The National Center for Advancing Translational Sciences: Catalyzing Translational Innovation in Rare Disease Research

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National Organization for Rare Disorders Special Member Webinar
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The Best of Times, the Worst of Times

Fundamental science unprecedentedly advanced, but:

- Poor transition of basic or clinical observations into interventions that tangibly improve human health
- Intervention development failure-prone and expensive
- Poor adoption of demonstrably useful interventions

Enormous opportunity/need to deliver on promise of science for patients
Human Conditions with Known Molecular Basis

Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

~500 with therapy
Moore’s Law

The graph illustrates the exponential growth in the number of transistors on silicon chips. The y-axis represents the transistor count, while the x-axis shows the date of introduction. The trend line indicates a doubling of transistor count every two years. The curve shows the historical data from 1971 to 2011, with data points for various transistor counts and introduction dates.

Eroom’s Law

The number of new drugs approved by the FDA per billion US dollars (inflation-adjusted) spent on research and development (R&D) has halved roughly every 9 years since 1950.

To catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.
What is Translation?

*Translation* is the process of turning observations in the laboratory, clinic, and community into interventions that improve the health of individuals and the public—from diagnostics and therapeutics to medical procedures and behavioral changes.
What is Translational Science?

Translational Science is the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process.

NCATS studies translation as a scientific and organizational problem.
Some of the **scientific translational problems on NCATS’ to-do list**

- Predictive toxicology
- Predictive efficacy
- Derisking undruggable targets/untreatable diseases
- Data interoperability
- Biomarker qualification process
- Clinical trial networks
- Patient recruitment
- Electronic Health Records for research
- Harmonized IRBs
- Clinical diagnostic criteria
- Clinical outcome criteria (e.g., PROs)
- Adaptive clinical trial designs
- Shortening time of intervention adoption
- Methods to better measure impact on health (or lack thereof)
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Some of the organizational/cultural translational problems on NCATS’ to-do list…

- Data transparency/release
- IP management
- Integration of project management
- Incentives/credit for team science
- Incentives/credit for health improvements
- Education/Training (scientific and cultural)
- Collaborative structures
  - Public-private partnership models
The Scope of Rare Diseases

• ~7000 diseases
  » ~80% mendelian genetic
  » ~50% onset in childhood
  » ~250 new rare diseases identified each year
• Population prevalence ~8% (US ~25M; EU ~30M, World 350M)
• Definition of “rare disease” varies by country
  » Absolute prevalence: USA<200,000; Japan<50,000; S Korea <20,000…
  » Percentage prevalence: EU<5 in 10,000; Australia<1 in 2000…
• <5% of rare diseases have a regulatorily approved treatment
  • USA ~300 diseases
  • At current rate 3-5 newly treatable diseases/yr… >1000 yrs to all
**NCATS Office of Rare Diseases Research**

- **ORDR Mission:** Accelerate the translation of rare disease science to benefit patients

- **Major Programs and Initiatives:**
  - Rare Diseases Clinical Research Network (RDCRN) Program
  - Genetics And Rare Diseases (GARD) Information Center
  - Global Rare Diseases Patient Registry Data Repository (GRDR)
  - NCATS Scientific Conferences Program
  - NCATS Toolkit Project
  - Bench to Bedside Awards
Office of Rare Diseases Research

About GARD

The Genetic and Rare Diseases Information Center (GARD) is a program of the National Center for Advancing Translational Sciences (NCATS) and is funded by two parts of the National Institutes of Health (NIH): NCATS and the National Human Genome Research Institute (NHGRI). GARD provides the public with access to current, reliable, and easy-to-understand information about rare or genetic diseases in English or Spanish.

Find Out How GARD Information Specialists Can Help

Patients, Families and Friends

Healthcare Professionals

Researchers

https://www.rarediseasesnetwork.org/
Rare Disease Patient Toolkit Project

- Provide centralized web portal to online tools and resources that patient groups can readily access to accelerate their work
- Focus on tools/resources across the drug development process
- “How-to” perspective, e.g. “How To Establish and Utilize a Patient Registry”
Save the Date of Sept. 8, 2017!

Join us for the NCATS Toolkit for Patient-Focused Therapy Development: Demonstration and Dissemination Meeting

On Sept. 8, 2017, NCATS will launch a new, centralized online resource portal that will enable patient groups to make progress along the entire translational science spectrum, no matter where they might be in that process. The NCATS Toolkit for Patient-Focused Therapy Development: Demonstration and Dissemination Meeting will take place from 9 a.m. to 4 p.m. ET on the NIH campus in Bethesda, Maryland. The event will enable the rare diseases and other patient communities to learn more about the toolkit, including how it can streamline their therapeutic development activities. Participants also will have the opportunity to provide input into how the toolkit can be refined, expanded and made even more useful.

Developed in collaboration with patients and rare disease advocates, the toolkit is a centralized online portal for resources and tools that will cover the broad therapy development landscape, including:

- How to establish a patient registry;
- How to drive patient-focused discovery and pre-clinical research and development;
- How to work with NIH and the Food and Drug Administration; and
- How to conduct post-market surveillance.

Register now and learn more >
https://events-support.com/events/NCATS_Toolkit_Meeting
NCATS Division of Preclinical Innovation
A Collaborative Engine

Project Entry Point

Target Validation ➔ Assay Dev ➔ Probe/Lead Development ➔ Lead Optimization ➔ Preclinical Development

Target ➔ RNAi ➔ NCGC ➔ Therapeutics for Rare/Neglected Dis (TRND)

DPI Program

Assay, Chemistry Technologies ➔ BrIDGs

Tox21 (Systems Toxicology)

Repurposing ➔ Repurposing

Paradigm/Technology Development

Deliverables

- Genome-wide RNAi systems biology data
- Chemical genomics data
- Stem cell tools/data
- Leads for therapeutic development
- Approved drugs effective for new indications
- New drugs for untreatable diseases
- Small molecule and siRNA research probes
- Predictive in vitro toxicology profiles
- Drugs suitable for adoption for further development
- Novel clinical trial designs

More efficient/faster/cheaper translation and therapeutic development
First-in-class GALK Inhibitors for Classic Galactosemia

1. Galactosemias: Rare autosomal recessive disorders in which the body cannot properly metabolize galactose

   - GALT
     - Type II galactosemia (GALK deficient)
     - Mild phenotype
     - Classic galactosemia
     - Severe phenotype
     - Type III galactosemia
     - Severe phenotype

   α-D-galactose

   Deficiencies cause

   - Classic Galactosemia - most common & severe of the galactosemias (~1 in 30,000-60,000 births)
   - Results from GALT deficiency
   - Lethal without dietary galactose restriction
   - Leads to mental deficits, ovarian dysfunction
   - No current therapy

2. GALK as a drug target

   α-D-galactose

   Type II galactosemia
     - GALK
     - Mild phenotype

   Classic galactosemia
     - GALT
     - Severe phenotype

   Hypothesis: GALK inhibition will phenocopy Type II Galactosemia in Classic Galactosemics, leading to milder, more easily manageable disease

3. GALK high-throughput inhibitor screen

   - Screened 350,000+ compounds for human GALK inhibition
   - Performed med chem on top active scaffolds
   - Further refinement to improve ADME/PK

   Hit
     - GALK IC₅₀: 7.6 uM
     - Solubility: <1 ug/mL

   Lead
     - GALK IC₅₀: 330 nM
     - Solubility: 64 ug/mL

   ADME:
     - Kin. Sol: 64 ug/mL
     - RLMS t₁/₂: >30 min
     - MLMS: 93% rem @ 15 min

   In vivo PK:
     - 47 mg/kg, IP
     - t₁/₂: 1.73 hr
     - Cmax: 226 uM
     - AUCinf 28,358 h* ng/mL
Compounds very effectively lower gal-1-p levels in Classic Galactosemia primary patient fibroblasts with no galactose challenge (clinically relevant).

**Primary Patient Cell Data**

- **SPR demonstrating high affinity GALK binding of lead**
- **Human GALK co-crystal w/ lead**
- **CETSA demonstrating on-target binding of GALK in cells**

**Lead characterization & cellular activity**

**Patient cell activity and upcoming in vivo models**

- **WT vs mutant mouse ovary histopathology**

Ratio of non-galactosylated IgG (G0) to monogalactosylated IgG (G1) in wild type (red boxes) vs GalT-gene trapped (GalT-“knockout”) (GK, blue boxes) mice.
In collaborative relationships with disease foundations enable drug discovery strategies for early-stage (gateway) translation

A. Develop assays to phenocopy molecular hallmarks of pathology leveraging disease knowledge and advances in molecular biology
B. Analysis and progression strategies for evaluation of approved drugs, investigational agents, large diversity libraries and complex chemical libraries (e.g., NPEs)

Training, grant support and outreach to strengthen competencies in translational research in new and established investigators

- Foundation-sponsored Post-doctoral training opportunities
Assay development strategies for PBD-ZSD

Pathophysiology

Increased VLCFA levels and decreased plasmalogen levels in blood & tissues

Genetic & molecular basis

Mutations in people with ZSD

- PEX1: 59.3%
- PEX9: 10%
- PEX10: 5.5%
- PEX12: 5.7%
- PEX13: 3.2%
- PEX19: 2.2%
- PEX21: 0.7%
- Other: 12.6%

Common mutations:
- PEX1 p.G843D
- PEX1 p.770fs

In cytoplasm of immortalized fibroblasts from patient

- In peroxisomes of the same patient fibroblasts rescued by small molecule treatments

Diosmetin Control

IN Cell Analyzer HCA System Software

Inglese (NCATS), Hacia, Braverman
Drug Repurposing

Target → Screen → Hit → Lead → Lead Optimization → Preclinical Development → Clinical Trials → FDA approval

1-2 years?

>500,000 compounds, 15 yrs

3000 drugs
NCATS Comprehensive Repurposing Program
“Systematizing Serendipity”

The NCGC Pharmaceutical Collection: A Comprehensive Resource of Clinically Approved Drugs Enabling Repurposing and Chemical Genomics

Ruili Huang,* Noel Southall,* Yuhong Wang, Adam Yasgar, Paul Shinn, Ajit Jadhav, Dac-Trung Nguyen, Christopher P. Austin†

Small-molecule compounds approved for use as drugs may be “repurposed” for new indications and studied to determine the mechanisms of their beneficial and adverse effects. A comprehensive collection of all small-molecule drugs approved for human use would be invaluable for systematic repurposing across human diseases, particularly for rare and neglected diseases, for which the cost and time required for development of a new chemical entity are often prohibitive. Previous efforts to build such a comprehensive collection have been limited by the complexities, redundancies, and semantic inconsistencies of drug naming within and among regulatory agencies worldwide; a lack of clear conceptualization of what constitutes a drug; and a lack of access to physical samples. We report here the creation of a definitive, complete, and nonredundant list of all approved molecular entities as a freely available electronic resource and a physical collection of small molecules amenable to high-throughput screening.
Identification of Drug Modulators Targeting Gene-Dosage Disease CMT1A

Sung-Wook Jang, Camila Lopez-Anido, Ryan MacArthur, John Swaren, and James Inglese

Purpose: The structural integrity of myelin formed by Schwann cells in the peripheral nervous system (PNS) is required for proper nerve conduction and is dependent on adequate expression of myelin genes including peripheral myelin protein 22 (PMP22). Consequently, excess PMP22 resulting from its genetic duplication and overexpression has been directly associated with peripheral neuropathy called Charcot-Marie-Tooth disease type 1A (CMT1A), the most prevalent type of CMT. Here, in an attempt to identify transcriptional inhibitors with therapeutic value toward CMT1A, we developed a cross-validating panel of orthogonal reporter assays, firstly luciferase (Fluc) and β-galactosidase (βLac), capable of recapitulating PMP22 expression, utilizing the intrinsic regulatory element of the human PMP22 gene. Each compound from a collection of approximately 3,000 approved drugs was tested at multiple titration points to achieve a pharmacological end point in a 1536-well plate quantitative high-throughput screen (qHTS) format. In conjunction with an independent counter-screen for cytotoxicity, the design of our orthogonal screen platform effectively contributed to the selection and prioritization of active compounds, among which three drugs (tenofovir, obinutuzumab, and boronulin) exhibited marked reduction of endogenous Pmp22 mRNA and protein. Overall, the findings of this study provide a strategic approach to assay development for gene-dosage diseases such as CMT1A.

INDUCION AND REVERSAL OF MYOTONIC DYSTROPHY TYPE 1 PRE-MRNA SPLICING DEFECTS BY SMALL MOLECULES

Jessica L. Childs-Disney, Ewa Stepniak-Konieczna, Tuan Tran, Ilyas Yildirim, Haileung Park, Catherine Z. Chen, Jason Hopkins, Noel Southall, Juan J. Marugan, Samir Patel, Wei Zheng, Chris P. Austin, George C. Schatz, Krzysztof Sobczak, Charles A. Thornton, and Matthew D. Disney

Purpose: To identify small-molecule drugs that can induce the expression of the myotonic dystrophy type 1 (DM1) protein dystrophia myotonica protein kinase (DMPK) by reversing its aberrant mRNA splicing defects.

Identification of repurposed small molecule drugs for chordoma therapy

Menghang Xia, Ruili Huang, Srialla Sakamuru, David Alcorta, Ming-Huang Cho, Dae-Hee Lee, Deric M Park, Michael J Kelley, Josh Sommer, and Christopher P Austin

Purpose: To identify small-molecule drugs that can repurpose for chordoma therapy.

Keywords: chordoma, NCIC Pharmaceutical Collection, cell viability, caspase 3/7, U-CH1, U-CH2, qHTS
NCATS Stem Cell Translation Laboratory

Overcoming systemic barriers to clinical application of iPSCs

• Part of NIH Common Fund Regenerative Medicine Program
• Goal: Bring iPS cells closer to clinical applications in drug discovery and regenerative medicine by developing characterization standards, improved iPSC differentiation protocols
• Cutting-edge technologies (e.g. qHTS, single cell proteomics, next-gen sequencing) and multidisciplinary team approach (e.g. biologists, chemists, engineers, bioinformaticians)
• SCTL is seeking new collaborations to help achieve common goals in iPS cell biology in a faster and more coordinated fashion (e.g. comprehensive cell characterization, functional maturation)

Ilyas Singeç
NCATS Therapeutics Development Programs

Therapeutics for Rare and Neglected Diseases (TRND)
Bridging Interventional Development Gaps (BrIDGs)

Model: Collaboration between NCATS labs with preclinical drug development expertise and external organizations with disease area/target expertise

Projects:
Entry from Probe to IND-enabling
Exit by adoption by external organization for completion of clinical development
Serve to develop new generally applicable platform technologies and paradigms

Eligible Collaborators:
Academic, Non-Profit, Government Lab, Biotech, Pharma
Ex-U.S. applicants accepted
NCATS TRND Project

Aromatic L-Amino Acid Decarboxylase Deficiency

- Collaborator: Agilis Biotherapeutics
- Gene Therapy: single dose AAV-hAADC injection into putamen
- AADC: Profound Developmental Failure
  - Extremely limited muscle strength, control and movement
  - Seizure-like symptoms (oculogyric crises)
  - Lifelong care and frequent hospitalizations
  - Severe forms have catastrophic course (average life expectancy of 4-8 yrs)

- Challenges to develop AAV-AADC
  - Ultra-rare disease (underdiagnosed) - small market
  - Stereotactic surgery in infant brains
  - Regulatory: phase 1 and phase 2 human data outside of U.S.

- TRND collaboration catalyzing development of AAV-AADC
  - 18 AADC patients received GT with some remarkable clinical responses
  - Project initiation, May, 2016
  - GMP grade AAV-AADC manufacturing production
  - GLP bio-distribution and toxicology testing in rodents
  - Patient finding / epidemiology study
  - FDA EOP2 meeting July 2017
Development of LUM-001 as a Treatment for Creatine Transporter Deficiency (CTD)

- Collaborator: Lumos Pharma
- Disease: X-linked cerebral creatine deficiency caused by mutations in the creatine transporter encoded by the SLC6A8 gene
  - Reduced creatine levels in brain leads to decreased levels of ATP needed as energy source
  - Severe intellectual disability and developmental delay
- No currently approved therapies

**PreClinical Studies**

**Question:** Does LUM-001 reach therapeutic concentrations in brain?
- *In vitro* cell uptake studies
- *In vivo* $^{14}$C-LUM-001 PK/distribution study
- PK/ADME
- Bioanalytical method development
- CMC/formulation
- Toxicology

**Clinical Studies**

- Multi-site Natural History Study: Lumos, NCATS, UPenn, and Duke
- Centralized data management using NIH Clinical Trials Database and biological sample collection

**Outcomes**

- Natural History study initiated Oct. 2016
- Lumos received funding to support further clinical development
  - Welcome Trust Award
  - VC funding
Why Drugs Fail in Development

**Drug Failure Modes**
- Efficacy: 28%
- Safety: 55%
- Strategic: 7%
- Commercial: 5%
- Operational: 5%

**Human toxicities found in animals**

- Skin
- Cardiovascular
- Endocrine
- Gastrointestinal
- Haemopoietic
- Hepatic
- Neurological
- Urinary
- Other

*Arrowsmith and Miller, Nature Reviews Drug Discovery, Volume 12, 569 (2013)*

*Cook et al., Nature Reviews Drug Discovery, Volume 13, 419 (2014)*
Human Tissue Chip Program

**Goal:** develop biochips to test for safe, effective drugs

- **Phase 1:** Individual chips
- **Phase 2:** Cell incorporation and organ integration

- **Current focus:**
  - Integration (DARPA and NIH); insight/expertise (FDA); compound testing, validation
  - Partnerships (MTA: GSK; Pfizer; AZ; MOU: IQ Consortium)
  - Adoptions of the tech to the community
Barth Syndrome Heart on a Chip Model

A

B

C

D

E

Normal Contractility of Wild Type Tissues

Impaired Contractility of Diseased Tissues

Rescued Contractility of Diseased Tissues

1 mm

1 mm

Teste stress (Pa) (peak syst - diast, gated)

Peak systolic stress (Pa)

modmRNA

nGFP

TAZ

nGFP

TAZ

nGFP

TAZ

WT1

BTHH

Galactose

Glucose

National Center for Advancing Translational Sciences

NIH
Modeling the mitochondrial cardiomyopathy of Barth syndrome with induced pluripotent stem cell and heart-on-chip technologies

Gang Wang¹,¹⁴, Megan L McCain²,¹⁴, Luhan Yang²,³, Aibin He¹, Francesco Silvio Pasqualini², Ashutosh Agarwal², Hongyan Yuan², Dawei Jiang¹, Donghui Zhang¹, Lior Zangi¹, Judith Geva¹, Amy E Roberts¹,⁴, Qing Ma¹, Jian Ding¹, Jinghai Chen¹, Da-Zhi Wang¹, Kai Li¹, Jiwu Wang⁵,⁶, Ronald J A Wanders⁷, Wim Kulik⁷, Frédéric M Vaz⁷, Michael A Laflamme⁸, Charles E Murry⁸-¹⁰, Kenneth R Chien¹¹, Richard I Kelley¹², George M Church²,³, Kevin Kit Parker²,¹³ & William T Pu¹,¹³

Study of monogenic mitochondrial cardiomyopathies may yield insights into mitochondrial roles in cardiac development and disease. Here, we combined patient-derived and genetically engineered induced pluripotent stem cells (iPSCs) with tissue engineering to elucidate the pathophysiology underlying the cardiomyopathy of Barth syndrome (BTHS), a mitochondrial disorder caused by mutation of the gene encoding tafazzin (TAZ). Using BTHS iPSC-derived cardiomyocytes (iPSC-CMs), we defined metabolic, structural and functional abnormalities associated with TAZ mutation. BTHS iPSC-CMs assembled sparse and irregular sarcomeres, and engineered BTHS ‘heart-on-chip’ tissues contracted weakly. Gene replacement and genome editing demonstrated that TAZ mutation is necessary and sufficient for these phenotypes. Sarcomere assembly and myocardial contraction abnormalities occurred in the context of normal whole-cell ATP levels. Excess levels of reactive oxygen species mechanistically linked TAZ mutation to impaired cardiomyocyte function. Our study provides new insights into the pathogenesis of Barth syndrome, suggests new treatment strategies and advances iPSC-based in vitro modeling of cardiomyopathy.
Next Phase Tissue Chip Initiatives

- **Tissue Chip Testing Centers (2016-2018)**
  - Tech transfer and testing at 2 independent centers (Texas A&M and MIT)

- **Tissue Chips for Disease Modeling (2017–2022)**
  - Develop tissue chip models of human diseases, particularly rare
    - Using human primary or induced pluripotent stem cell sources
  - Use to test effectiveness of candidate therapeutics

- **Tissue Chips in Space (2017–2021)**
  - Partnership with Center for the Advancement of Science in Space (CASIS)
  - Adapt, refine chips for on-flight experiments at the International Space Station U.S. National Laboratory
    - To understand diseases (e.g. bone, muscle, aging) prevalent on earth and accelerated in space
International Rare Diseases Research Consortium (IRDiRC)

- Established 2011 to maximize global coordination and cooperation in rare disease research
  - Members from Europe, North America, Asia, Australia, Middle East
  - Each funder supports its own research

- Initial focus on developing common scientific and policy frameworks

- 2011-2020 objectives:
  - 200 new therapies for rare diseases by 2020
  - Means to diagnose most rare diseases by 2020
  - Achieved in 2017 → new objectives formulated
IRDiRC Consortium Assembly

- Western Australia Department of Health
- European Organisation for Treatment & Research on Cancer, EORTC
- Canadian Institutes for Health Research
- Genome Canada
- BGI
- Chinese RD Research Consortium
- WuXi AppTec
- E-Rare 2 Consortium
- European Commission
- Academy of Finland
- Agence Nationale de la Recherche, ANR
- Fondation maladies rares
- French Muscular Dystrophy Association, AFM
- Lysogene
- Children's New Hospitals Management Group
- Federal Ministry of Education and Research
- Shire
- Chiesi Pharmaceuticals
- Istituto Superiore de Sanita
- Telethon Foundation
- Japan Agency for Medical Research and Development, AMED
- National Institutes of Biomedical Innovation, Health and Nutrition, NIBIOHN
- Saudi Human Genome Project
- Netherlands Organisation for Health Research and Development
- Korea National Institute of Health
- National Institute of Health Carlos III, ISCIII
- Roche
- National Institute for Health Research
- Food and Drug Administration, FDA
- National Cancer Institute, NCI, NIH
- National Center for Advancing Translational Sciences, NCATS, NIH
- National Eye Institute, NEI, NIH
- National Human Genome Research Institute, NHGRI, NIH
- National Institute of Arthritis and Musculoskeletal and Skin Diseases, NIAMS, NIH
- National Institute of Child Health and Human Development, NICHD, NIH
- National Institute of Neurological Disorders and Stroke, NINDS, NIH
- NKT Therapeutics
- Pfizer
- PTC Therapeutics
- Sanford Research
- EURORDIS
- National Organization for Rare Diseases
- Genetic Alliance
IRDiRC Goals 2017–2027

VISION: Enable all people living with a rare disease to receive diagnosis, care, and therapy within one year of coming to subspecialty medical attention

GOAL 1: All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently undiagnosable individuals will enter a globally coordinated diagnostic and research pipeline.

GOAL 2: 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options.

GOAL 3: Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients.
More information on IRDiRC

- http://www.irdirc.org/
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