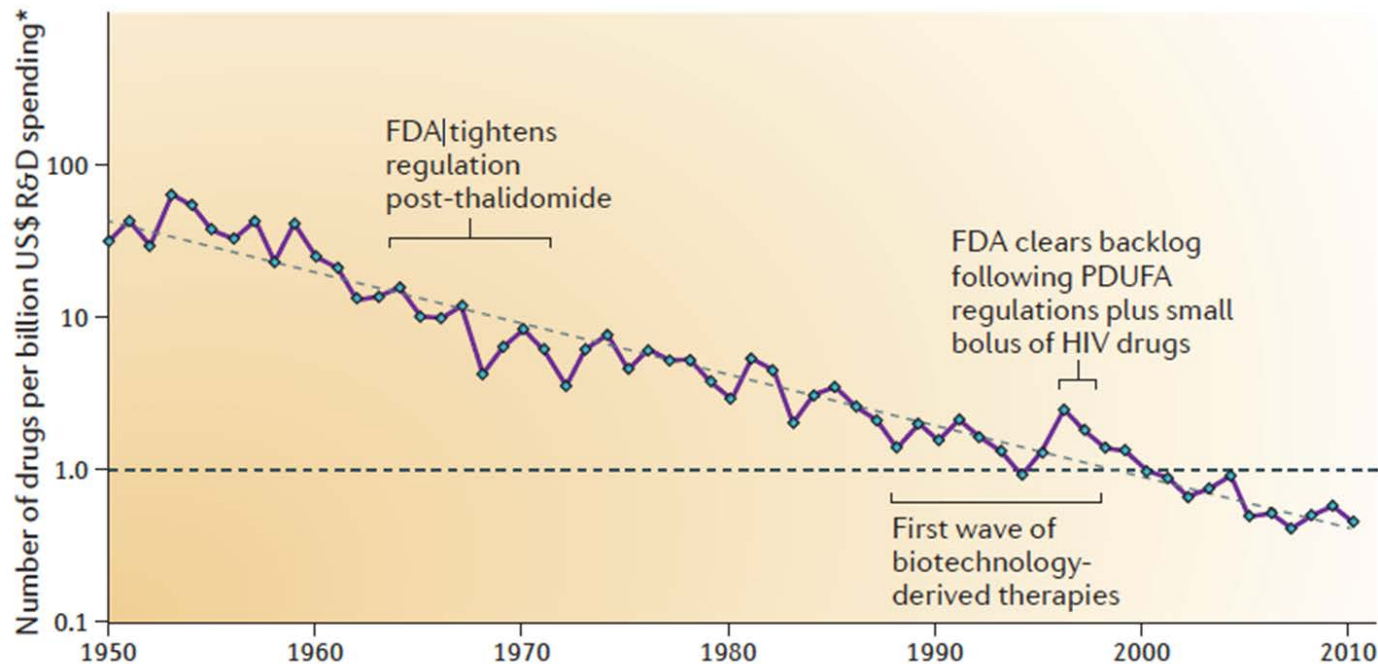
Source: *Academic-Industry Partnerships for Biopharmaceutical Research & Development: Advancing Medical Science in the U.S.*
Tufts Center for the Study of Drug Development & Tufts University School of Medicine



Medical Devices and Materials

- Manufacturers of medical devices and implantable materials
 - *Face similar regulatory & financial challenges*
 - *Rarely have internal resources for biologic safety testing*
- Interactions of materials with the immune system, complement and the coagulation cascade can have safety implications
- Academic centers offer exhaustive testing of pathways for uncommon diseases

Changing Pharmaceutical Landscape



Source: *Diagnosing the decline in pharmaceutical R&D efficiency*. Scannell et al. *Nature Reviews Discovery*, March 2012.

- Many of the common diseases already have effective drugs that are now coming off-patent
- Pharmaceutical industry is forced to develop products for less common diseases which pose new development and economic challenges



Current Challenges for Pharma Industry

- Less common diseases require more basic and translational research in order to develop effective drugs
- Patients are more difficult to recruit for uncommon disease clinical trials
- Drug development costs are similar (\$1 billion or more) despite the potential market being smaller
 - *More expensive health economics*
 - *More difficult to convince payers to reimburse for therapy*



Industry-Academic Synergies

Industry Need

Less common diseases require more basic and translational research in order to develop effective drugs

Academic Advantage

Majority of research in uncommon diseases is already occurring in the academic setting



Industry-Academic Synergies

Industry Need

Patients are more difficult to recruit for uncommon disease clinical trials

Academic Advantage

Academic centers are referral centers for patients with uncommon diseases and often have specialty clinics devoted to these diseases



Industry-Academic Synergies

Industry Need

Drug development costs remain similar despite the potential market being smaller, resulting in much more expensive drugs

Academic Advantage

Clinicians in academic centers are early adopters of new therapeutics and are often less sensitive to the economic issues of community-based practices. They are also involved in professional education



Industry-Academic Synergies

Industry Need

Companion diagnostics are often needed to achieve reasonable health economics and convince payers to reimburse for therapy

Academic Advantage

Highly skilled laboratory directors working with translational researchers can rapidly develop companion diagnostics



Industry-Academic Synergies

Industry Need

The small market and reimbursement for companion diagnostics make them less attractive to many IVD companies and large reference laboratories

Academic Advantage

The mission of academic centers to advance science, educate and meet clinical needs gives them greater latitude and flexibility compared with commercial entities



Academic Center Infrastructure

Clinical

Laboratory

Basic Science

Biobank

Research Database

Office of Research

Academic Center Infrastructure

Clinical

- Expert clinicians
- Access to patients
- Clinical trials
- Market for leading-edge assays
- Imaging

Laboratory

Basic Science

Biobank

Research Database

Office of Research



Academic Center Infrastructure

Clinical

Laboratory

- Expert lab directors
- Assay development
- Commercialization
- Sample testing

Basic Science

Biobank

Research Database

Office of Research

Academic Center Infrastructure

Clinical

Laboratory

Basic Science

- Expert scientists
- Discovery
- Animal models and facilities
- Nextgen sequencing and computational biology

Biobank

Research Database

Office of Research



Academic Center Infrastructure

Clinical

Laboratory

Basic Science

Biobank

Standardized collection and storage of specimens

Research Database

Office of Research

Academic Center Infrastructure

Clinical

Laboratory

Basic Science

Biobank

Research Database

Annotation of samples with data from health record and research studies

Office of Research

Academic Center Infrastructure

Clinical

Laboratory

Basic Science

Biobank

Research Database

Office of Research

IRB, Honest Broker, Tech Transfer

Academic Center Infrastructure

Clinical

- Expert clinicians
- Access to patients
- Clinical trials
- Market for leading-edge assays
- Imaging

Laboratory

- Expert lab directors
- Assay development
- Commercialization
- Sample testing

Basic Science

- Expert scientists
- Discovery
- Animal models and facilities
- Nextgen sequencing and computational biology

Biobank

Standardized collection and storage of specimens

Research Database

Annotation of samples with data from health record and research studies

Office of Research

IRB, Honest Broker, Tech Transfer



Academic Partnership Considerations

- Leadership support of partnering institutions is vital
- Flexible, long-term strategic partnerships work best
- Have a shared vision and strategy



Academic Partnership Considerations

- Encourage cross-fertilization of ideas
- Focus on execution rather than on intellectual property (IP) initially
- Create cross-functional operational teams
- Focus on synergies and a win-win alliance
- Utilize project management across organization



Academic Partnership Models

Commonly used

- Unrestricted grants
- Fee-for-service

Increasingly popular

- Corporate venture capital funds
- Academic drug discovery centers

Emerging

- Risk sharing
- Competitive grants



Case Studies

- Imaging Study: Lung Nodules
- IVD Case Study: Lung Cancer
- CLIA Lab for Hire Case Study:
Age-related Macular Degeneration (AMD)
- Pharmaceutical Case Study:
Pulmonary Fibrosis



Case Studies

- **Imaging Study: Lung Nodules**
- IVD Case Study: Lung Cancer
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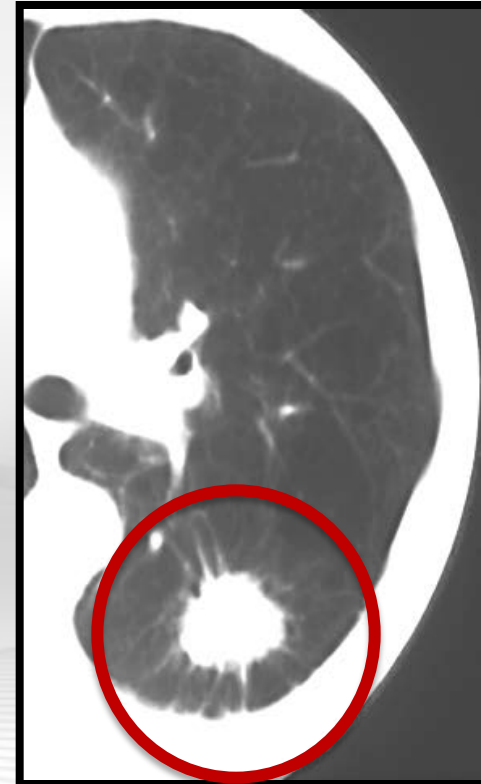
Lung Cancer

- Lung cancer is the leading cause of cancer death in men and women
- Less than 25% patients diagnosed early (Stage I/II)
- 5-year survival rates better in patients diagnosed at an early stage



Imaging Study: Lung Nodules

- NLST showed a benefit to screening high risk patients with CT scan, however, national expense would be prohibitive
- Need more specificity to reduce follow up expense for detected nodules
- Need better differentiation of ground glass lesions



Imaging Study: Lung Nodules

Imaging equipment manufacturer

- Configurable software platform
- Equipment at reduced cost

National Jewish Health

- Image analysis of COPDgene cohort
- PET-CT study of nodules
- Dosage study for ground glass nodule detection

Imaging Study: Lung Nodules

Win

Imaging Company

- Better analysis software for detecting potentially malignant nodules
- Data on efficacy of CT-PET in differentiating malignant lesion
- Dosage for groundglass lesions

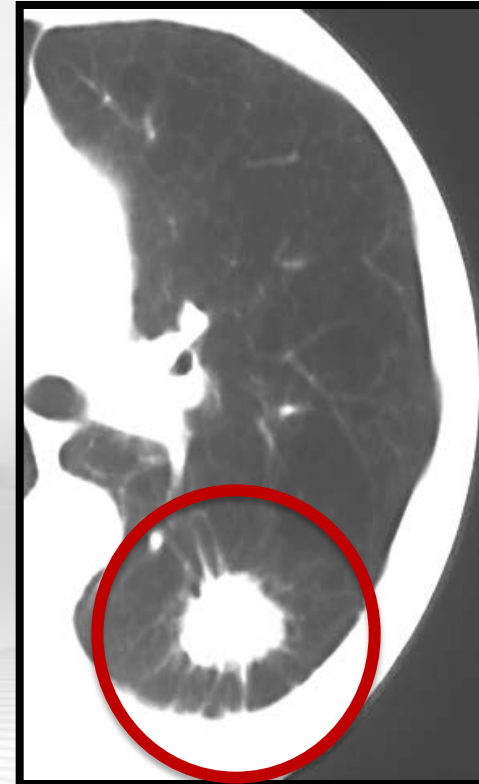
Win

National Jewish Health

- More effective clinical screening
- Publications
- Reduced our cost for equipment purchase
- Lung nodule registry

IVD Case Study: Lung Cancer Unmet Need

- CT is an expensive way to screen all high-risk patients for lung cancer
- CT scans detect many nodules, most of which are benign
- Need low-cost diagnostic test to risk stratify patients for screening
- Combining biomarker test with CT imaging may be optimal





IVD Case Study: Partnership Synergies

Diagnostics Company

- miRNA discovery
- Intellectual property
- Technology expertise
- Capital

National Jewish Health

- Clinical leadership in lung cancer
- Access to high-risk COPD patient population
- Extensive biobank tied to research database
- Biomarker validation expertise



IVD Case Study: Partnership Rewards

Win

Diagnostics Company

- Independent validation of miRNA biomarkers
- Potential IVD test for clinical and trial markets

Win

National Jewish Health

- Enhanced miRNA biomarker validation capabilities
- Potential early lung cancer screening LDT for high-risk patients
- Contract services revenue



Case Studies

- Imaging Study: Lung Nodules
- IVD Case Study: Lung Cancer
- **CLIA Lab for Hire Case Study:
Age-related Macular Degeneration (AMD)**
- Pharmaceutical Case Study: Pulmonary Fibrosis

CLIA Lab Case Study: AMD



- Age-related Macular Degeneration (AMD) is leading cause of blindness for people over the age of 55 in the Western world
- In 2010, there were about 2 million people in the U.S. with late-stage AMD (*Source: National Eye Institute*)
- Known genetic markers account for 70% of the risk of development



CLIA Lab Case Study: AMD Diagnostics Unmet Need

- No early genetic test for AMD diagnosis
- Need for prognostic test to stratify high-risk patients for aggressive clinical management

CLIA Lab Case Study: Partnership Synergies

Diagnostics Company

- Intellectual property
- Strong sales and marketing team

National Jewish Health

- Test development
- CAP/CLIA/ISO 15189 certified laboratory
- Billing & client services



CLIA Lab Case Study: Partnership Rewards

Win

Diagnosics Company

- Break into U.S. market
- Test revenue
- Access to clinical & research resources for the next generation assay development

Win

National Jewish Health

- Enhanced capabilities of molecular diagnostic services
- New market penetration
- Test revenue

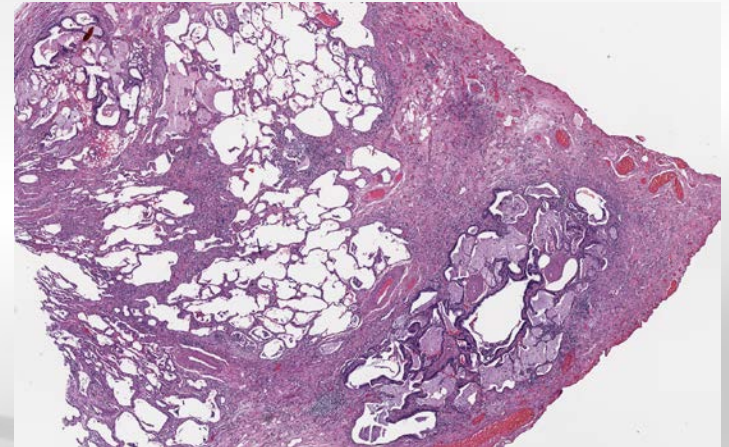


Case Studies

- Imaging Study: Lung Nodules
- IVD Case Study: Lung Cancer
- CLIA Lab for Hire Case Study: Age-related Macular Degeneration (AMD)
- **Pharmaceutical Case Study: Pulmonary Fibrosis**

Pharma Case Study: Pulmonary Fibrosis

- Pulmonary fibrosis is a fatal disease. Lung tissue becomes thickened and scarred
- Prevalence in United States 130,000- 200,000
- Approximately 50,000 new cases per year in the U.S.
- 40,000 Americans die each year



Source: 2013 Pulmonary Fibrosis Foundation, pulmonaryfibrosis.org



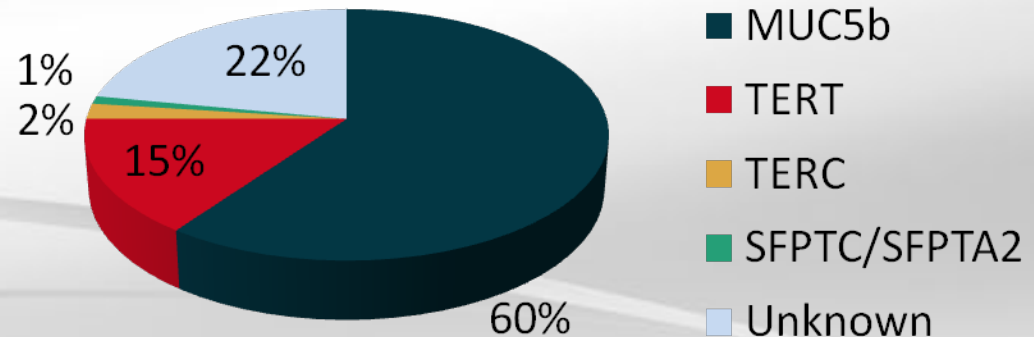
Pharma Case Study : Pulmonary Fibrosis Unmet Need

- No definitive genetic test to diagnose Pulmonary Fibrosis
- Current genetic tests such as TERT, TERC, SFTPC and SFTPA2 are used to assess familial risk
- No prognostic test currently available

Pharma Case Study: MUC5b as a Prognostic Marker

- MUC5b SNP accounts for approximately 60% of genetic risk in both Idiopathic and Familial Pulmonary Fibrosis
- Improved survival in Pulmonary Fibrosis patients carrying MUC5b SNP

PF Genetic Factors





Pharma Case Study: Partnership Synergies

Pharmaceutical Company

- Patient samples from clinical trial
- Drug development expertise in Pulmonary Fibrosis

National Jewish Health

- Clinical leadership in Pulmonary Fibrosis
- Gene discovery
- Biomarker validation expertise
- Molecular diagnostics expertise

Pharma Partnership: Partnership Rewards

Win

Pharmaceutical Company

- Better understanding of disease mechanism
- IP revenue
- Potential CDx development

Win

National Jewish Health

- Prognostic test for patients
- Risk assessment test for family members
- Better understanding of disease mechanism
- Test and IP revenue



Summary of Academic-Industry Partnerships

Win

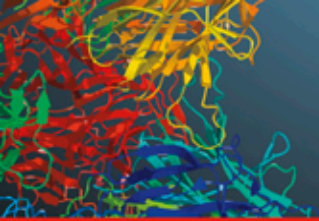
Industry Partner

- Access to patients & biospecimens
- Access to Key Opinion Leaders
- IP revenue
- Faster, more innovative product development

Win

Academic Partner

- Funding for research
- Access to capital
- Revenue from new diagnostic tests
- Entry to new markets



Questions