The Power to Cure: Therapeutic Innovation in Academia

Leslie Molony, Ph.D.
November 7, 2011
Sanford-Burnham Medical Research Institute: *a Non Profit Basic Research Institute*

- 3 Sites of operation
- 1200 People (over 500 Ph.D. scientists)
- 80+ Faculty
- $166 MM annual operating budget in 2011
## Grants are the Major Revenue Source

<table>
<thead>
<tr>
<th>SOURCE</th>
<th>* Percent (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Federal Research Grants</td>
<td>58.0%</td>
</tr>
<tr>
<td>Other Research Grants</td>
<td>8.9%</td>
</tr>
<tr>
<td>Philanthropy</td>
<td>12.6%</td>
</tr>
<tr>
<td>Investment Income</td>
<td>3.0%</td>
</tr>
<tr>
<td>Licenses, Partnerships</td>
<td>17.5%</td>
</tr>
<tr>
<td>State Funding, Other</td>
<td></td>
</tr>
</tbody>
</table>

*Of Annual Operating Budget*
Novel Combination of Talents

High-Impact Research
- Diabetes & Obesity
- Neuroscience & Aging
- Cardiovascular Disease
- Oncology
- Inflammation
- Infectious Diseases
- Stem Cell Center

Early Stage Drug Discovery
- Pharmaceutical Experience
- Business Development Expertise
- Entrepreneurial Environment

Translational Research
- Collaborations with Clinical Scientists – Florida Hospital and the Joint TRI
Research Areas - Orlando

- Cardiometabolic Diseases
- Diabetes and Obesity
- Cancer
- Fibrosis and Vascular complications

SBMRI Local Metrics
- 17 Invention Disclosures
- ~ 140 Publications
- $52M in Research $

Since Inception in Orlando

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**Orlando Sentinel**

*Burnham doctors' discovery could lead skin cancer treatment*

November 01, 2010 | By Linda Shrievess

American doctors perform about 8 million skin biopsies each year, searching for skin diseases. Now a scientist at Sanford Burnham has discovered a piece of genetic material called a microRNA that could help doctors detect and someday treat melanoma, the deadly skin cancer.

Call it the case of the disappearing microRNA, but what Perera discovered may prove to be very important.

When Perera studied healthy melanocytes — the cells that produce pigment in our skin — he found that they contained microRNA 211. But when he looked at melanoma — cancerous melanocytes — he found the microRNA had disappeared.

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Since Inception in Orlando
State-of-the-Art Technologies

Enable Target & Drug Discovery

- Robotic ultra-high throughput and high content screening
  - Screen > 300K wells/day
  - Libraries of > 700K chemicals
    - MLPCN center
    - NCI screening center
    - Sanford-Burnham libraries

- Genomics/proteomics technology to relate gene expression to drug activity

- Fully staffed with industry-trained scientists

Filling the Drug Discovery Pipeline

- Discover therapeutics for unmet medical needs
- Develop novel reagents that will expedite discovery
Superior Technology Infrastructure

**Animal Resources**
- Animal Care & Procedures
- *In Vivo* Analysis
- Cardiometabolic Phenotyping
- Model Organisms

**Cell Imaging & Analysis**
- Cell Imaging
- Histology and Molecular Pathology
- Flow Cytometry

**Stem Cells**
- Embryonic Stem Cells
- Electrophysiology

**Genomics**
- DNA Analysis
- Microarray & QPCR
- HT DNA sequencing

**Functional Genomics**
- siRNA/shRNA screening
- Viral Vectors

**Proteomics & Metabolomics**
- Proteomics
- Metabolomics

**Bioinformatics & Data Management**
- Bioinformatics
- Cheminformatics
- 3D Computational Modeling

**Structural Biology**
- Crystallography
- Nuclear Magnetic Resonance
- Protein Production & Analysis

**Conrad Prebys Center For Chemical Genomics**
- Assay Development
- Libraries and HT Screening
- Ultra-HTS
- High Content Screening
- Cheminformatics

**Medicinal Chemistry & Pharmacology**
- Medicinal Chemistry
- SAR by NMR
- Experimental Pharmacology

**31 core facilities**
Translational Research
Guides Drug Development

This collaboration provides the most advanced translational capabilities among non-profit institutions

- Bridges the typical silos of clinical and laboratory research
- Translates fundamental biomedical discoveries into new therapies
- Implements therapeutic strategies tailored to the individual patient
- Expedites clinical trial design and therapeutic strategies in a research setting (Phase 0)
Parallel Metabolic Phenotyping

Metabolic Phenotyping In the Laboratory

Metabolic Phenotyping In the Clinic

Bridging Bench to Bedside...... and Bedside To Bench.
Metrics for Phenotype in Humans and Mice:

Research Cores
1. Clinical Operations
2. Clinical Research Unit (inpatient)
3. Database and Bioinformatics
4. BioRepository
5. Metabolism & Energy expenditure
6. Imaging & body composition
7. Recruiting

- Metabolomics
- Transcriptome
- Proteomics
- Epigenome
- Next Gen. Seq.
Metabolomics – Precision Medicine

- Metabolites are ~8000 substances
- Mass Spectrometry
- Measures changes in Genomics and Environment.....
- Can detect changes after therapeutic treatments.....
Representative Drug & Target Discovery Programs

• Adipogenesis and Energy Metabolism Targets
  • Sheila Collins, Dev Sikder

• Epigenetic Regulation
  • Sepideh Khorasanizadeh, Ranjan Perera

• Myocyte-driven energy regulation, cardiac myocyte as well as skeletal (exercise in a pill; the couch potato mouse)
  • Kelly, Osborne, Rastinejad, Gulick, L. Smith

• Glucose metabolism and Insulin Regulation
  • Julio Ayala, Judith Altarejos, Fred Levine (Islet Biology), Bjorn Tyrberg, Zhen Jiang

• Cancer Metabolism
  • Jorge Moscat, Dan Kelly, Adam Richardson, Jeff Smith, etc.
Goals of Partnerships with Industry & Current Partners

Innovate further along the discovery pathway

- Disease Pathway interrogation
- Specific novel target discovery and validation
- New approaches to diagnostics and personalized medicine
- High content, phenotypic and UTS screening
- Hit Identification using CPCGC

- Takeda Pharmaceutical Co., Ltd./Florida Hospital
  - Novel Peripheral obesity targets
- Ortho McNeil Janssens
  - Alzheimer’s Disease and Neuropsychiatric Disease specific targets
Sanford-Burnham in Florida

- Well respected scientists engaged in medical research at universities and institutes in Florida
- Extensive, well managed healthcare systems
- Collaborative, multi-disciplinary communities
  - Simulation, VR
  - Optics and imaging applications
  - Space sciences
- Florida biosciences community
  - Political collaboration (FRC)
  - Entrepreneurial environment
  - Growing advocacy for Life Sciences
Our mission is to conduct world-class collaborative research dedicated to finding cures for human disease, improving quality of life, and thus creating a legacy for our employees, partners, donors and community.