# RARE DRUG DEVELOPMENT SYMPOSIUM

## A PARTNERSHIP BETWEEN PENN MEDICINE ORPHAN DISEASE CENTER AND GLOBAL GENES

PHILADELPHIA, PENNSYLVANIA

## NETWORKING AND CUREACCELERATOR LIVE!

CureAccelerator Live! Rare Disease is a philanthropic pitch competition that gives attendees the chance to vote for the next breakthrough repurposing treatment. Finalists will be Principal Investigators presenting their clinical repurposing projects for patients with rare diseases. The winner, announced that evening, receives up to $50,000 in funding.

Congratulations to our Finalists:

**Margherita Baldassarri, MD, University of Siena:** Repurposing a Generic Diuretic to Treat Alport Syndrome

**Kim Nichols, MD, St. Jude Children's Research Hospital:** Repurposing a Blood Cancer Drug to Treat an Immune Disorder in Children

**Kevin Winthrop, MD, MPH, Oregon Health and Science University:** Repurposing a Leprosy Drug to Treat a Rare Lung Infection

**Stephen Wong, PhD, Houston Methodist Research Institute:** Repurposing a Heart Failure Drug for Pediatric Brain Cancer

**DAY 1: 5:00 PM – 8:00 PM**

- OPEN NETWORKING WITH EXHIBITORS, POSTERS AND RECEPTION
- PITCH BY CUREACCELERATOR LIVE! FINALISTS, FOLLOWED BY EXPERT PANEL Q&A
- VOTING, ANNOUNCEMENT OF WINNER AND NETWORKING

**Location:** Liberty Ballroom B

# RARE DRUG DEVELOPMENT SYMPOSIUM MEETING AGENDA

## DON'T STOP INNOVATING: PATIENTS' ROLE IN BREAKTHROUGH IDEAS

**DAY 2: 7:00 AM – 8:00 AM** *Sponsored by:* STRONGBRIDGE BIOPHARMA

### BREAKFAST, REGISTRATION, POSTERS, EXHIBITORS AND NETWORKING

**Location:** Foyer

**DAY 2: 8:00 AM – 8:15 AM**

### WELCOME -- INNOVATION TAKES A COLLABORATIVE EFFORT

**Location:** Liberty Ballroom A

Join us as we give a quick overview of the sessions for the 2019 RARE Drug Development Symposium -- all focused on where and how rare disease communities can actively create and champion innovations in research and drug development to realize advances in disease understanding and therapy development.

**DAY 2: 8:15 AM – 9:15 AM**

**SESSION #1**

### WHERE DOES INNOVATION HAPPEN IN RESEARCH AND DRUG DEVELOPMENT?

**Location:** Liberty Ballroom A

The process of research and drug development is rapidly changing as we continue accelerating into an era of patient-driven research, early and frequent collaboration among patients, researchers, and industry, and new therapeutic approaches that may revolutionize our understanding and approach to rare disease. This opening session will give an overview of the research and development process for rare disease, with a focus on the steps where innovation is rapidly occurring, the stakeholders advancing innovations, and how patients and advocates play a role.

**DAY 2: 9:30 AM – 10:20 AM**

**SESSION #2.1**

## INTRODUCTION TO GENOMICS: WHY IT MATTERS FOR DIAGNOSIS, UNDERSTANDING AND TREATMENT DEVELOPMENT

**Location:** Liberty Ballroom A

Your complete genome is all the genetic information in your cells -- about three billion pairs of DNA bases. Most rare diseases and conditions result from a change in one or more of these DNA bases. This change can be small, but in rare disease, the effects, even from a small change, are tremendous. Identifying the change that is the cause of a rare disease and understanding how that change causes a rare disease are critical steps in research and therapy development. This session will provide an introduction to the science of genomics, or the study of the genome, and its role in research and development for rare diseases.

**DAY 2: 9:30 AM – 10:20 AM**

**SESSION #2.2**

## RARE GENETIC INSIGHTS INTO COMMON DISEASE: SHARED TRAITS CAN LEAD TO WAYS TO COLLABORATE AND EXPAND RESEARCH

**Location:** Liberty Ballroom B

There can be molecular or genetic connections among some rare diseases and more common diseases, which means that understanding the rare disease can also provide insight into understanding the common disease. Identifying these potential connections can expand the number of researchers and the funding dedicated to both the rare disease and the common disease. In this session, hear about examples of expanding research and collaborations on rare disease through sharing efforts with more common diseases.

**DAY 2: 10:20 AM – 10:50 AM** *Sponsored by:* NOVARTIS

## NETWORKING COFFEE BREAK WITH POSTER PRESENTATIONS AND EXHIBITORS

**Location:** Foyer

**DAY 2: 10:50 AM – 11:40 AM**

**SESSION #3.1**

## WHERE TO BEGIN: MODELING RARE DISEASE

**Location:** Liberty Ballroom A

One of the first steps on the path to developing a rare disease treatment is developing an animal model of the disease. Animal models can help confirm the cause and effects of a rare disease and can also serve as an initial test for potential treatments. Mouse models are often discussed, but there are several other types of common, and not-so-common, animal models. This session will discuss considerations in developing animal models for rare disease.

**DAY 2: 10:50 AM – 11:40 AM**

**SESSION #3.2**

## BUILDING UNDERSTANDING OF RARE DISEASE THROUGH NEXT-GENERATION MODELS

**Location:** Liberty Ballroom B

Tools for understanding rare diseases, and ultimately for developing and testing potential therapies, are rapidly evolving. As a complement to animal models, induced pluripotent stem cells, organs-on-a-chip, and other new approaches to modeling rare disease can help to understand the mechanisms of rare diseases. This session will discuss these new approaches and how they could be used in rare disease research and development.

**DAY 2: 11:45 AM – 1:00 PM** *Sponsored by:* HORIZON

## NETWORKING LUNCH WITH POSTER PRESENTATIONS AND EXHIBITORS

**Location:** Horizon Rooftop Ballroom

**DAY 2: 1:00 PM – 1:50 PM**

**SESSION #4.1**

## CLINICAL TRIALS: HOW TO USE NATURAL HISTORY DATA

**Location:** Liberty Ballroom A

Natural history studies track the course of a disease over time, helping to understand the full range of disease presentation and symptoms, even potentially identifying specific subgroups in a rare disease. This data and understanding can be very helpful when designing clinical trials for testing a potential treatment, and even, in some cases, be used in place of a placebo-control in a clinical trial. This session will discuss these and other ways natural history data can inform clinical trials for rare disease.

**DAY 2: 1:00 PM – 1:50 PM**

**SESSION #4.2**

## CLINICAL TRIALS: WHAT IS A MASTER PROTOCOL AND HOW CAN IT BE USED FOR RARE DISEASE

**Location:** Liberty Ballroom B

The standard approach to clinical trials is to develop a design that will answer the question of how does one drug compare to standard of care treatment. This can lead to a slow and sometimes inefficient process. One potential approach to streamlining the clinical trials process is a master protocol, or a test of multiple drugs simultaneously and/or testing a therapy in multiple populations at the same time. Learn more about this innovative approach to clinical trials and how it may be used for rare disease.

**DAY 2: 2:00 PM – 2:50 PM**

**SESSION #5.1**

## FINDING NEW USES FOR INNOVATIONS: REPURPOSING THERAPIES

**Location:** Liberty Ballroom A

Drug development is an expensive and lengthy process. One approach to addressing these challenges of drug development for rare disease is to test therapies that have already been approved for a new rare disease indication, or drug repurposing. Learn more about this approach to drug development through a discussion of successes and continuing efforts.

**DAY 2: 2:00 PM – 2:50 PM**

**SESSION #5.2**

## ARE YOU READY TO DISCOVER NEW THERAPIES?

**Location:** Liberty Ballroom B

Certain milestones must be met before pursuing the development of a new therapy can be a viable option. What are those milestones, and how do you know the research has reached a point where developing a potential target is possible?

**DAY 2: 2:50 PM – 3:20 PM** *Sponsored by:* Spark Therapeutics

## NETWORKING COFFEE BREAK WITH POSTER PRESENTATIONS AND EXHIBITORS

**Location:** Foyer

**DAY 2: 3:20 PM – 4:10 PM**

**SESSION #6**

## KEEPING INNOVATION GOING: ONE IS NOT ENOUGH

**Location:** Liberty Ballroom A

Rare disease patients deserve multiple research approaches to their disease and continued efforts to innovate and improve therapeutic options. Hear about the importance of continuing to pursue research and development and new collaborations even when there are dedicated research efforts and effective therapies in development or available.

**DAY 2: 4:10 PM – 4:30 PM**

## THANK YOU, MEETING CLOSE

**Location:** Liberty Ballroom A

the orphan disease center

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