

The Children's Hospital of Philadelphia Frontier Programs

October 2017

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Outline

- Origin of the CHOP Frontier Programs Initiative
- Basic elements of Frontier Programs
- Current Frontier Programs

Frontier Programs Background

- ~15 years ago, CHOP introduced “Luminary Programs” as a strategy to:
 - highlight distinctive clinical care
 - attract patients nationally and internationally
 - contribute to the reputation and health of the institution
- The CHOP strategic planning process in FY2013 concluded that distinctive clinical programs should remain a priority but should transition to another form

Background

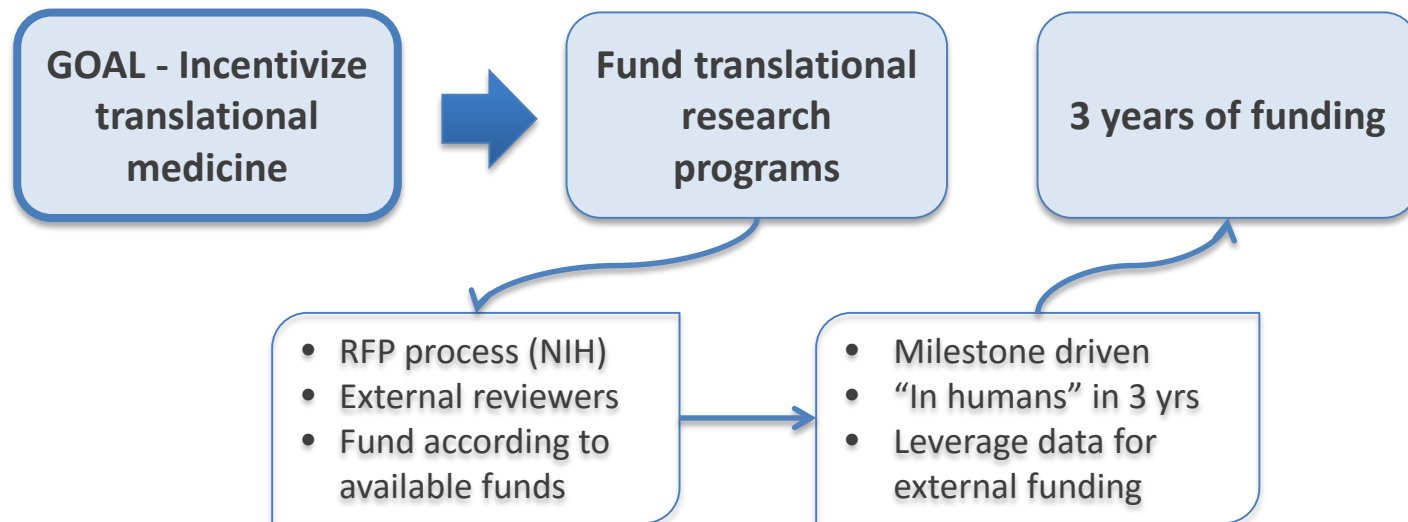
During the FY13 strategic planning process, high priorities included...

- Innovation would be the leading differentiator for CHOP in the market place
- Innovation would be defined as bringing novel approaches to clinical treatment and novel business models into practice
- A continued focus on operational excellence and customer intimacy would be critical

Background

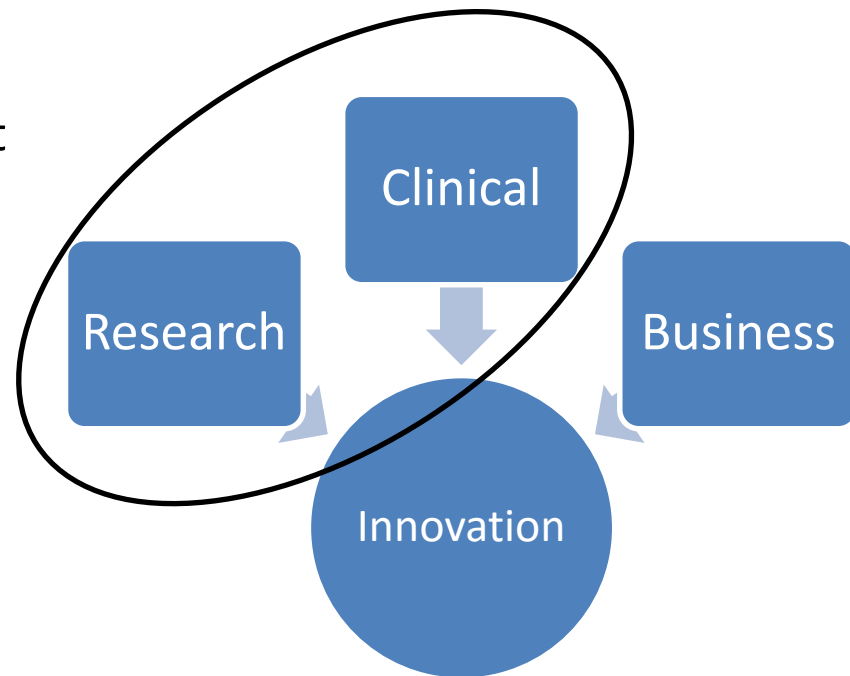
During the FY13 strategic planning process ...

- Discussions on research innovation (that would lead to clinical innovation) included a proposal for a different model of funding
- Allocate dollars and encourage faculty to compete for funding based on their best ideas



Background

- As we convened in 2014 to discuss how to support clinical innovation at CHOP, we began to merge the funding concept for research innovation with clinical innovation
- We noted that Luminary Programs shared common themes but had followed different development paths
 - Strong clinical champion
 - Delivery of extraordinary care
 - National and international patient draw
 - National and international reputation
 - Multidisciplinary program design
 - Impact on financial health of CHOP
- We agreed to conduct focus groups to gain input into how best to broadly support research and clinical innovation



Goal: Increase the pace of development and the number of “luminary” programs

Focus Group Recommendations

- *Process*: Further pursue process with competitive component to support innovation across clinical, research, and business dimensions.
- *Language*: Consider “impact” or “innovation” or “frontier” instead of “luminary programs.”
- *Funding Stipulations*: Attend closely to criteria for funding and metrics for determining ‘success.’
- *Infrastructure*: Consider how to broaden the input into innovation at CHOP, how to facilitate early support in idea/proposal development, and review of COI, IP, and tech transfer.
- *Dollars*: Sort out how much, for how long, and from where.

Proposal – “Frontier” Programs

■ **Components**

- Distinctive clinical program that provides extraordinary care
- Associated translational research program
- Potential for commercialization

■ **Suggested pilot**

- CART19/cancer immunotherapy program

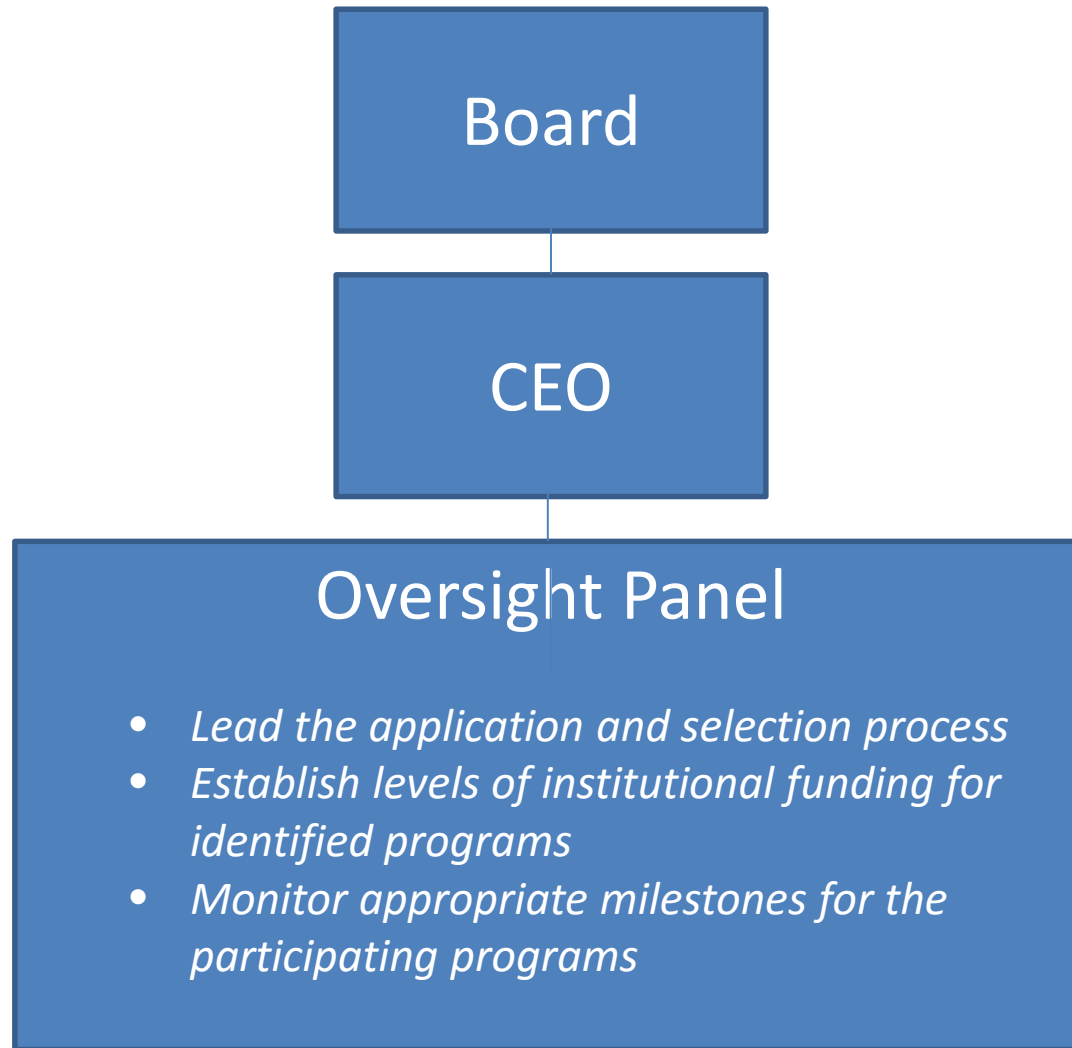
■ **Future**

- 2 programs per year, for total of 10 programs over a 5 year period

Proposal – Funding Approach

- \$5M in FY16 budget
- \$10M/year for FY17-FY21 allocated in 7-year financial plan for Frontier Programs and Office of Entrepreneurship and Innovation
 - *Selected programs will be eligible to receive up to \$1M in year 1 and \$1.5M per year for years 2 and 3*
- Potential future funding sources
 - Successful Frontier Programs
 - Philanthropy/Endowment
 - Royalty income
 - Angel investors

Proposal – Governance Structure



Frontier Programs

- Frontier Programs proposal was approved by CEO Council in spring 2015
- Plan to launch CART19/Cancer Immunotherapy Program as a pilot Frontier Program in July 2015
- Plan to identify an additional pilot program in FY16

Frontier Programs Overview

- Designated Frontier Programs must have three essential components:
 - A distinctive clinical program that provides extraordinary care, generally involving children with complex conditions
 - An associated translational research program that facilitates further advancement of the extraordinary care
 - A return on investment that can support ongoing innovation
- Frontier Programs objectives:
 - Will differentiate CHOP because of their unique combination of translational research and exceptional clinical care
 - Will be vital to CHOP's mission, reputation, and financial well-being and contribute to our success regionally, nationally, and internationally.

Frontier Program Oversight Panel

- Frontier Programs Oversight Panel responsible for:
 - Selection of programs
 - Assignment of funding and other resources
 - Monitoring of program progress (milestones, metrics)
- Oversight Panel members
 - Physician-in-Chief & Chair, Department of Pediatrics (Chair)
 - Surgeon-in-Chief & Chair, Department of Surgery
 - COO
 - CFO
 - CSO
 - SVP for Outpatient and Clinical Services
 - SVP for Strategy & Planning
 - VP for Entrepreneurship & Innovation

Frontier Programs Application Process

- Applicants are asked to submit the following:
 - **Program Summary, including:** clinical innovation and differentiators, associated translational research, overall clinical and research goals of the program, use of existing clinical and research platforms, and opportunities for commercialization and other forms of return on investment
 - **Proposed Business Plan for 2-3 years, including:** volume projections, hospital clinical revenue, current/anticipated research revenue, resource requirements (*clinical and research*), financial analysis and proposed budget (*separate clinical and research components*), and anticipated program market share
 - **Proposed milestones and metrics (*Research, Clinical, Programmatic*)**

Frontier Programs Overview

- **FY16 Pilot Programs**

- CART19/Cancer Immunotherapy Program
- Cardiac Center - Lymphatic Imaging and Intervention Program

Frontier Programs Overview

■ **FY16 Pilot Programs**

- CART19/Cancer Immunotherapy Program
- Cardiac Center - Lymphatic Imaging and Intervention Program

■ **FY17 Programs**

- Inflammatory Bowel Disease Program
- Thoracic Insufficiency Program

■ **FY18 Programs**

- Mitochondrial Medicine Program
- Pediatric Airways Program
- Neonatal Chronic Lung Disease Program

Steve Grupp, MD, PhD

Program Goals:

- Develop and deploy CART19: targeted/engineered T cell therapy for acute lymphoblastic leukemia (ALL), the most common childhood cancer
- Expand cell therapy to treat newly diagnosed high risk ALL, AML, other malignancies
- Develop combination cell therapy to attack more than 1 target and decrease relapse risk
- Combine checkpoint inhibitors with CAR T cells against solid tumors to overcome limitations in cell trafficking to solid tumors
- Remain most active & innovative pediatric cell therapy program in world

Yoav Dori, MD and Max Itkin, MD

Program Goals:

- Define relationship between abnormalities in anatomy of lymphatic circulation and disease
- Develop approaches and imaging agents to refine imaging of lymphatic circulation
- Develop interventions to circumvent lymphatic abnormalities (occlusive techniques, ligation of vessels, lymphatic duct pump)
- Treat complications of congenital heart disease (e.g. plastic bronchitis, chylothorax) and expanding list of congenital abnormalities (e.g. chylothorax, lymphangiomatosis, protein losing enteropathy, etc)

Robert Campbell, MD

Program Goals:

- Provide multispecialty care for patients with life threatening spine and chest wall deformities associated with respiratory failure (scoliosis, inherited skeletal diseases, other genetic diseases)
- Capitalize on VEPTR (**vertical expandable prosthetic titanium rib**) device
- Establish a basic research animal model program to facilitate development of new devices
- Develop advanced patient imaging techniques to assess outcomes
- Expand understanding of relationship between treatment regimens, growth, pulmonary function, activities of daily living
- Examine correlation between genetic determinants and outcomes

Robert Baldassano, MD

Program goals:

- Provide multidisciplinary care for patients with inflammatory bowel disease (IBD) (currently >1,400 patients)
- Identify novel genetic, microbiome, and environmental factors that cause IBD and very early onset IBD (VEO-IBD)
- Identify the immunologic mechanisms that lead to VEO-IBD
- Identify and explore precision targeted therapies in animal models of IBD and in humans (MAbs, novel compounds)

Marni Falk, MD

Program goals:

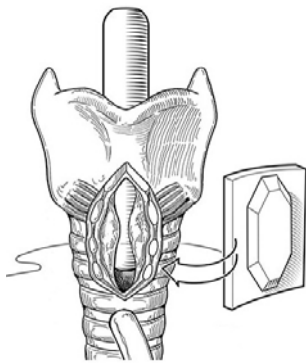
- Provide integrated multidisciplinary clinical care to patients with mitochondrial disease
- Develop new diagnostic tests for patients with mitochondrial disease (molecular, biochemical, histologic/anatomic)
- Pursue basic, translational, and clinical research to develop novel treatments and improve the health of patients of all ages living with mitochondrial disease (no current proven treatments)



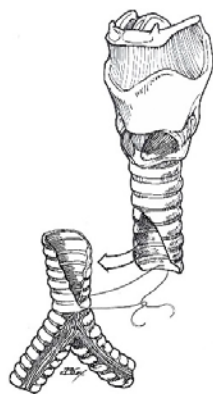
Ian Jacobs, MD

Program goals:

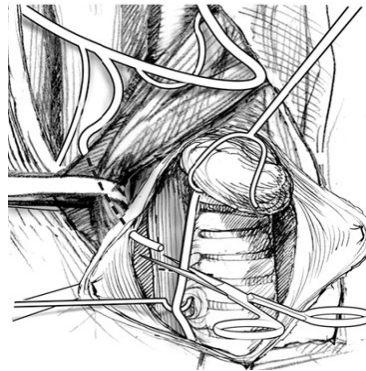
- Provide state-of-the-art medical and surgical interventions for patients with airway disease, using endoscopic and open airway procedures
- Expand neonatal airway program and pediatric voice program
- Develop novel cartilage substitutes using tissue engineering techniques
- Develop novel treatment strategies for lithium battery injury



**Laryngotracheal
Reconstruction**



**Slide
Tracheoplasty**



Reinnervation

William Fox, MD

Program goals:

- Expand internationally recognized multi-disciplinary program dedicated to the treatment of severe chronic lung disease, a common complication of prematurity (35% of infants <1500 g)
- Examine therapeutic efficacy of partial liquid ventilation using perflubron (CHOP holds IND for safety and feasibility trial)

Impact of Frontier Programs

- Growth in cancer immunotherapy, lymphatics, thoracic insufficiency, and IBD patient populations
- FDA approval of Kymriah (tisagenlecleucel) for B-cell ALL in children and adults up to age 25 years
- Application of lymphatic treatment to novel conditions
- Initiation of clinical trial of novel treatment for VEO IBD
- Progress toward development of improved VEPTR device for thoracic insufficiency

Impact of Frontier Programs

- International Medicine Program
- National partnerships
- Facility planning

Questions?