Repurposing and Rescue Conference

REGISTER

November 14-15, 2022 | Washington, DC

Learn about the latest technologies, partnerships and advances propelling the field of repurposing and re-shaping drug discovery and development

Conference Agenda

November 14, 2022

(all times Eastern Standard Time)

8:05 am Chairperson's Opening Remarks
Aris Persidis, Ph.D., President, **Biovista**

8:15 am A Drug Repositioning Approach for Parkinson's Disease Using Patient Derived Cells
In the talk Dr. Mortiboys will outline a drug repositioning screening approach for Parkinson's Disease, starting with and validating in various patient derived cellular systems. This will include one example of ursodeoxycholic acid (UDCA) which is now in clinical trials for Parkinson's Disease, followed by more recent repositioning screens highlighting multiple compound classes with different novel mechanisms.
Heather Mortiboys, Ph.D., Professor of Cellular Neuroscience & Metabolism, **University of Sheffield**

8:45 am Converting Generics Into Patentable NCEs Without Falling into the 505(1)b Trap
An Achille's heel in the realm of drug repurposing has been the lack of exclusivity, unless one has an orphan drug designation. Even then there are the issues of off label prescriptions. In an attempt to address this we have developed a path that creates a NCE with composition of matter claim for the generic drug and yet allows for a slightly modified 505(b)2 development path. Additionally, the NCEs generated are highly soluble and often improve the PK profile of the original drug. Presentation will cover various examples that illustrates the concept.
Sundeep Dugar, Ph.D., President & Chief Executive Officer, **Aayam Therapeutics**

9:15 am Drug Repurposing Strategies for Global Health: Screening of the ReFRAME Library to Identify COVID-19 Interventions
The ReFRAME library is a comprehensive repurposing library of 12,000 compounds generated with support from the Bill & Melinda Gates Foundation to accelerate discovery of treatments for global health. With the onset of the COVID-19 pandemic, Calibr shared the library with virology labs around the world to accelerate the discovery of therapeutic interventions. They also rapidly developed in-house screens to identify drugs that could be repurposed as treatments for SARS-CoV-2 infection and as advanced starting points for medicinal chemistry optimization. In this talk Dr. Bakowski will discuss the development and results of their anti-SARS-CoV-2 screening cascade.
Malina Bakowski, Ph.D., Principal Investigator, **Calibr at Scripps Research**

9:45 am Solid-Form Drug Repurposing
Drug repurposing is often directed to developing new methods or formulations of pre-existing drug substances, often taking for granted the drug itself. As such, the focus of repurposing efforts is not directed to altering drug substance, especially in the small molecule context. However, by altering the solid-state properties of a drug substance, it is often possible to alter its delivery properties and, at the same time, create valuable new drug substance intellectual property. This presentation will discuss the strategy of deploying new sr molecule solid form drug substance patents for drug repurposing.

A target-based drug discovery strategy has led to a bias away from low molecular weight (MWT) drug discovery. Analysis of the ACS chemistry registration system shows that most low MWT drugs were first made in the time era before target-based drug discovery. Therapeutic activity among most low MWT drugs was identified in the era of phenotypic drug discovery when drugs were selected based on their phenotypic effects and before in vitro screening, mechanism of action considerations and experiences with fragment screening became known. The common perception that drugs cannot be found among low MWT compounds is incorrect based on both drug discovery history and our own experience with MLR-1023. We posit that low MWT compounds are more suited to identification of new therapeutic activity using phenotypic screens provided that the phenotypic screening method has enough screening capacity. Among ideal drug repositioning candidates, pleiotropic activity is far more likely due to on-target effects arising where a single target mediates multiple therapeutic benefits.
Christopher Lipinski, Ph.D., Scientific Advisor, **Melior Discovery**

11:15 am Fast-Tracking Affordable Cancer Treatments Using AI

Reboot Rx is the tech nonprofit startup dedicated to saving the lives of cancer patients using repurposed generic drugs. Repurposing FDA-approved generics represents a major opportunity to quickly improve outcomes for cancer patients and reduce healthcare costs. Hundreds of non-cancer generics have already shown promise for treating cancer across thousands of published studies, but it would take years to manually review the data and identify the most promising drugs for repurposing. Reboot Rx is building AI technology for rapid evidence synthesis and prioritization of drug candidates. Dr. Kleiman will discuss how Reboot Rx is partnering with cancer patients and oncologists to identify the top candidates to advance into the clinic.
Laura Kleiman, Ph.D., Founder & CEO, **Reboot Rx**

11:45 am Repositioning in the Time of Covid-19: Mapping the Opportunities

Long a tool in pharma development, repositioning is enjoying focused interest in Covid-19 times. What are lessons learned from Covid-19 repositioning? What is the opportunity map for the field going forward? Specific examples and technology approaches will be discussed.
Aris Persidis, Ph.D., President, **Biovista**

12:15 pm Luncheon

1:15 pm PANEL SESSION: Investing in Repurposing: Strategies for Influencing the Capital Markets' Perception of Drug Repurposing

David H. Crean, Ph.D., MBA, Managing Director, **Equitos Venture Partners**
Michael Derby, Managing Partner, **TardiMed Sciences**
Sven Jacobson, MBA, CEO, **Woolsey Pharmaceuticals**, Founder & CEO, **Martin Pharmaceuticals**

2:00 pm Creating Impact in Minority and Underserved Communities Through Repurposing

The value of repurposing is more evident today than ever before, not just in finding possible treatments for the COVID-19 pandemic, but also its ability to serve a broader purpose. Currently, there is increased attention on both the health disparities in communities of color as well as a racial gap in the funding of minority researchers in the US. Cures Within Reach's Diversity, Equity and Inclusion efforts are designed to help address both health disparities and building the pipeline of fundable minority researchers through proof-of-concept repurposing trials.
Clare Thibodeaux, Ph.D., Vice President, Scientific Affairs, **Cures Within Reach**

2:30 pm Drug Repositioning Using a Transcriptome-based AI Platform

In this presentation Dr. Kim will discuss the technology Oncocross has developed and its application in revolutionizing the drug development process. Oncocross is repurposing drugs by mining diseases against given drugs and mining drugs against diseases to treat rare, intractable diseases and diseases without treatments. Oncocross developed an AI platform that utilizes whole set of RNA gene expression patterns at transcriptome level of cells and human biopsy & blood samples to perform comparative and anti-symmetrical analysis to predict a mode that can reverse the unbalanced gene back to the normal state. Using their technology, they identified OC-501 and OC-504, both marketed drugs, as a possible drug combination for Sarcopenia. They performed in vitro and in vivo validation, and licensed-out to Korea Pharma, which recorded the first L/O deal of an AI-developed repurposed drug candidate from an AI biotech to a traditional pharmaceutical company in Korea. Korea Pharma recently submitted Phase IIa clinical trials IND for aged sarcopenia in Korea and they are currently conducting a phase I clinical trial in Australia for global license.
Yi Rang Kim, MD, Ph.D., Founder & Chief Executive Officer, **ONCOCROSS**

3:00 pm Refreshment/Networking Break

3:30 pm Investment Transactions in 505(b)(2) Product Pathways: An Investor's Perspective

In the session, you'll learn about:
Which asset classes are deploying capital to develop products and companies
Why investors allocate capital into projects that offers competitively differentiated products and market exclusivity which address unmet

Tuberculosis (TB) remains a major public health threat with an estimated 1.5 million annual deaths worldwide. TB drugs fail to shorten the lengthy treatment duration and display poor efficacy against multi drug resistant (MDR) M. tuberculosis (Mtb). To overcome these challenges, instead of targeting resistance prone microbial components with antibiotics, host directed therapy (HDT) is envisioned to target conserved host factors and enhance the ability of the human host to fight infections. Screening of the COVID Box library identified 5 drugs with relatively high activity against Mtb within infected macrophages. Several show low micromolar inhibition of Mtb growth inside infected macrophages without toxicity to the host cell or activity against Mtb grown in Broth. A hit anti Parkinson drug was further characterized and its molecular target in macrophages was revealed using combined pharmacological and genetic approaches. We have shown that it is possible to identify novel TB drugs from an arsenal of repurposed drugs and identify and their corresponding target. To conclude, we suggest that repurposing of existing drug is a cost-effective strategy for finding a drug candidate for HDT against TB which offers advantages of reducing high attrition rates and accelerating anti-infective drug development.
Yossef Av-Gay, Ph.D., Professor, Departments of Medicine and Microbiology and Immunology, **University of British Columbia**

4:30 pm Retargeting Drugs Through Synthetic Lethality

Genetically targeted therapies have emerged as a major force in the treatment of cancer over the past decade. They account for a significant proportion of oncology approvals in the US and currently benefit nearly one if five cancer patients. The vast majority of approved targeted therapies inhibit oncogenic transformation driven by gain-of-function mutations that aberrantly activate a specific pathway. Unfortunately, approximately two thirds of cancers are caused by loss of function mutations that cannot be easily targeted through these conventional approaches. At Leapfrog Bio we are taking advantage of synthetic lethality by drugging an essential target that compliments a mutation in another gene within the tumor. This approach allows us to target tumors caused by loss of function drivers. Our unique pharmacogenomic approach yields novel insights into the biology of cancers while simultaneously identifying small-molecule compounds to effectively treat mutations in tumor suppressor genes. I will share insights that we believe are crucial to successfully retargeting drugs to treat genetically defined tumors, including a focus on synthetic lethality with driver genes, pre-clinical validation, and companion diagnostics for patient selection.
Tomas Babak, Ph.D., Chief Executive Officer, **Leapfrog Bio**

5:00 pm Cocktail Reception

November 15, 2022

(all times Eastern Standard Time)

8:30 am Chairperson's Opening Remarks

Dr. Bruce Bloom, Chief Collaboration Officer, **HealX**

8:35 am Repurposing Beta-lactams for Buruli Ulcer Therapy, From the Bench to the Clinic

Drug discovery and development for NTDs (Neglected Tropical Diseases) is not an attractive activity to the pharma industry due to low returns compared to pharmaceutical standards, as such, new approaches need to be implemented. Repurposing clinically approved might help to speed up the development of new therapies for NTDs. Beta-lactams are one of the largest groups of antibiotics available today with an exceptional record of clinical safety in humans. Dr. Ramon-Garcia and his team recently demonstrated that beta-lactams combined with rifampicin and clarithromycin are synergistic in vitro against M. ulcerans. They thus focused on amoxicillin/clavulanate, which is oral, suitable for treatment in adults and children, and readily available with an established clinical pedigree. In this presentation, the scientific rational behind the progression of amoxicillin/clavulanate will be discussed as a new anti-BU treatment in combination with current oral BU therapy, rifampicin and clarithromycin, together with the steps undertaken for its validation, including a multi-country pragmatic clinical trial in West Africa (the BLMs4BU trial. PMID: 35804454) with the final goal of country adoption of the new therapy.
Santiago Ramon-Garcia, Ph.D., ARAID Investigator, **Research and Development Agency of Aragon (ARAID) Foundation**

9:05 am COVID-19 Brought Worldwide Attention to Repurposing - Will VCs and Pharma Rethink the Value of the 505(b)(2) Pathway?

Federal funding for COVID-19 treatments required product availability within six months for testing in human clinical trials. Given this rapid timeline, repurposing came to the forefront of all research efforts. Big pharma rapidly embraced various approaches for assessing opportunities within their portfolio of approved products and initiated studies with COVID-19 patients in record time. Without accepting repurposing as a development strategy, pharma would not have been able to introduce any treatments for the pandemic.
John Seman, Chief Executive Officer, **Revitale Pharma**

9:35 am Academic Drug Repurposing: Perspectives from Industry and Philanthropy

The goal of this session is to share over two decades of experience working with and funding academic researchers and clinicians advancing drug repurposing from both the philanthropic and industry perspectives, including real world examples of what has work and what has not. We will discuss examples from solicitation of projects all the way through negotiating contracts from both industry and

10:30 am Panel Session: Drug Repurposing and Repositioning in a Global Setting: EATRIS and NewFound, a Vision for a Global Collaboration.

The goal of this session is to highlight international effort in repurposing/repositioning medicines through accessing global translational science expertise. Approximately 3000 medicines have been approved globally for human use. Many of these agents have multiple pharmacological activity in related diseases and share biological targets and pathways to benefit patients without known therapies, and in some cases at lower cost. Although pharmaceutical companies have practiced "internal repurposing and positioning "of their proprietary molecules very successfully, public-private partnerships in doing so are still limited and fraught with challenges. REMEDI4ALL (https://remedi4all.org) and NewFoundMed (https://newfoundmed.org/) are new initiatives that have partnered to enable a global effort to address challenges and opportunities in scientific, regulatory, and business models that currently exist, and create a vision for moving forward with new models. Leaders with decades of experience in drug discovery, development and regulatory issues will participate in this session to discuss the current landscape and engage the audience to increase awareness and promote collaborations between governments, academia, industry, and non-profit organizations globally.

Chairpersons: G. Sitta Sittampalam, Ph.D., Senior Advisor to the Director, Office of The Director, National Center for Advancing Translational Sciences, **National Institutes of Health**

Dr. Bruce Bloom, Chief Collaboration Officer, **HealX**

Panelists:

Martin de Kort, Ph.D., Senior Scientific Program Manager Translational Medicine & Drug Development, **EATRIS**

Jaykumar Menon, Chair and Co-founder, **Open Source Pharma Foundation**

12:00 pm Luncheon

12:50 pm Establishing and Running a Highly Active Global Drug Repurposing Clinical Trial Program in Parkinson's Disease

How drug repurposing, currently involving a portfolio of >40 drugs, each of which addresses a different neuroprotective biological target, is being used to identify therapeutics that may slow disease progression. Lessons learned, problems solved during the decade of the program, and results of recent patient trials will be discussed. Examples will be drawn from our GLP-1, GBA and cAbl clinical trial programs. We will also outline the efficiency benefits of our multi-arm, multi-stage clinical trial designs, as well as our drug screening programs that identify appropriate therapeutics to enter into these trials.

Dr. Richard Wyse, Director of Clinical Development, **Cure Parkinson's**

1:20 pm The Benefits of Collaborative Business Efforts on 505(b)(2) Repurposing

The pandemic has demonstrated how important repurposed products can be in producing rapid action and innovation to meet market needs. However, sadly many repurposed products never gain the support needed to make it to market. As we move into the next stage, how can we use collaboration to expand the number of repurposed and repositioned products that make it to market? In this session Mr. Casey will discuss achieving success through meaningful collaborations. Learn how to create more innovation, efficient processes, increased success, and improve communication by:

♦ Building partnerships with competitors, other businesses, non-profits and government regulators to move your repurposing business forward

♦ Using collaboration to amplify your voice

♦ Collaborating to create rapid action and innovation in days and weeks instead of months

♦ Using collaboration to problem solve

Stephen Casey, Founder & CEO, **The 505(b)(2) Platform**

1:50 pm Drug Repurposing Roadmap That Optimizes Patient Impact Through Collaboration

The Cure Drug Repurposing Collaboratory (CDRC) is a US FDA funded public-private partnership initiated in June 2020 and led by the non-profit Critical Path Institute (C-Path). The Collaboratory brings together a diverse group of global stakeholders to advance drug repurposing by systematically capturing real-world data from off-label drug usage to inform future clinical trials for diseases of high unmet medical need. The ultimate goal is to provide an open and transparent roadmap that supports regulatory submissions to expand drug labels for diseases with limited or no treatment options.

Smith Heavner, Ph.D., RN, Scientific Director, CURE Drug Repurposing Collaboratory (CDRC), **Critical Path Institute**

2:20 pm Rethinking Translational Drug Discovery and Repurposing Through the Use of AI/Machine Learning-Supported Biology

Small animal models have been extensively used to better understand basic and fundamental aspects of human biology. Decades of work have shown that small animal models are relevant in vivo models of choice to catalyze preclinical drug discovery efforts, and more recently, drive therapies into a clinical setting. Now, in conjunction with artificial intelligence and machine learning, they are proving to be valuable tools to improve our understanding of the underlying causes of human disease, and in our efforts to accelerate drug development and repurposing into them.

James Doyle, Ph.D., Co-Founder and CEO, **Modelis**

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