Welcome
Dr. Hashem El-Serag &
Dr. Christie Ballantyne
3:30 pm  Overview by Dr. Ballantyne & Welcome by Dr. El-Serag
3:35 pm  College Vision, Dr. Adam Kuspa
3:45 pm  CTSA submission, Dr. Chris Amos
3:55 pm  Submitting Successful Multi-Investigator R01s, Dr. Melissa Bondy
4:00 pm  Submitting Successful U01s, Dr. Matthew Ellis
4:05 pm  Precision Medicine, Dr. Richard Gibbs
4:15 pm  Data Warehouse, Mr. Lee Leiber
4:25 pm  Advanced Technology Cores, Dr. Dean Edwards
4:35 pm  Breakout Sessions, Dr. Laura Petersen
4:37 pm  Break
4:45 pm  Breakout Session 1
5:50 pm  Breakout Session 2
6:50 pm  Break
7:10 pm  Wrap Up
College Vision
Dr. Adam Kuspa
BCM’s Ongoing Strategic Planning for Precision Population Health

Health Information Exchange/Bioinformatics: Data Security and Quality

Population Health

Enterprise Data Warehouse

Analytics

Ethics/Policy: Consent, Governance, Transparency, Engagement, Access, Aligned Incentives

Science/Discovery

Precision Medicine

Translational Medicine

Population Health Science/Discovery

Precision Medicine

Translational Medicine

Population Health

Science/Discovery

Precision Medicine
# Proposed New Graduate Programs

All Program names are provisional; To be determined by Program Faculty

<table>
<thead>
<tr>
<th>Program Name</th>
<th>Focus Areas</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quantitative &amp; Computational Biology</td>
<td>Data science; Bioinformatics; Computational biology; Biomedical informatics, text mining</td>
</tr>
<tr>
<td>Genetics &amp; Genome Sciences</td>
<td>Genetic basis of human disease; Genomics; Bioinformatics; Epigenetics; Genetic model systems</td>
</tr>
<tr>
<td>Cancer &amp; Cell Biology</td>
<td>Cancer biology, oncogenes, tumor biology; Cell biology, cell cycle; cell signaling</td>
</tr>
<tr>
<td>Neuroscience</td>
<td>Neuroscience, (molecular &amp; computational); Neural development &amp; disease; Technologies &amp; models</td>
</tr>
<tr>
<td>Immunology &amp; Microbiology</td>
<td>Microbiology; Microbiome; Immunotherapy; Autoimmunity; Antibiotic resistance; Vaccines</td>
</tr>
<tr>
<td>Chemical &amp; Structural Biology</td>
<td>Pharmacology; Biochemistry; Drug delivery &amp; discovery; Medicinal chemistry; Molecular probes</td>
</tr>
<tr>
<td>Development, Disease Models and Therapeutics</td>
<td>Physiology; Disease: mechanisms, prevention &amp; treatment; Cell &amp; gene therapy; Tissue regeneration; Stem cell biology; Muscle, Cardiovascular, Reproductive Biology</td>
</tr>
</tbody>
</table>
Proposed Program Organization

- **Program Director (Faculty)**
  - Co-Directors
  - Program Administrator(s)

- **Dean of GSBS**

- **Provost of BCM**

- **Graduate Education Committee (Faculty)**

- **Graduate Faculty**
  - Graduate Students
  - 15-18 per year, 80-100 total
  - Welcome Events
  - Student Seminar series
  - Journal Clubs, Workshops
  - Teambuilding
  - Theme Retreat

- **Departments and Centers**
  - Research Support
  - Department/Center Seminars
  - Retreats/Symp./Research Day

- **Internal Education Advisory Committee (Chairs and Center Directors)**

- **Baylor College of Medicine/GSBS**
  - Library Services
  - Student Support
  - Career Center
  - Professional Skills
  - Mentor Training

- **Core Labs & Vivaria**
  - Research Support
  - Advanced Technology
Institute for Clinical and Translational Research (ICTR)

Needs
- Ability to perform first in man and early phase trials
- Recruitment of biomedical informatics faculty
- Adequate Biostatistics, Epidemiology and Research Design team
- Improved access to patients and clinical records, including clinical affiliates
- Communication between the informatics environment at Baylor Clinic, BSLMC, Harris Health, and TMC

Opportunities
- Develop analytics and data mining capabilities related to EMR (DEEP6?)
- Assist in data integration between Baylor Clinic, BSLMC and Harris Health
- Collaborate with OOR/OCR and Research IT to implement TMC wide clinical trials pilot
- Participate in development of a precision medicine laboratory with the DLDCC to support the development of approaches for evaluating specific features of an individual’s disease by evaluating drug response, gene edited, cellular based studies
Driving Strategy Within the Research Mission – Precision Medicine

**Information flow**

1. **Genes**
   - Genomic profiling
   - Single cell genomics
   - Gene vector

2. **Transcripts**
   - Transcriptionomics
   - RNA in situ hybridization

3. **Proteins**
   - Discovery proteomics
   - Antibody proteomics
   - Drug discovery
   - Protein production

4. **Metabolites**
   - Metabolomics
   - Drug metabolism
   - Metabolism
   - Cytometry & cell sorting
   - Cell-based screening
   - In vivo modeling

**Core Labs**
- Genomic profiling
- Single cell genomics
- Gene vector

**Academic Units**
- Genetics Department
- Therapeutic Innovation Center (THINC)
- Cell Biology Department (MCB)
- Precision Oncology
- Center for Drug Discovery (CDD)
- Center for Molecular Discovery (ACMD)
- Cell & Gene Therapy (CAGT)

**Companies**
- Baylor Genetics
- Diversigen
- Coactigon
- TBD
- Forma Therapeutics
- FGH BioTech
- Cell Medica
CTSA Submission
Dr. Chris Amos
Daniel L. Duncan Institute for Clinical and Translational Research and the CTSA Program

Christopher I. Amos, Ph.D.
Ashok Balasubramanyam, M.D.
Clinical and Translational Science Awards Program

- CTSA Program provides critical resources and support needed to strengthen the entire spectrum of our nation’s clinical and translational research enterprise

- CTSA Program biomedical research institutions — called “hubs” — provide core resources, essential mentoring and training, and opportunities to develop innovative approaches and technologies designed to re-engineer existing capabilities.

- Program support and collaborative initiatives harmonize efforts, foster collaboration and strengthen this network to improve the quality, safety, efficiency and speed of clinical and translational research nationally.

- BCM has applied several times previously unsuccessfully – major barriers were i) lack of accessible electronic medical records to support clinical research, ii) bad timing related to clinical partners, iii) insufficient depth of biomedical informatics research and faculty engagement, iv) ties with Human Genome Center and TCH are strengths
Organizational Framework for CTSA Programs

- Domain Task Forces aligned with strategic goals
- NIH, FDA and community members
- Organizationally guided by the Steering Committee
- Working Groups already launched
NCATS Initiatives

Innovation for Multi-site Studies: Participant Recruitment
RFA TR 15-004 – CTSA Network Recruitment Innovation Centers (RICs)

The problem: Slow recruitment delays most NIH-funded trials

The approach: Build national recruitment capacity using data from the Electronic Health Record (EHR) to find potential trial participants who meet entry criteria

NIH Partner: NLM

Study planning phase
- Data-driven site selection
- Feasibility analysis

Study implementation phase
- Privacy and IRB compliant recruitment plan
- Funded expert staff to help with implementation
NCATS Initiatives

RFA-TR-15-002 - CTSA Network Trial Innovation Centers (TICs)

**The problem:** Study start-up takes too long

**The approach:** Streamline, standardize and centralize processes

**NIH Partner:** NIA
Major CTSA Program Initiatives

- Trial Innovation Network – Focus on initiating clinical trials more rapidly, cost efficiently and with more innovation includes RICs, TICs and CTSA Program Hubs
- CTSA Program Collaborative Awards (CCIAs) supporting collaboration among two or more CTSA Program Hubs
- Common metric program to improve impact of the CTSA program

Maximum Award for BCM is $4.1 M, but can be increased with partnership with other institutions (MDACC = $2.4 M, UT Houston = $1.7 M, UH = $0.4M, Methodist = $0.3 M, Rice = $0.2M, TSU = $0.03M, THI =0.02M) Maximum allowable award is $7M. Must also be matched by >50% institutional support
## Elements of CTSA Application

<table>
<thead>
<tr>
<th>Component Types Available in ASSIST</th>
<th>Page Limits</th>
</tr>
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<tbody>
<tr>
<td>Overall</td>
<td>12 pages</td>
</tr>
<tr>
<td>Admin Core (use for A. Administrative Core)</td>
<td>12 pages</td>
</tr>
<tr>
<td>CTR Core (use for B. Informatics)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for C. Community and Collaboration)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for D. Translational Endeavors)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for E. Research Methods)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for F. Hub Research Capacity)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for G. Network Capacity)</td>
<td>6 pages</td>
</tr>
<tr>
<td>CTR Core (use for H. Optional Functions)</td>
<td>6 pages</td>
</tr>
<tr>
<td>Inst Career Dev (use for I. Institutional Career Development Core)</td>
<td>25 pages</td>
</tr>
<tr>
<td>NRSA Training (use for J. NRSA Training Core)</td>
<td>25 pages</td>
</tr>
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</table>
Goals of the ICTR

- Catalyze interdisciplinary, collaborative clinical and translational research
- Create a robust, readily accessible infrastructure to support multidisciplinary, collaborative clinical and translational research
- Attract, educate and train individuals in clinical, translational, and collaborative research
- Improve human health and healthcare practice
- Advance BCM’s Population Health – Precision Medicine Initiatives
Submitting Successful Multi-Investigator R01s
Dr. Melissa Bondy
Multi-Investigator R01s

- Grants are investigator initiated
- Usually 2-3 PIs (one must be a contact PI)
- Budgets are usually over $500K
- Must identify the NIH Institute and a Program Officer to help shepherd the proposal through the ARA approval process (Program needs at least 8 weeks - Plan ahead)
- Approval requires the Aims page, Budget and Justification, Abstract, Data Sharing Plan, and cover letter
- Official Institutional approval is not needed
- Once approved submit by the grant deadline
- Good Luck
Submitting Successful U01 Grants
Dr. Matthew Ellis
Submitting Successful UO1s

U01 Research Project Cooperative Agreement

- Supports discrete, specified, circumscribed projects to be performed by investigator(s) in an area representing their specific interests and competencies
- Used when substantial programmatic involvement is anticipated between the awarding Institute and Center
- One of many types of cooperative agreements
- No specific dollar limit unless specified in FOA

Funding Opportunity Title

Proteogenomic Translational Research Centers for Clinical Proteomic Tumor Analysis Consortium (U01)
Precision Medicine
Dr. Richard Gibbs
Getting Genomics in the Adult Clinic:

Richard Gibbs AC Ph.D.,
Baylor College of Medicine - Human Genome Sequencing Center
Faculty Fellow (2016-17)
Texas A&M University Institute of Advanced Studies

- BCM is a joint owner of Baylor-Miraca Genetics Laboratories (BMGL)
- BCM owns stock in Codified Genomics
Clinical Genetics Catch 22:

1: Need large numbers of well characterized research participants for discovery,

2: The best source is the clinic,

3: Need discovery to drive clinical relevance:
High frequency
Few Actionable Alleles
Missing ‘Heritability’

Mendel
 ‘Oligogenic’?

Low frequency/High impact
Not related to sporadic cases
in a simple way

The ‘missing heritability’?

CHILDREN
SIMPLE

ADULTS
HARD

High frequency
CD/CV (GWAS)

2011 1st quarter
TECHNOLOGY 2000 -> 2018

Human Genome Project

Reference sequence

WGS

James D. Watson

Individual variation

WGS

Desmond Tutu
(Khoisan & Bantu genomes)

Variation in populations

WGS

James R. Lupski
Genome Medicine (2013) 5:57

Locus/Alleles for known genetic disease

WGS

Beery twins
Sci Transl Med (2011) 3(87);87re3.

Locus/alleles to inform and guide medical treatment

WGS

WES: ASXL3/AHDC1
AJHG,

de novo AD Discovery

WES

WES: Diagnostic laboratory

Clinical service

WES

100,000’s of Research WGS

Discovery in Complex Disease

WGS/WES
Cumulative CMG Disease Gene Discovery

~200 novel discoveries per year
1 novel discovery per 30 WES
~60,000 BCM EXOMES

RESEARCH
(34,000)

HGSC
HUMAN GENOME SEQUENCING CENTER
CLINICAL LABORATORY

DISCOVERY
~75% ‘Undiagnosed’

DIAGNOSIS
(12,000)

BCM-JOHNS HOPKINS CENTER FOR MENDELIAN GENOMICS
(7,100)

James Lupski
David Valle

Christine Eng

Yaping Yang

WGL Sample Volume through Jan 2016
Discovery Impact on Molec Dx, ClinVar and GTR Entries

Unsolved Clinical Cases feed discovery

Discovery Feeds Clinical Diagnoses
WES and diagnosis of children demonstrated that merging of research and clinic is the recipe for accrual of large numbers of participants and rapid progress!

THE ‘MARS’ GENETICS MISSION:

Establishing routine genomics in the adult clinic in order to boost the number of available, well phenotyped genomes for research studies.
2018 – Round Two of Cardiac Disease Panel

- Interpreting/Reporting improved
- Cost 60% of 2014
- Turn-around-time 50%

158 Genes: 20 ‘sub categories’ with 3 – 45 genes in each (overlapping)

CASE 1: 20 y.o. female, ER after seizure-like episode - actually arrhythmias; mutation in KCNQ1; Long QT Syndrome, Type 1

CASE 2: Anueyrm case: Mutation in TGFBR1 (Loeys-Dietz syndrome: LDS) NOT Marfans i.e different management

CASE 3: 20 y.o. student ‘abnormal lipids blood work’ at school health fair: severe LDLR mutation – radical change for patient and family.
Clinical ⇔ Research Enterprises

- Tremendous success in pediatrics, where clinical application feeds discovery
- Extended to adults in CVD Pilot
- Immediate utility
- Bringing Adults into the Genomics Clinic
Data Warehouse
Mr. Lee Leiber
Learning Health System Vision

Health Information Exchange/Bioinformatics: Data Security and Quality

Data Web

Participant(s)

Population Health

Translational Medicine

Science/Discovery

Analytics

Ethics/Policy:
Consent, Governance, Transparency, Engagement, Access, Aligned Incentives

Precision Medicine
What makes up the “Data Web”? 

- BCM Enterprise Data Warehouse (EDW)
- Greater Houston HealthConnect
- Deep6 AI (TMC)
- Cosmos
- Other Affiliates

Options for Data Depend Heavily on Use Case and Scope!
Other Affiliates

- Currently working to map out processes specific to affiliate to access data (if outside GHH dataset)
- Data likely will *NOT* reside in BCM data warehouse
Next Steps

- Continue identification of interesting data sets and plan ingestion into BCM EDW
- Continue field mapping for Cosmos and Happy Together
- Implement GHH Integration and MPI (Master Patient Index)
- Develop strategy for Research Data Center for Compute, HPC, and Storage (with associated governance and charge models)
- Engage Research community for needs assessment around other “Big Data” needs (e.g., Hadoop infrastructure, etc.)
- Need to develop resource plan to support technology, methodologies, governance, etc.
Advanced Technology Cores (ATC)

Executive Director
Dean P. Edwards, Ph.D.
Professor Molecular & Cellular Biology

https://www.bcm.edu/research/advanced-technology-core-labs/lab-listing
Organizational Structure Institutional Advanced Technology Cores (ATC)

SR VP & Dean of Research
Adam J. Kuspa, Ph.D.

Executive Director
Dean P. Edwards, Ph.D.

Administrator
Jennifer McCullough
(Admin team)

Core Directors Operations Committee

Faculty Oversight Committee

Scientific Advisory Committees

Advanced Technology Cores (26)
Faculty Directors/Co-Directors & Research Staff (125)
## Operating Budget FY18

<table>
<thead>
<tr>
<th>Revenue source</th>
<th>Amount ($)</th>
<th>% of Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Grants (P30 Centers/CPRIT/NIH Consortium)</td>
<td>$3,634,257</td>
<td>27%</td>
</tr>
<tr>
<td>Institutional (ATC/DLDCCC-Duncan gift)</td>
<td>$4,489,663</td>
<td>33%</td>
</tr>
<tr>
<td>Chargebacks (user grants)</td>
<td>$5,570,799</td>
<td>40%</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>$13.7M</strong></td>
<td><strong>100%</strong></td>
</tr>
</tbody>
</table>

26 ATC Cores: subset of 12 are DLDCCC Shared Resources
## Major Equipment

### New equipment purchases last 3 years ($7.21M)

<table>
<thead>
<tr>
<th>Year</th>
<th># of items</th>
<th>College</th>
<th>Grants (NIH S10, CPRIT, FDN)</th>
</tr>
</thead>
<tbody>
<tr>
<td>FY16</td>
<td>19</td>
<td>$1.3M</td>
<td>$0.82M</td>
</tr>
<tr>
<td>FY17</td>
<td>20</td>
<td>$0.82M</td>
<td>$1.57M</td>
</tr>
<tr>
<td>FY18</td>
<td>13</td>
<td>$1.5M</td>
<td>$1.2M</td>
</tr>
<tr>
<td>Totals</td>
<td></td>
<td>$3.62M</td>
<td>$3.59M</td>
</tr>
</tbody>
</table>

### Annual cost to maintain equipment (FY18)

<table>
<thead>
<tr>
<th>Service contracts</th>
<th>Depreciation</th>
<th>Leases</th>
<th>Total costs</th>
<th>% total operating budget</th>
</tr>
</thead>
<tbody>
<tr>
<td>$1,343,941</td>
<td>$553,000</td>
<td>$780,919</td>
<td>$2,667,860</td>
<td>19.4%</td>
</tr>
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</table>
What we do:
[Support research and grants for a broad range of faculty]

Metrics CY17

<table>
<thead>
<tr>
<th>Total # cores</th>
<th>Publications supported by cores</th>
<th>BCM faculty usage</th>
<th>% BCM usage</th>
</tr>
</thead>
<tbody>
<tr>
<td>26</td>
<td>465</td>
<td>1,100</td>
<td>85%</td>
</tr>
</tbody>
</table>

User grants awarded with data generated by cores and/or specific aims requiring core services.

<table>
<thead>
<tr>
<th>Total new grants</th>
<th>Agencies</th>
<th>Annual directs</th>
</tr>
</thead>
<tbody>
<tr>
<td>139</td>
<td>NIH, NCI, DOD, ACS, CPRIT &amp; FDNs</td>
<td>$40M</td>
</tr>
</tbody>
</table>
Upcoming plans and opportunities

• Plans for new ATC cores (next FY):
  - Zebrafish (genomic editing models)
  - Glycomics (global changes in carbohydrate moieties)
  - Epigenetics (global histone PTMs, ChIP-seq, DNA methylation, chromatin structure)

• Grant submissions for direct support of core operations and equipment
    (6 grants for $4.26M total: metabolic cage system, 9.4T MRI, mass spectrometer for proteomics, imaging mass spectrometer, super-resolution microscope).
  - CPRIT Core Facility Award $6M for 5 years (due Jan 30, 2019)
    One/Institution. Internal LOI and competition.
  - P30 NCI Cancer Center Support Grant: Competing Renewal (9/2019)
    Currently supports 12 Cores for ~$1M/year

• Any P30 Center grant, PPG or consortium grant can include an ATC Core
Questions?

Thank you.

https://www.bcm.edu/research/advanced-technology-core-labs/lab-listing
Breakout Sessions
Dr. Laura Petersen
## Breakouts

<table>
<thead>
<tr>
<th>Time</th>
<th>Room A</th>
<th>Room B</th>
</tr>
</thead>
<tbody>
<tr>
<td>4:45 pm</td>
<td><strong>Session 1</strong> Precision Medicine</td>
<td><strong>Session 2</strong> Population Health &amp; Health Disparities</td>
</tr>
<tr>
<td></td>
<td>Dr. Melissa Bondy &amp; Dr. Matthew Ellis</td>
<td>Dr. Laura Petersen &amp; Dr. Wolfgang Winkelmayer</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Dr. Chris Amos &amp; Dr. Hashem El-Serag</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Dr. Christie Ballantyne &amp; Dr. Dennis Villareal</td>
</tr>
<tr>
<td>5:50 pm</td>
<td><strong>Cardio Metabolic/ Inflammation</strong></td>
<td></td>
</tr>
<tr>
<td></td>
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<td></td>
</tr>
</tbody>
</table>
Breakout Session
Questions

1. What are the existing strengths?
2. What are the collaborative opportunities for BCM & TMC in this area?
3. What are the relevant funding opportunities we want to pursue?
Precision Medicine - Opportunities to link biospecimens to clinical phenotypes

- Inventories of Biospecimens “virtual repository”
  - Prospective targeted collections
  - Versus front door consent?
  - common specimen management system
- Statistical Genetics and Informatics – opportunities for expansion
- Laboratory support for biomarker development
CTSA

- Optimize community engagement – identify clinical focus areas, establish work groups and learn from existing areas of excellence ex. Cancer center
- BCM data warehouse – test ability to obtain data and utilize resources for targeted project areas
- Identify existing informatics staff/resources at BCM, with option to consolidate then develop informatics working groups
Population Health & Health Disparities

- Population Health Strengths / Theme
  - Obesity and related diseases
  - Cross-disciplinary collaborations

- Population Health Strengths / Theme
  - Exploring Conditions Related to Disparities

- Collaborations
  - Capitalize on Houston Diversity!
Cardio Metabolic/Inflammation

- Transgenerational Study for patients with cardiometabolic risk, will enable intervention in younger generations
- Availability of several cohorts: ARIC, LookAhead, Sprint, CADRE, maternal fetal, repositories, registries, databases
- Research across lifespan
Thank you!

Output from this meeting

• Summary report
• Creation of focus groups