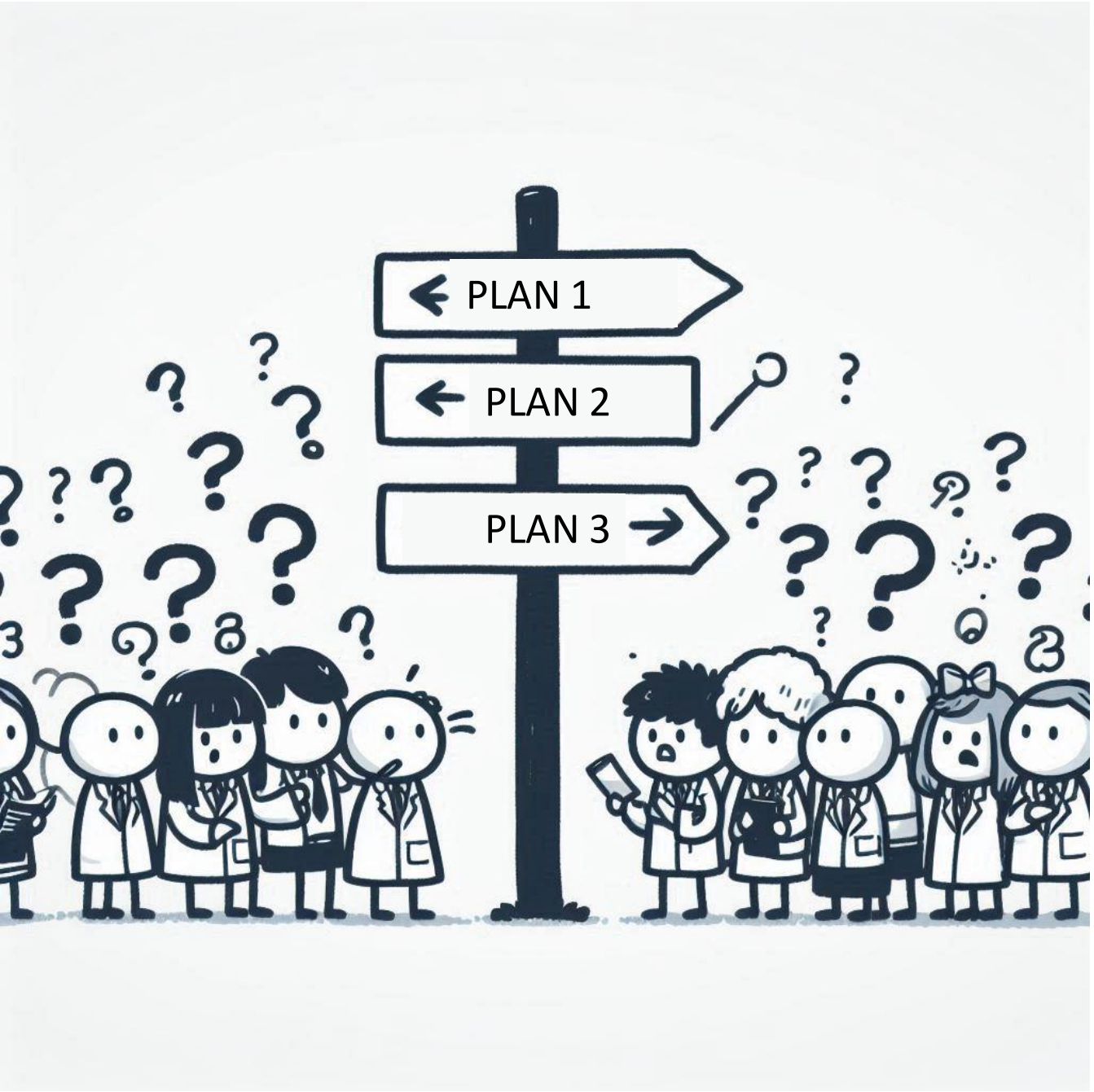


Guiding Research with the Goal of Translation

David M. Groppe, PhD

Clinical Data Scientist
Persyst Development Corporation

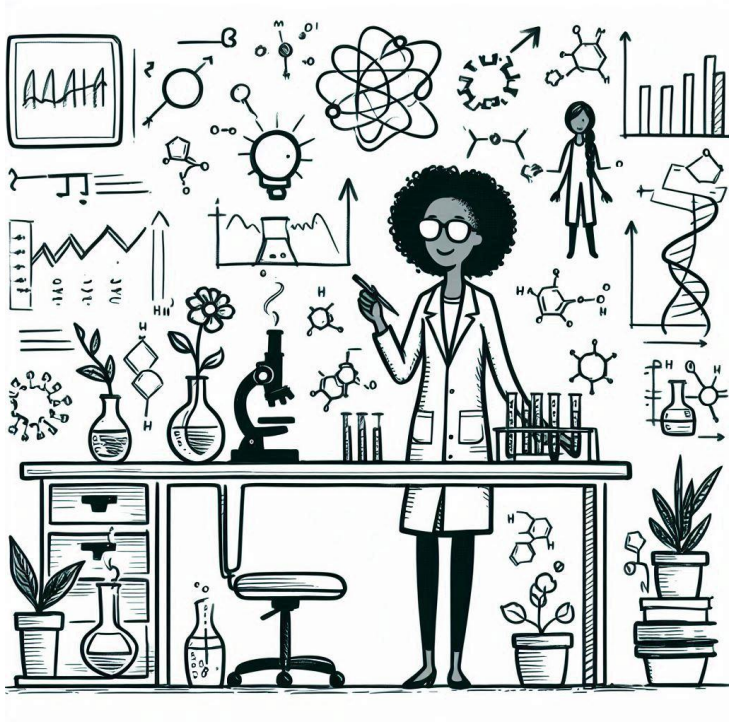
Presentation Outline

- What is Translational Research?
- Some Key Translational Issues
  - -Commercial Value
  - -Regulatory Pathway
  - -Intellectual Property
- Hypothetical Case Study: *An AI Diagnostic*
- Take Home Points

Learning Objectives

- Better understand key considerations (e.g., intellectual property) for translating research into clinical applications
- Spark ideas on how to design and execute research so that it is more easily translatable into clinical application

What is Scientific Translation?



“Translation is the process of turning **observations** in the laboratory, clinic and community into **interventions** that improve the health of individuals and the public — from diagnostics and therapeutics to medical procedures and behavioral changes.”

National Center for Advancing Translational Sciences:
https://ncats.nih.gov/about/about-translational-science

Multiple Definitions of Translational Research

New diagnostics & treatments (T1)
“effective translation of the new knowledge, mechanisms, and techniques generated by advances in basic science research into new approaches for prevention, diagnosis, and treatment of disease is essential for improving health.”

Dissemination of Best Practices (T2)
“ensuring that new treatments and research knowledge actually reach the patients or populations for whom they are intended and are implemented correctly "

Woolf, Steven H. "The meaning of translational research and why it matters." *Jama* 299.2 (2008): 211-213.

Multiple Definitions of Translational Research

New diagnostics & treatments (T1)
“effective translation of the new knowledge, mechanisms, and techniques generated by advances in basic science research into new approaches for prevention, diagnosis, and treatment of disease is essential for improving health.”

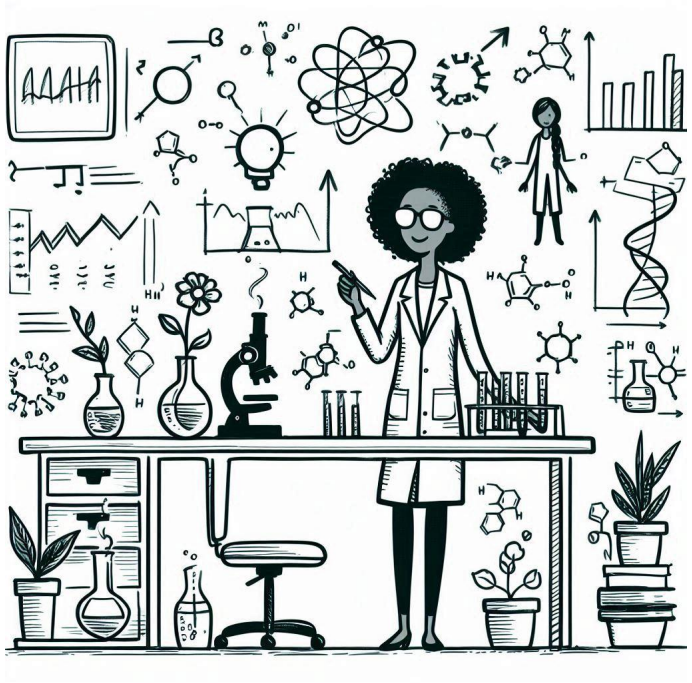
Dissemination of Best Practices (T2)
“ensuring that new treatments and research knowledge actually reach the patients or populations for whom they are intended and are implemented correctly "

Woolf, Steven H. "The meaning of translational research and why it matters." *Jama* 299.2 (2008): 211-213.

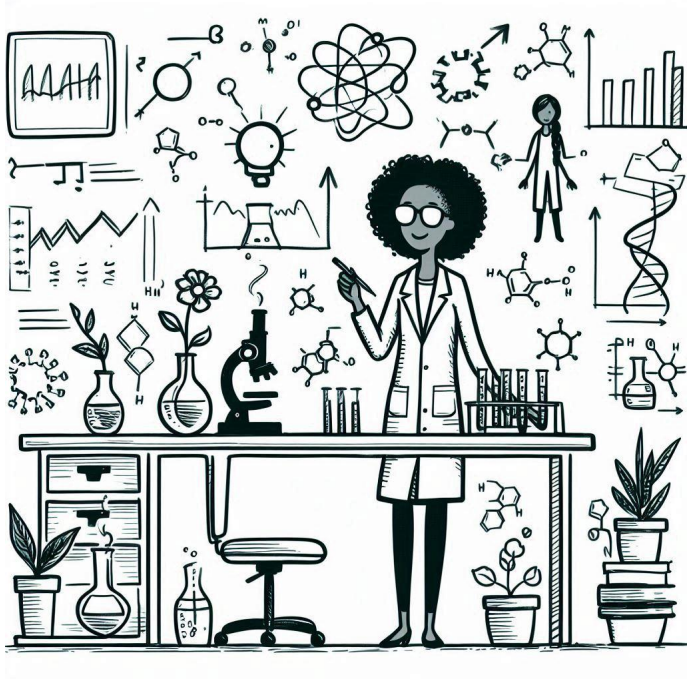
Presentation Outline

- What is Translational Research?
- Some Key Translational Issues
  - -Commercial Value
  - -Regulatory Pathway
  - -Intellectual Property
- Hypothetical Case Study: *An AI Diagnostic*
- Take Home Points

Key Translational Issues



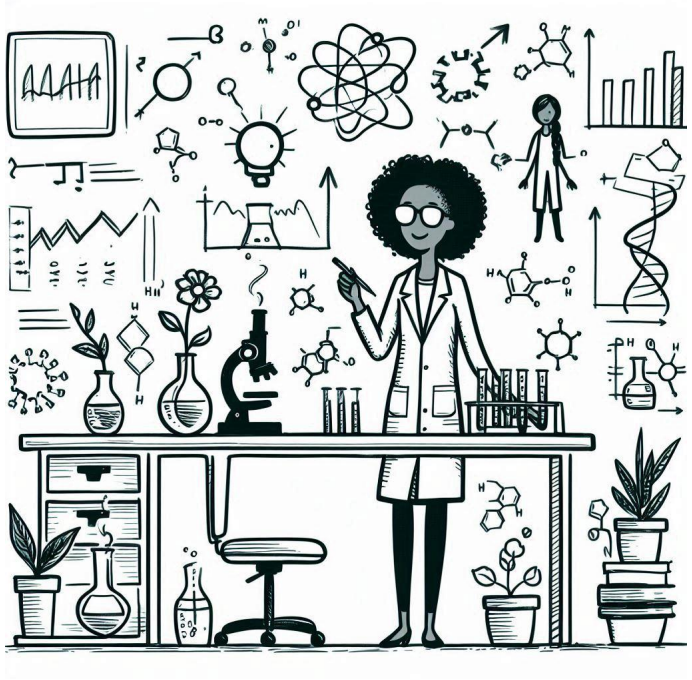
Key Translational Issues



1.Value

- Who will pay for it (hospitals, insurers, patients)?
- How much will they pay for it?
- How big is the market?
- What's the competitive advantage?

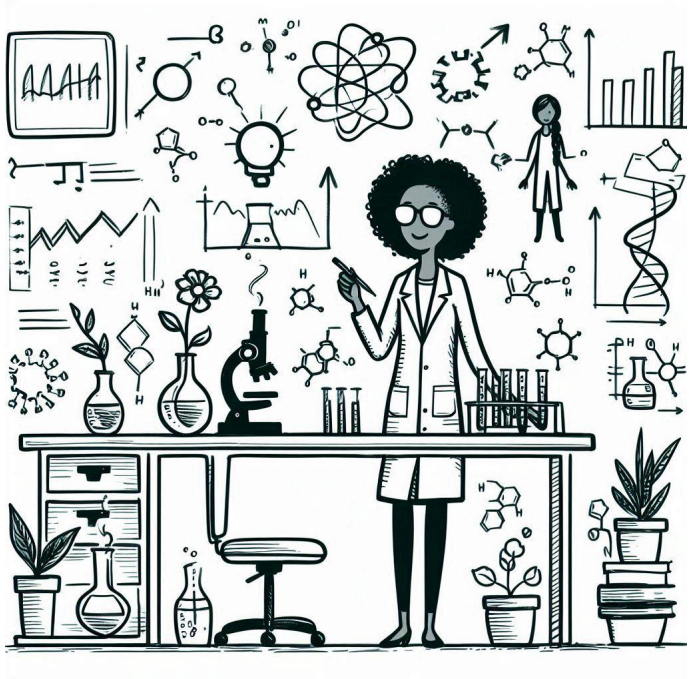
Key Translational Issues



1.Value
2.Regulatory

- What kind of drug (new drug, investigational new drug)?
- What class of medical device (I, II, or III)?
- Is there an existing product that can be compared against to simplify approval?
- How much would regulatory approval process cost?

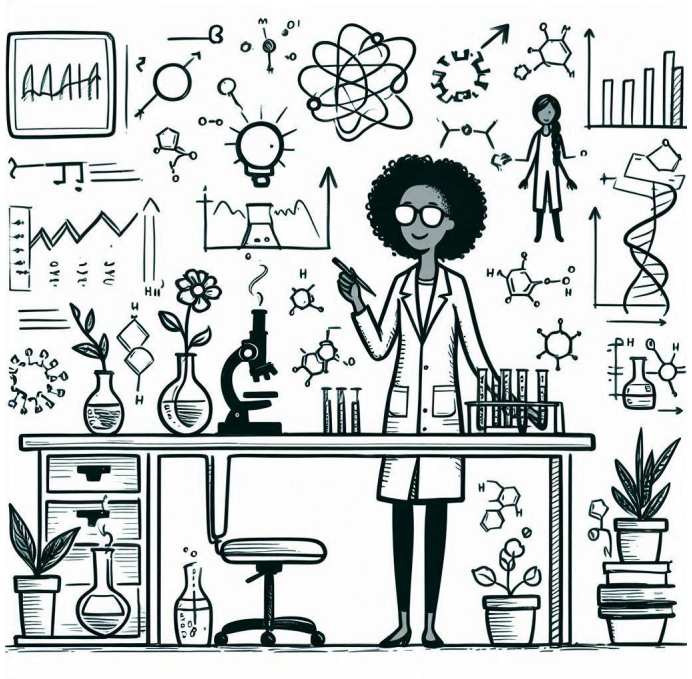
Key Translational Issues



1. Value
2. Regulatory
3. Intellectual Property

- Is the innovation patentable?
- Does the innovation infringe on existing patents/require licensing of IP?
- Are the data used for validation licensed for commercial use?

Key Translational Issues



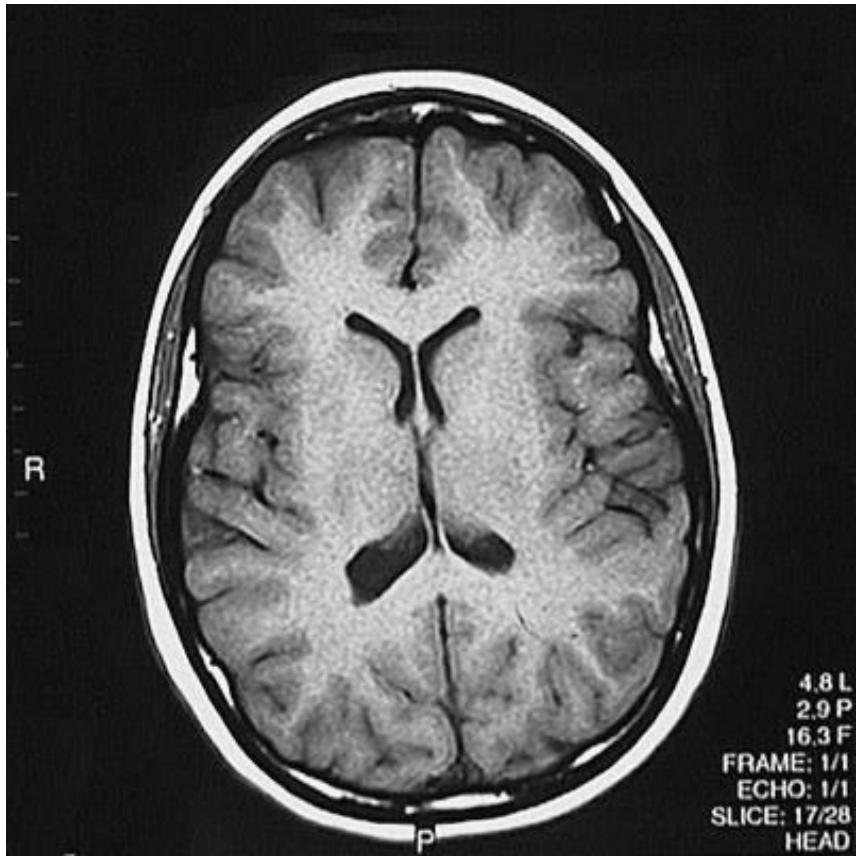
1. Value
2. Regulatory
3. Intellectual Property

A Systematic Problem: Academic scientists typically have little-to-no expertise in these domains.

Presentation Outline

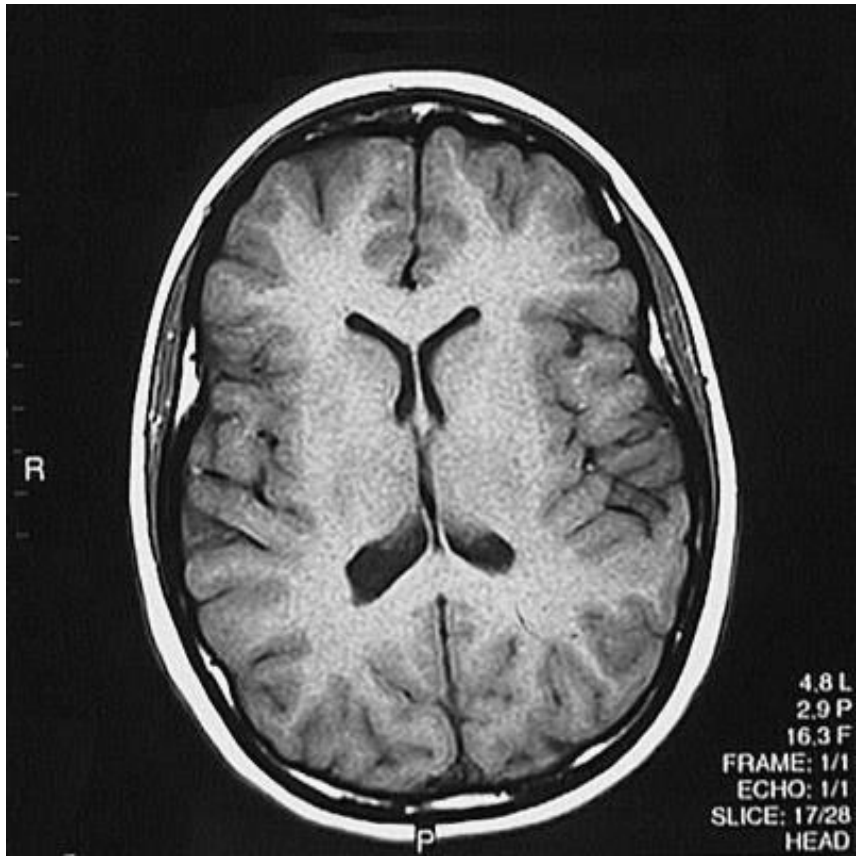
- What is Translational Research?
- Some Key Translational Issues
  - -Commercial Value
  - -Regulatory Pathway
  - -Intellectual Property
- Hypothetical Case Study: *An AI Diagnostic*
- Take Home Points

Hypothetical Example: *An AI Diagnostic*



Early diagnosis of Condition X from an MRI by an AI algorithm

Hypothetical Example: *An AI Diagnostic*

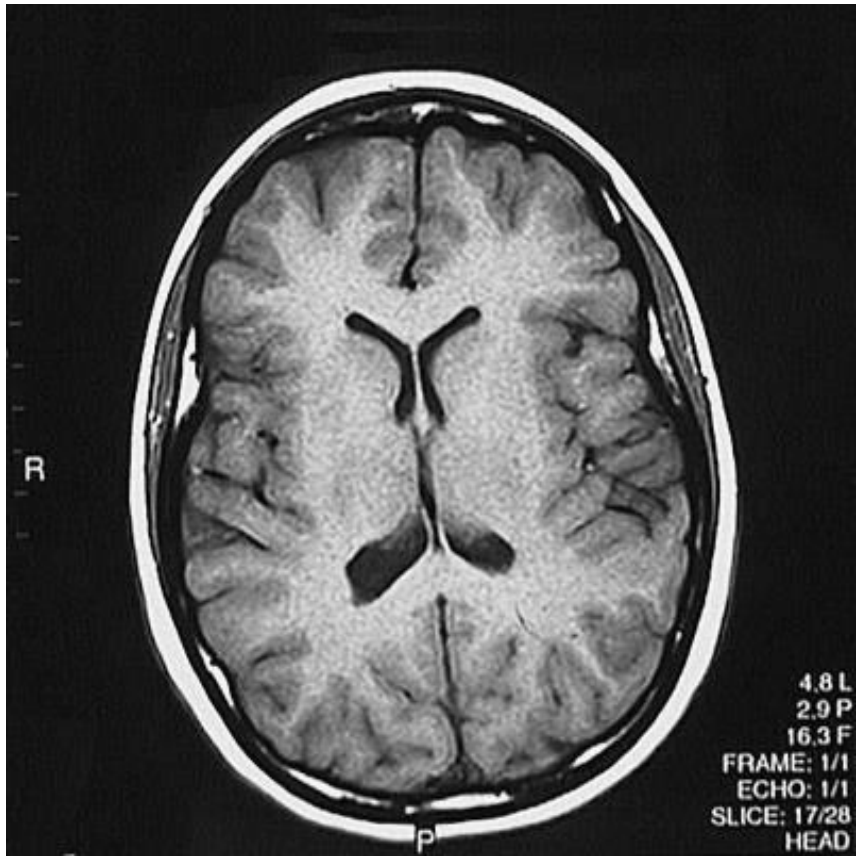


Early diagnosis of Condition X from an MRI by an AI algorithm

Common Research-Design Problems:

- Little-to-no research into the commercial value of the algorithm
- No research into the pathway for regulatory approval
- No research into whether or not the algorithm is patentable or infringes on existing patents
- Developed using popular research software that is not licensed for commercial use
- Validated using unrealistic, sanitized data from one hospital
- Data not licensed for commercial use

Hypothetical Example: *An AI Diagnostic*

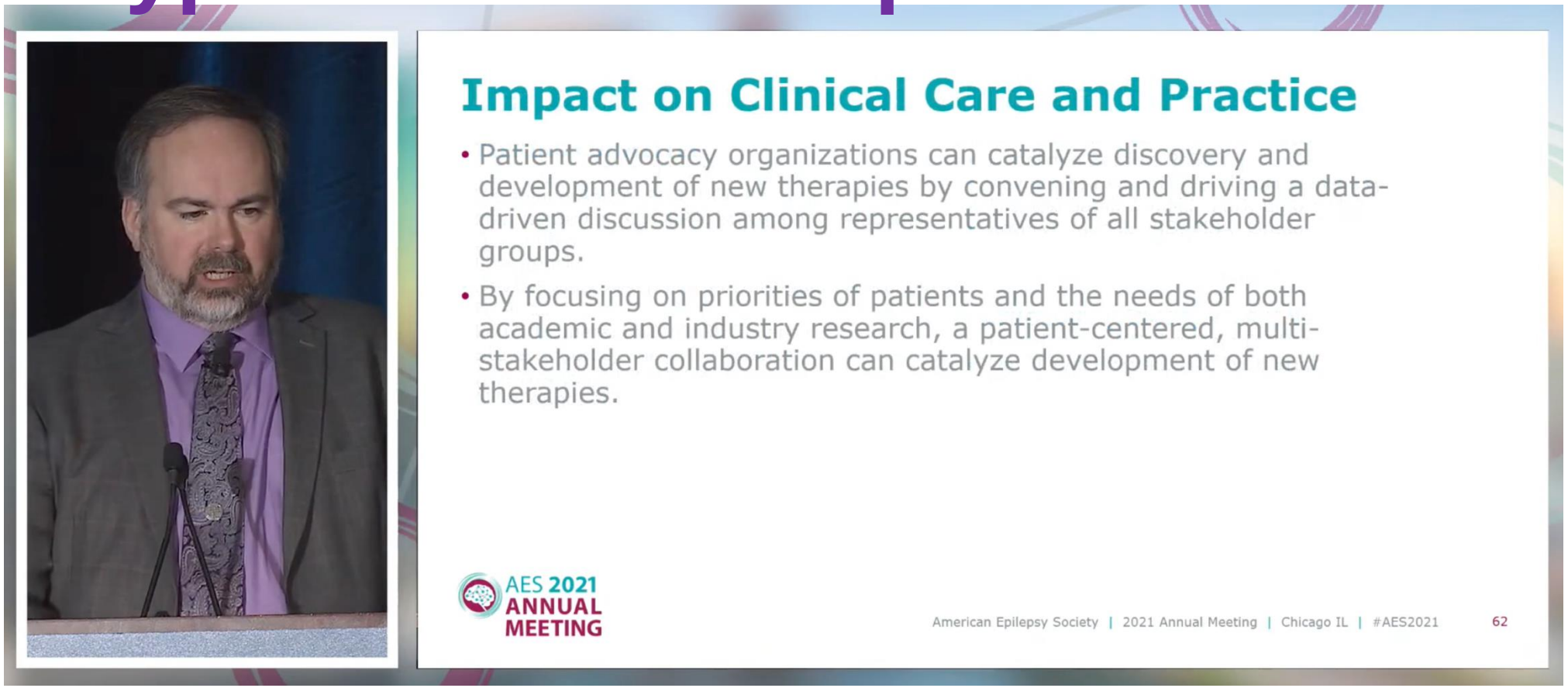


Early diagnosis of Condition X from an MRI by an AI algorithm

Translation-Guided Approach

- Talk to possible industry partners early in development
- Research what the regulatory pathway would be
- Determine if use of the algorithm is billable or would save hospitals money
- Estimate the market size
- Patent the algorithm or ensure that the algorithm does not infringe on existing patents
- Use software libraries licensed for commercial use that would be compatible with the software currently used by commercial partners/hospitals
- Use realistic (non-sanitized) data licensed for commercial use from multiple hospitals

Non-Hypothetical Examples:



Youtube: **2021 American Epilepsy Society Translational Research Symposium: Overcoming Systemic Barriers to Translating Research:**

- Steve Roberds: *Tuberous Sclerosis Complex Alliance*
- Chris Austin: *National Center for Advancing Translational Sciences*
- Elizabeth Donner: *Ontario's Epilepsy Project ECHO*

Presentation Outline

- What is Translational Research?
- Some Key Translational Issues
  - -Commercial Value
  - -Regulatory Pathway
  - -Intellectual Property
- Hypothetical Case Study: *An AI Diagnostic*
- Take Home Points

Take Home Points

1. **When designing research plans, consider key translational issues:**
   - Commercial value
   - Regulatory pathway
   - Intellectual property (IP)
2. **Foster collaboration between researchers, clinicians, patient advocates, & industry**
3. **Have a plan for commercial use of the research:**
   - If no clear plan: freely license research products/data for commercial use (e.g., Creative Commons license)