

The Changing Nature of Healthcare R&D

Bryn Roberts
Global Head of Data & Analytics
Roche Information Solutions

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Agenda

1. Healthcare Context
2. Transformation in Healthcare R&D
3. Resolution and Relevance for Disease Management
4. Concluding Remarks and Discussion

Healthcare Context

- Challenges facing healthcare systems
- Healthcare 5.0 & Digital Transformation

Health- and social-care systems are breaking!

Major challenges facing healthcare systems around the world

Healthcare costs



Aging population



Access and equity



Value-based care



Patient experience & engagement



Staffing shortages & burnout



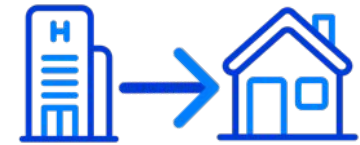
Pandemic preparedness & prevention



Health & Social Care coordination



Non-traditional settings



Health technology implementation



Data silos



Cybersecurity threats



Aging populations

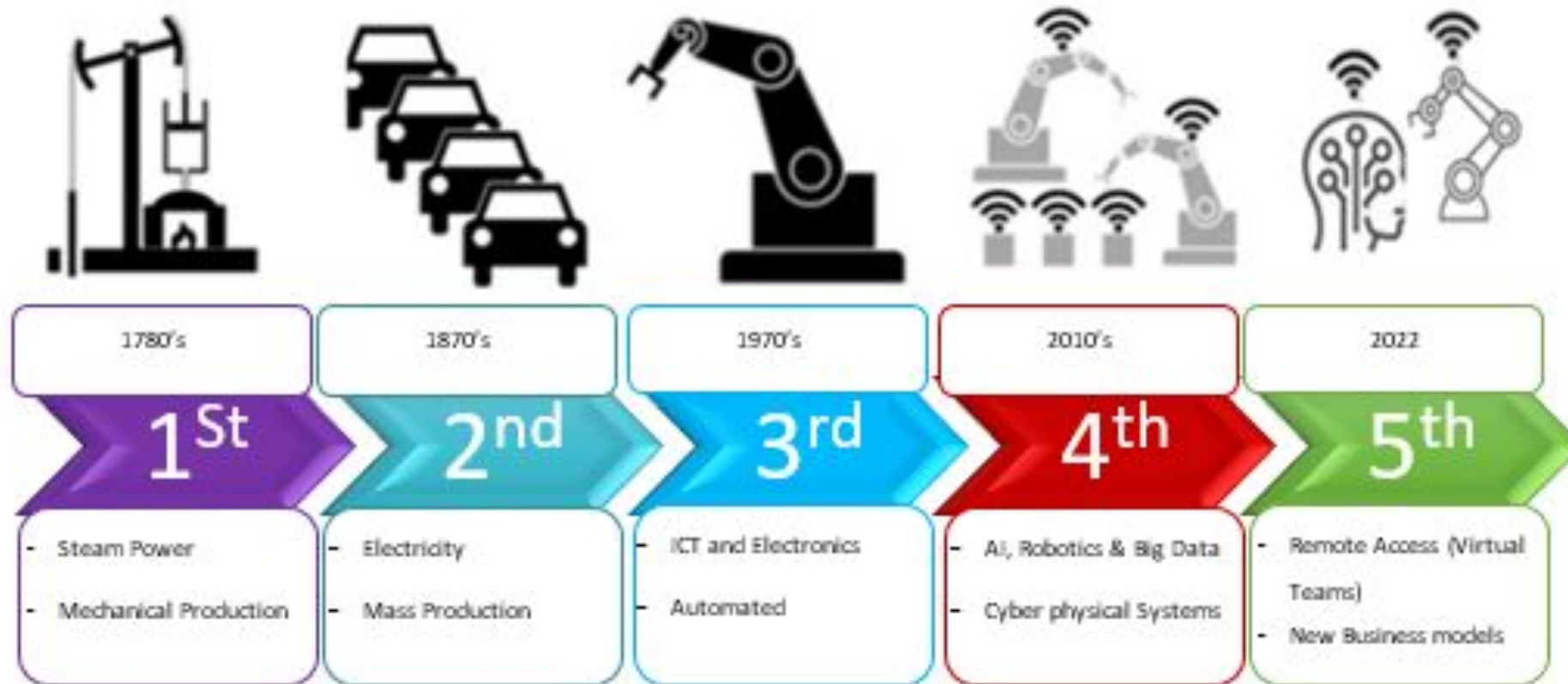
An insurmountable challenge under current paradigms



- Average life expectancy is 73 - the average healthy life expectancy is only 63
- The over 80s are set to triple by 2050
- Preventative measures are urgently needed to ease the pressure on health and social care systems

Industry 5.0

The context for digital transformation



<https://hbatsh.medium.com/the-next-industrial-revolution-is-comin-742325c71409>

1780'
S

10

1870'
S

10

1970'
S

4

2010'
S

1

2020'
S

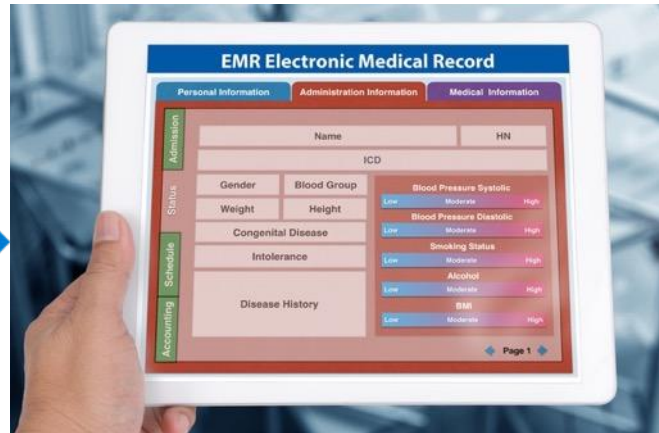
Healthcare 5.0?

A new age of healthcare



Digital Transformation

Foundations for data-driven activities and organisations



Digitisation

Converting data from analogue to digital form

Digitalisation

Leveraging digital technologies to create added value in processes, decision-making, communication, etc.

AI applications are rapidly expanding in healthcare

Latest newsflows demonstrate progress many diseases and settings

Medical News Today

Type 2 diabetes: AI model predicts onset within 12 hours

Researchers recently created an artificial intelligence model that predicts diabetes onset with 12 hours of blood glucose data collected...



The New York Times

Doctors Are Using ChatGPT to Improve How They Talk to Patients

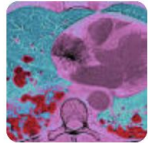
Despite the drawbacks of turning to artificial intelligence in medicine, some physicians find that ChatGPT improves their ability to...



NBC News

Promising new AI can detect early signs of lung cancer that doctors can't see

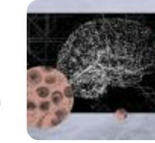
Researchers in Boston are on the verge of what they say is a major advancement in lung cancer screening: Artificial intelligence that can...



Medical News Today

Parkinson's: AI tool may spot signs in blood 15 years early

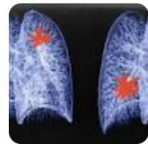
A new AI tool may be able to detect signs of Parkinson's disease in blood samples up to 15 years before the onset of symptoms,...



Medical News Today

Lung cancer: AI tool Sybil may be able to predict risk within 6 years

A new AI tool named Sybil may be able to predict lung cancer risk 1-6 years after screening with one low-chest computed tomography scan,



The Wall Street Journal

Generative AI Makes Headway in Healthcare - WSJ

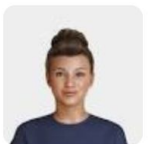
The University of Pittsburgh Medical Center plans to roll out a generative artificial intelligence-based tool from Abridge AI that helps...



World Health Organization (WHO)

WHO and partners launch world's most extensive freely accessible AI health worker

The World Health Organization, with support from the Qatar Ministry of Health, today launched the A.I.-powered WHO Digital Health Worker,...



Florence 2.0

Medical News Today

Stroke: Researchers use AI model to predict a person's 10-year risk

In a new study, researchers used artificial intelligence (AI) and a single chest X-ray to predict a person's 10-year risk of dying from a...

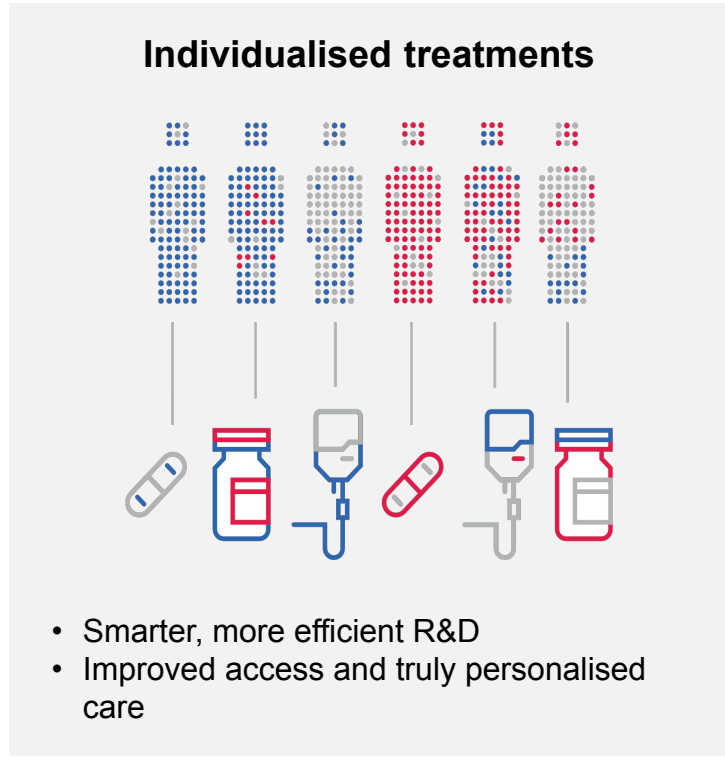
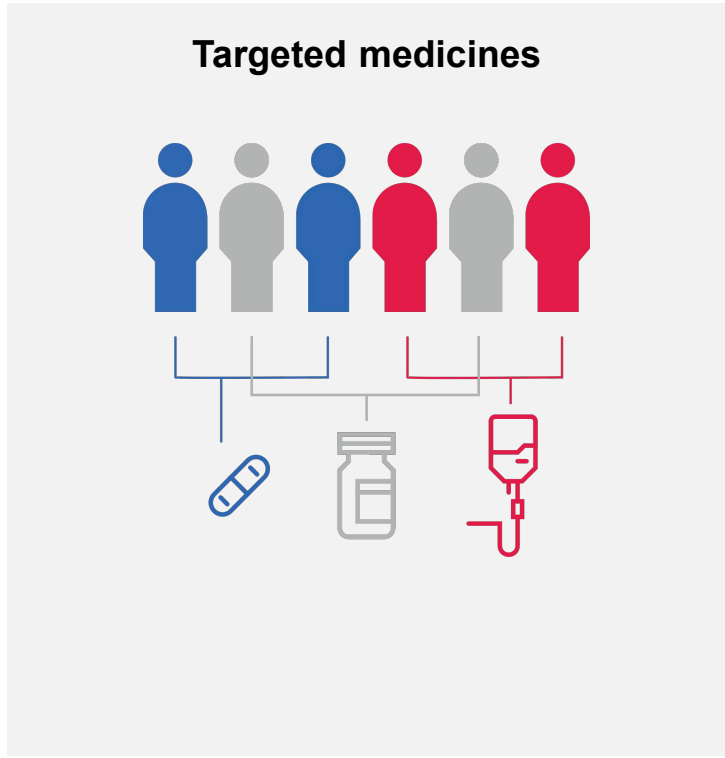
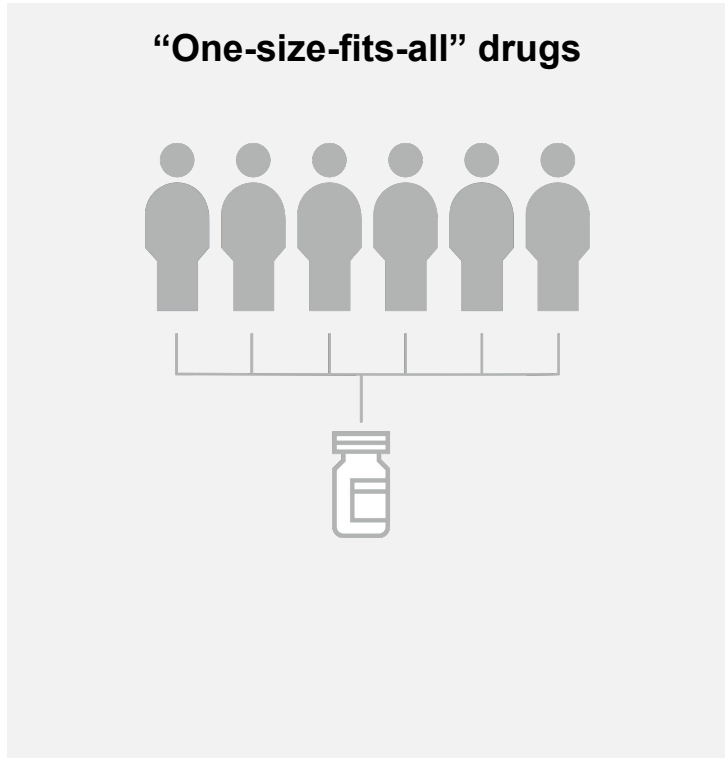


Transformation in Healthcare R&D

- Personalised healthcare
- Scientific and technological transformation
- R&D productivity

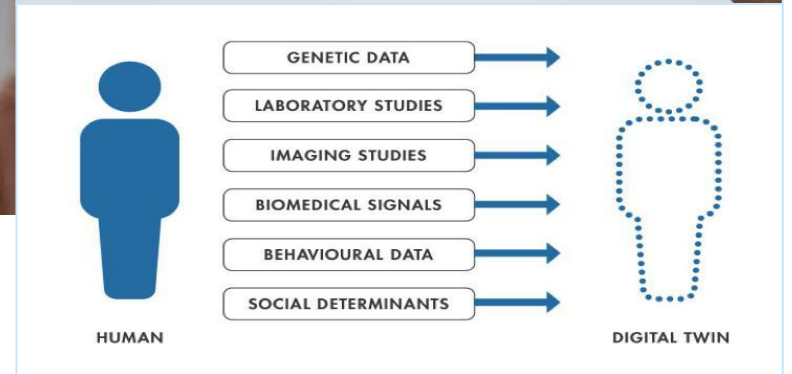
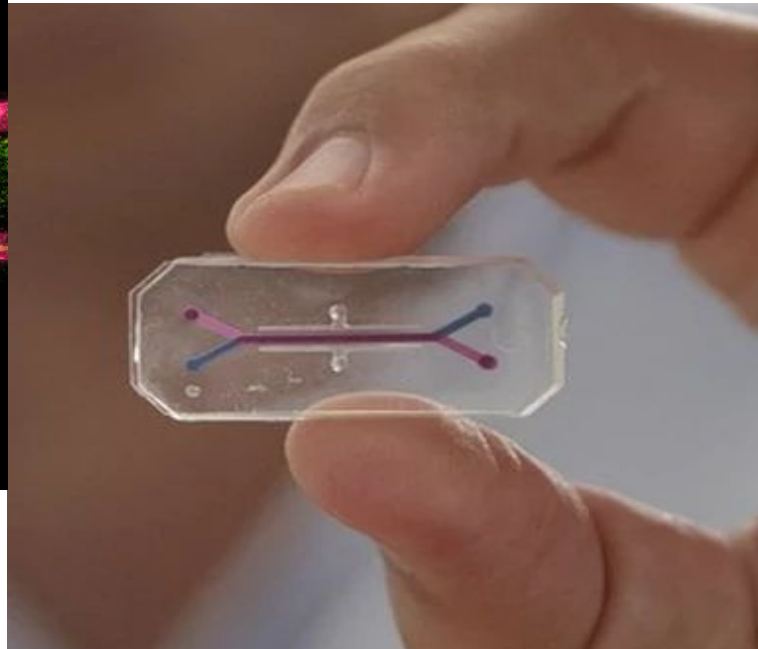
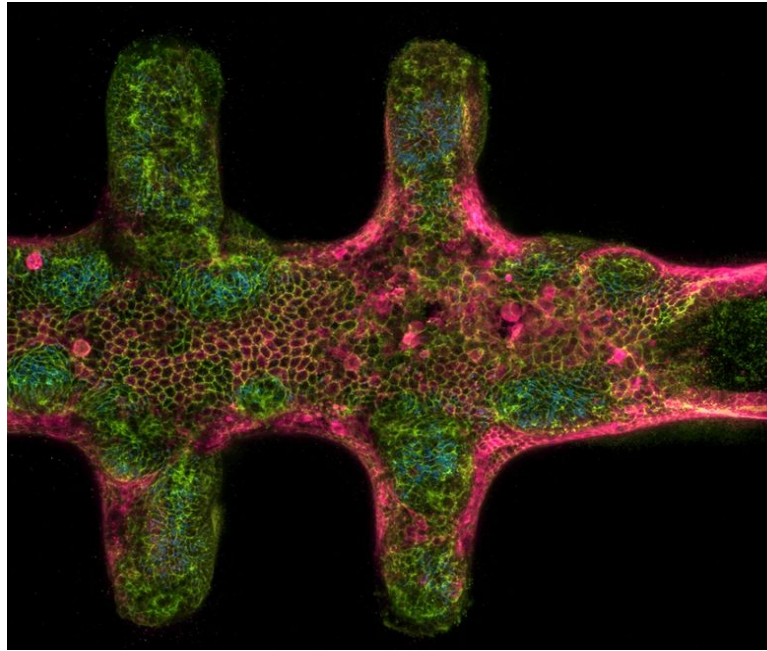
Personalised healthcare

Data drive our understanding of the individual – genotypic and phenotypic



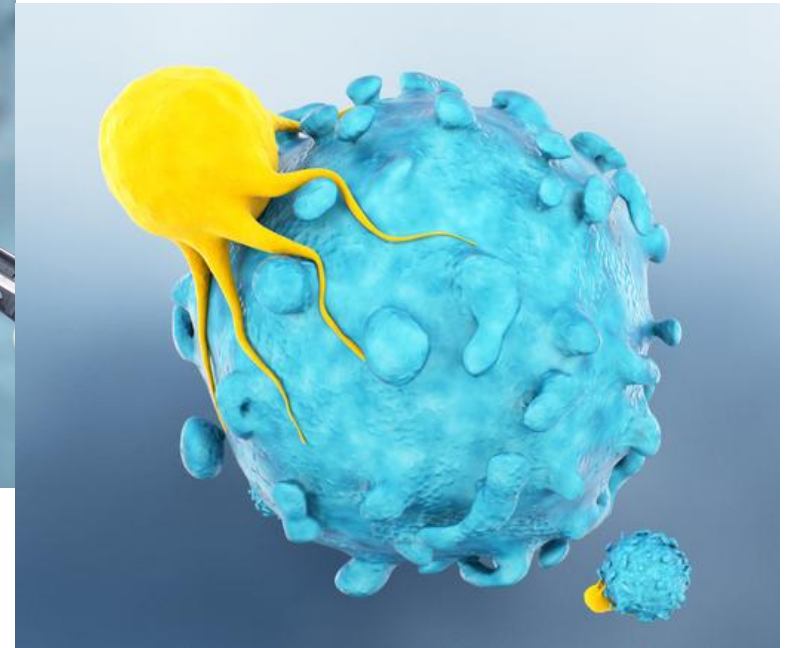
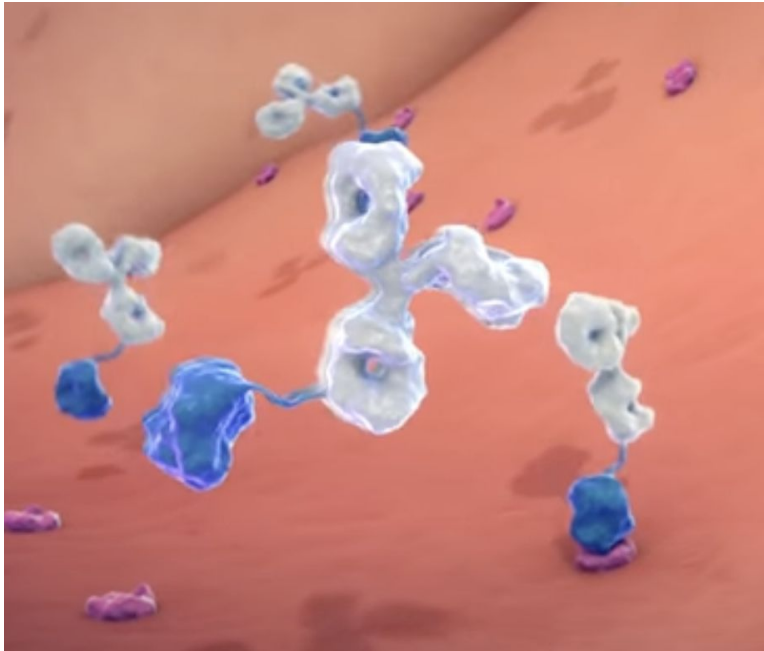
Human model systems and translational bioengineering

Organoids, human-on-a-chip, digital twins, etc.



New and more exotic modalities

Advanced antibody formats, oligos, gene and cell therapies, mRNA vaccines, etc.



Laboratory digitalisation and automation

Augmenting quality, productivity, SHE, compliance, etc.



Pharma R&D productivity challenge

Despite the progress in science and technology, industry productivity continues to be a major challenge

Drug Discovery Today • Volume 28, Number 10 • October 2023

FEATURE



Analysis of pharma R&D productivity – a new perspective needed

Alexander Schuhmacher^{1,2,*}, Markus Hinder³, Alexander von Stegmann und Stein², Dominik Hartl^{4,5}, Oliver Gassmann²

¹ Technische Hochschule Ingolstadt, THI Business School, Esplanade 10, DE-85049 Ingolstadt, Germany

² University of St. Gallen, Institute of Technology Management, Dufourstrasse 40a, CH-9000 St. Gallen, Switzerland

³ Novartis, Global Drug Development Patient Safety, Forum 1, CH-4002 Basel, Switzerland

⁴ University of Tübingen, Hoppe-Seyley-Strasse 1, 72076 Tübingen, Germany

⁵ Granite Bio, Aeschenvorstadt 36, 4051 Basel, Switzerland

R&D productivity continues to be the industry's grand challenge. We analyzed the R&D input, output, and outcome of 16 leading research-based pharmaceutical companies over 20 years (2001–2020). Our analysis shows that pharma companies increased their R&D spending at a compound annual growth rate of 6% (2001–2020) to an average R&D expenditure per company of \$6.7 billion (2020). The companies in our investigation launched 251 new drugs representing 46% of all CDER-related FDA approvals in the past 20 years. The average R&D efficiency of big pharma was \$6.16 billion total R&D expenditures per new drug. Almost half of the leading companies needed to compensate for their negative R&D productivity through mergers and acquisitions.

On average, the big pharma companies in this sample, during the period 2001-2020:

- Spent **\$4.4bn per year** on R&D
- Launched **0.78 new drugs per year**
- Had an R&D spend **CAGR of 6%**

And... almost half of the 16 leading companies had a **negative return** on their R&D spend

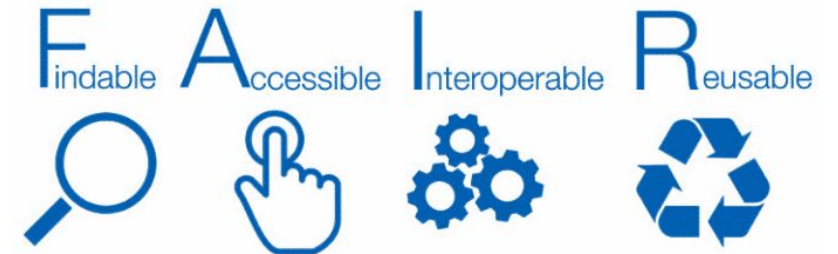
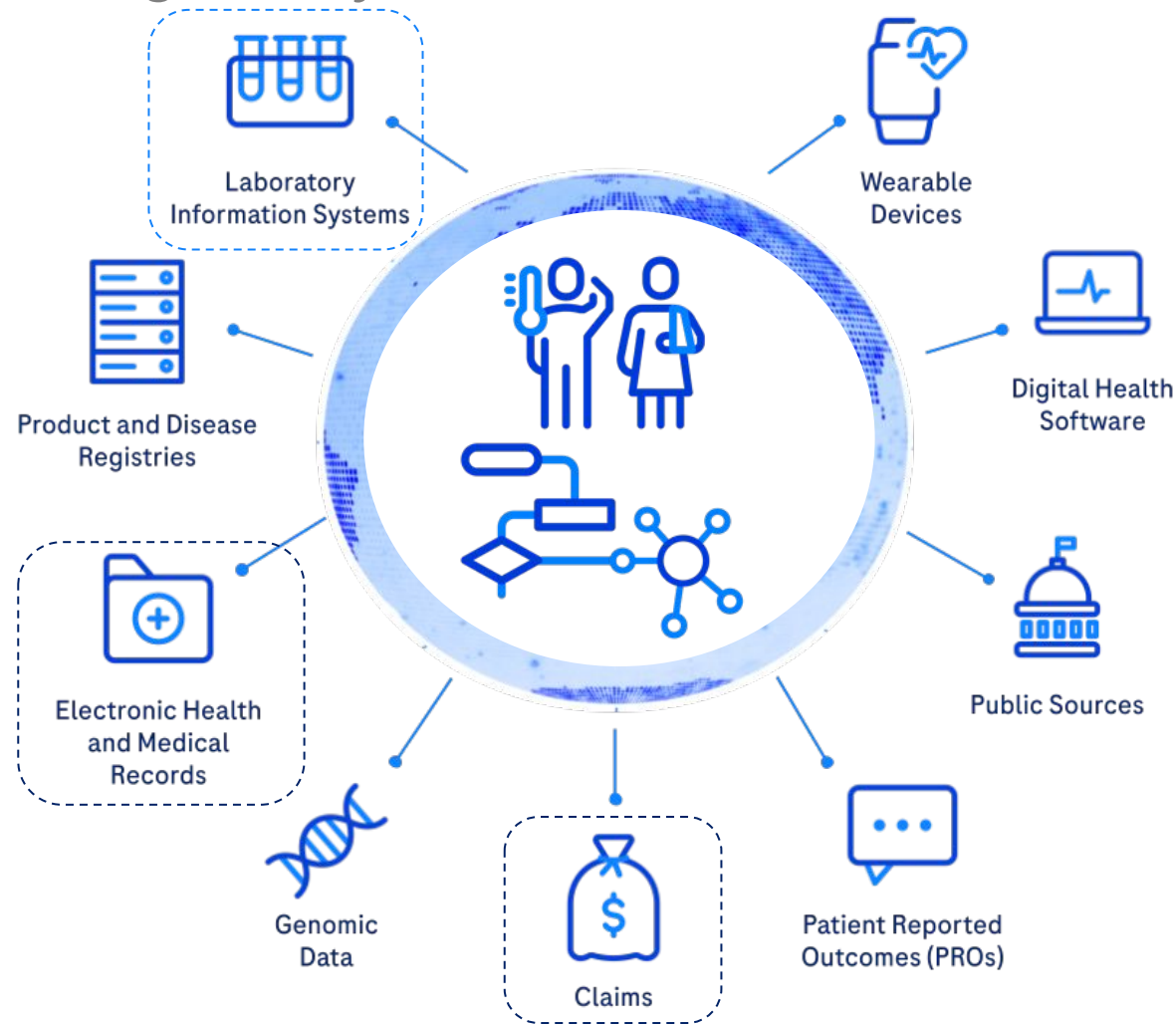
FEATURE

Resolution and Relevance for Disease Management

- Multimodal real world data
- Longitudinal patient records
- Remote patient monitoring and digital biomarkers

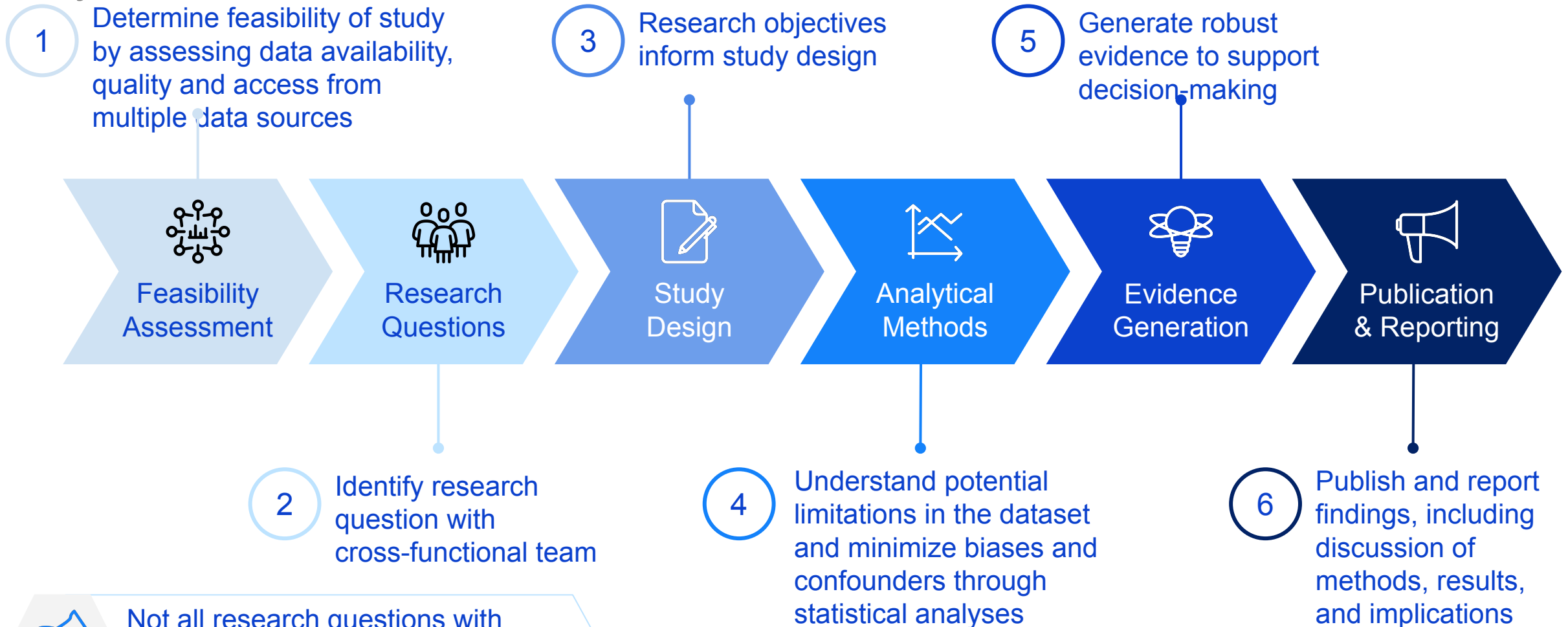
There are many different types of RWD sources

New sources are emerging continuously to strengthen evidence generation in R&D and regulatory



Conducting a RWD study involves scientific rigor at every step

Maintaining robust methodology and promoting transparency/traceability are key

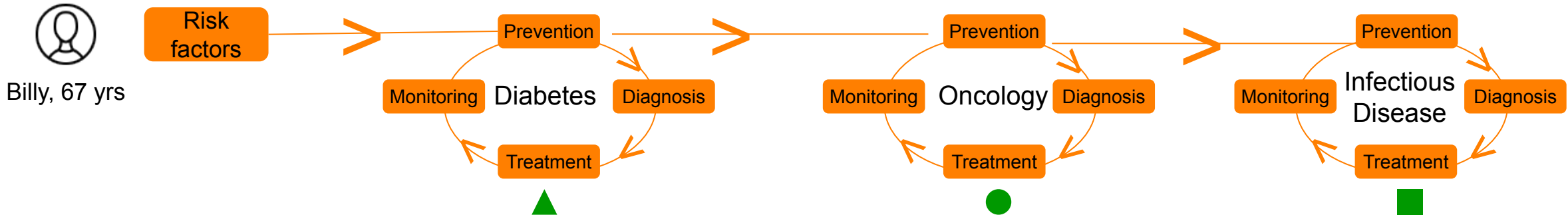


Not all research questions with regulatory intent can be addressed by RWD

Longitudinal Patient Record (LPR)

Integrating and activating data to enable contextualized analytics and insights

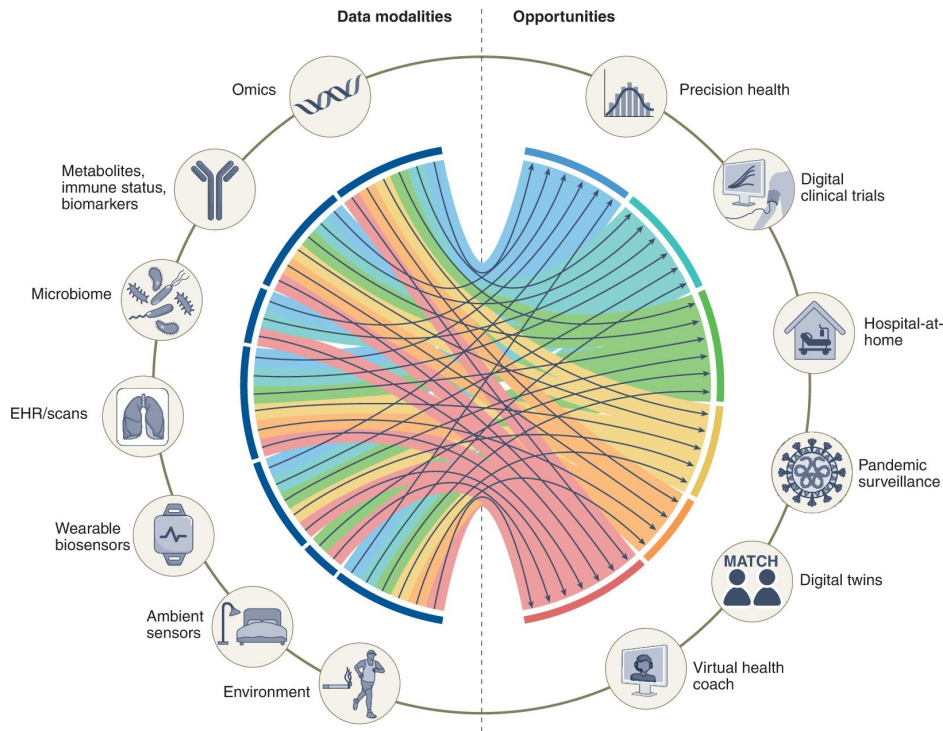
Illustrative



The promise of AI in healthcare

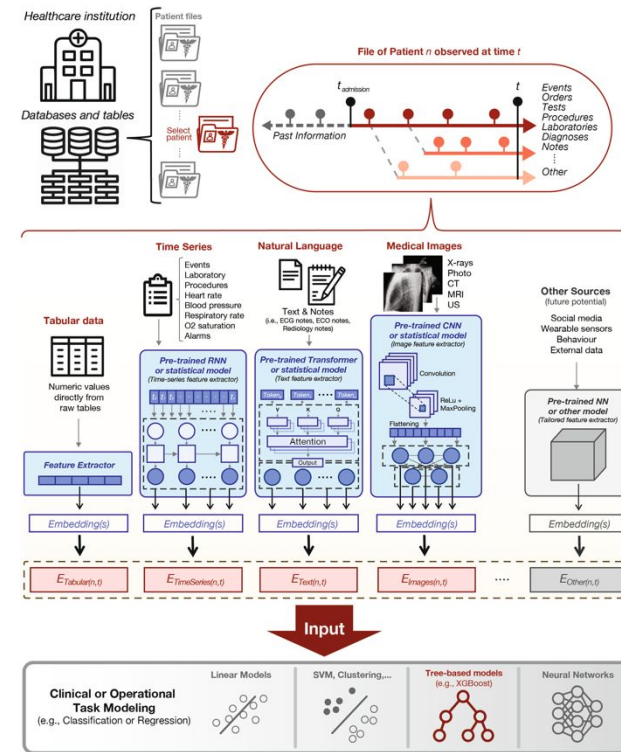
Leveraging multimodal data for clinical and operational benefits

Data modalities and opportunities for multimodal biomedical AI



Integrated multimodal artificial intelligence framework for healthcare applications

Holistic Artificial Intelligence in Medicine (HAIM) framework



Transforming clinical research with remote patient monitoring

Two clinic visits in the year of a patient with Parkinson's Disease



Day in the life of a patient with weak symptoms

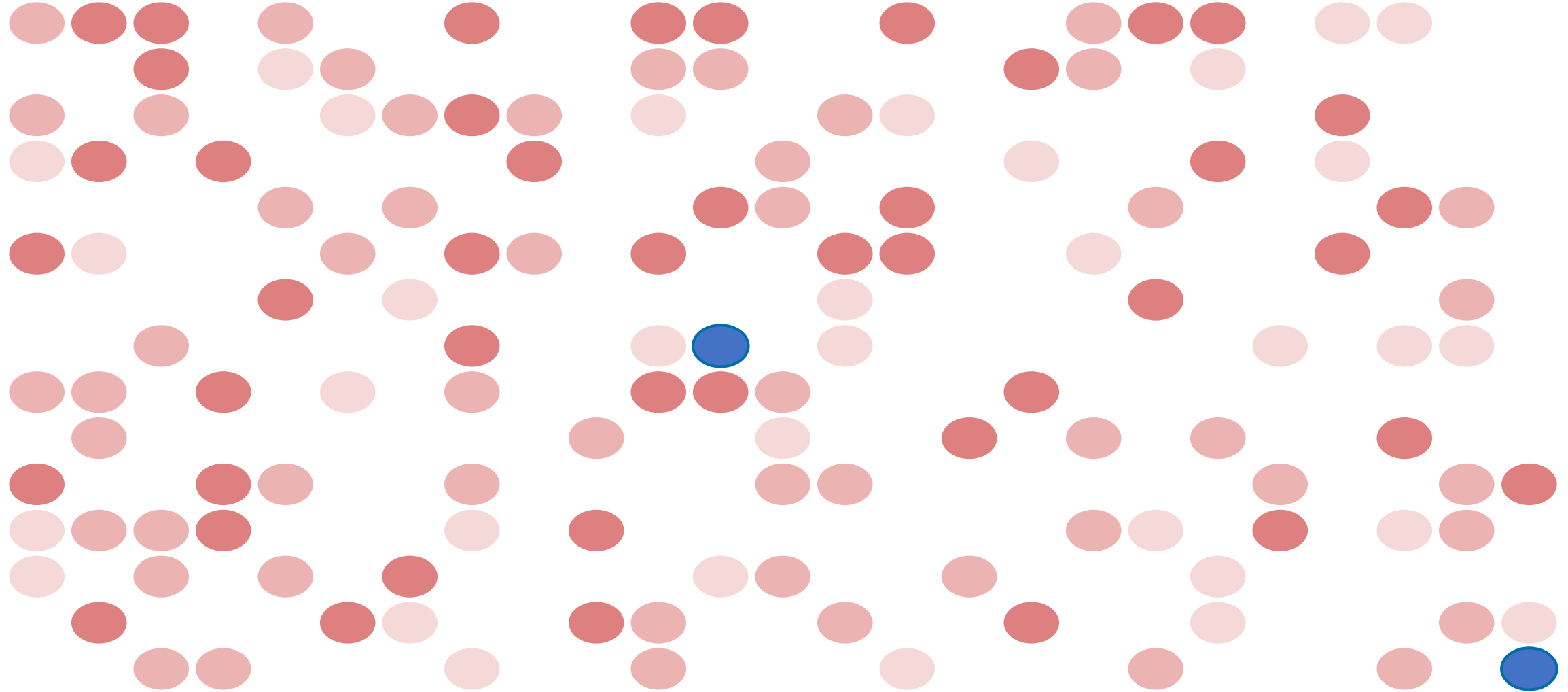




Day with a visit to the clinic/physician



Transforming clinical research with remote patient monitoring

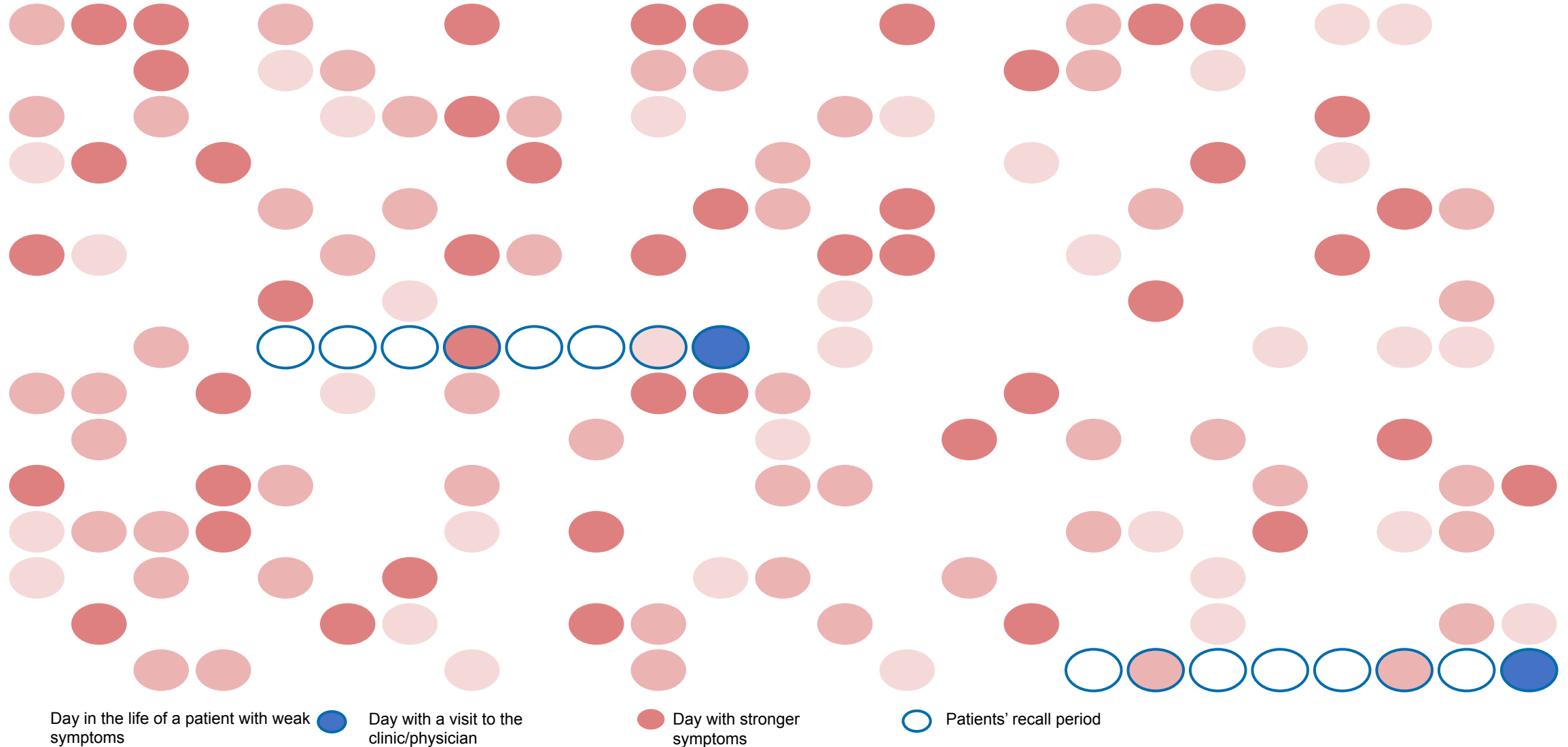
Daily fluctuations in symptom severity



Day in the life of a patient with weak symptoms  Day with a visit to the clinic/physician  Day with stronger symptoms

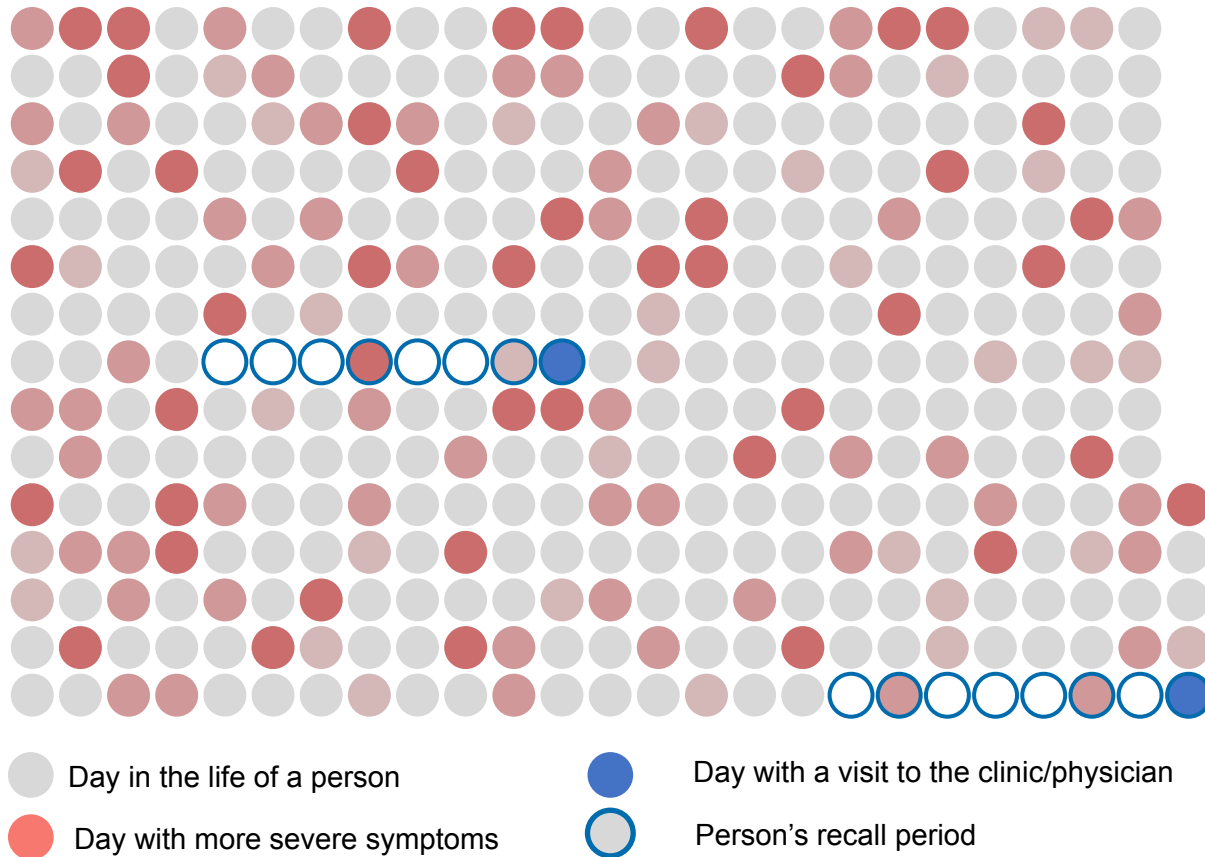
Transforming clinical research with remote patient monitoring

Limited insight into disease severity and progression



Challenges with clinical outcome assessments

In Parkinson's disease drug development today



Challenges with clinical outcome assessments can lead to... ¹⁻³

- More patients needed in trials
- Longer trial durations
- Inconclusive trial results

1. Dorsey ER, Venuto C, Venkataraman V, Harris DA, Kieburtz K. Novel methods and technologies for 21st-century clinical trials: a review. *JAMA Neurol.* 2015 May;72(5):582–8.

2. Parkinson study group QE3 Investigators; Beal MF, Oakes D, Shoulson I, Henchcliffe C, Galpern WR, et al. A randomized clinical trial of high-dosage coenzyme Q10 in early Parkinson disease: no evidence of benefit. *JAMA Neurol.* 2014;71(5):543–52.

3. Goetz CG, Tilley BC, Shaftman SR, Stebbins GT, Fahn S, Martinez-Martin P, et al. Movement disorder society UPDRS revision task force. movement disorder society-sponsored revision of the unified Parkinson's disease rating scale (MDS-UPDRS): scale presentation and clinimetric testing results. *Mov Disord.* 2008 Nov 15;23(15):2129–70.

Digital Biomarkers can help address these challenges



Frequency

How often measurement is repeated
Daily measurement can capture fluctuating symptoms



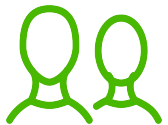
Resolution

Smallest increment that can be measured
High precision sensor measurement may reveal clinically meaningful differences and avoid floor/ceiling effects



Accuracy

Closeness of measurement to actual value
Sensors can directly measure the behaviors and physiological characteristics of interest



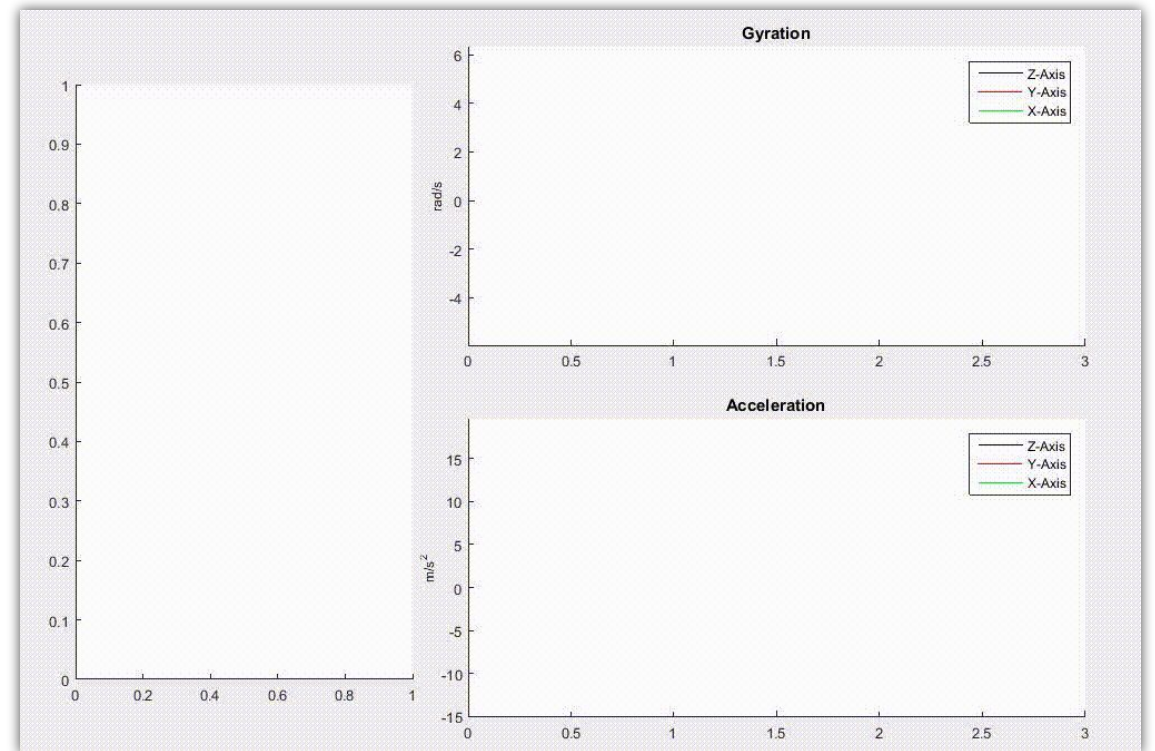
Reliability

Consistency of measurement
Sensors can provide highly consistent measurements of everyday behaviors



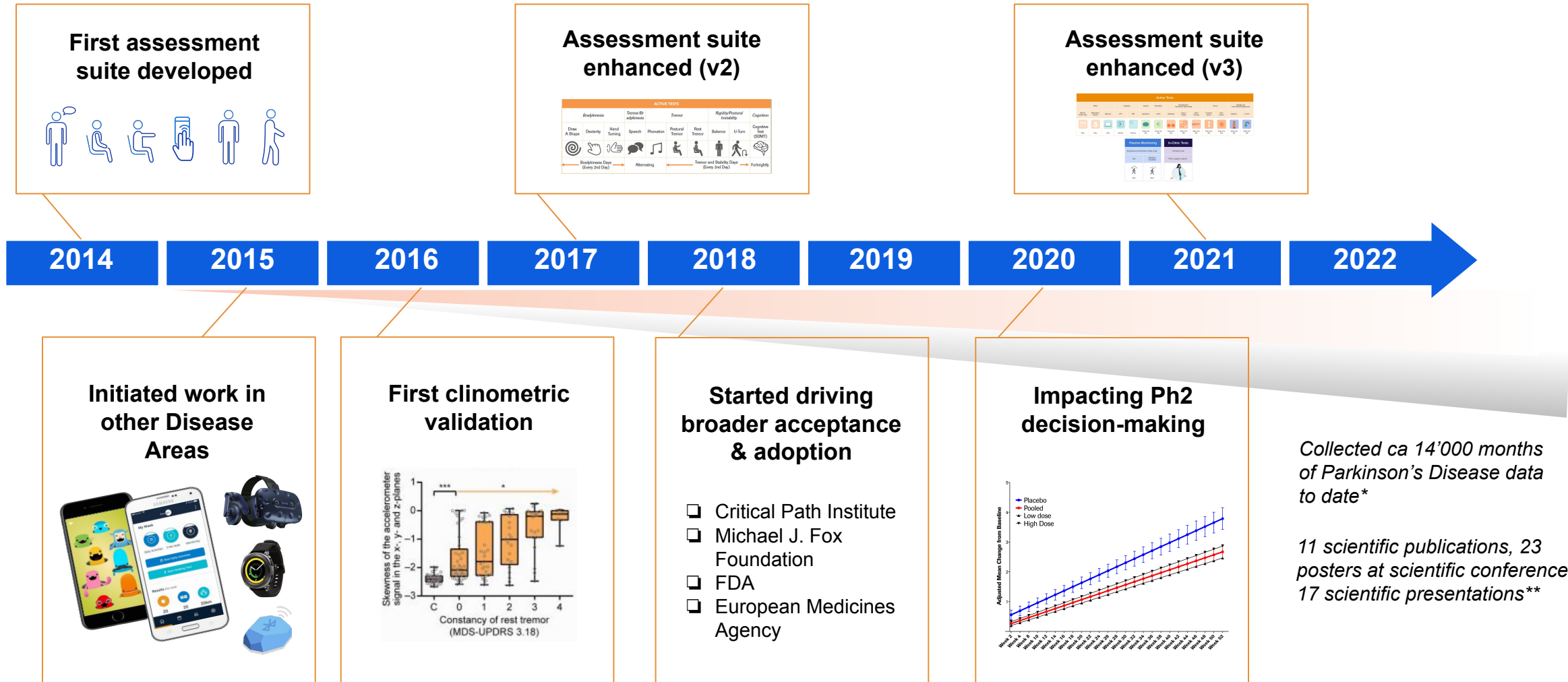
Relevance (ecological validity)

Generalisability of measurement to real-life
Patients can perform tasks at home and are measured during their typical, daily activities



Roche PD Digital Biomarker Solution development timeline

Collected 14,000 months of data and 11 scientific publications to date



*Roche data on file

**See publication table in the references section

The PD Digital Biomarker Assessment

Developed with clinical- and patient-expert input

Active Tests													
PROs			Cognition		Speech	Phonation	Bradykinesia: Dexterity & Upper Body			Tremor		Stability and Lower Body Bradykinesia	
Missed Active Test	Medication tracker	Daily Qs	IPS	TMT	Questions	Voice	Dexterity	Draw a Shape	Hand Turning	Postural tremor	Rest tremor	Balance	U-Turn
Daily	Daily	Daily	Monthly	Monthly	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day	Every 2nd day

Passive Monitoring	
Bradykinesia and Activities of Daily Living	
Gait	Mobility & Sociability
Daily	Daily

In-Clinic Tests
Full Active Tests
PROs, Cognition, Speech...



PD Digital Biomarker Solution shows path to 70% sample size reduction

In a simulated trial with a digital biomarker motor score as the primary endpoint over UPDRS, sample sizes were reduced by ~70% and trial duration by 16 months, reducing overall Ph 2 trial costs¹

	Trial powered on MDS-UPDRS	Trial powered on dBM score	Digital benefit	Assumptions
Sample size	550	166	70% fewer Participants	<ul style="list-style-type: none"> - Newly diagnosed PD patients, not on dopaminergic treatment - Minimal detectable difference (MDD) = 25% - 2-arm study randomized 1:1 - 24 week study duration - Type I error = 0.2 - Power = 0.8 (i.e. 80% of the times you run the same trial, you would detect a 25% or larger treatment effect)
Trial duration (months)	28	12	2.3x faster	
Trial cost			Ca. 66% savings	

1. Abstract and poster accepted at AD/PD in March 2023

HD Digital Biomarker Solution shows 80% sample size reduction

Accelerating timelines, whilst reducing costs and patient burden

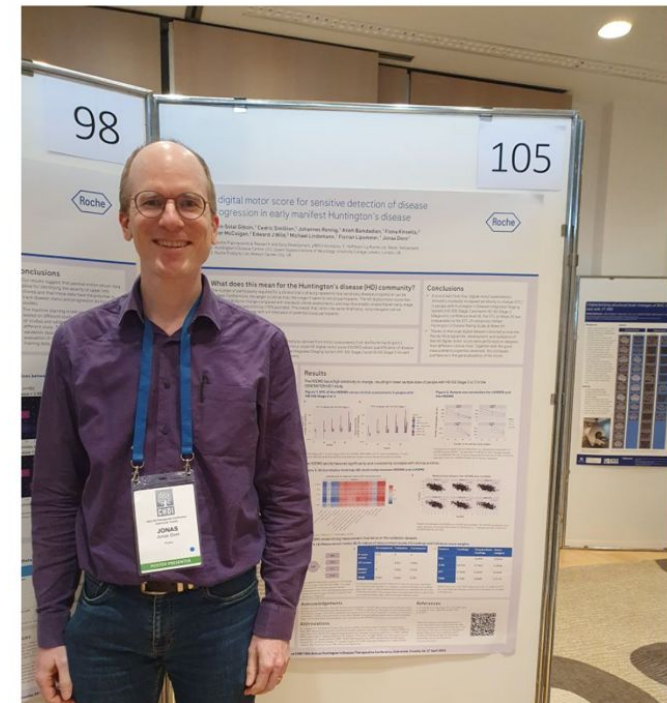


Jonas Dorn • 1st

Digital Biomarker Technology Lead at Roche

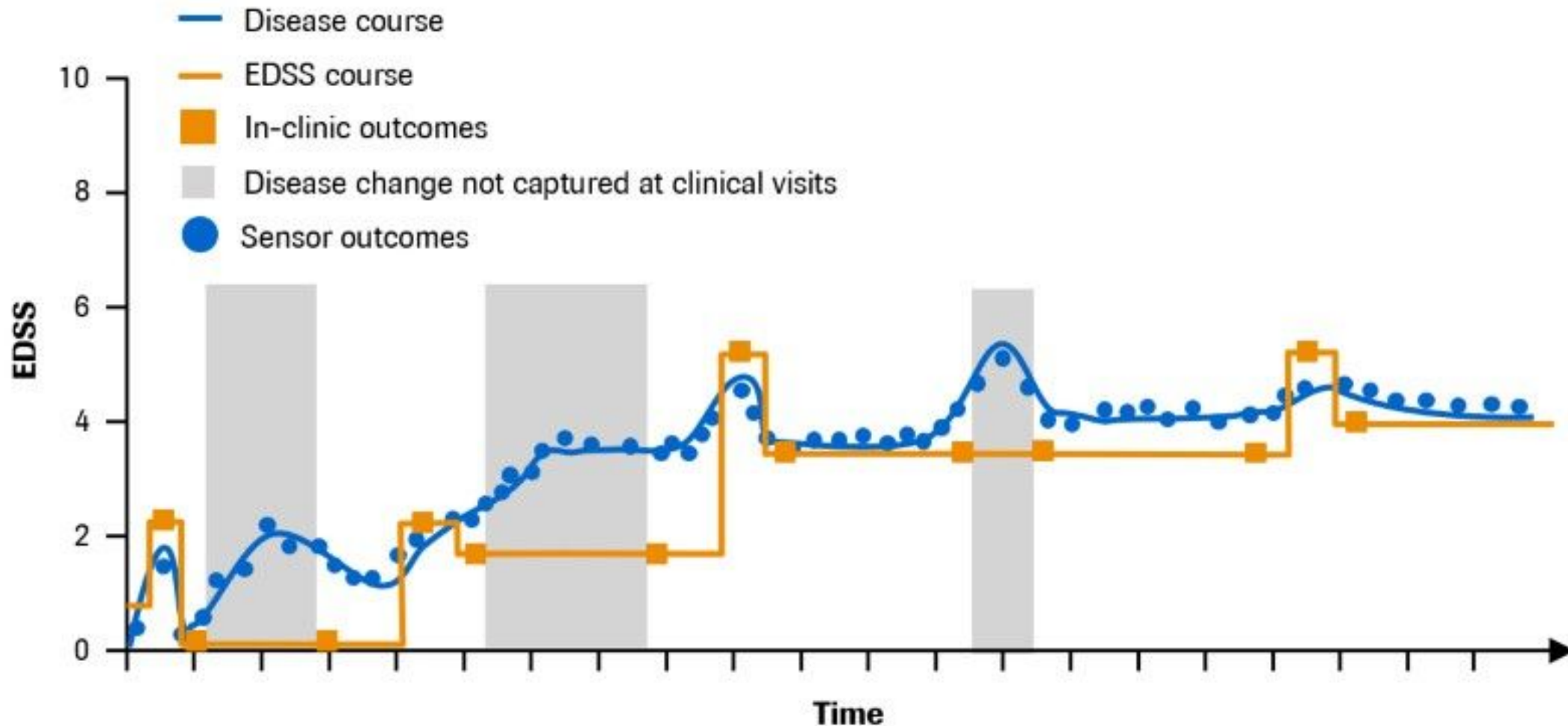
3w • 

In case anybody is still on the fence about whether digital biomarkers are worth developing: How about running clinical trials with 80% fewer patients? At [#chdi2023](#) we share our latest data and analysis on how [#Roche's #digitalbiomarker](#) approach can accelerate [#huntingtonsdisease #drugdevelopment](#) by reducing the number of [#patients](#) needed in trials ([Louis-Solal Giboin](#), [Cedric Simillion](#), [Johannes Rennig, PhD](#), [Atieh Bamdadian](#), [Fiona Kinsella](#), [PeterMcColgan](#), [Edward Wild](#), [Michael Lindemann](#), [Florian Lipsmeier](#)) If you want to know more, drop by the poster or contact me directly.



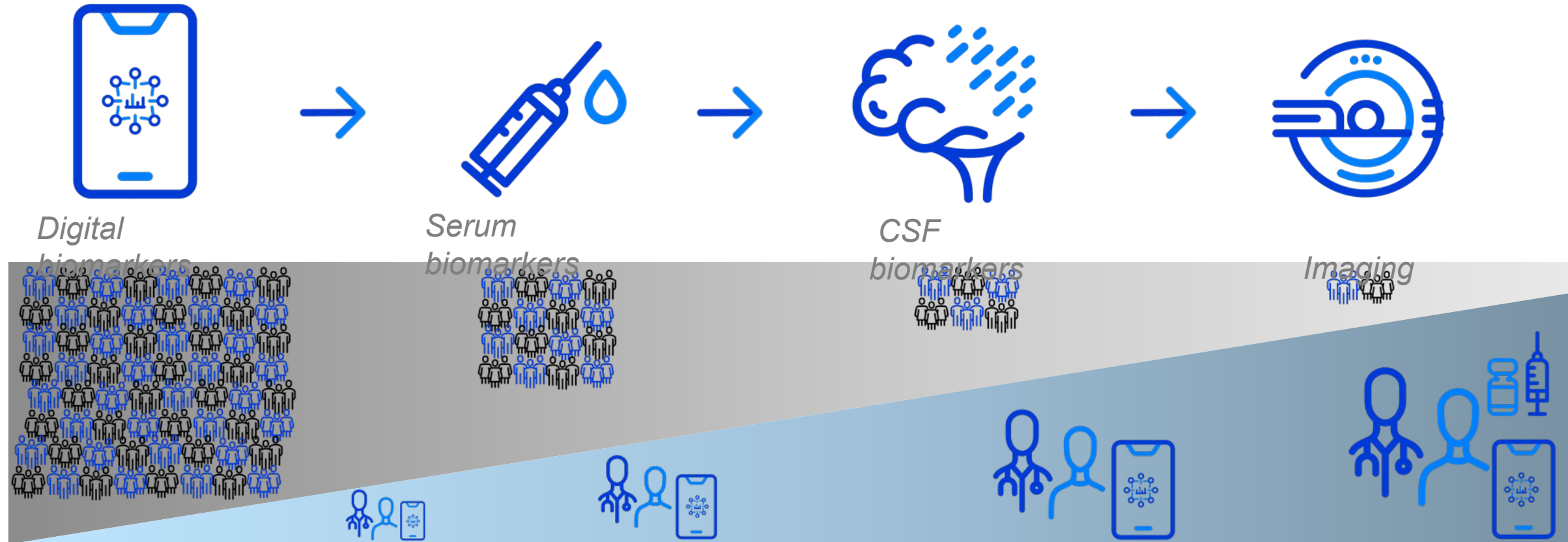
Digital Biomarkers in Multiple Sclerosis

Sensitive detection of subclinical disease activity



Potential role in disease detection and diagnosis

dBMs could be the entry point to future patient pathways through diagnosis and treatment of neurodegenerative conditions such as AD



- Earlier detection and intervention options
- Reduced burden and cost of detection and diagnosis

- More objective and precise diagnosis
- Monitor patient progression and response

Increasing relevance and resolution in the lab

Longitudinal and real-time monitoring of laboratory experiments

chemical reactions



in vivo environmental conditions



Concluding remarks

Summary: “the changing nature of healthcare R&D”

With a little help from our friend. 

- **Shift to Personalized Medicine:** Healthcare R&D is increasingly focused on personalized treatments, leveraging genomics and individualized therapies.
- **Data-Driven Insights:** Data analytics, machine learning, and AI are driving healthcare R&D, providing valuable insights for drug discovery and patient care.
- **Telemedicine and Remote Monitoring:** Telemedicine and remote patient monitoring are revolutionizing healthcare delivery, enhancing accessibility and research into remote care solutions.
- **Regulatory Adaptation:** Regulatory agencies are revising guidelines to accommodate the rapid pace of innovation in healthcare, ensuring the safe and effective development of novel therapies and digital health solutions.
- **Patient-Centricity:** Patients are playing a more significant role in healthcare R&D, contributing insights into their preferences and experiences, leading to more patient-oriented treatments.
- **Interdisciplinary Collaboration:** Collaboration between researchers, clinicians, engineers, and data scientists is fostering innovation and accelerating the development of breakthrough treatments and technologies.

Questions & Discussion



Doing now what patients need next