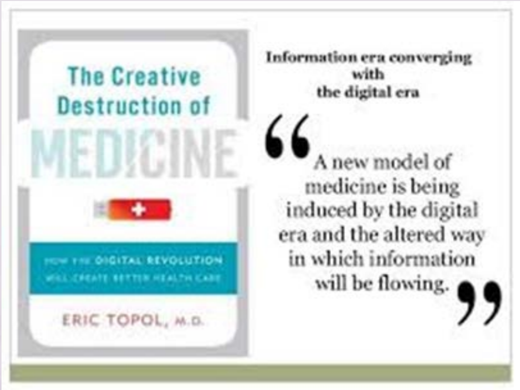
RETHINKING RESEARCH IN A DATA RICH ENVIRONMENT



William Riley, Ph.D.

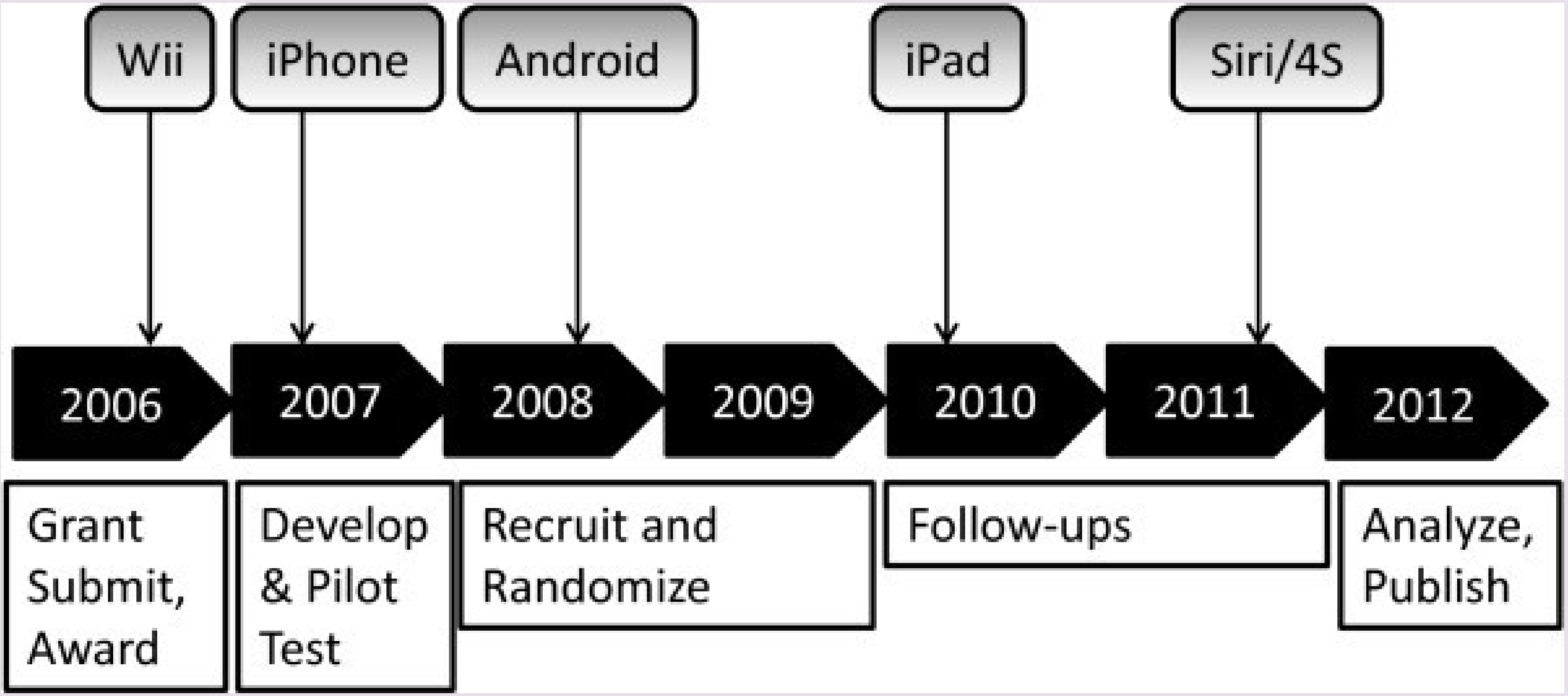
Acting Director, Office of Behavioral and Social Sciences Research
Chief, Science of Research and Technology Branch
National Cancer Institute

OFFICE OF BEHAVIORAL AND SOCIAL SCIENCES RESEARCH
NATIONAL INSTITUTES OF HEALTH

RESEARCH METHODS IN A DATA POOR ENVIRONMENT

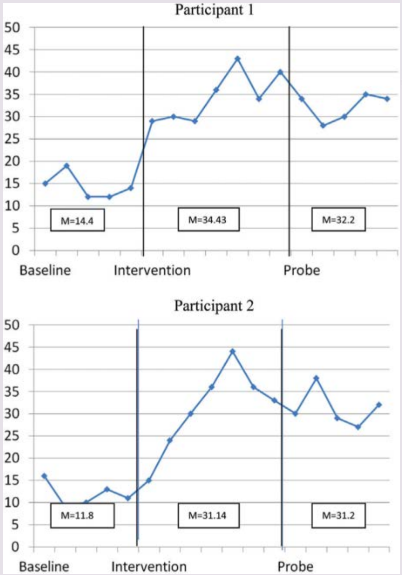
- Limited and costly data collection opportunities
- Focus on prospective design and data collection
- Predominately cross-sectional or minimally longitudinal designs (pre/post/fu)
- Unable to assess or control myriad confounds
- Control by random assignment

TECHNOLOGY OUTPACES RCTS

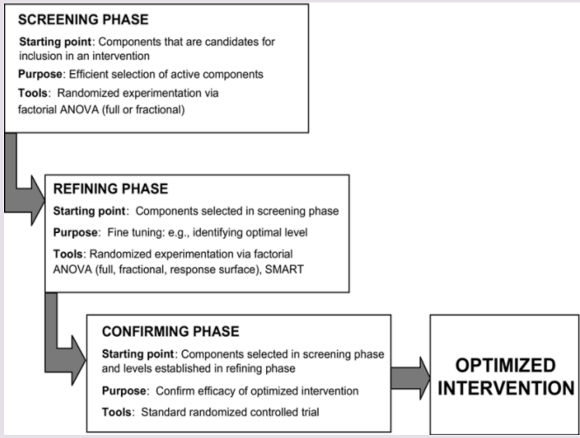


Riley, et al., (2013) Rapid, Responsive, Relevant Research. Clin Transl Med.

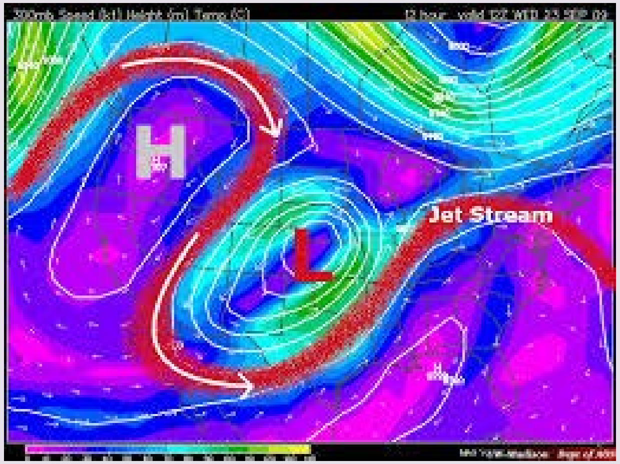
RAPID, RESPONSIVE, RELEVANT (R3) RESEARCH



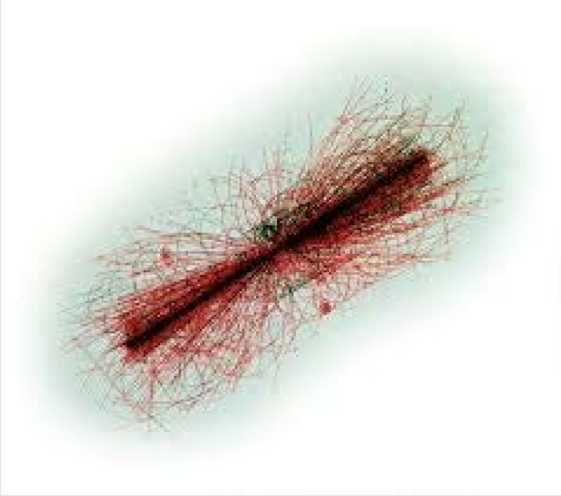
- Stakeholder Relevance
  - Evaluability Assessment (participatory research)
  - Relevant Outcomes
  - Citizen-Scientist Panels (feedback throughout)
- Design Issues
  - Replace pilots with single case and optimization trials
  - Consider within-subject designs and alternatives to two-arm RCT
  - Leverage technology for automated RCTs (A-B testing)
  - Report proximal outcomes
- Review Issues
  - Streamline review process (both grants and articles)
  - Encourage reviewers to consider innovative designs that speed research
  - Streamline IRB, especially for low-risk studies, and rapid modifications
- Infrastructure Issues
  - Data standards and CDEs to facilitate data sharing
  - Rapid learning systems
  - Create national biobank/cohort (Precision Medicine)
  - Use practice networks and EHRs for recruitment and test beds
  - Improved research dissemination systems

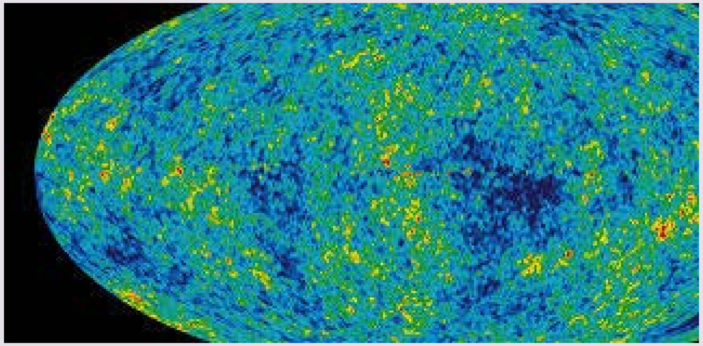


RESEARCH METHODS IN A DATA RICH ENVIRONMENT



- Temporally Dense
- Noisy But Precise
- Computational
- Explanatory and Predictive



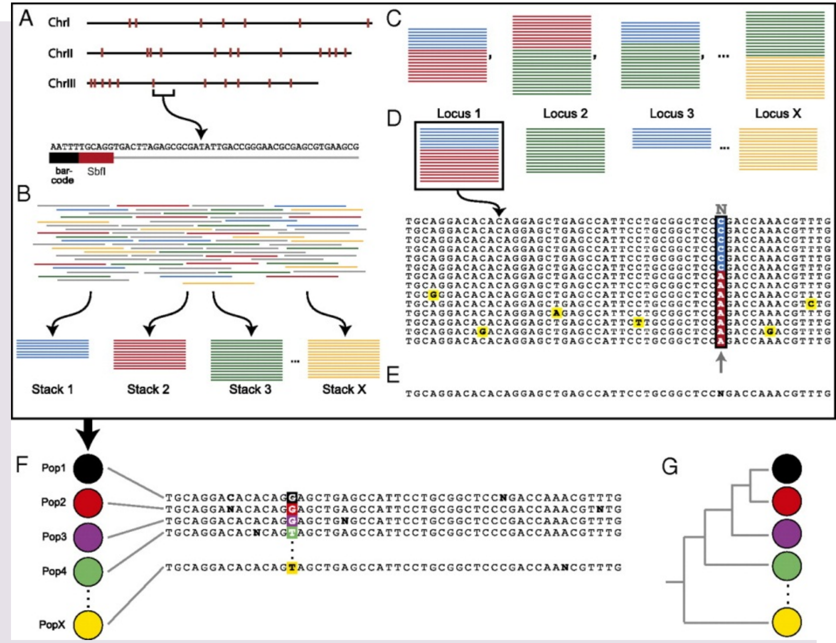


RAPID LEARNING SYSTEMS

- IOM Workshop on "Learning Healthcare Systems" (2006)
  - "structural inability of evidence to keep pace with the need for better information to guide clinical decision making"
- Use EHR practice-based data to answer practice-based scientific questions
- Incorporate rapid RCTs for comparative effectiveness trials
- Large and representative data to predict outcomes
- Automated adverse event surveillance

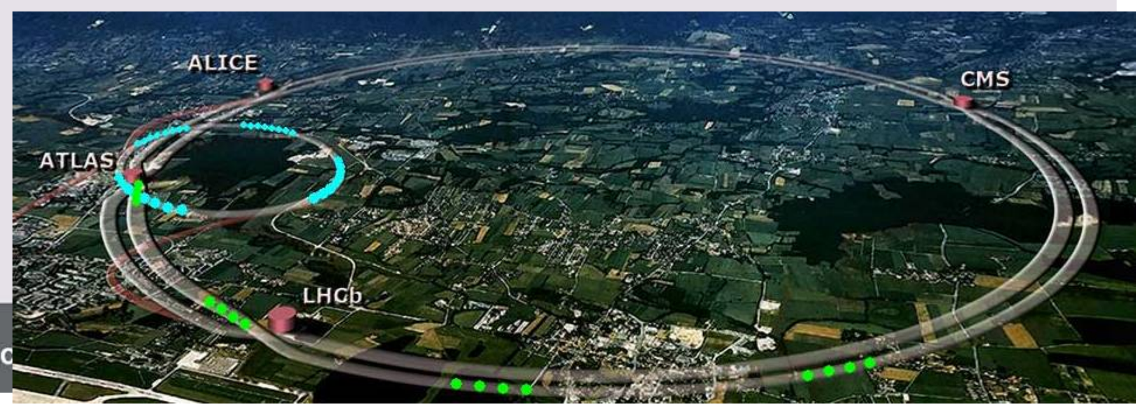
A DATA RICH HEALTH RESEARCH ENVIRONMENT

- What if we built a health monitoring system comparable to the natural science research infrastructures
- What if the data from Electronic Health Records:
  - Documented outcomes as much as it did reimbursable procedures
  - Seamlessly integrated across health systems and platforms
  - Were truly owned and managed by patients (and donated for research purposes)
  - Linked to national health surveys and other relevant databases (e.g., mortality)
  - **Extended beyond the clinical encounter and included or linked to passively sensed health information**



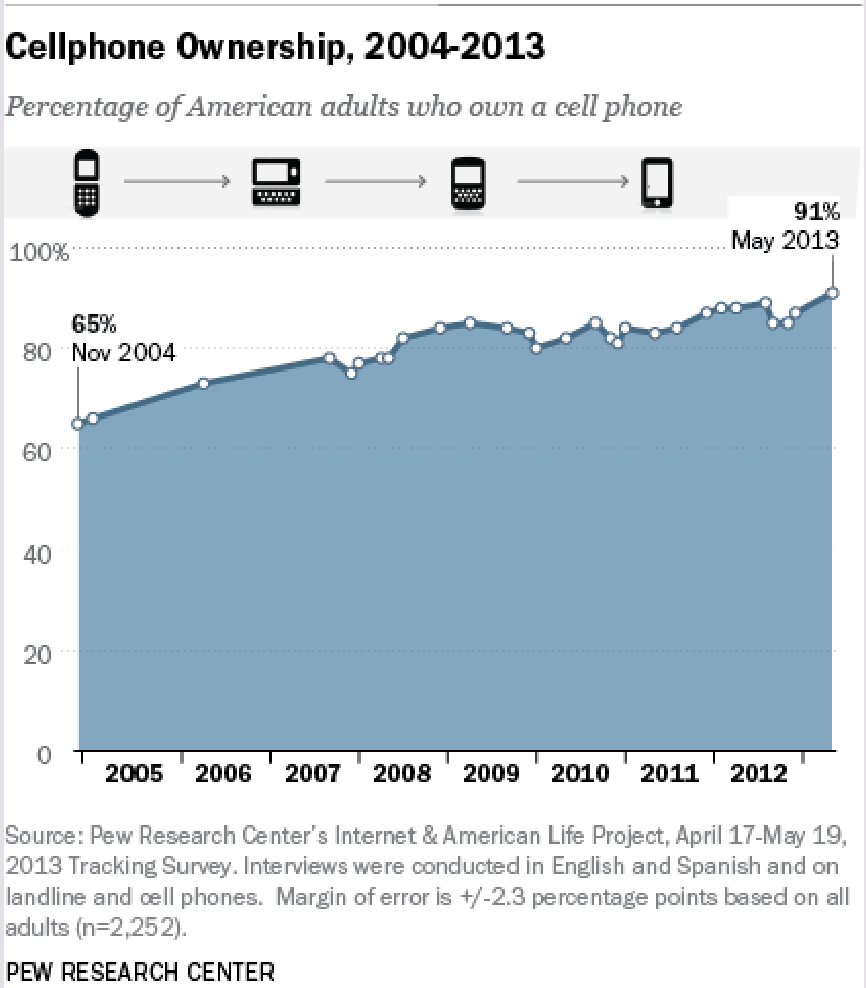
"Nearly all the grandest discoveries of science have been but the rewards of accurate measurement." Lord Kelvin, 1872

E OF BEHAVIORAL AND SOCIAL SCIENC



CURRENT LANDSCAPE

From Topol et al, JAMA 2015



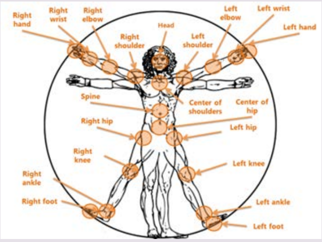
	2010	2015	2020
World Population, billion	6.8	7.2	7.6
No. connected			
Devices, billion	12.5	25	50
Devices, per person	1.8	3.5	6.6
No. of smartphone subscriptions, billion	0.5	3.0	6.1
No. of wireless hotspots, millions	3.0	47	500
No. of transistors, millions/chip, nm	16/40	19/16	22/8
No. of sensors	20 million	10 billion	1 trillion
No. of individuals sequenced	<10	400000	5 million

CURRENT LANDSCAPE

- **World Gone Mobile**
  - Pervasive
  - Rapidly advancing
  - Inter-networked computing and communications environment
  - Extreme power at both the user and cloud level
  - Highly customizable
  - Simple user interfaces and interactions
  - Combined with secure communications enables novel opportunities for engagement and extensive longitudinal data collection.
  - Result is:
    - Entirely New Type of Science
    - Dramatically Lower Measurement Costs and Participant Burden
    - Automated Engagement and Support for Participants

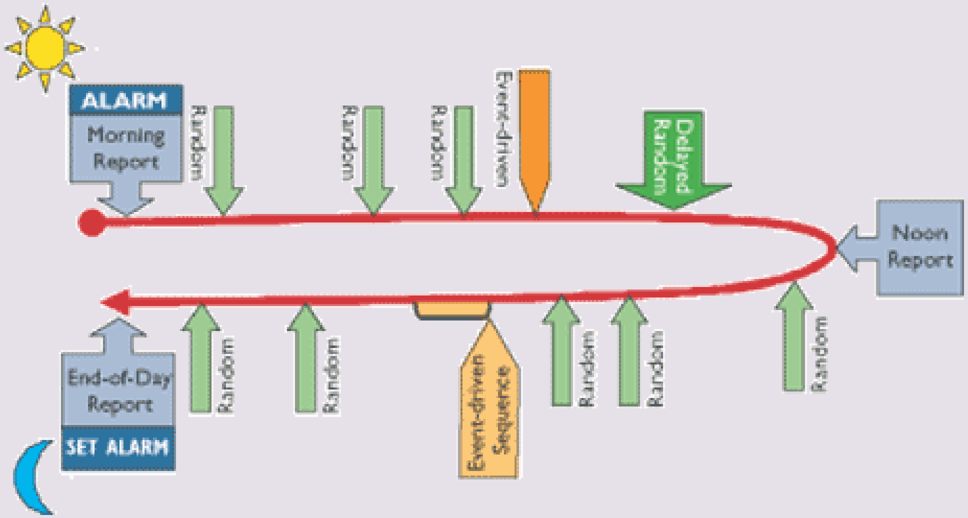
NEW TECHNOLOGIES FOR DATA RICH BIOMEDICAL AND BEHAVIORAL SCIENCE

- Rapidly accelerating technology development
  - Ecological Momentary Assessment (EMA) methods improved and delivered on cell phones
  - Capture of digital traces from daily interactions with technology
    - Social media
    - Call data records
    - Passive sensors
  - Sensors that can passively and continuously monitor health indices, behaviors, and environments in context
    - Environmental sensors
    - Location and movement sensors
    - Substance use sensors
    - Physiological sensors
    - Subcutaneous metabolic sensors



  - Applications of computational modeling and new statistical modeling approaches that provide the analytic capabilities for intensive longitudinal (temporally dense) data.

ECOLOGICAL MOMENTARY ASSESSMENT



Ginexi EM et al. The Promise of Intensive Longitudinal Data Capture for Behavioral Health Research. Nicotine & Tobacco Research 2014 16 (4): S73-S75.

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Dynamic Tools to Measure Health Outcomes from the Patient Perspective

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Researchers

Provides efficient, reliable, and valid assessments of adult and child (pediatric) self-reported health

- Common Questions About PROMIS and Its Instruments
- PROMIS Instruments Selected References
- PROMIS In Research

Clinicians

Provides data about the effect of therapy that cannot be found in traditional clinical measures

- Common Questions About PROMIS and Its Instruments
- PROMIS for Clinicians
- Select Publications
- Computer Adaptive Test (CAT) Demonstration

Patients

Measures what you are able to do and how you feel

- More on PROMIS
- What Patient Reported Outcomes (PROs) Are
- PROMIS Measures

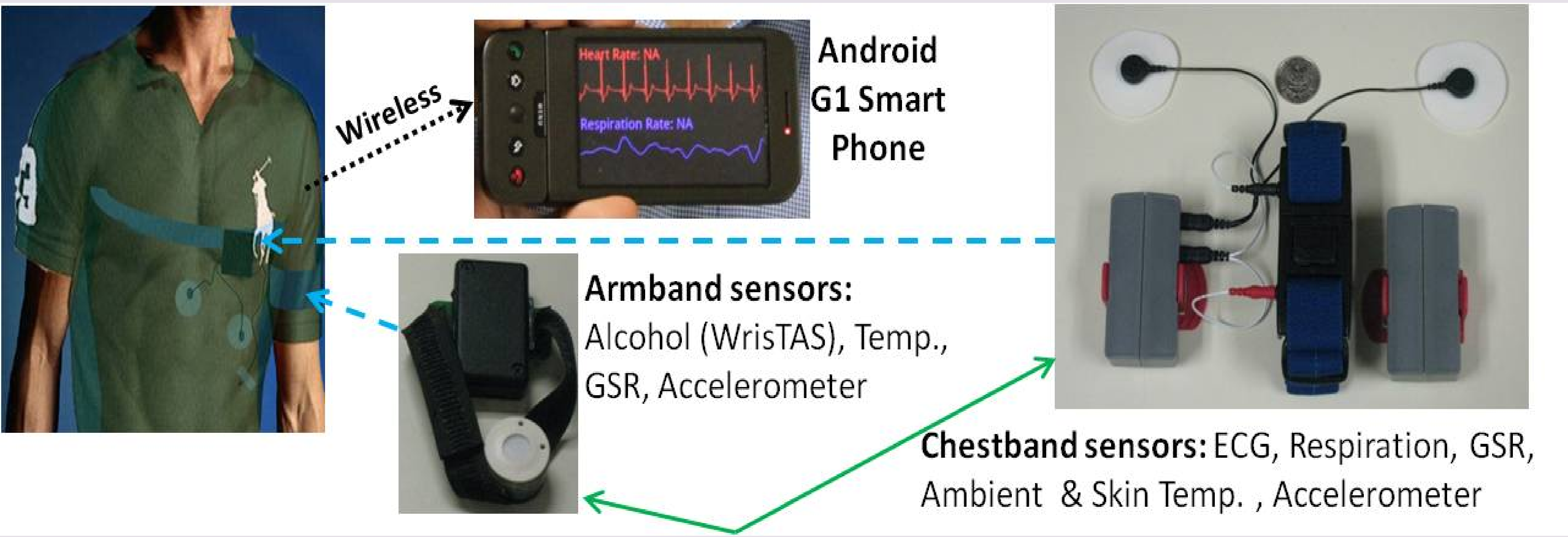
ARCHIVAL BIG DATA SOURCES IN THE BEHAVIORAL SCIENCES “DIGITAL BREADCRUMBS” (PENTLAND, MIT)

- Data Traces gleaned from consumer-based data sources
  - Social Media (Twitter, Facebook)
    - Twitter opens up its 200 million users with 500 million tweets per day to researchers (2/10/2014)
  - Internet Searches (Google)
  - Cell phone Use (# calls and texts)
  - Cable Box Data (hours of TV)
  - Auto Black Box data (miles driven, seat belt use)

Wireless Sensor Possibilities

ration From Wactlar et al., 2011

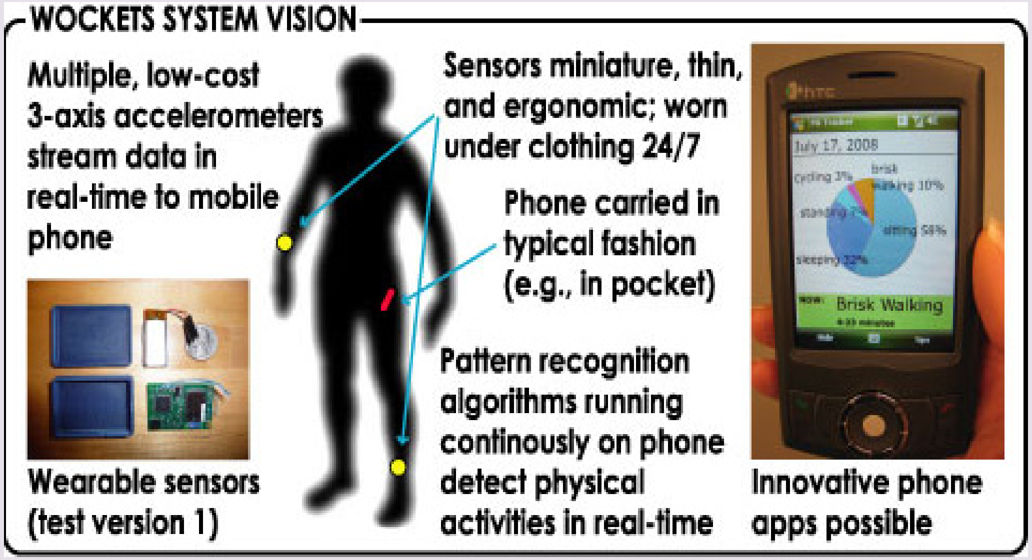
PSYCHOPHYSIOLOGY



Autosense
Santosh Kumar
University of Memphis

POPULATION SCALE ACTIVITY MEASURES

- Population-scale measurement of physical activity
- Miniature, low-cost devices that measure human motion using redesigned accelerometers in a user-friendly format



Stephen Intille, PhD,
Northeastern University
NHLBI, U01HL091737

EMERGING TECHNOLOGIES AND ASSAYS FOR ADHERENCE MONITORING

Xhale SMART "breathalyzer" for GRAS drug taggants

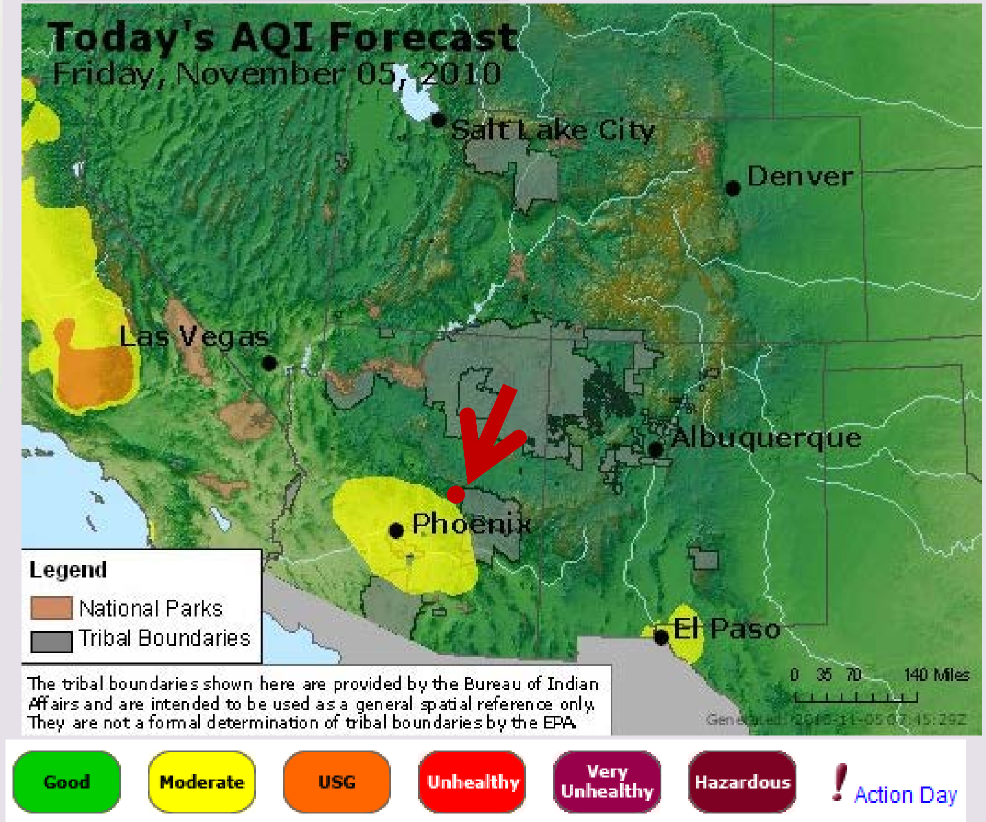
Drug (metabolite) concentrations via hair samples or dried blood spots

Proteus pill microchips and sensor

GlowCaps

WEARABLE CHEMICAL SENSOR SYSTEM

http://www.airnow.gov

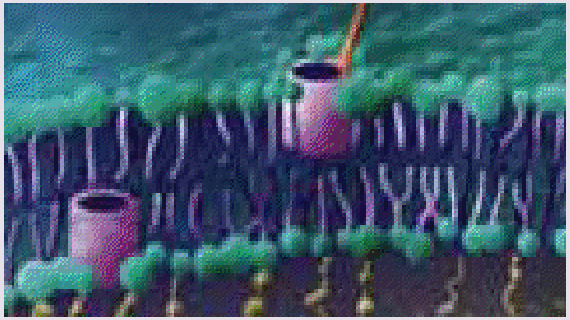


- Chemical exposure varies by context, need personal exposure
- Selective detection of VOCs (hydrocarbon and acid vapors)
  - Sensitive: ppb – ppm
  - Real-time: sec. – min.
  - Spatially resolved
  - Wearable: cell phone size
  - Cell phone based interface

Nongjian Tao, Arizona State University, NIEHS, U01 ES016064

IMPLANTABLE BIOSENSORS

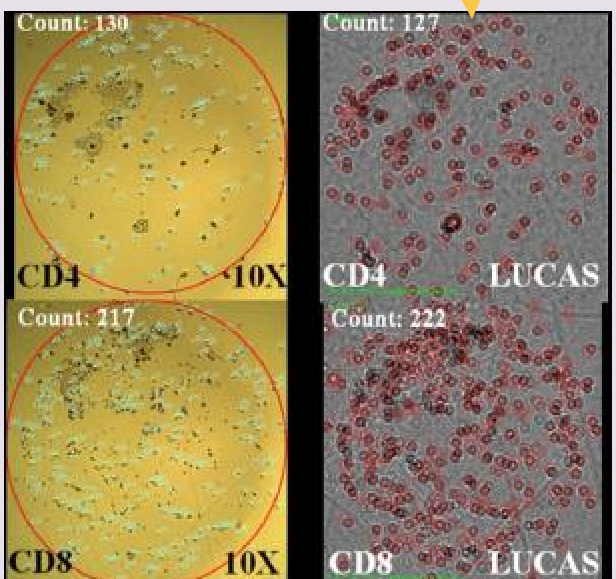
- Measurement of analytes (glucose, lactate O2 and CO2) that indicate metabolic abnormalities
- Miniaturized wireless implantable biosensor that continuously monitors metabolism
  - Inserted by needle subcutaneously
  - Operated remotely using a PDA
  - Multi-analyte sensor
  - One month continuous monitoring

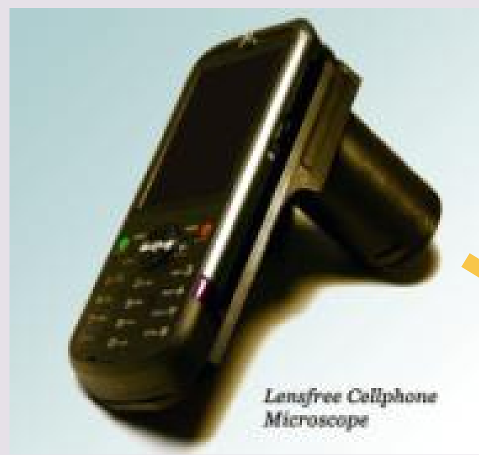


Diane J. Burgess, University of Connecticut NHLBI, R21HL090458

LUCAS- MOBILE MICROSCOPE

Problem: Create a low-cost quality microscope to use in low resources settings.
Solution: A specially-developed lens fits to a cell phone to create a microscope
Field testing: Malawi, Mozambique and Brazil





Cell phone transmits image

Co
au
int
remote site

Karin Nielsen, UCLA, FIC, R24TW008811

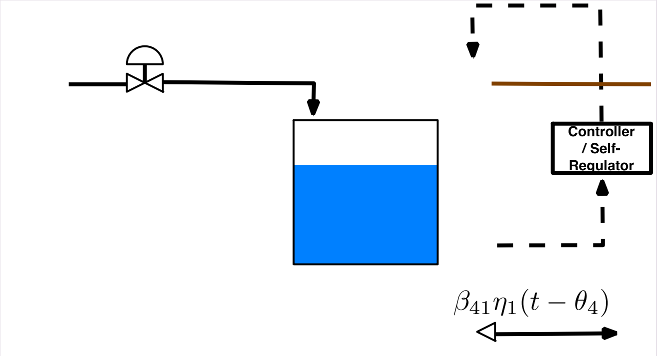
HAMMERS LOOKING FOR NAILS

Can We Use these Mobile Health Technologies to:

- Better Characterize Phenotypes (e.g., augment or extend EHR)
- Better Characterize Outcomes (e.g., patterns of response)
- Better Characterize Treatments (e.g., adherence)
- Extend Treatment Predictors beyond Genomics (e.g. physiological variability)
- More Precisely and Intensively (and with less respondent burden) Characterize Behavioral Risk Factors and Environmental Exposures that Interact with (or overwhelm) Genetics
- Fully Engage Participants as Partners (data control and feedback)

NEW ANALYTIC APPROACHES

- Temporally Dense, Intensive Longitudinal Data
- Greater reliance of statistical approaches that embrace variability over time
  - Latent Curve Models
  - Time Varying Effect Models
- Big Data Approaches
- Shift to Computational Dynamic Modeling



QUESTIONS?