Recap & Potential Future Strategies in the Innovation of Drug R&D for Prevalent Chronic Diseases

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Guiding Themes

1. Patient Engagement and Access
2. Technology Solutions
3. Clinical Research Approaches
4. Research Applications

Patient Engagement and Access

- The knowledge gaps that discourage investment in R&D for prevalent, chronic disease are far too big for any one company or institution to fill
- When early research and drug development are not representative and inclusive, we miss out on knowledge, increase risks, and limit or forego widespread benefit
- Patient advocacy organizations and community-based organizations can help
  - Establishing research priorities
  - Identifying where new tools and more knowledge are needed
  - Organizing (and sometimes helping to fund) collaborations
  - Recruiting patients
  - Disseminating research results
- Incentives need to be aligned for both the patient and research communities

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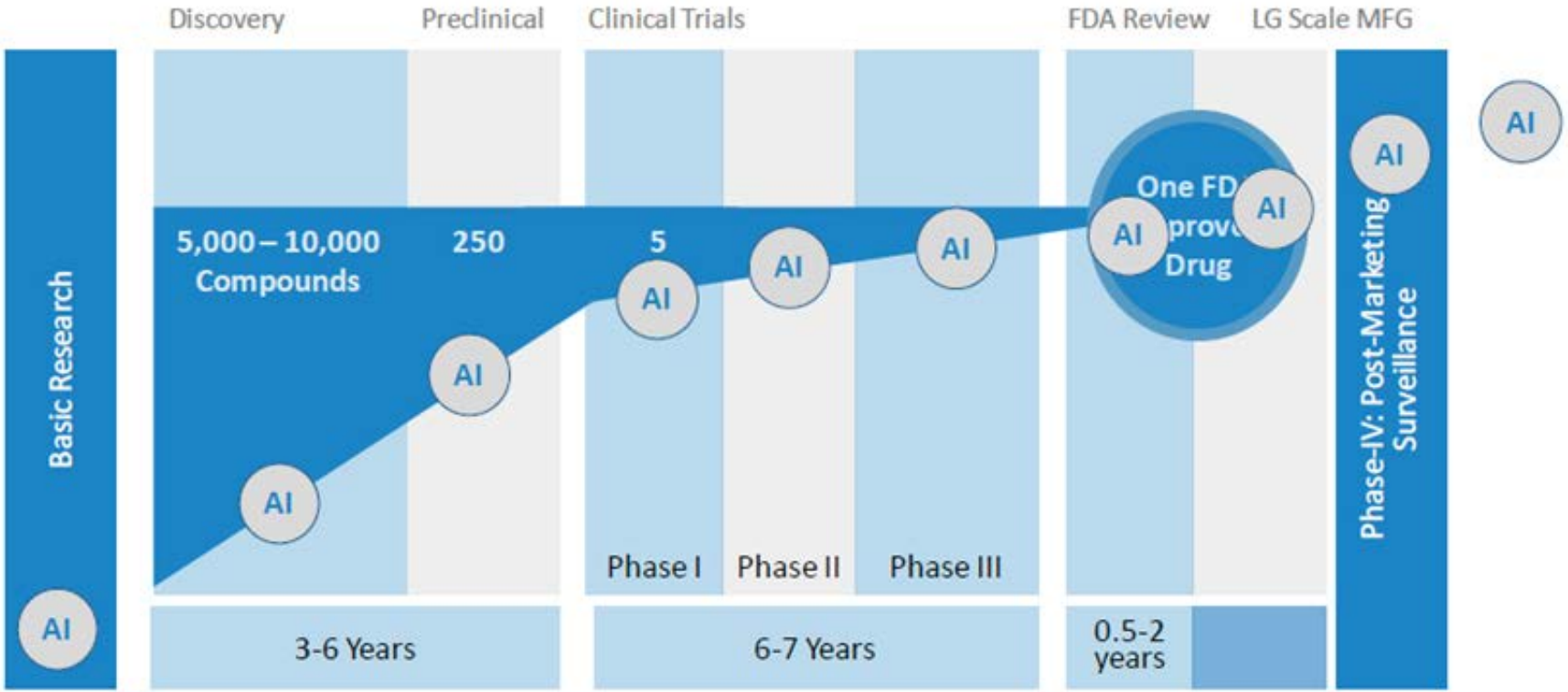
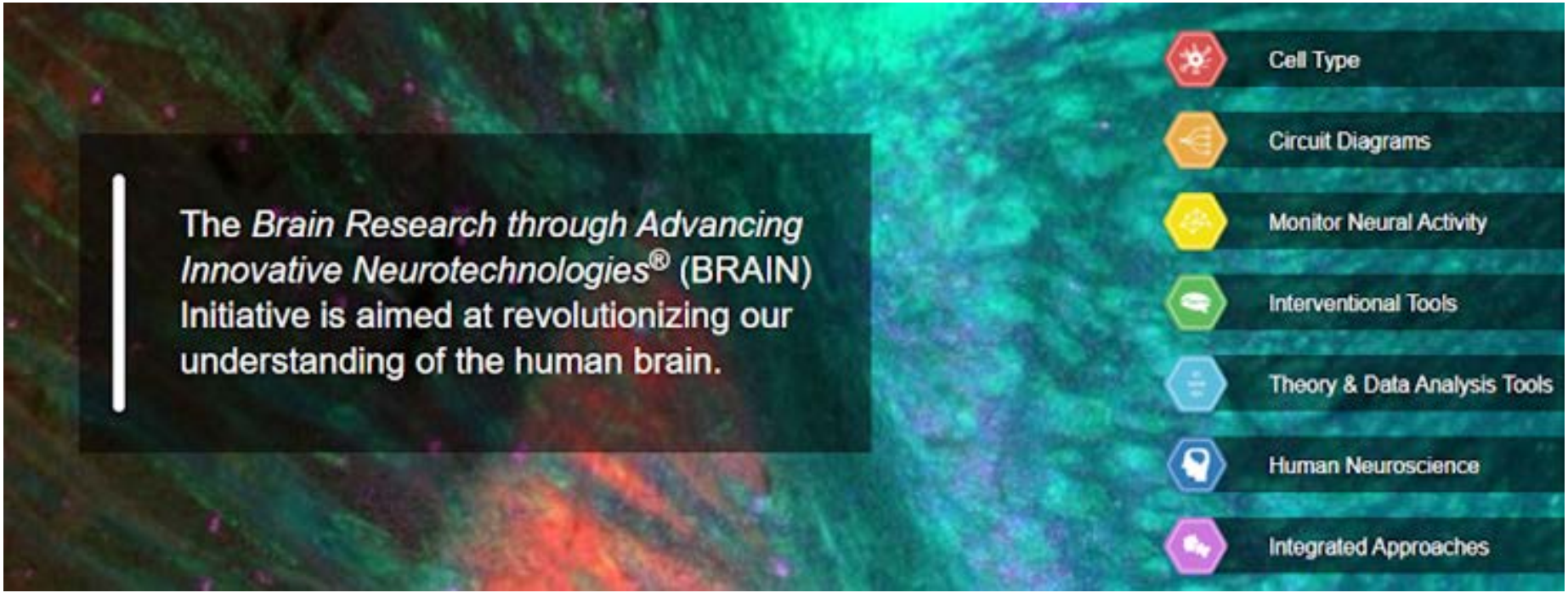
Technology impact on chronic diseases is growing

- Uncertainty from discovery to market so AI can help in multiple places
- Pathogenesis not well known for chronic diseases so AI helps discovery
- Low financial incentive so AI can save money & time
- Ability to generate more data feeds AI algorithms
  - E.g. BRAIN Initiative
  - E.g. Patient Diversity
- Knowing the gaps in knowledge, directs data collection e.g. ADME
- “Nuts & Bolts” challenges in formatting and sharing data, privacy and large number of potential sub-populations

AI APPLICATIONS IN DRUG DISCOVERY & DEVELOPMENT

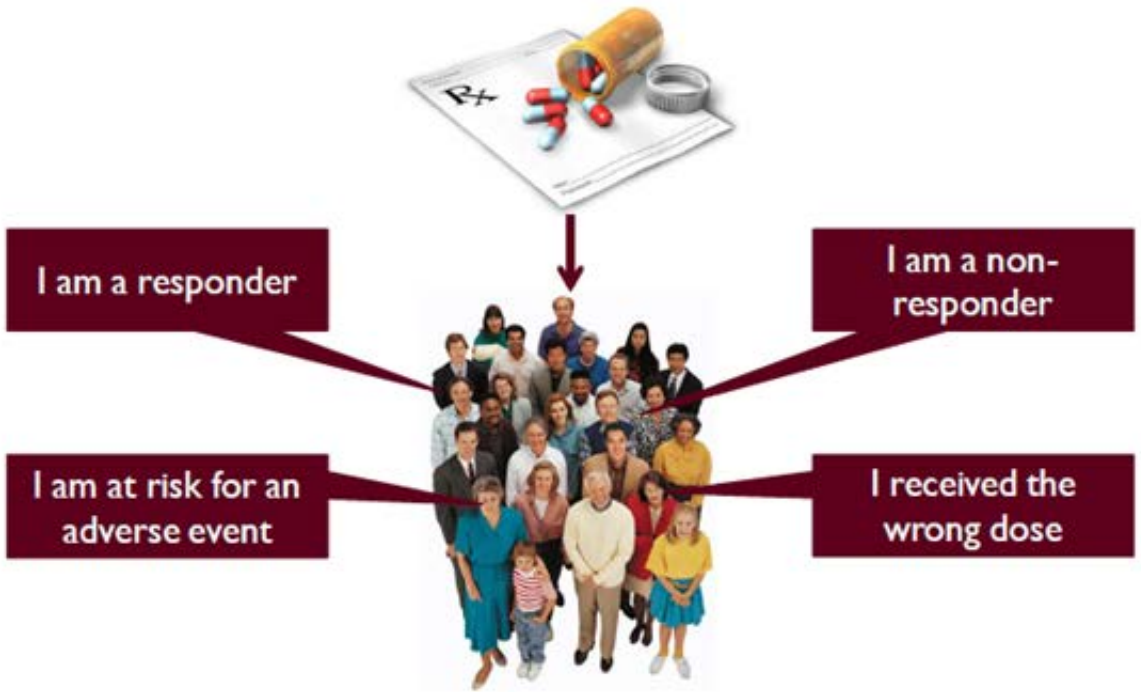


Slide originally presented by Andrew Radin, twoXar Pharmaceuticals



Perspective on NIH's BRAIN Initiative originally presented by John Ngai, The BRAIN Initiative

Precision Medicine and Pharmacogenomics



Slide originally presented by Erica Woodahl, University of Montana

New Types of Data: Data from Novel Experiment Systems

FDA/CDER Microphysiological Systems Laboratory

Review | Open Access

Liver Microphysiological Systems for Predicting and Evaluating Drug Effects

Alexandre J. S. Ribeiro, Xinning Yang, Vikram Patel, Rajnikanth Madabushi, David G. Strauss

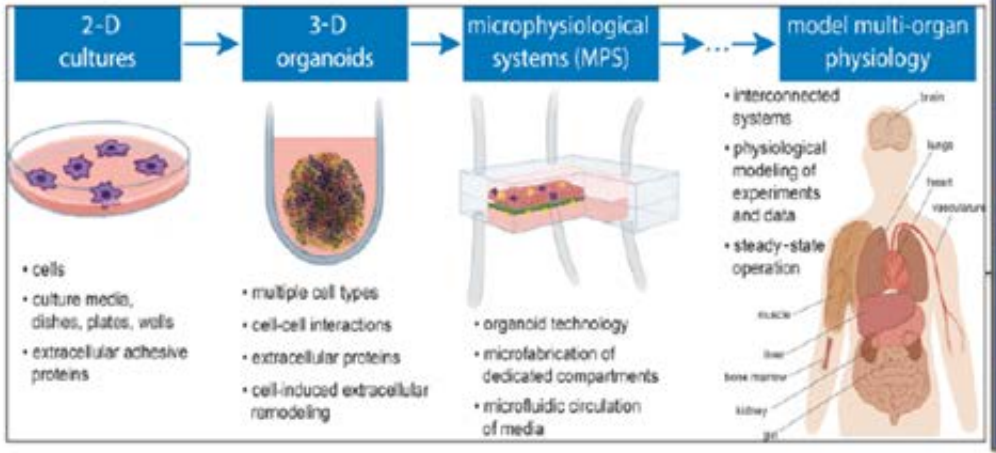
Clinical Pharmacology & Therapeutics 2019;106:139-47.

ARTICLE | Open Access

Characterizing the Reproducibility in Using a Liver Microphysiological System for Assaying Drug Toxicity, Metabolism and Accumulation

Andres Rubiano, Amruta Indapurkar, Ryosuke Yokosawa, Alina Miedzik, Barry Rosenzweig, Ayesha Arefin, Chloe M. Moulin, Keri Dame, Neil Hartman, Donna A. Volpe, Murali K. Matta, David J. Hughes, David G. Strauss, Tomasz Kostrzewski, Alexandre J.S. Ribeiro

Clinical & Translational Science 2020 [epub].



Slide courtesy of Dr. David Strauss

Slide originally presented by Qi Liu, FDA

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Clinical Research Approaches

- A patient-centered approach is necessary to designing clinical trials
  - Encourage engagement at every stage
  - Builds trust

- How do you involve patients during drug development?
  - Advise on the concept
  - Review proposals
  - Disseminate results

- Lessons from COVID
  - Decentralized trial designs to reach a broader audience
  - Lessons in leveraging clinical data to contribute to real world evidence
  - Maintain collaborations between payors, regulators, sponsors

Clinical Research Approaches

- It is important to intervene early in chronic disease
  - Innovative therapies are focused on treating earlier in disease progression
  - Consider patient journey and importance of quality of life
  - Value-based care - managing risk long-term

- New technologies produce multiple endpoints
  - Establish clear plans for collecting and analyzing data

- Clinical trials are becoming increasingly complex, but there is room for innovation in the way they are conducted and analyzed that is acceptable to regulators

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Research Applications