5.1 Overview of Biomedical Research

What is Biomedical and Health Informatics? - http://informatics.health/
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Biomedical research – who and where?

- Academic medical centers – researchers, usually faculty, at universities and other medical centers
- Government research laboratories
- Private industry – usually focused on product research and development
- Others – growing involvement and leadership by patient groups and others, facilitated by the Internet and social media, e.g., PatientsLikeMe

“Basic” vs. “clinical” science and other distinctions and terminology

- Historical distinction of
  - Biological vs. clinical
  - “Wet” vs. “dry” lab
- Organizing principle of schools of medicine
  - Basic science departments mostly PhDs vs. clinical departments mostly MDs
- Other important terminology
  - Biobanks (Annaratone, 2021)
  - Registries (Labkoff, 2024)
  - Team science (Cline, 2020) – sometimes at odds with academic promotion pathway
  - Implementation science (Rubin, 2023)
- Many roles for informatics to fit in

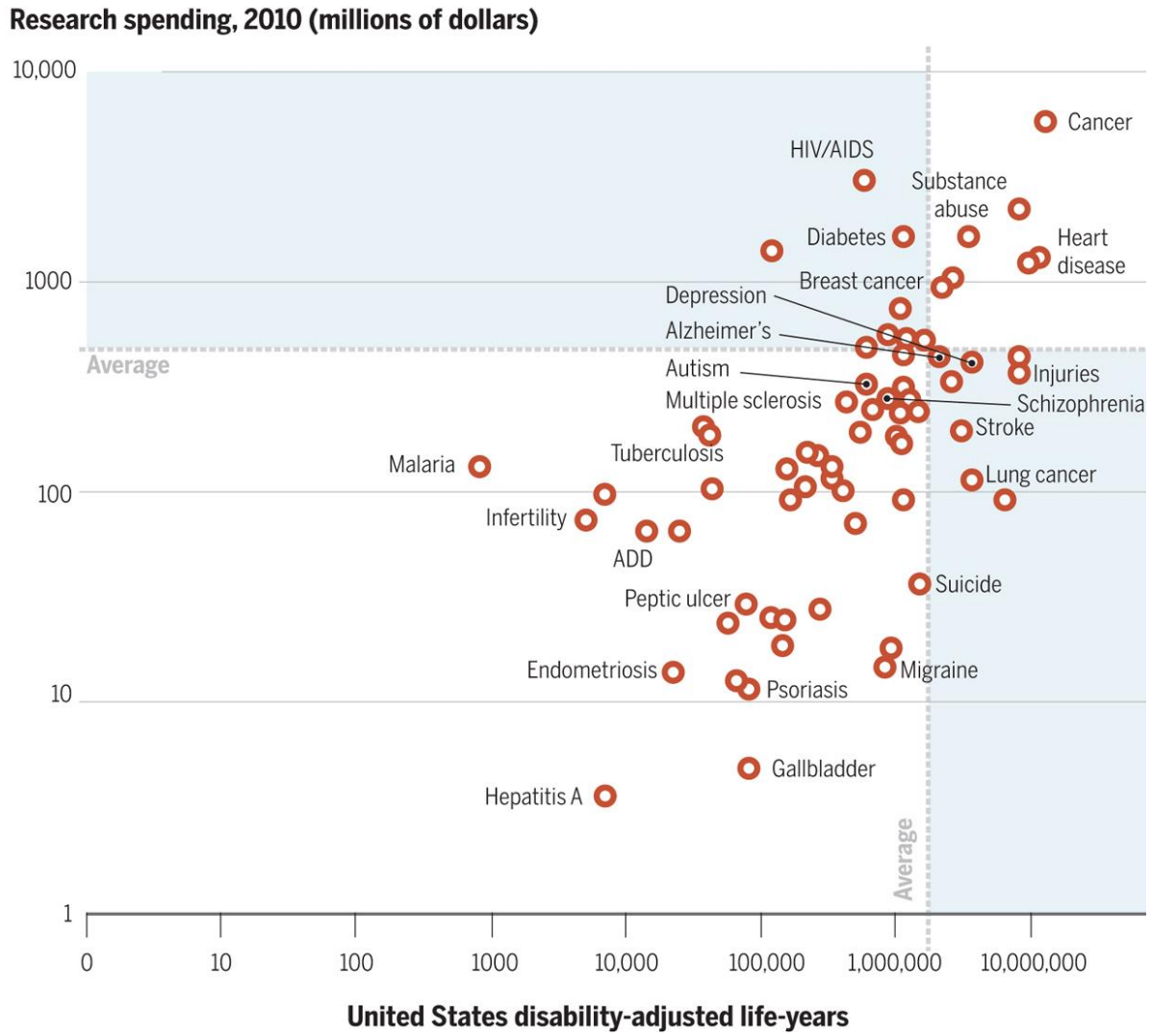
Biomedical research funding – mostly government

- In US, Department of Health & Human Services (HHS) includes (among others)
  - National Institutes of Health (NIH)
  - Agency for Healthcare Research & Quality (AHRQ)
  - Centers for Disease Control and Prevention (CDC)
- Other countries, especially wealthier ones, have government funding as well, e.g.,
  - United Kingdom Medical Research Council (MRC)
  - Canadian Institutes of Health Research (CIHR)
  - Singapore National Medical Research Council (NMRC)
  - South Africa Medical Research Council (SAMRC)

National Institutes of Health (NIH)

- Annual budget in 2024 was ~$48 billion, ~83% of which goes to fund extramural research
  - Organization and budget
- Consists of 27 institutes and centers (ICs)
  - Most of which are disease-focused, e.g.,
    - National Cancer Institute (NCI)
    - National Heart, Lung, and Blood Institute (NHLBI)
  - But some of which are more generic, e.g.,
    - National Library of Medicine (NLM) – 175 years of contributions (Slomski, 2011, Brennan, 2016; Humphreys, 2021)
    - National Institute of Biomedical Imaging and Bioengineering (NIBIB)

NIH spending on diseases vs. burden (Kaiser, 2015)



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Non-governmental research organizations

- Quasi-public
  - Patient-Centered Outcomes Research Institute (PCORI)
    - Created under Affordable Care Act
    - Funded by tax on health insurance plans (public and private)
    - Funded for $3.5B through 2019, extended to 2029
- Private non-profit – many organizations; some larger ones include
  - Robert Wood Johnson Foundation
  - Howard Hughes Medical Institute
  - American Cancer Society
  - American Heart Association
  - In UK, Wellcome Trust

Some NIH initiatives especially pertinent to informatics

- Common Fund
  - Evolution of NIH Roadmap to "identify major opportunities and gaps in biomedical research that no single institute at NIH could tackle alone but that the agency as a whole must address"
- AllOfUs (formerly, Precision Medicine Initiative)
  - (Collins, 2015; Denny, 2019; Mayo, 2023)
  - Approach to disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle
  - Goal to create research cohort of >1M American volunteers who will share genetic data, biological samples, and diet/lifestyle information, all linked to their EHRs

Another NIH initiative – Clinical and Translational Science Award (CTSA)

- From NIH National Center for Advancing Translational Science (NCATS)
- Goal: accelerate translation of research into clinical care and community
- About 60 centers funded around US to facilitate clinical and translational research
- Informatics important?
  - Centers required to have a biomedical informatics component
  - Funded Clinical Data to Health (CD2H) that coalesced efforts around National COVID Cohort Collaborative (N3C; Haendel, 2021)

National Patient-Centered Clinical Research Network (PCORnet)

- (Fleurence, 2014; Collins, 2014)
- Started with 13 Clinical Data Research Networks (CDRNs) involving two or more health systems and functioning as integrated research networks; data to be accessible by other researchers
- Consolidated into 9 clinical research networks and 2 health plan research networks (Forrest, 2021) – about and data

Other initiatives

- Sentinel Initiative – US FDA effort to track safety of drugs, biologics, and medical devices
  - Outgrowth was Observational Medical Outcomes Partnership (OMOP), which developed widely used data model now under purview of Observational Health Data Sciences and Informatics (ODHSI) (Hripcsak, 2015)
- Million Veteran Program (MVP) – aims to collect blood samples and clinical data from 1M US veterans (Gaziano, 2016)
- UK Biobank (Bycroft, 2018; Joseph, 2023) collecting clinical and biological data on over half-million citizens since 2006

Other initiatives (cont.)

- Health and Human Heredity in Africa (H3Africa) – NIH- and Wellcome-funded project to advance genomic and clinical science in Africa (Mulder, 2018)
  - Aimed to overcome "helicopter science" (Moodley, 2022)
- H3Africa Bioinformatics Support Network (H3ABionet) – bioinformatics support network for H3Africa (Mulder, 2017)
- Data Science Initiative for Africa (DSI Africa) – NIH-funded project to build human and data capacity in Africa
  - Includes OHSU collaboration with University of Cape Town to develop new graduate program

Other initiatives (cont.)

- 21st Century Cures Act (Hudson, 2017; Kesselheim, 2017)
  - Passed by Congress in late 2016 by large bipartisan majorities
  - Provided additional funding for
    - Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative
    - Precision Medicine Initiative (PMI) – now AllOfUs
    - Cancer Moonshot
    - Regenerative medicine
- Advanced Research Projects Agency for Health (ARPA-H; Collins, 2021)
  - Supporting transformative high-risk, high-reward research to drive biomedical and health breakthroughs – ranging from molecular to societal to provide transformative solutions for all patients

Benefits from research

- NIH and other government research provides important basic research that industry does not fund
  - Of 26 “transformative” drugs approved between 1984-2009, most were developed by academic researchers funded by NIH (Kesselheim, 2015)
  - 354 of 356 (99.4%) drugs approved by FDA between 2010-2016 received some NIH funding; over 90% of funding represented basic research related to biological targets for drug action (Galkina Cleary, 2018)
  - Economic return on investment – each $1 spent generates $2.56 in new economic activity (United for Medical Research, 2025)
- Cancer death rates continue to decline (Sherman, 2025)

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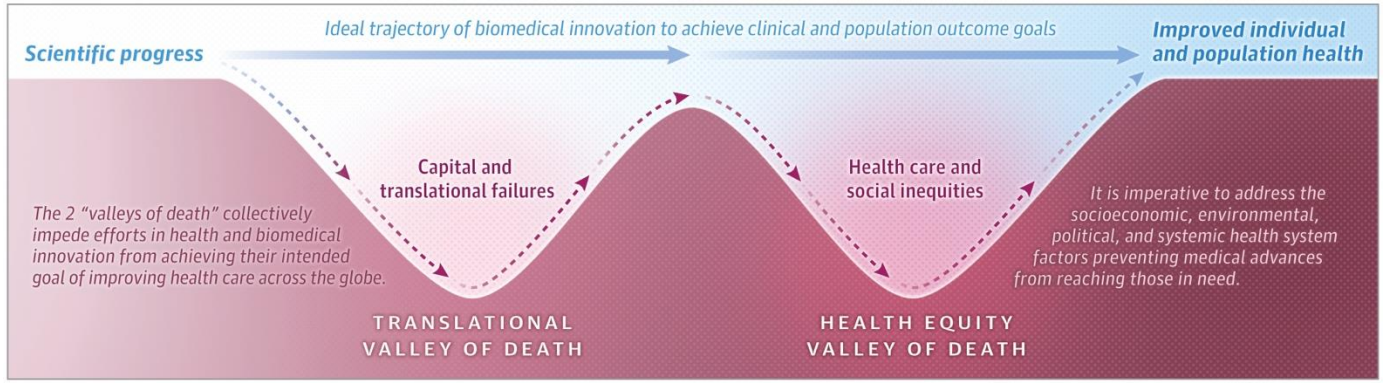
Challenges for research

- "Chasms" for research – translational and equity (Dzau, 2024)
- Reproducibility and replicability (NAP, 2019)
  - Opportunities for EHR data to replicate clinical studies (Wang, 2022)
- Need for meta-research (Ioannidis, 2024)?



		Data: Same	Data: Different
Code: Same		Reproducible	Replicable
Code: Different		Robust	Generalisable

(Adler-Wagstyl, 2018)

Challenges (cont.)

- Career support for scientists (Daniels, 2018), especially clinician scientists (Utz, 2022; O'Rahilly, 2023); solutions include (NAP, 2018)
  - Create predictable and sustainable funding
  - Broaden career paths for trainees
  - Mentoring by established scientists
- National Academy of Medicine (NAM, 2024) advocated strategies for addressing challenges
  - Strategic vision
  - Funding
  - Health equity
  - Coordination and convergence science
  - Workforce development
- What portends future of research support and funding (Walensky, 2025; Laine, 2025; Altmann, 2025)?