Health Data for Clinical Trials and Real World Evidence

Dean Summerfield
Senior Vice President, Real World & Commercial Solutions EMEA

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Addressing today's healthcare challenges requires better data

The healthcare environment is more challenging due to confluence of factors

Need to contain **rising costs**

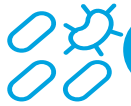
Increasing **need for evidence** generation to meet stakeholder demands, often within **short timelines**

Need for **up-to-date** real-world data with no extra burden for physicians

Need for **RWE early** in the product lifecycle

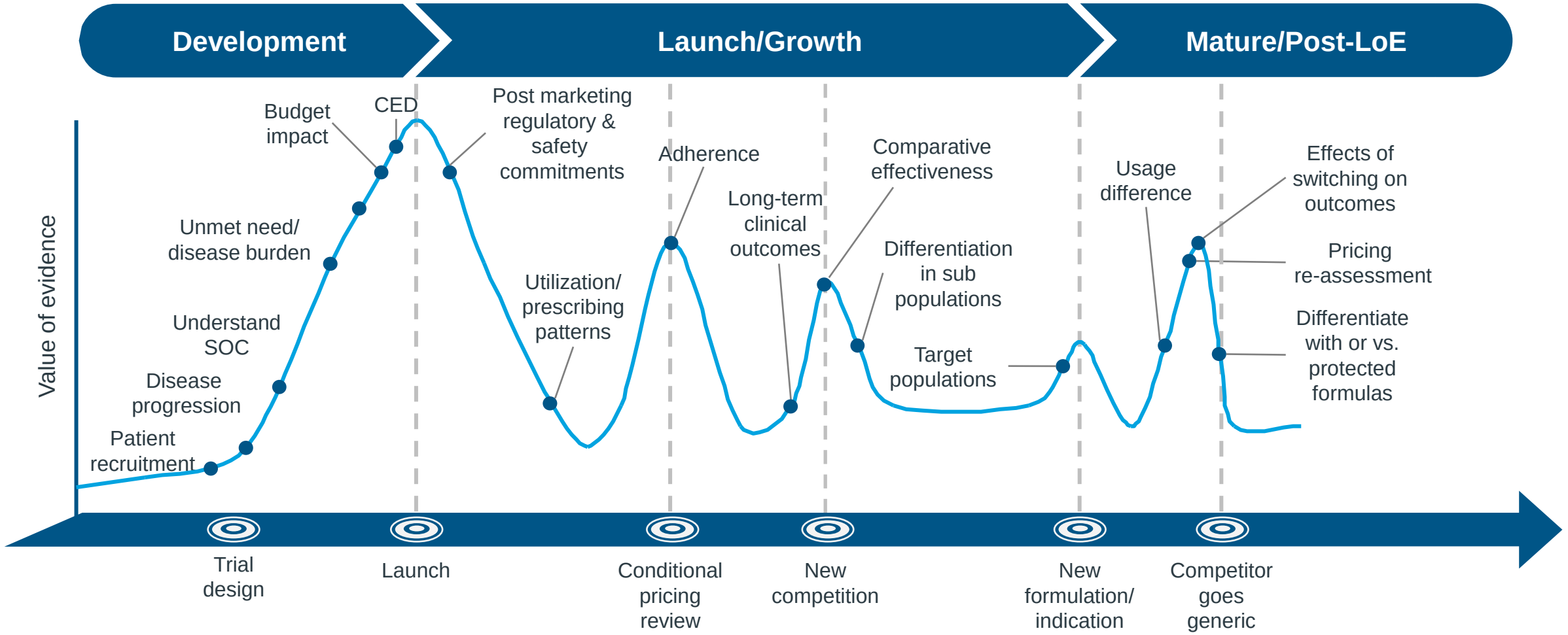
Reliance on **evidence-based medicine** & health technology assessment to drive payer decisions

Proliferation of **competing drugs** in the same therapeutic areas



Increasing demand for evidence on **rare/ultra-rare diseases**

Life Sciences companies seek higher quality data to inform key decisions across the product life cycle

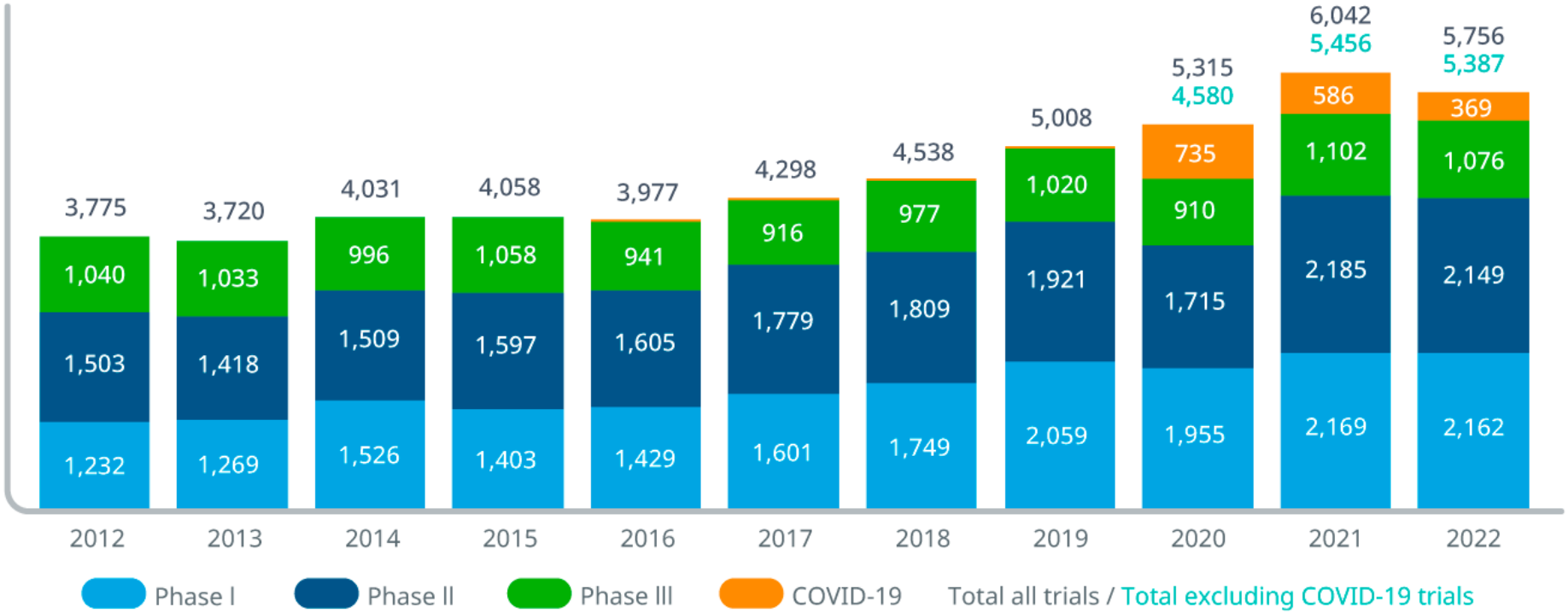


SOC: Standard of care; CED: Continued evidence development; Source: IQVIA expertise

Clinical trial activity and investment continues to grow, particularly in early stage trials

40% increase in Phase I-III trials over last 10 years

Total number of clinical trial starts by phase, 2012–2022



Source: Citeline Trialtrove, Jan 2023.

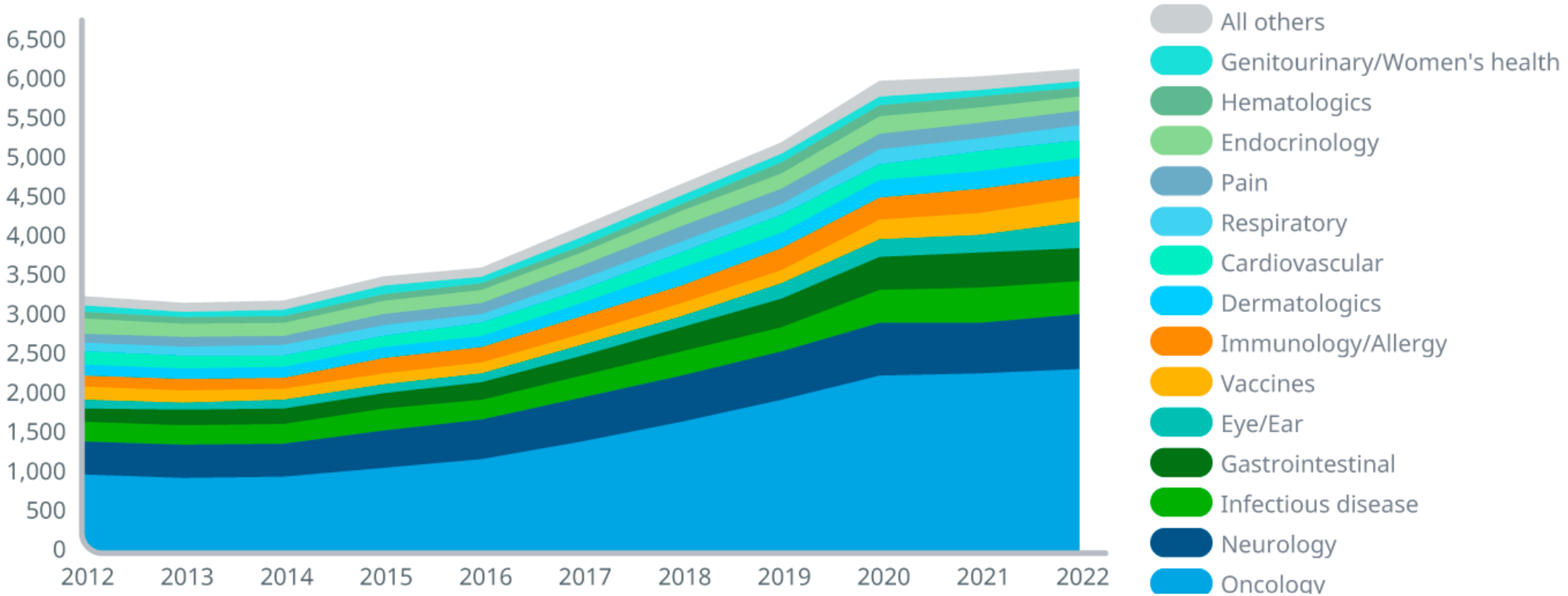
Notes: Phase II includes Phases I/II, II, IIa, IIb. Phase III includes Phase II/III and III. Terminated trials are included to track the activity still involved with their initiation, partial execution and termination. Trials were industry sponsored, interventional trials and device trials were excluded.

Report: Global Trends in R&D 2023: Activity, Productivity, and Enablers. IQVIA Institute for Human Data Science, February 2023.

Research in all therapeutic areas increased over the last decade

~35% of pipeline in oncology; ~2/3rds of pipeline in onco, neurology, ID and gastrointestinal disease

Number of pipeline products Phase I to regulatory submission by therapeutic drug class, 2012-2022



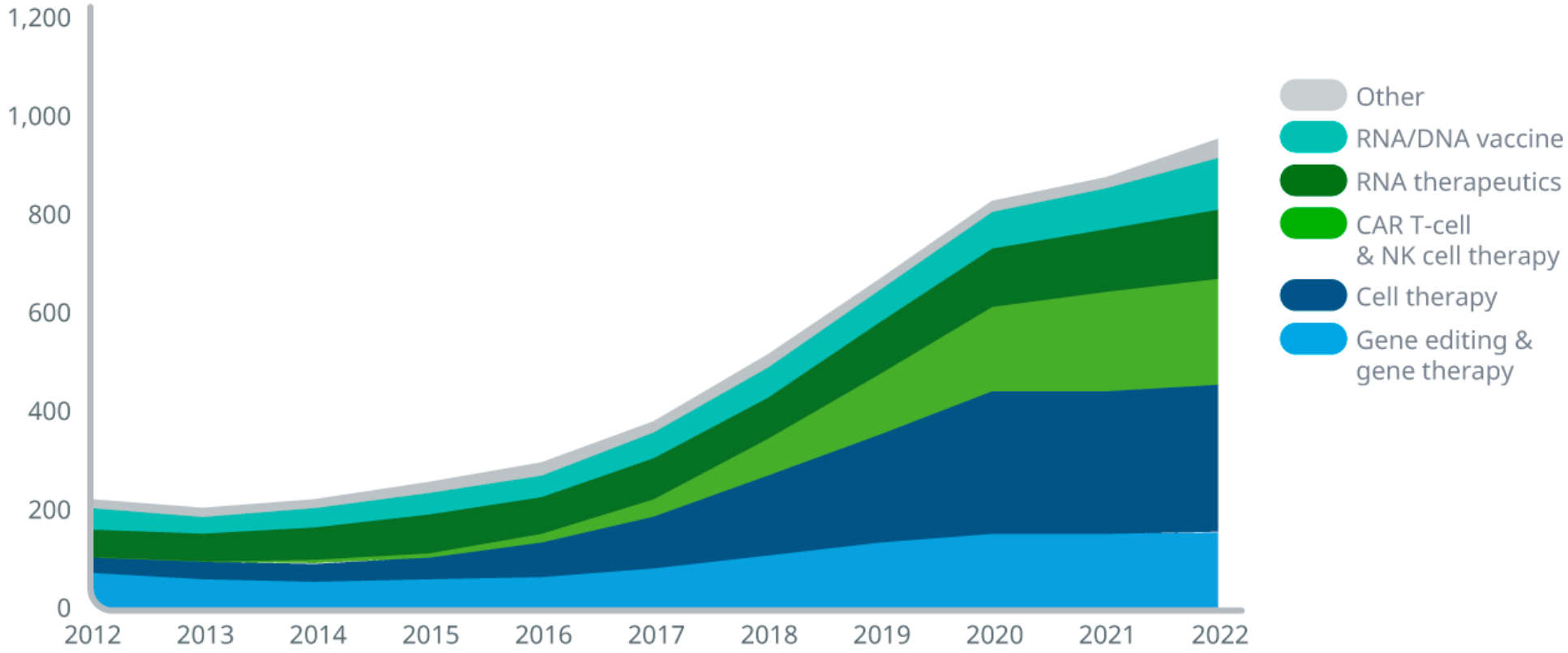
Source: IQVIA Pipeline Intelligence, Dec 2022; IQVIA Institute, Jan 2023.

Notes: Includes drugs with an active research program, with phase determined by the highest phase of research regardless of indication. Oncology includes supportive care. Neurology includes central nervous system disorder treatments and mental health treatments but does not include pain management or anesthesia.

Report: Global Trends in R&D 2023: Activity, Productivity, and Enablers. IQVIA Institute for Human Data Science, February 2023.

Advanced therapies, cell, gene and RNA therapies, are now ~15% of the trial pipeline increasing demand for newer forms of data

Next-generation biotherapeutics Phase I to regulatory submission pipeline by mechanism, 2012–2022



Source: IQVIA Pipeline Intelligence, Dec 2022; IQVIA Institute, Jan 2023.

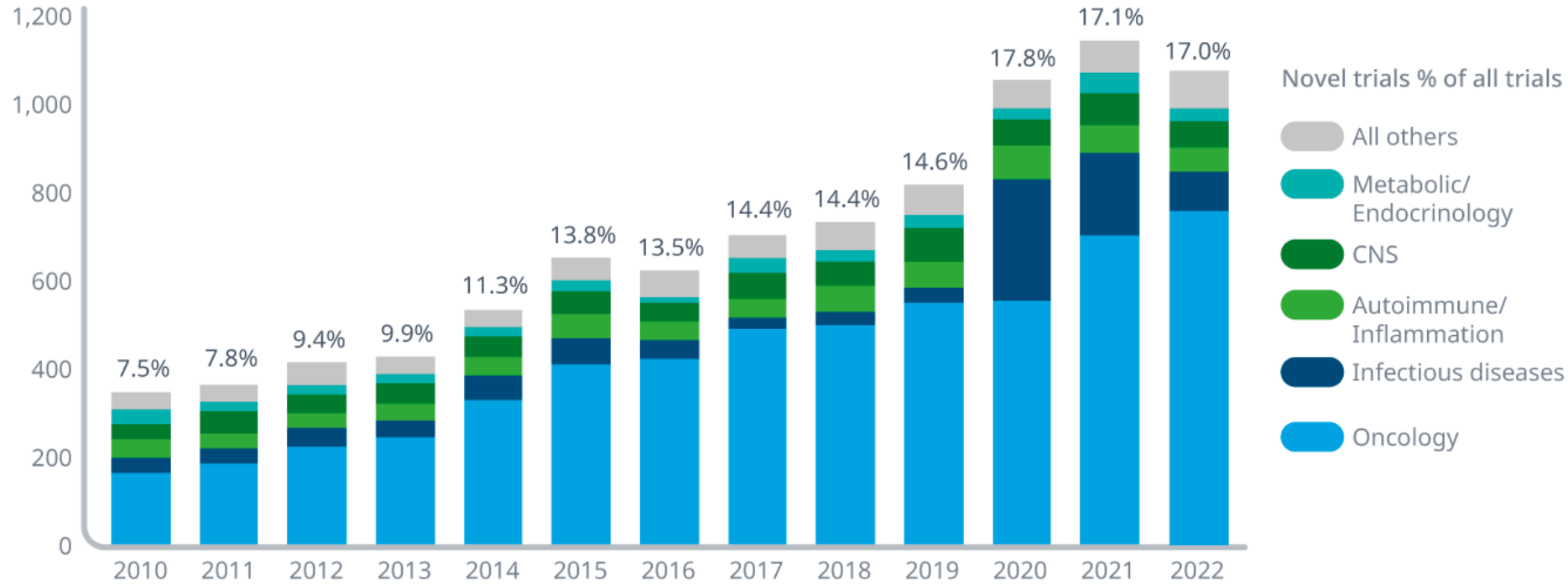
Notes: Includes drugs with an active research program, with phase determined by the highest phase of research regardless of indication. Other includes oligonucleotides and other less common next-generation biotherapeutics.

Report: Global Trends in R&D 2023: Activity, Productivity, and Enablers. IQVIA Institute for Human Data Science, February 2023.

Commercial clinical trials increasingly adapting novel designs, which place additional requirements on data generation

Includes umbrella, basket, adaptive, master protocol and other designs

Novel trial design starts by year and therapy area, 2010–2022



Source: Citeline Trialtrove, IQVIA Institute, Jan 2023.

Notes: Trials were industry sponsored, interventional trials and device trials were excluded. Novel trial designs include umbrella, basket, adaptive, master protocol, dose escalation + dose expansion studies using a range of keyword strings.

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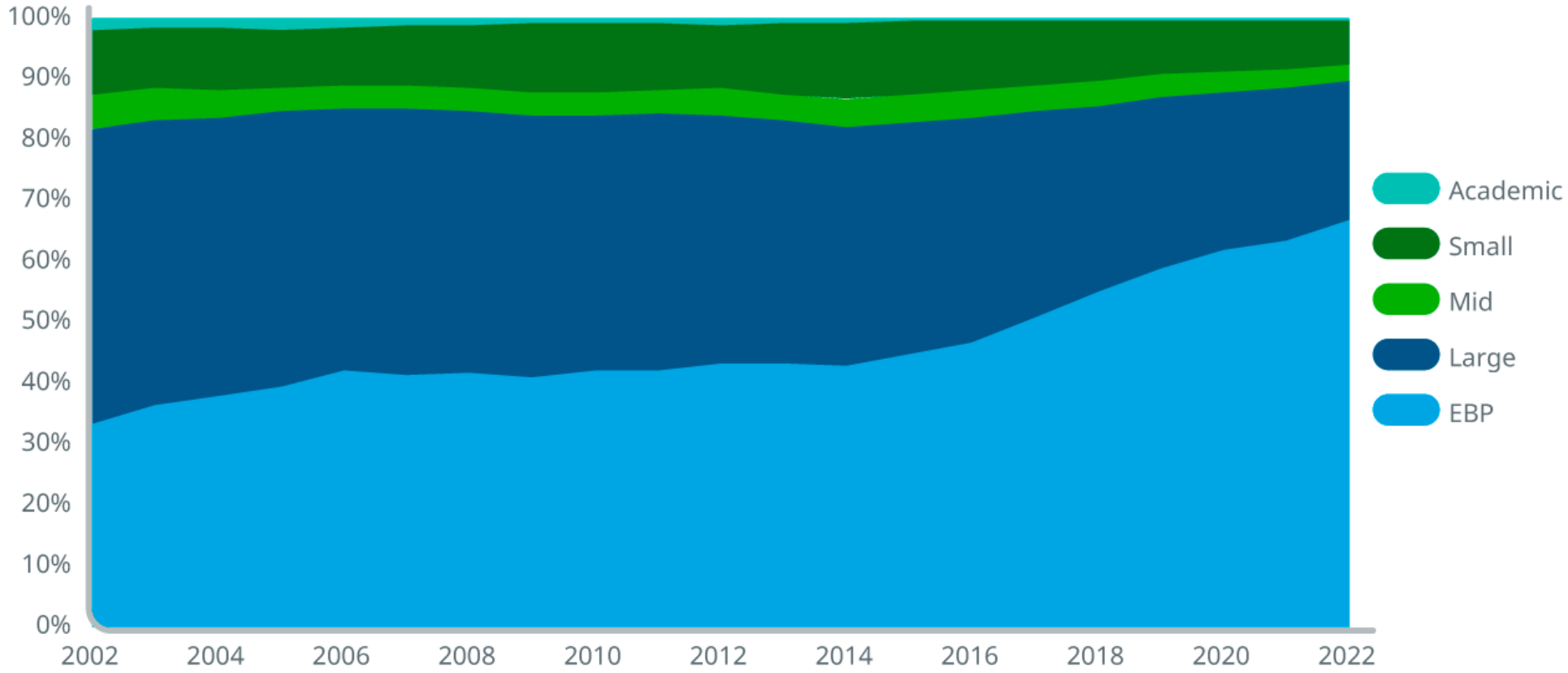
Emerging biotech companies (EBPs) now represent a record 65% of the clinical development pipeline

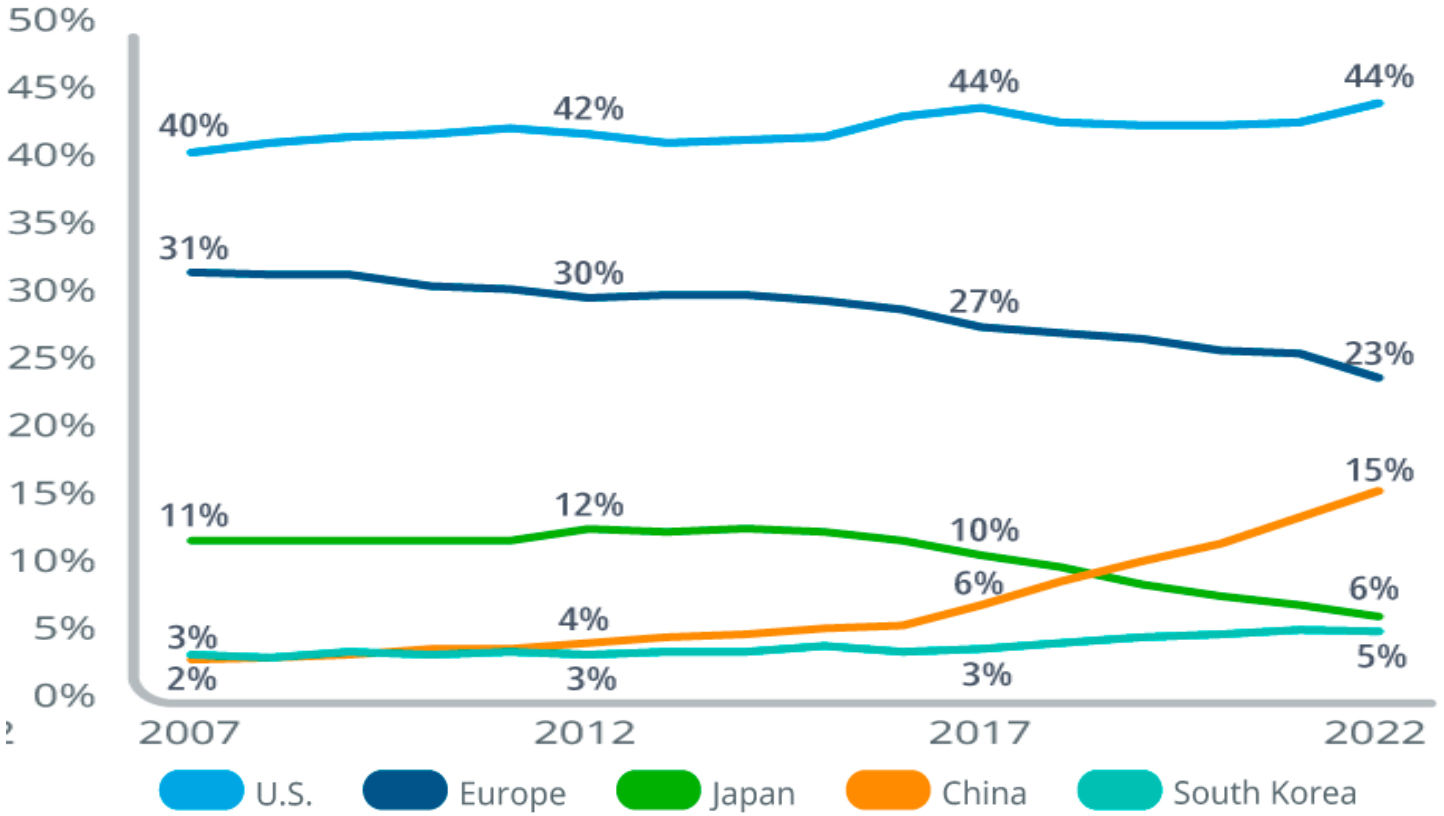
Share of Phase I to regulatory submission pipeline by company segment, 2002 - 2022



Source: IQVIA Pipeline Intelligence, Dec 2022; IQVIA Institute, Jan 2023.
Notes: Includes drugs with an active research program, with phase determined by the highest phase of research regardless of indication. Company segment when two or more companies are involved is determined by the larger sales segment.
Report: Global Trends in R&D 2023: Activity, Productivity, and Enablers. IQVIA Institute for Human Data Science, February 2023.

Asia, particularly China, is an increasing source of novel active substances; Europe declining

Number of drugs and country share of pipeline Phase I to regulatory submission based on company headquarter location, 2007–2022



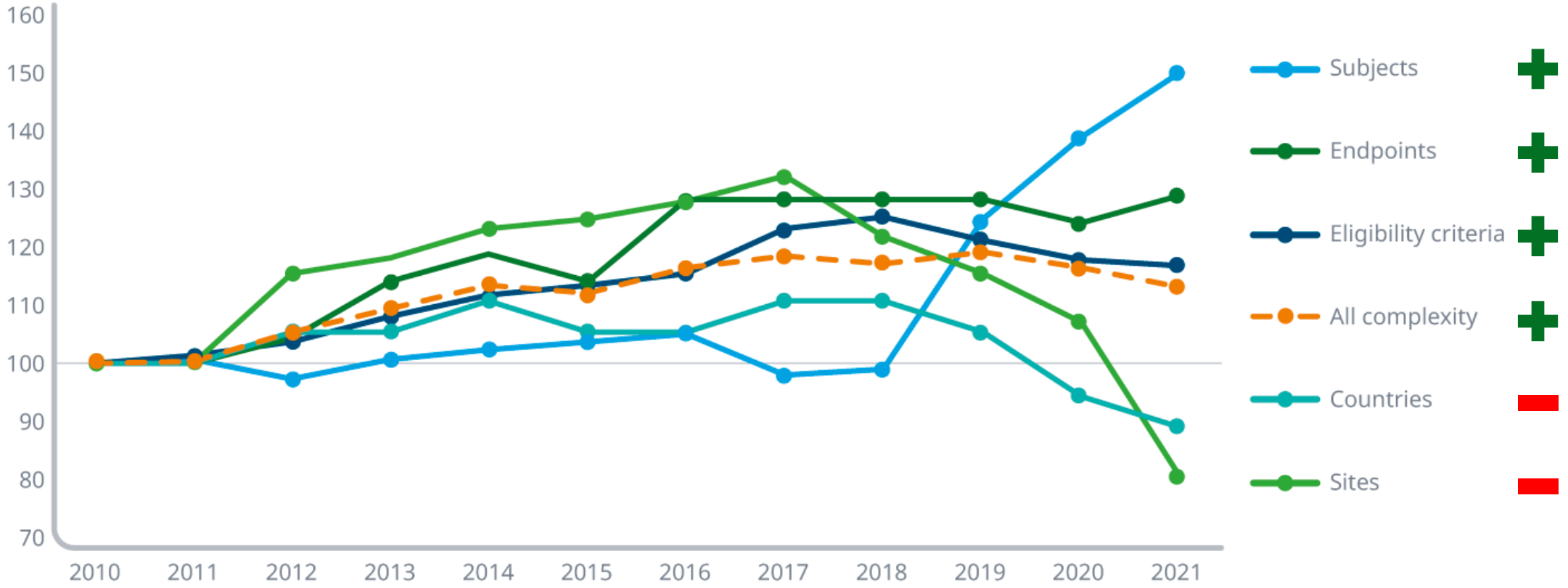
Source: IQVIA Pipeline Intelligence, Dec 2022; IQVIA Institute, Jan 2023.

Notes: Includes drugs with an active research program, with phase determined by the highest phase of research in each year regardless of indication. Each company involved in a drug's development is counted individually, so products with more than one company involved are counted more than once and may be included in more than one region. Europe is defined as any country in continental Europe.

Report: Global Trends in R&D 2023: Activity, Productivity, and Enablers. IQVIA Institute for Human Data Science, February 2023.

Clinical trial complexity has increased across multiple dimensions over the last 10 years

Elements of complexity indexed to 2010 values, all phases, 2010-2021

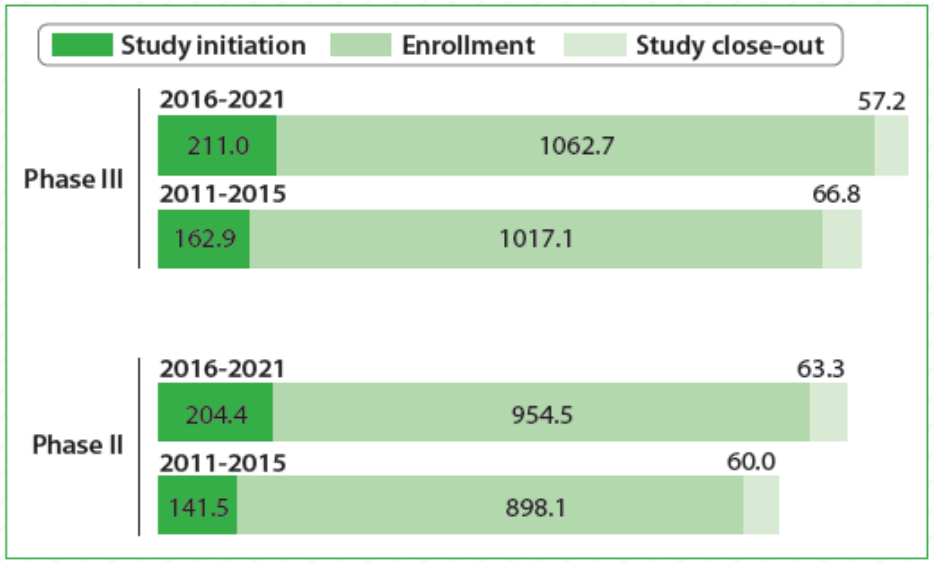


Source: Citeline Trialtrove, IQVIA Institute, Jan 2022.
Notes: Terminated and withdrawn trials were excluded from the analysis. Trials were industry sponsored and interventional. Diagnostics, behavioral therapies, supplements, devices, and medical procedures were excluded. Infectious diseases excludes vaccines
Report: Global Trends in R&D: Overview through 2021. IQVIA Institute for Human Data Science, February 2022

Recent Tufts report demonstrates the continued rise in protocol design scope and execution burden

Trial durations increasing, especially in initiation

(mean clinical trial duration in days by phase)



Source: Tufts Center for the Study of Drug Development

37% increase in endpoints in Ph III trials since 2015

(endpoints and procedures per protocol by phase)

	Total endpoints at DBL*	Total procedures*	Percent non-core procedures†
Phase II			
2011-2015	20.7 (0.6)	219.4 (0.6)	17.9%
2016-2021	19.5 (0.7)	259.2 (1.2)	26.1%
Phase III			
2011-2015	18.9 (0.7)	187.6 (0.7)	24.7%
2016-2021	25.8 (0.8)	266.3 (0.9)	23.1%

Notes: DBL=Database lock; CoV=Coefficient of Variation

* Mean (CoV)

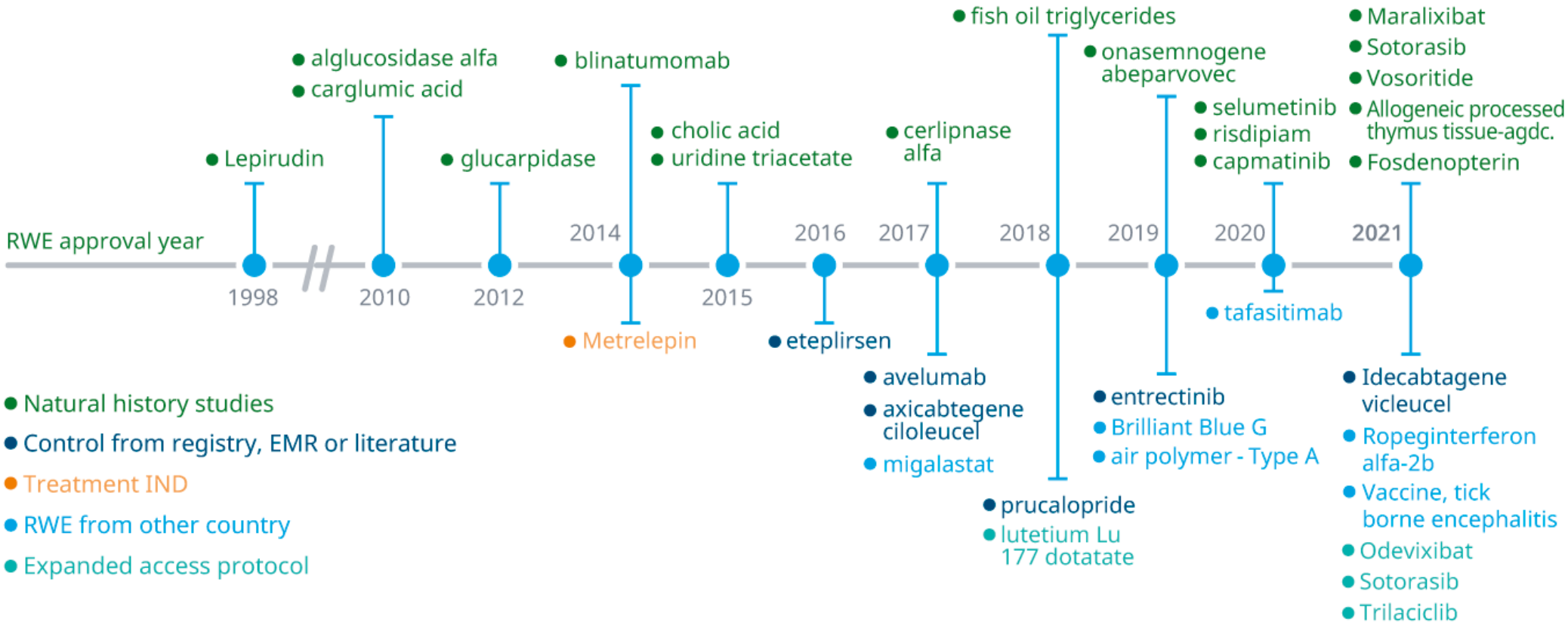
† Non-core procedures are those that support ancillary secondary, tertiary, and exploratory endpoints as well as safety and efficacy procedures not associated with primary or key secondary endpoints

Source: Tufts Center for the Study of Drug Development

Source: Tufts Center for the Study of Drug Development; Impact Report May/June 2023

Real-world evidence is increasingly part of regulatory submissions and included in the approval decisions

Timeline of U.S. FDA approvals of novel active substances (NASs) based on real-world evidence (RWE)



Source: IQVIA Institute, Jan 2022.

Notes: Collected from public sources relating to the approval trials for medicines. Data collected under a treatment IND or expanded access protocol has been considered a form of RWE by the FDA, such as in rare disease settings where there is little chance of a prospective trial. RWE approvals shown here include those granted after approval (e.g., carglumic acid 2010 RWE but drug was a 2006 launch).

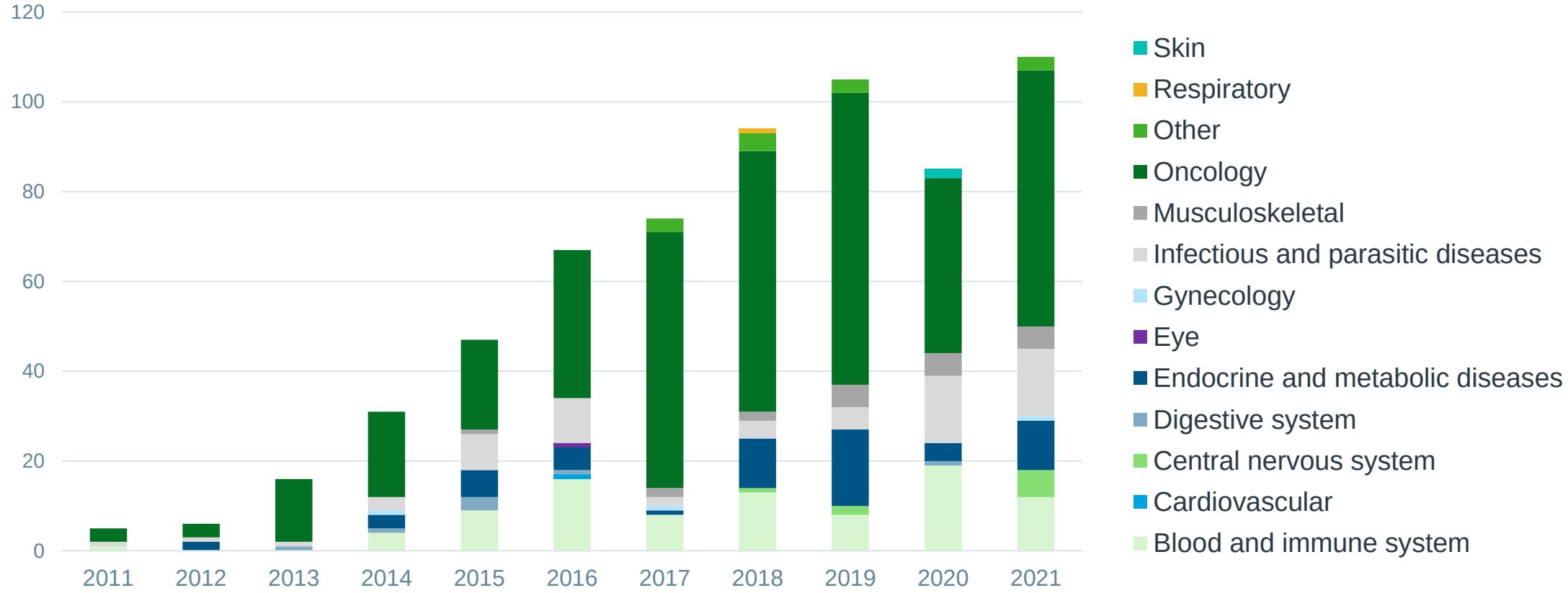
Report: Global Trends in R&D: Overview through 2021. IQVIA Institute for Human Data Science, February 2022

Use of real world data in payer submissions is also increasing

For example, growth in single arm trials drives a higher need for external comparator studies

Single arm trial submissions to HTA bodies globally by therapy area, 2011-2021

N=640 Single arm trials covering >400 drug/indication combinations



Inclusion criteria = Single-arm study; Original submission or extension of indication; All countries; 2011–2021
Source: IQVIA HTA Accelerator analysis, March 2022

Diversity in clinical development has many dimensions, driving a need for better connected healthcare data

Diversity Objective

- Identify epidemiology differences in prevalence and outcomes
- Measure differential biological responses
- Identify underlying genetic mutation propensities

Diversity Objective

- Study participants representative of disease population
- Equitable access to clinical research as a care option
- Identification of response and safety in important subpopulations

Diversity Objective

- Track access and use of medicine by subpopulations
- Assess outcomes by subpopulations
- Measure reductions in outcomes disparities by subpopulation

Pharma company research executives use a variety of factors in choosing countries and sites for clinical research

Prevalence Pre-scoping: data-driven patient population availability

Trial Performance: Speed and Quality	**Expertise Network:** Knowledge and Advocacy	**Regulatory Framework:** Value and Innovation
• Set-up and activation timelines • Patient recruitment and retention • Diversity and Rare diseases Recruitment • Digitalisation, DCT, niche capabilities	• Role of KOLs • End-to-end impact of in Market Access and Launch Excellence • Associations and advocacies, etc.	• Registration and Approval policies • Clinical trial design innovation • Access and reimbursement • Tax benefits, grants

Cost components: not a top-priority (i.e., "quality and speed over costs")

Source: IQVIA White Paper, July 2022 - *Attracting Investment in Clinical Development: How pharmaceutical companies make clinical development location decisions, and what healthcare policy makers can do to attract commercial clinical investment.*

EU driven initiatives to further develop Europe as a competitive centre for innovative clinical research

Non-exhaustive list of initiatives

European Commission

EUROPEAN HEALTH DATA SPACE

#EUDigitalHealth

BRIEFING

EU Legislation in Progress

European Parliament

Artificial intelligence act

DARWIN EU

And diverse collaborations to address global public health challenges and, in particular, those in LMICs

Non-exhaustive list of initiatives

HERA

HEALTH EMERGENCY PREPAREDNESS AND RESPONSE AUTHORITY

European Commission

EU FAB

Network of vaccine producers for future health emergencies

#EU4Health

European Commission

European Commission

Tackling Antimicrobial Resistance in a One Health approach

JUNE 2023

CEPI

IQVIA and CEPI collaborate to strengthen global research preparedness to advance the 100 Days Mission

11 Oct 2023 By CEPI News

IQVIA

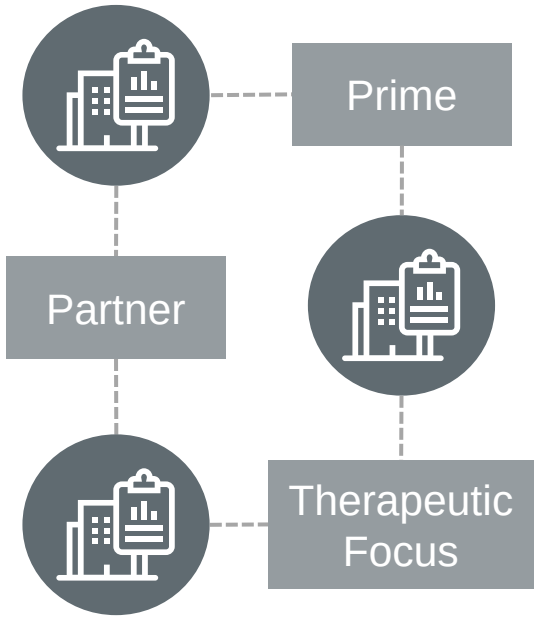
AFRICA HEALTH SUMMIT

Advancing Africa's health through data, technology and innovation

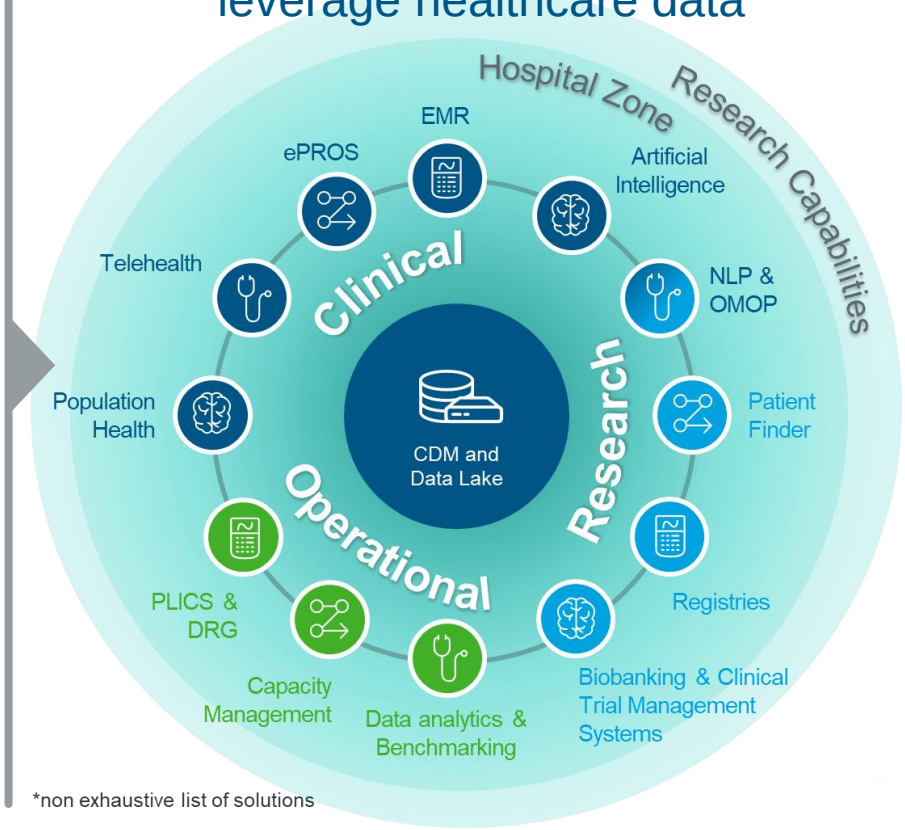
11-12 October 2023 | Kigali, Rwanda

Networks of “Connected Sites” are critical to meet the demands of today’s research

Preferred Site Networks



Powered by technology to leverage healthcare data



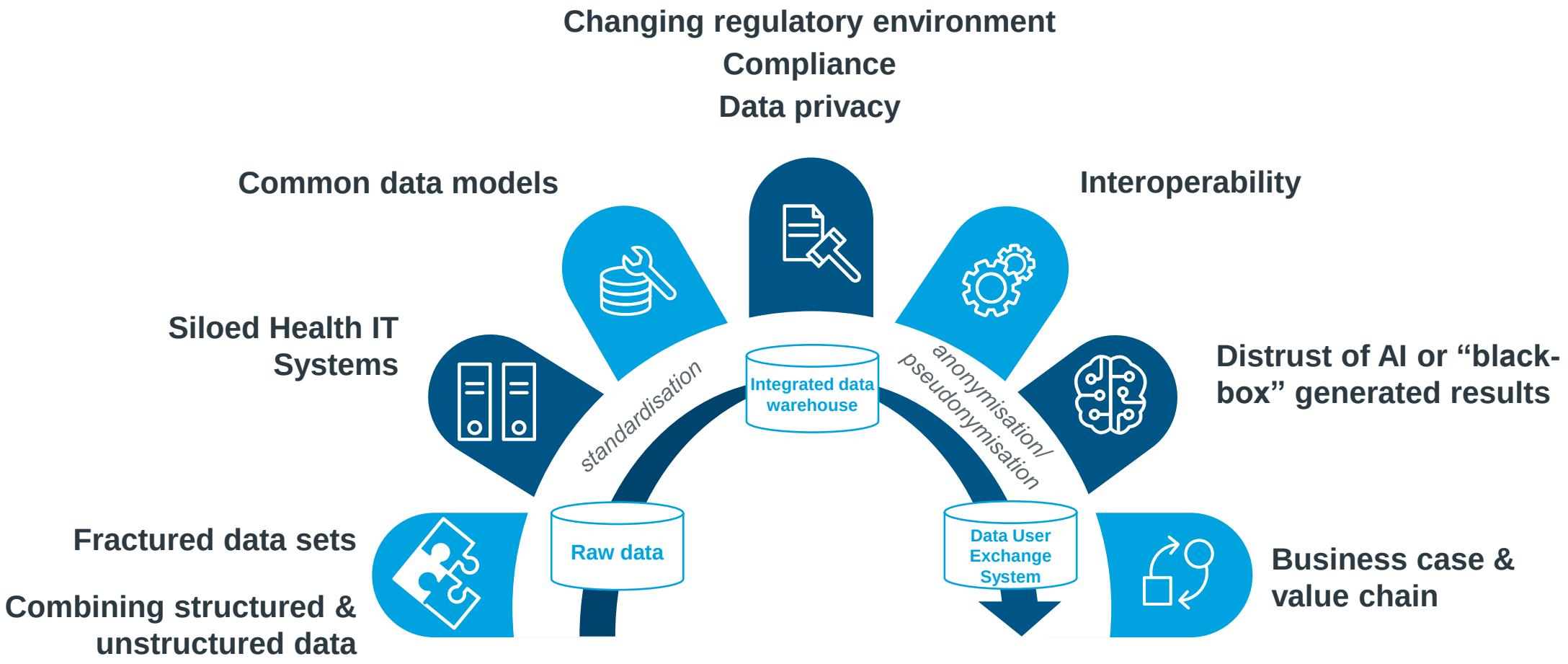
*non exhaustive list of solutions

Compelling Benefits and Results

- Faster recruitment
- Improved patient and site experience
- Reduced administrative burden
- Facilitates Clinical Research as a Care Option

Integrating all available data across multiple systems to help drive improved **care delivery**, **operational efficiencies,** and cohort centred **research** initiatives.

Healthcare systems must overcome several data & technology challenges to realise the full potential



Working together we can unleash the tremendous potential of health data to accelerate research and improve patient outcomes.