Leveraging an Academic-Industry Partnership for Commercial Success
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**
  - 1940s

- **IgE “Allergy Antibody” discovered**
  - 1970s

- **Low-cost culture medium for tuberculosis developed**
  - 1980s

- **First method of diagnosing asthma**
  - 1950s

- **T cell gene identified**
  - 1990s

- **Genetic roots of pulmonary fibrosis discovered**
  - 2000s
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

<table>
<thead>
<tr>
<th>Pharmaceutical Companies</th>
<th>Academic Centers</th>
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<tbody>
<tr>
<td>Bristol-Myers Squibb</td>
<td>Vanderbilt University</td>
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<tr>
<td>Elan</td>
<td>Cambridge University</td>
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<tr>
<td>Johnson &amp; Johnson</td>
<td>Queensland University</td>
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<tr>
<td>Novartis</td>
<td>University of Pennsylvania</td>
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<tr>
<td>Roche</td>
<td>Cook Medical, Biomet</td>
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<tr>
<td>Sanofi</td>
<td>UCSF</td>
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<tr>
<td>Sanofi</td>
<td>Brigham &amp; Women’s Hospital, Harvard Medical School</td>
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• 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
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
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
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 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 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

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Office of Research
Academic Center Infrastructure

- Clinical
- Laboratory
  - Expert lab directors
  - Assay development
  - Commercialization
  - Sample testing
- Basic Science

Biobank
Research Database
Office of Research

Advanced Diagnostic Laboratories National Jewish Health®
Academic Center Infrastructure

Clinical

Laboratory

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

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Annotation of samples with data from health record and research studies

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Academic Center Infrastructure

Clinical
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- Imaging

Laboratory
- Expert lab directors
- Assay development
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- Sample testing

Basic Science
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- 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

Advanced Diagnostic Laboratories National Jewish Health®
Academic Partnership Considerations

- Leadership support of partnering institutions is vital
- Flexible, long-term strategic partnerships work best
- Have a shared vision and strategy
• 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
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- CLIA Lab for Hire Case Study: Age-related Macular Degeneration (AMD)
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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

<table>
<thead>
<tr>
<th>Imaging Company</th>
<th>National Jewish Health</th>
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<tbody>
<tr>
<td>• Better analysis software for detecting potentially malignant nodules</td>
<td>• More effective clinical screening</td>
</tr>
<tr>
<td>• Data on efficacy of CT-PET in differentiating malignant lesion</td>
<td>• Publications</td>
</tr>
<tr>
<td>• Dosage for groundglass lesions</td>
<td>• Reduced our cost for equipment purchase</td>
</tr>
<tr>
<td></td>
<td>• Lung nodule registry</td>
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</table>
• 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

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<tr>
<td>• Independent validation of miRNA biomarkers</td>
<td>• Enhanced miRNA biomarker validation capabilities</td>
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<tr>
<td>• Potential IVD test for clinical and trial markets</td>
<td>• Potential early lung cancer screening LDT for high-risk patients</td>
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<td>• Contract services revenue</td>
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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
• 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
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

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<td><strong>Diagnostics Company</strong></td>
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<tr>
<td>• Break into U.S. market</td>
<td>• Enhanced capabilities of molecular diagnostic services</td>
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<tr>
<td>• Test revenue</td>
<td>• New market penetration</td>
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<tr>
<td>• Access to clinical &amp; research resources for the next generation assay development</td>
<td>• Test revenue</td>
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**Win Win**
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, SFTP C and SFTPA2 are used to assess familial risk
- No prognostic test currently available
• 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
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

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<td>• Better understanding of disease mechanism</td>
<td>• Prognostic test for patients</td>
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<tr>
<td>• IP revenue</td>
<td>• Risk assessment test for family members</td>
</tr>
<tr>
<td>• Potential CDx development</td>
<td>• Better understanding of disease mechanism</td>
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<td>• Test and IP revenue</td>
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## Summary of Academic-Industry Partnerships

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<th>Win Industry Partner</th>
<th>Win Academic Partner</th>
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<tr>
<td>• Access to patients &amp; biospecimens</td>
<td>• Funding for research</td>
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<td>• Access to Key Opinion Leaders</td>
<td>• Access to capital</td>
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<tr>
<td>• IP revenue</td>
<td>• Revenue from new diagnostic tests</td>
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<tr>
<td>• Faster, more innovative product development</td>
<td>• Entry to new markets</td>
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Questions