Let’s Talk Therapeutics:
How Urgency & Collaboration Lead to Therapeutic Strategies
Speaker: Hans Christinger, Ovid Therapeutics
August 17, 2019

Visit kif1a.org/2019Conference to watch a recording of this presentation.
Support TREATMENT for Susannah

Managing Expectations with a Home Remodeling Project

Accessible Homes

On the Way to a Rare Disease Cure, Parents Tackle the High Price Tag of Research

RARE DISEASE

Medical ID

Susannah Rosen
Agenda

- What is biotechnology?
- What does a biotechnology company do?
- Urgent collaboration
What is a Biotechnology?

**Definition of biotechnology (Merriam Webster)**

1: the manipulation (as through genetic engineering) of living organisms or their components to produce useful usually commercial products (such as pest resistant crops, new bacterial strains, or novel pharmaceuticals)

*also* : any of various applications of biological science used in such manipulation
Innovation in Science & Medical Application

The manipulation (as through genetic engineering) of living organisms or their components to produce useful usually commercial products.
Innovation in Science & Medical Application

*also*: any of various applications of biological science used in such manipulation

New understanding of specific receptors & their importance in the brain

New understanding of what is meaningful change in syndromes and how to measure change in clinical trials
What Were the Rules & Regulations for Developing “Biotechnology” Medicines in 1976?

There were none!

Biotech companies, regulatory agencies, physicians, and the community came together and figured it out.
What Does A Biotechnology Company Do?
Our Purpose is to Improve Care Through Meaningful Medicines
Understand as Much as Possible About A Patient’s Experience

Need
- What is the child experiencing?
  - Development, movement, sleep, communication...
- What is the cause?
  - How might we address the underlying cause?
- Does the medicine deliver meaningful change in people, safely?
- Can we measure and the impact of medicines in cells and animals?
- Can we replicate the situation in cells and animals?

Gene

Cell “model”

Animal “model”

Clinical trial
A Community - We Bring Together the Best Resources and Minds to Help Create a Medicine – Ovid Example
Learn from Research into Similar Genes / Proteins, to Expedite Development of a Medicine
Understand Normal and Atypical Function to Inform Strategies for the Development of Medicines
Approaches to Develop a Medicine

• Replace gene
• Knock down gene
• Modulate KIF1A activities and interactions
• Modulate pathway and compensate for KIF1A
Screen Options to Find a Potential Medicine

- Small Molecule
- Gene Therapy
- ASO
- iPSC Testing
- Mouse Model
- Safety Screens
- microRNA
Urgent Development Path to a Medicine

- **Pre-clinical**
  - Sequenced Genomes
  - Cell Lines - iPSC
  - Mouse Model
  - Safety Screens

- **Non-clinical**
  - IND-Enabling

- **Clinical**
  - Natural History
  - “Rare” Model

- **Regulatory**
  - Compassionate Use/ Phase I/II

- **CMC**
  - Partner with CMO