



# Bootcamp Agenda

May 14-16, 2024

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Research is the  
Key to Help  
Patients

There are many  
types of  
“research”  
important for  
drug discovery?



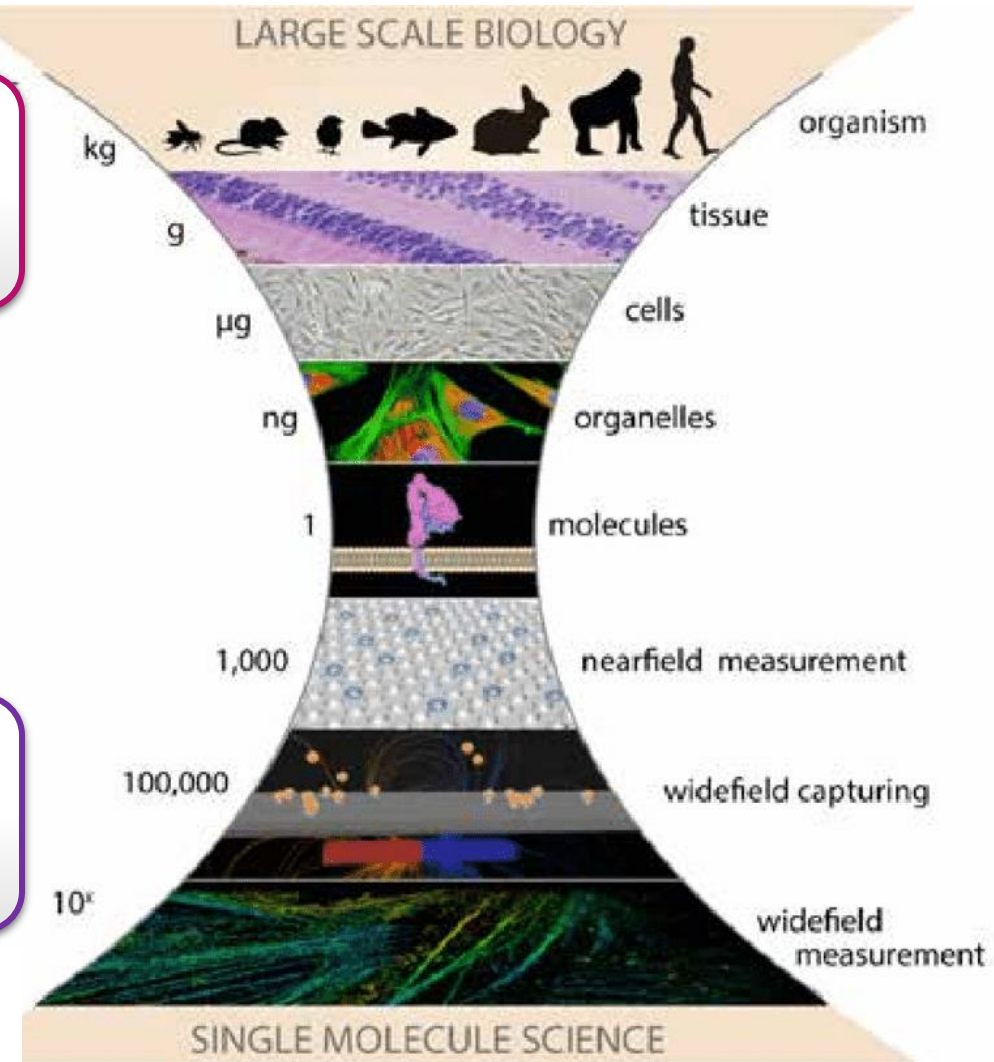
# Discovery Research is Basic Science

How does it all work? (Body, mind, normal situation)

- Study people at organismal, organ systems, cellular, and molecular levels
- Utilize tractable models (animals, cells) to gain a deeper understanding

Why do I feel sick? (What is different for me than normal?)

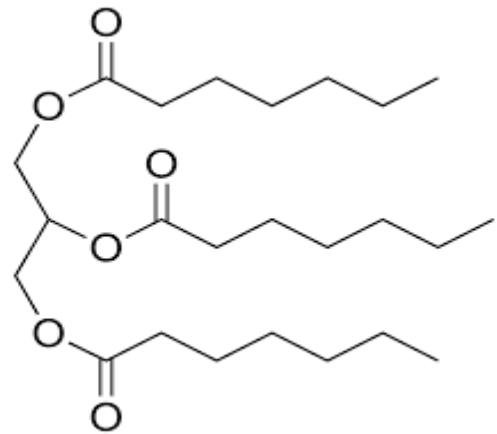
- Studied at all levels - but the goal is to reduce how you are feeling to a molecular target



# Translational Research

## *Modalities – what kind of medicines can we make?*

### Small Molecule



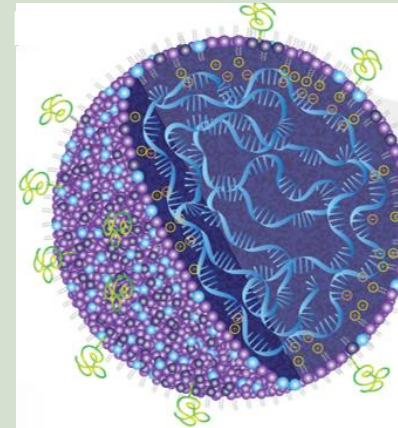
- Chemical compound
- <1 kDa
- Simple manufacturing

### Protein Biologic



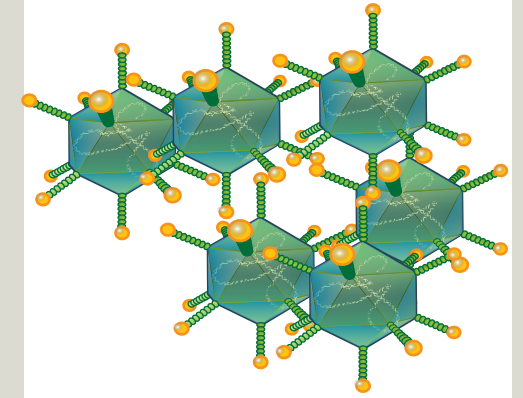
- Proteins or antibodies
- Antibody can bind a target and block its function

### Nucleic Acid



- Oligonucleotides
  - Bind to RNA/DNA
- siRNA – bind to RNA and chop it
- mRNA – gene replacement

### Gene Therapy



- Deliver DNA to a target cell in an AAV-viral capsid
- One-shot therapy – long-term durability

# Day 1 Agenda

## *Defining Research Goals*

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- Presentation Skills – connection, garner interest and support
- Patient Diagnosis – Paul Kruszka
  - Genetics underpinning why I feel sick.
  - With this knowledge, I can find more people like me.
- Therapeutic Modalities
  - Small Molecules & Repurposing – Bruce Bloom
  - Oligonucleotides – Scott Dindot
  - Gene Therapy and Gene Editing – Matt Fuller
- Table Discussion – what modalities are best candidates for your indication?

# Day 2 Agenda

## *Translational Research to Enable Clinical Research*

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- Biomarkers - Binodh DeSilva
- Animal Models – Maggie Wright
- Table Discussion – Identifying Research Priorities – what should we fund first?
- Perspective of an Experienced Rare Disease Drug Developer – Emil Kakkis
- IND-Enabling Studies – Marcus Andrews
- Regulatory Overview – Sandra Nino-Siddens
- Cellular Models – Angels Almenar

# Clinical Research

What is Clinical Research? – testing the therapeutic in people

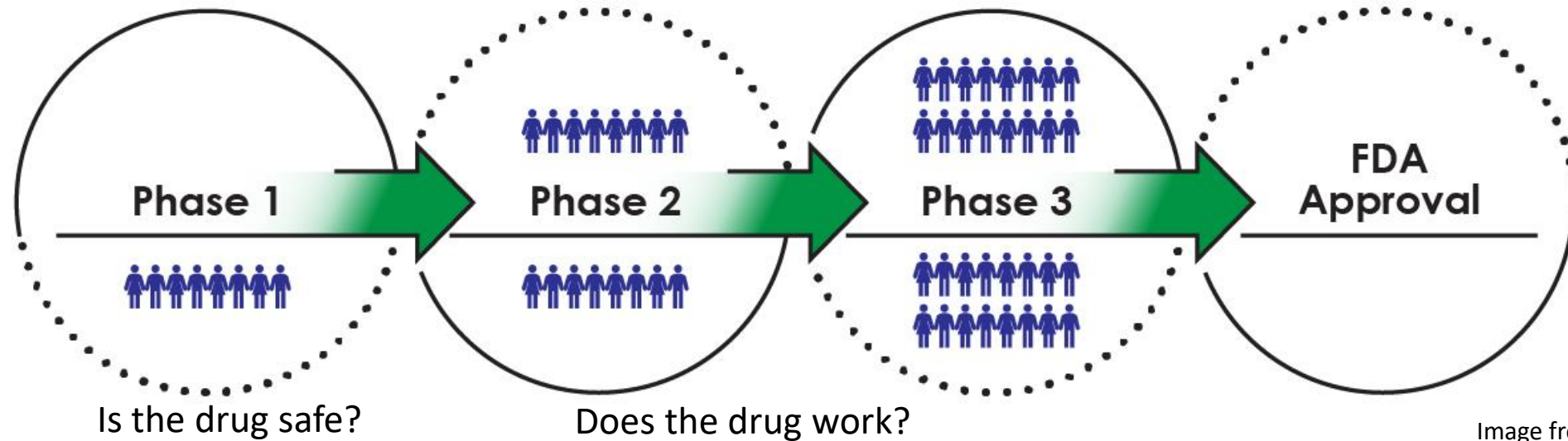


Image from Concert Pharma. website

## Day 2-3

Clinical Endpoints – Ali Skrinar

Clinical Trial Design – Lawrence Meritt

Manufacturing – Dennis Huang

# Day 2-Day 3 Agenda

## *Finding the Resources & Practical Considerations*

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- Legal and IP Panel Discussion – Paul Wickman, Brei Gussack, Christina McDougall
- Collaborating with Academia – Scott Dindot
- Natural History Studies & Registries – Farid Vij
- Patient Identification – Lukas Lange
- Funding Panel Discussion – Tom Kassberg, Neil Kumar, Beryl Cummings



# Day 3 Agenda

## *Advice from Experienced & Successful Foundation Leaders*

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- Mika Graglia – Early Research Funding; SynGAP Research Fund
- Charlene Son Rigby – Navigating Foundation Dynamics; STXBP1 Foundation and Global Genes
- Casey McPherson – Parent Journey; To Cure a Rose and Multiple Companies
- Julia Vitarello – From Mila to Millions; Mila’s Miracle Foundation

Requires





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# Here we GO!



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