A Few Housekeeping Notes

• Q&A time will follow presentation

• During the presentation, use the chat box in WebEx to ask questions

• Please remain muted unless you need to speak. This minimizes background noise.

• When you speak, please identify yourself by name and work area to help others recognize you.

• This WebEx is being recorded and will be posted on the CTSI website
Welcome to Duke CTSI Grand Rounds

July 20, 2018
Moderated by Rebbecca Moen
Chief Administrative Officer, Duke CTSI

Please note that you have been muted upon entry to this WebEx.
Click the microphone by your name in the participant list to unmute as needed.

This presentation is being recorded.
Project Management and Funding for Translational Research

Vonda Rodriguez, PhD, PMP
Director of Operations, CTSI Accelerator
The Duke Clinical and Translational Science Institute is the administrative hub for Duke’s Clinical Translational and Science Award (CTSA).

One of 57 hubs nationwide.

Leadership:

- Ebony Boulware, PI CTSA Grant & Director
- James McNamara, Co-PI CTSA Grant
- Jennifer Li, Co-PI CTSA Grant
- Rebecca Moen, CTSI Chief Administrative Officer
- Lynn Sutton, CTSI Director of Operations

Source: National Center for Advancing Translational Science
Translational Process

**Duke Investigator with an idea**

- Research progresses from bench to clinical trial
- Researcher raises non-dilutive follow-on funding to support trials and R&D
- Duke
- **NewCo Creation**
  - CEO retained
  - SBIR funds secured
  - License agreement with Duke, Angel, VC, Strategic Partner
- OLV licenses to strategic partner
- Students build business case, compete nationally

**Program Management**
- Project management
- Business plan
- Grant preparation
- Regulatory
- Biostatistics
- OLV & COI Coordination

**Oversight Committee**
- Quarterly reports
- Progress to plan
- Program portfolio
- Project selection

**Side Car Funds**
- IP Research & Consultants

**CTSI and COULTER Pilot funds milestone driven value creation**

**Content for this graphic: National Center for Advancing Translational Science**

**Duke Clinical & Translational Science Institute**

[Link to ctsi.duke.edu]
Pilot Programs

Provides translational funding, technical expertise, project management and strategic logistics to move Duke discoveries from bench to bedside.

Pilot Programs
Collaborative Funding
CTSI Accelerator Leadership

Oversight Committee
Chair: Barry Myers

Members: Bruce Sullenger, Kim Blackwell, Dani Bolognesi, Rosa Gonzalez-Guarda, Jim McNamara, Charlie Gersbach and Ryan Shaw

Janet Bettger, PhD
Faculty Co-Director

Barry Myers, MD, PhD
Faculty Co-Director
Director of Innovation

Lynn Sutton, MS, PMP
Director Operations, CTSI

Vonda Rodriguez, PhD, PMP
Director Ops, CTSI Accelerator

CTSI Accelerator

Duke Clinical & Translational Science Institute
ctsi.duke.edu
Lesia O’Hara, Maria Iglesias de Ussel, Jillian Hurst, Anita Grissom, KK Lam, Tarun Saxena, Emily Miller, Jennifer Bond, Vonda Rodriguez, Juliana Layzer
The CTSI Accelerator team comprises 10 project leaders, an associate program leader and a senior program coordinator with over 150 years combined experience.

Combined, our team has earned:
9 PhDs; 1 PharmD, 4 PMPs; 1 MSPH; 1 MS, 1 BS

Project leaders work alongside Duke investigators to ease administrative burden, provide technical expertise, project management and strategic logistics and focus.
## Award programs managed or co-managed by CTSI Accelerator

<table>
<thead>
<tr>
<th>Award program Name</th>
<th>MEDx</th>
<th>Coulter Program</th>
<th>CTSA Translational</th>
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* Duke, Foundation, CTSA, Duke Health System; We are currently working to expand to other programs
CTSI Funding Programs

Application periods announced regularly in the CTSI newsletter, at ctsi.duke.edu and myRESEARCHhome

Collaborative Awards
1-year support up to $50k

Translational Accelerator Research Funding Agreements
1-year support up to $150k

Transformative Funding Agreements
2-year support up to $500k
<table>
<thead>
<tr>
<th>Type of Funding</th>
<th>Max Amount</th>
<th>Purpose</th>
<th>Process Oversight</th>
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<tr>
<td>Duke/UNC Collaborative Pilots</td>
<td>$50,000*</td>
<td>Develop collaborations between institutions</td>
<td>Faculty Directors: Li, Carey, Buse, Program Officer: Anita Grissom</td>
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<tr>
<td>Duke/NCSU Collaborative Pilots</td>
<td>$50,000*</td>
<td>Develop collaborations between institutions</td>
<td>Faculty Directors: Myers, Horowitz, Program Officer: Anita Grissom</td>
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<tr>
<td>Carolinas Collaborative Pilots (Duke, UNC, WF, MUSC)</td>
<td>$100,000*</td>
<td>Utilize component electronic health record data warehouses to accelerate translational research</td>
<td>Faculty Directors: Li, Carey, Buse, McClain, Brady, Program Officer: Anita Grissom</td>
</tr>
<tr>
<td>Translational</td>
<td>$150,000</td>
<td>Move projects along translational spectrum</td>
<td>Faculty Director: Myers, Program Officer: Tarun Saxena</td>
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<tr>
<td>Transformative</td>
<td>$500,000</td>
<td>High-impact, high-risk innovations</td>
<td>Faculty Director: Myers, Program Officer: Tarun Saxena</td>
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</table>

*Includes $25k/project from collaborating institutions
Where does the funding go?

100+ Duke investigators funded 40+ distinct areas

Pratt School of Engineering
- Biomedical Engineering
- Electrical & Computer Engineering

School of Nursing

Trinity College of Arts & Science
- Biology
- Chemistry

School of Medicine
- Anesthesiology
- Biochemistry
- Biology
- Biostatistics & Bioinformatics
- Cell Biology
- Dermatology

Department of Medicine
- Cardiology
- Clinical Pharmacology
- Gastroenterology
- General Internal Medicine
- Geriatrics
- Hematological Malignancies and Cellular Therapy
- Hematology
- Infectious Diseases
- Medical Genetics
- Medical Oncology
- Nephrology
- Neurology
- Pulmonary, Allergy, Critical Care
- Rheumatology and Immunology

- DGHI-Global Health Institute
- Nicholas School of Environment
- Institute for Genome Sciences & Policy
- Immunology
- Molecular Genetics & Microbiology
- Neurobiology
- Neurology
- Obstetrics & Gynecology
- Ophthalmology
- Orthopaedic Surgery
- Pathology
- Pediatrics
- Pharmacology & Cancer Biology
- Psychiatry & Behavioral Sciences
- Radiation Oncology
- Radiology
- Surgery
Funding Process

Request for Applications is Announced
- Not all have LOIs
- LOIs are encouraged
- Consultation with PL

RFA

LOI
- Translational
- Transformative

Applications Received
- Submit Applications

Application Evaluations
- Reviewers Assigned
- Evaluated using MRP
- Applications reviewed
- Funding/Move Forward recommendations made

Study Section
- Study Section consists of all reviewers and invited experts from relevant disciplines

Oral Presentation
- Invited Applicants
- List of areas to address
- Funding recommendations

Award
- Assign Project Leader
- Notify Awardees
- Finalize budgets, Aims
- Initiate paperwork
- Kick-off Meeting

Based on budget and requirements

Reviewers are experts across Duke from basic science, clinical, technology and operations

All applicants will receive valuable critique provided by the Study Section

Duke Clinical & Translational Science Institute
ctsi.duke.edu
I benefited tremendously last year from the application process. As a result, the application I submitted, although not funded by CTSI, was funded by a private foundation a little later in the year.

I would like to apply this year for the CTSI application. I noticed the format and RFA somewhat changed from last year. I could really benefit from your group’s input on two ideas that I have and how to best structure the application to enhance my likelihood of success.
How can we help?

**Bench**
- Basic and translational research

**Funding**
- Foundations, VC, Angel, CTSI, NIH

**IP**
- Market research, patents

**Risk Reduction**
- Risk assessment, strategic planning, aligning collaborations

**Execution**
- Preclinical work, contracts, GLP, GMP

**Bedside**
- Regulatory, clinical trials

DECREASING RISK

INTEGRATING TEAM SCIENCE

PROJECT LEADER

Duke Clinical & Translational Science Institute
ctsi.duke.edu
CTSI Accelerator Metrics

- $8.6 million awarded
- $162 million in follow-on funding
- 94 projects supported
- 31 Health Innovations to market

Coulter Partnership

$9.1 million awarded

$535 million in follow-on funding

44 projects supported

25 Health Innovations to market

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* Duke, Foundation, CTSA, Duke Health System; We are currently working to expand to other programs
**myRESEARCHproposal (MRP)**

- Increase efficiency
- Reduce errors
- Effort based
- User training and support

Users in 2013: 25
Users in 2018: 3400+

Projects in 2013: 4
Projects in 2018: 100+

25+ Duke departments and institutes are using myRESEARCHproposal!

**MRP** is a web-based tool used for streamlining the management of competitive grant application processes, nominations and other data collection mechanisms.

Contact us
MyResearchProposal@duke.edu

Anita Grissom
Lesia O’Hara

Duke Clinical & Translational Science Institute
ctsi.duke.edu
Who’s using MRP?

Duke Reach Equity Career Development Award
Duke-Coulter Translational Partnership
CTSI Population Health Improvement Awards

Children’s Health & Discovery Initiative At Duke University
Duke University Pratt School of Engineering
Duke University School of Nursing
Duke Institute for Health Innovation
Duke MEDx
Duke Biomedical Engineering
RTI International
UNC The North Carolina Translational & Clinical Sciences Institute
NC State University
NC Central University

Duke Clinical & Translational Science Institute ctsi.duke.edu
“The CTSI Accelerator has provided crucial contributions including the financial support toward enabling a phase I study for Pompe disease. In addition, interaction with CTSI Project Leader Jennifer Bond brought expertise in manufacturing, regulatory pathway and business development resulting in a commercial license—these interactions were invaluable to the project’s translation.”

2014: Pilot Award

Drug Therapy
✓ Favorable initial safety and efficacy data for adjunctive clenbuterol therapy in late-onset Pompe disease patients
✓ Plan for larger, Phase 2 study, pending licensing
✓ IND obtained with support of Duke ORAQ

2016: Transformative Funding

Gene Transfer for Rare Muscle Disease
✓ GMP manufacturing currently underway
✓ Phase 1 clinical trial begins Fall 2018
✓ IND obtained with support of Duke ORAQ
✓ Licensed to Actus Therapeutics
In Vivo Genome Editing with the CRISPR/Cas9 System Using Novel Muscle-Tropic AAV Vectors

• Successfully demonstrated use of AAVs to deliver CRISPR/Cas9 gene-editing tools in mice with Duchenne muscular dystrophy
• CRISPR/AAV combination successfully corrected muscles throughout the body, including the heart
• Featured online in the prestigious journal Science
• Follow-on funding received to continue the work

“The Duke/UNC award program was the ‘spark’ for this collaboration after many impromptu encounters with Aravind. The CTSI financial support, project leadership investment, and project outcomes led to an NIH R01 award as well as a large funded collaboration with Sarepta Therapeutics. The CTSI support was very instrumental to the project’s translation.”

2015: Duke/UNC Collaborative Pilot Award

In Vivo Genome Editing with the CRISPR/Cas9 System Using Novel Muscle-Tropic AAV Vectors

• Successfully demonstrated use of AAVs to deliver CRISPR/Cas9 gene-editing tools in mice with Duchenne muscular dystrophy
• CRISPR/AAV combination successfully corrected muscles throughout the body, including the heart
• Featured online in the prestigious journal Science
• Follow-on funding received to continue the work
Michael Cotten
Hypoxic-Ischemic Encephalopathy (HIE)

2009: Pilot Award
Autologous Cord Blood Cells for Brain Injury in Term Newborns
• Initiated enrollment in Phase 1 clinical trial
• Phase 1 study completed post-funding
• Multi-center Phase 2 in progress

Problem: Autologous cells are not available for ~ 2/3rds of HIE infants
Potential Solution: Duke Cellular Therapy Group has developed a mesenchymal stromal cell product (hCT-MSC) derived from umbilical cord tissue

2018: Pilot Award
hCT-MSC’s for Infants with Hypoxic-Ischemic Encephalopathy
This new award will fund
• hCT-MSC manufacturing
• Open label Phase 1 trial

“The collaborative study team, involving the neonatologists, cell therapy experts, CTSI project leadership and our neurodevelopmental follow on colleagues are excited to have this opportunity to continue our work. The CTSI Accelerator contributions integrated with the collaborative team is a unique combination of strengths here at Duke.”
Contact Us!

Office Hours, 3:00 – 5:00 p.m.
Third Thursdays, 251 MSRB1

cptsiconsultstudio@duke.edu

ctsi.duke.edu
Thank you for joining!

Please join us in the fall for our next CTSI Grand Rounds!