Podium Presentation Abstracts

Monday, March 28

Track II: Translational Research; Session 1: Resources and Collaborative Paradigms in Academia, Not-for-Profit and Industry

Session Chairs: Paul Burn, Sanford Research Center

Location: Osceola C

1:30 – 2:00 pm

Plenary: Putting Research into Practice

Paul Burn, Sanford Research Center

2:00 – 2:30 pm

Changing the Rules for the Research "Game" in Academia - Accelerating Discovery Through Novel Means of Collaboration

Mark Atkinson, The University of Florida

3:00 – 3:30 pm

Collaboration as a Central Strategy for Successful Translational Research

John Reed, Sanford-Burnham Medical Research Institute

3:30 – 4:00 pm

Challenges and Successes of Translational Research in the Public Domain: Retrospective and Prospective Analyses and Case Histories of "Probes to Leads" From Six Years of the Molecular Libraries Program

Thomas "T.C." Chung, Conrad Prebys Center for Chemical Genomics at Sanford-Burnham Medical Research Institute

4:00 – 4:30 pm

Translation in Action: MRCT's Centre for Therapeutics Discovery

Justin Bryans, MRC Technology

Putting Research into Practice

Paul Burn, Sanford Research Center

Many basic, academic research institutions, biotech companies, and industry have an exemplary track record of fostering environments that lead to the advancement of science resulting in scientific discoveries. Only a very few, however, are able and have succeeded in translating these early discoveries into clinical proof of concepts studies in humans and ultimately to the delivery of novel therapeutic approaches and medicines. This is true in particular for indications such as type 1 diabetes that are underrepresented in the program portfolios of Biotech and big Pharma companies. Here, we present "The Sanford Project" an emerging translational research initiative aimed at delivering a cure for type 1 diabetes within the setting of Sanford Health, the largest rural health care provider in the US.

Changing the Rules for the Research "Game" in Academia —Accelerating Discovery Through Novel Means of Collaboration

Mark Atkinson, The University of Florida

While many intellectual gains have occurred in the field of type 1 diabetes (T1D) research over the past four decades, the period for which we considered the disorder to be autoimmune in its nature, a means to unequivocally prevent or reverse the disease has yet to been identified. This, despite the formation of large consortia to identify genes forming genetic susceptibility for T1D, test therapeutic interventions, determine the natural history of the disease, and more. While some may disagree, this lack in cure-related progress has not likely been blocked by lack of research funding, as considerable support opportunities have been available, through governmental agencies, private foundations, and philanthropy. Beyond this, spontaneous animal models of T1D exist, pharmaceutical industry interest in the disease is appreciable, and research publications touting important discoveries of this disease (reported in an ever increasing number of journals) has never been higher. Yet again, the disease has no cure. One theoretical impediment to research progress involves the long held "competitive" nature of academia, a survival of the fittest mechanism that provides minimal (or unclear) rewards for collaboration. To that end and as a test of that hypothesis, a group of self-aggregating investigators recently formed (i.e., the Brehm Coalition) for the purpose of accelerating discovery in T1D through operations that are designed to reward collaboration over competition. Guiding principles, largely unique in settings of academia, include those of: self-aggregation; flexible management; intimate collaboration facilitated by frequent and convenient video conferencing; emphasis on competence of the group rather than that of individuals; respect and trust in each other (i.e., complete sharing of all data); dependence on each other for results; cooperation on and even performance in one another's experiments; and sharing of institutional cores and resources. In addition, emphasis is placed within the Coalition on training the next generation of researchers that will, to the best of their abilities, adopt these principles whenever possible, as well as in reaching out to fellow colleagues within a given institution for like purpose. While too early to claim long term success, early results for this means of approaching research appear promising across a variety of metrics (both intellectual and emotional) and hopefully, with time, this will result in a way to meaningfully impact this disorder. In addition, if proven successful, this method for approaching research with an emphasis on collaboration may find application across a wide variety of academic disciplines, accelerating discovery.

Collaboration as a Central Strategy for Successful Translational Research

John Reed, MD, Sanford-Burnham Medical Research Institute

Translating laboratory discoveries into innovative therapeutics and diagnostics is one of the greatest challenges in the biomedical research enterprise today. Successful translational research rarely is achieved by lone individuals, and more often requires multi-disciplinary teams, armed with the appropriate resources to challenge disease and achieve breakthroughs in healthcare. Several examples of strategies developed at Sanford-Burnham Medical Research Institute for promoting successful translational research will be provided, including a description of efforts in high-throughput screening (HTS) and drug discovery as well as initiatives in pharmacogenomics and biomarker discovery. Central to the strategy are (a) building technological infrastructure in the form of "shared resources" (core facilities) that provide investigators with access to advanced technologies (instrumentation, etc.) along with dedicated professional staff skilled in delivering high quality results and (b) a culture of collaboration, where scientists, physicians, and other professionals join forces to tackle unmet challenges in healthcare.

Challenges and Successes of Translational Research in the Public Domain: Retrospective and Prospective Analyses and Case Histories of "Probes to Leads" From Six Years of the Molecular Libraries Program

Thomas "T.C." Chung, Conrad Prebys Center for Chemical Genomics at Sanford-Burnham Medical Research Institute

We present a retrospective analysis of >90 HTS & Probe Development projects undertaken by Sanford-Burnham during 6 years of NIH's MLPCN to highlight the unique challenges and critical factors of screening in the academic milieu. Case histories of successful and unsuccessful projects exemplify solutions and lessons, as well as discovery & development of potent state-of-art probes with novel target or pathway specificity, linkage to other translational research programs (e.g. CBC) and prospects for novel therapies.

Translation in Action: MRCT's Centre for Therapeutics Discovery

Justin Bryans, MRC Technology

Academic based drug discovery is fast becoming a significant player in target validation and the delivery of new potential treatments for disease, and there are a number of centres enjoying considerable success in this field. The Medical Research Council Technology's Centre for Therapeutics Discovery (CTD) in the UK is one such centre. The downsizing of "Big Pharma" has enabled the CTD to acquire high quality drug discovery skills and mesh them with some of the World's best academic medical research scientists to create a high quality drug discovery capability within academia. There is massive potential within academic research for the discovery of novel drugs, but in order to unlock this and gain buy-in from Pharma, the targets and molecules of interest need to be progressed to a stage that addresses their concerns. The CTD's primary aim is to collaborate with academics and Pharma on "de-risking" both small molecule and antibody based research projects by progressing them to a point where the targets have been validated by proof of concept studies in vitro and/or in vivo. In so doing, CTD leverages its own expertise in screening, medicinal chemistry and antibody generation and humanisation in a truly synergistic model with academia and Pharma. A brief description of the processes, initiatives and recent successes will be described.