Rare Bootcamp™

Agenda

15 – 17 April 2025

ultragenyx

EVERYLIFE FOUNDATION FOR RARE DISEASES

A WARM THANK YOU TO ADDITIONAL RARE BOOTCAMP SPONSORS AND PARTNERS

Global Genes® Allies in Rare Disease

ALEXION AstraZeneca Rare Disease

Rentschler Biopharma

BIOMARIN

bridgebio

GeneDx

iXCells Biotechnologies

healx

Morgan Lewis

Day 1 Agenda
Defining Research Goals

- Presentation Skills – connection, garner interest and support
- Patient Diagnosis – Amanda Singleton
- Cellular Models – Nianwei Lin
- Animal Models – Lorelei Stoica

- Table Discussion: Identifying Research Priorities – what is known and what are the gaps?

Day 2 Agenda
Translational Research to Enable Clinical Research

- Therapeutic Modalities
  - Small Molecules & Repurposing – Bruce Bloom
  - Nucleotide Therapeutics – Janaiah Kota
  - Gene Therapy – Matt Fuller
  - Gene Editing – Shravanthi Madhavan
- Table Discussion – what modalities are the best candidates for your indication?
- IND-Enabling Studies – Marcus Andrews
- Biomarkers - Binodh DeSilva

Day 2 Agenda
Clinical Research

- Clinical Endpoints – Ali Skrinar
- Clinical Trial Design – Eric Crombez
- Regulatory Overview – Nicole Parker
- Small Scale Manufacturing – Tom Lauzon

Day 3 Agenda
Finding the Resources and Practical Considerations

- Policy and Public Affairs – Annie Kennedy
- Collaborating with Academia – Scott Dindot
- Legal and IP Panel Discussion – Brei Gussack, Mark Hayman, Sam McMahon
- Mika Graglia – Early Research Funding; SynGAP Research Fund
- Tom Kassberg – Late-Stage Funding
- Resources for Rare Diseases
  - Citizen Health – Kate Haldeman
  - COMBINEDBrain – Terry Jo Bichell
  - Gene Dx – Amanda Singleton
  - iXCells – Margaret Barlow
  - Odylia Therapeutics – Ashley Winslow
  - Probably Genetic – Caty Reid
  - RareRising – Kate Haugstad

Day 3 Agenda
Advice from Experienced and Successful Rare Disease Leaders

- Perspective of an Experienced Rare Disease Drug Developer – Emil Kakkis
- Charlene Son Rigby – Navigating Foundation Dynamics; STXBP1 Foundation and Global Genes
- Mika Graglia – Early Research Funding; SynGAP Research Fund
- Jocelyn Duff – Rare Parents as Drug Development Catalysts

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Here we GO!