The National Academies of SCIENCES ENGINEERING MEDICINE

HEALTH AND MEDICINE DIVISION

Board on Health Sciences Policy

Exploring Novel Clinical Trial Designs for Gene-Based Therapies - A Workshop

November 13, 2019

#RegenMedForum

Planning Committee Members

Krishanu Saha, Ph.D. (co-chair)
University of Wisconsin-Madison

Celia Witten, M.D., Ph.D. (co-chair)
U.S. Food and Drug Administration (FDA)

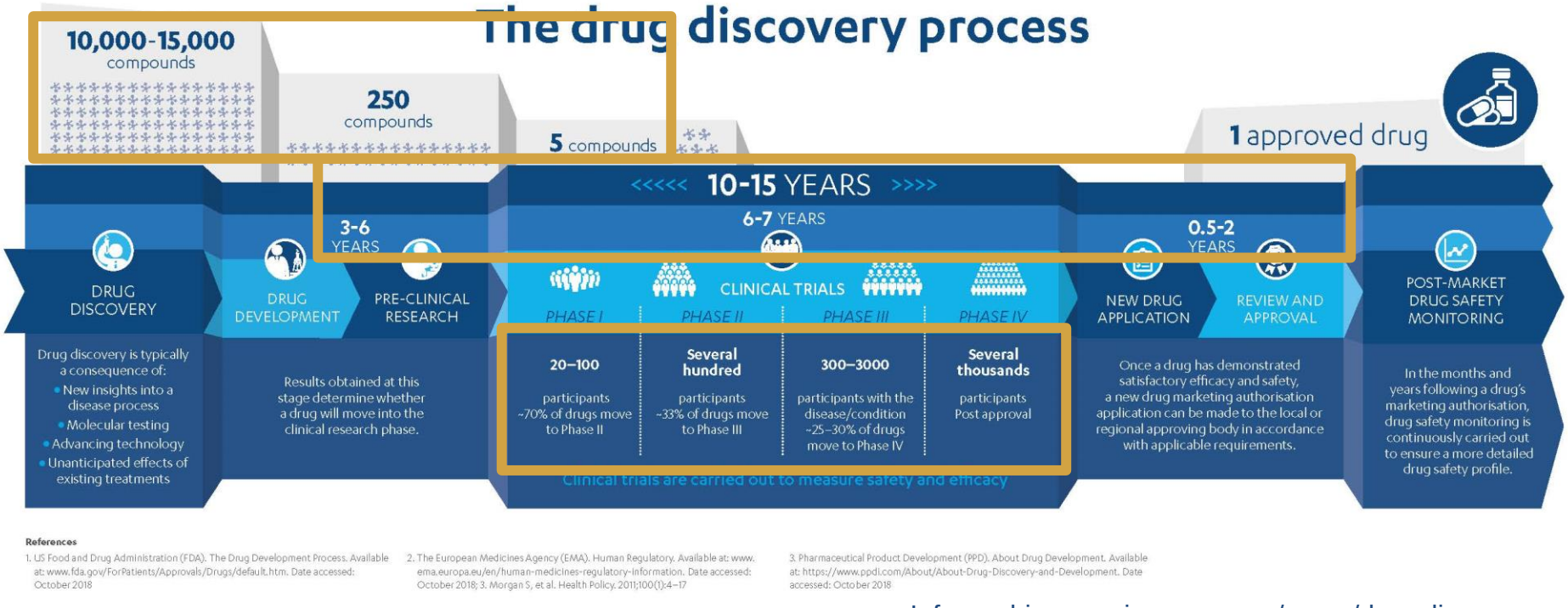
Mildred Cho, Ph.D.
Stanford University

Michael DeBaun, M.D., M.P.H.
Vanderbilt University

Cindy Dunbar, M.D.
National Heart, Lung, and Blood Institute (NHLBI)

Derek Robertson, M.B.A., J.D., C.H.C.
Maryland Sickle Cell Disease Association

Katherine Tsokas, J.D.
Johnson & Johnson



References

1. US Food and Drug Administration (FDA). The Drug Development Process. Available at: www.fda.gov/ForPatients/Approvals/Drugs/default.htm. Date accessed: October 2018

2. The European Medicines Agency (EMA). Human Regulatory. Available at: www.ema.europa.eu/en/human-medicines-regulatory-information. Date accessed: October 2018; 3. Morgan S, et al. Health Policy. 2011;100(1):4–17

3. Pharmaceutical Product Development (PPD). About Drug Development. Available at: https://www.ppdi.com/About/About-Drug-Discovery-and-Development. Date accessed: October 2018

Infographic: www.janssen.com/emea/drug-discovery

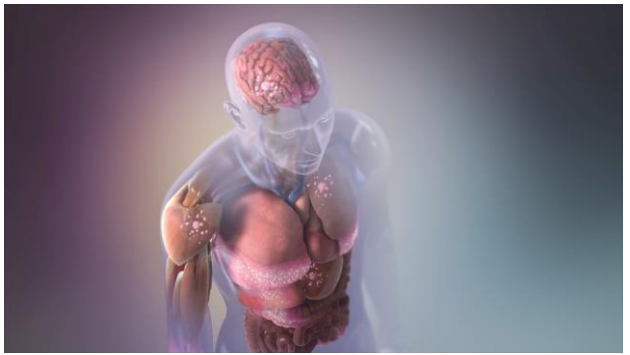
- The process for gene-based therapies is different: smaller numbers of compounds, patients, and years

Trials with smaller numbers

Layla Richards, leukemia, 2015
Photograph: Great Ormond Street Hospital/PA

Emily Whitehead, leukemia, 2013
Matt Chappell, HIV, 2014
Mila Makovec, Batten disease, 2019
Jaci Hermstad, ALS, 2019 (HR2855)
Victoria Gray, sickle cell, 2019
….

- Patients: *n* = 1-100
- Compounds: 1-10
- Time: 1-3 years
- Cells, viruses, editors, antisense oligos….



NIH Common Fund, SCGE

- Customizable to the genotype and/or patient population

Workshop Objectives

- Gain a better understanding of the design complexities and ethical issues associated with clinical trials for gene-based therapies
  - Transitioning to first-in-human trials
  - Determining the optimal starting dose
  - Optimizing therapeutic delivery
  - Communicating risks and benefits to patients and families

- Identify potential ways to improve the design of gene therapy clinical trials from the perspective of participants, product developers, regulators, and other key stakeholders

Reminders

- Please ask questions into a microphone and state your name/affiliation
- Speakers and Planning Committee members to meet for lunch in **Room 118**
  - Please let them pick up their lunch first