



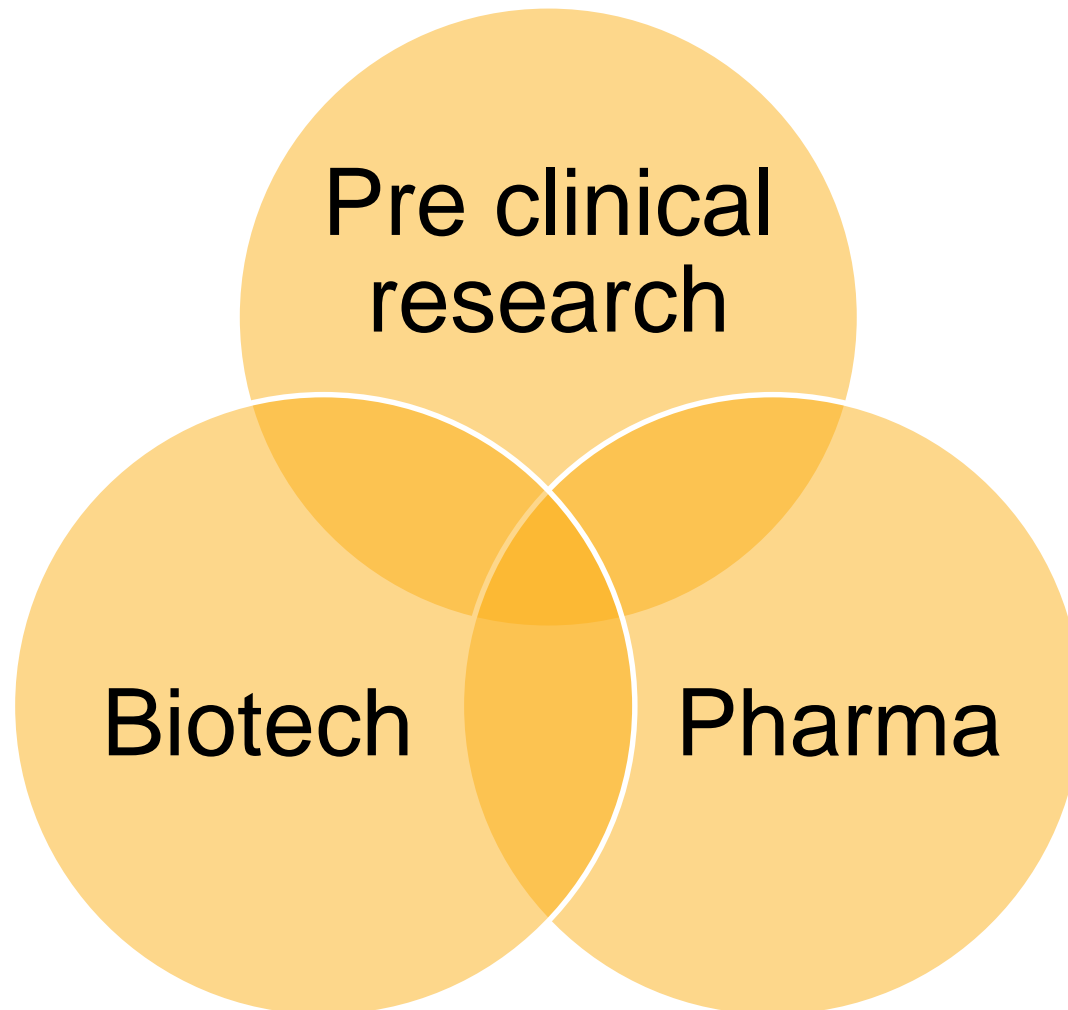
Big Data in Pharmaceutical Industry (Novartis)

Jean-Michel Gaullier, PhD

Sollentuna, August 3rd 2015

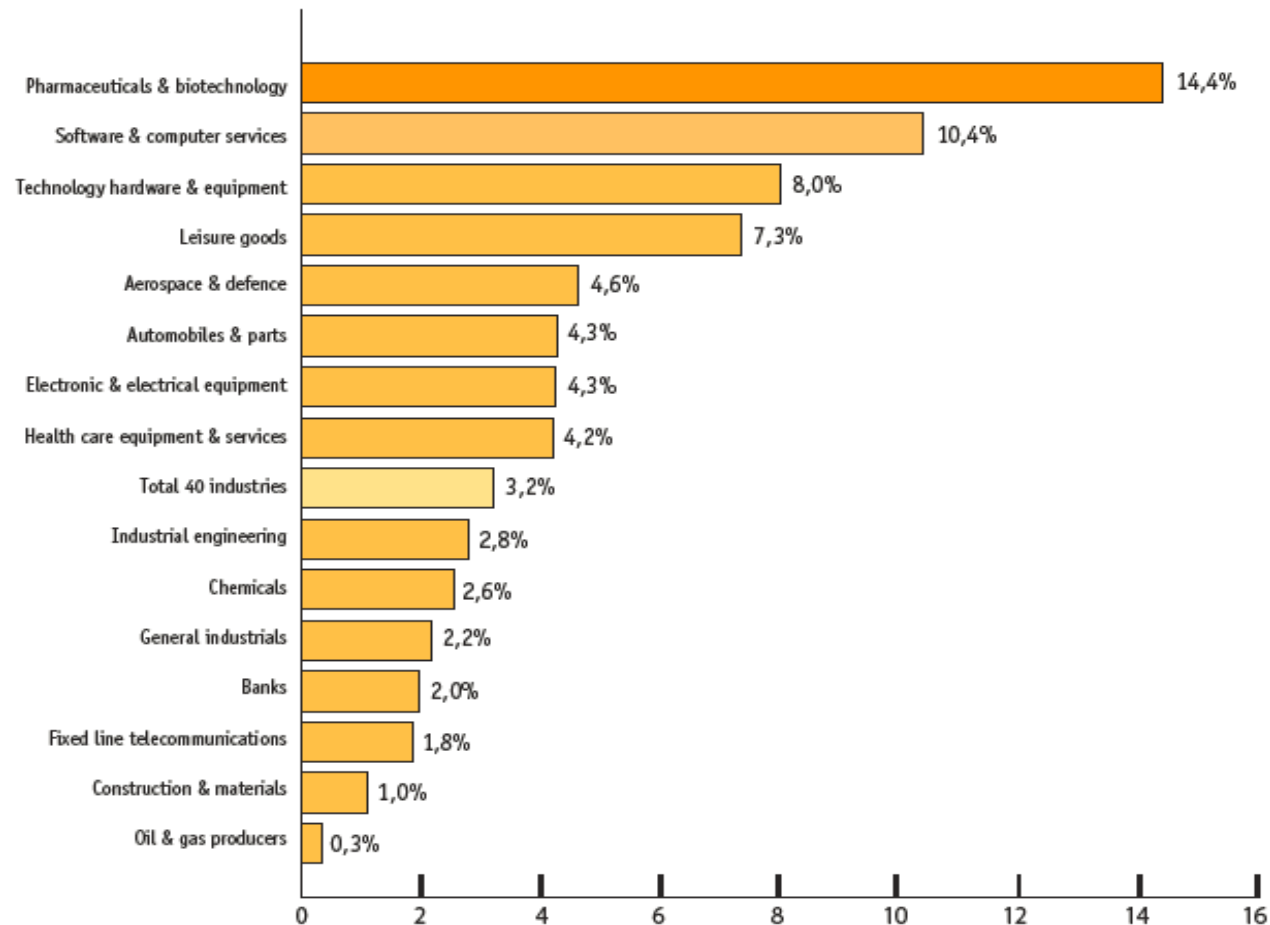
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Pharmaceutical Industry



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Innovation in Pharma & Biotech (R&D as percentage of net sales – 2013)



Note: Data relate to the top 2,500 companies with registered offices in the EU (633), Japan (387), The USA (804) and the Rest of the World (676), ranked by total worldwide R&D investment (with R&D investment above €15.5 million)

Source: The 2014 EU Industrial R&D Investment Scoreboard, European Commission, JRC/DG RTD

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Novartis



More than **119 000 associates** work at Novartis worldwide

Our workforce comprises people from **150 nationalities**

To encourage diversity, we continue to focus on the **promotion of women**, who made up 40% of Group company management in 2014

In 2014 we introduced revised **Novartis Values and Behaviors** that explain how we expect associates to act at work: innovation, quality, collaboration, performance, courage and integrity

Novartis is a world-leading healthcare company

Leading market position

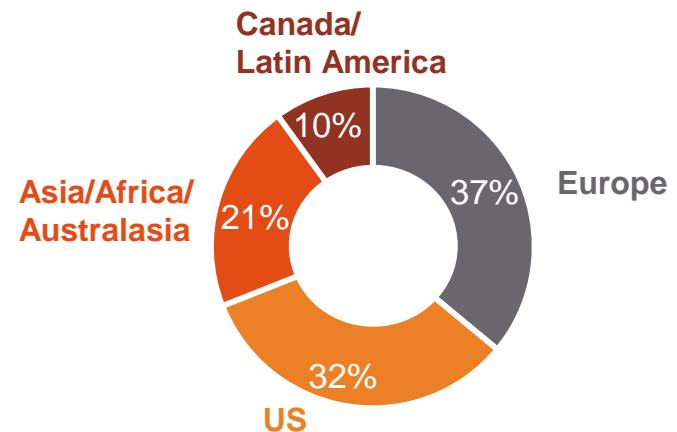
One of **25 largest** companies by market capitalization

Among **most respected** companies globally

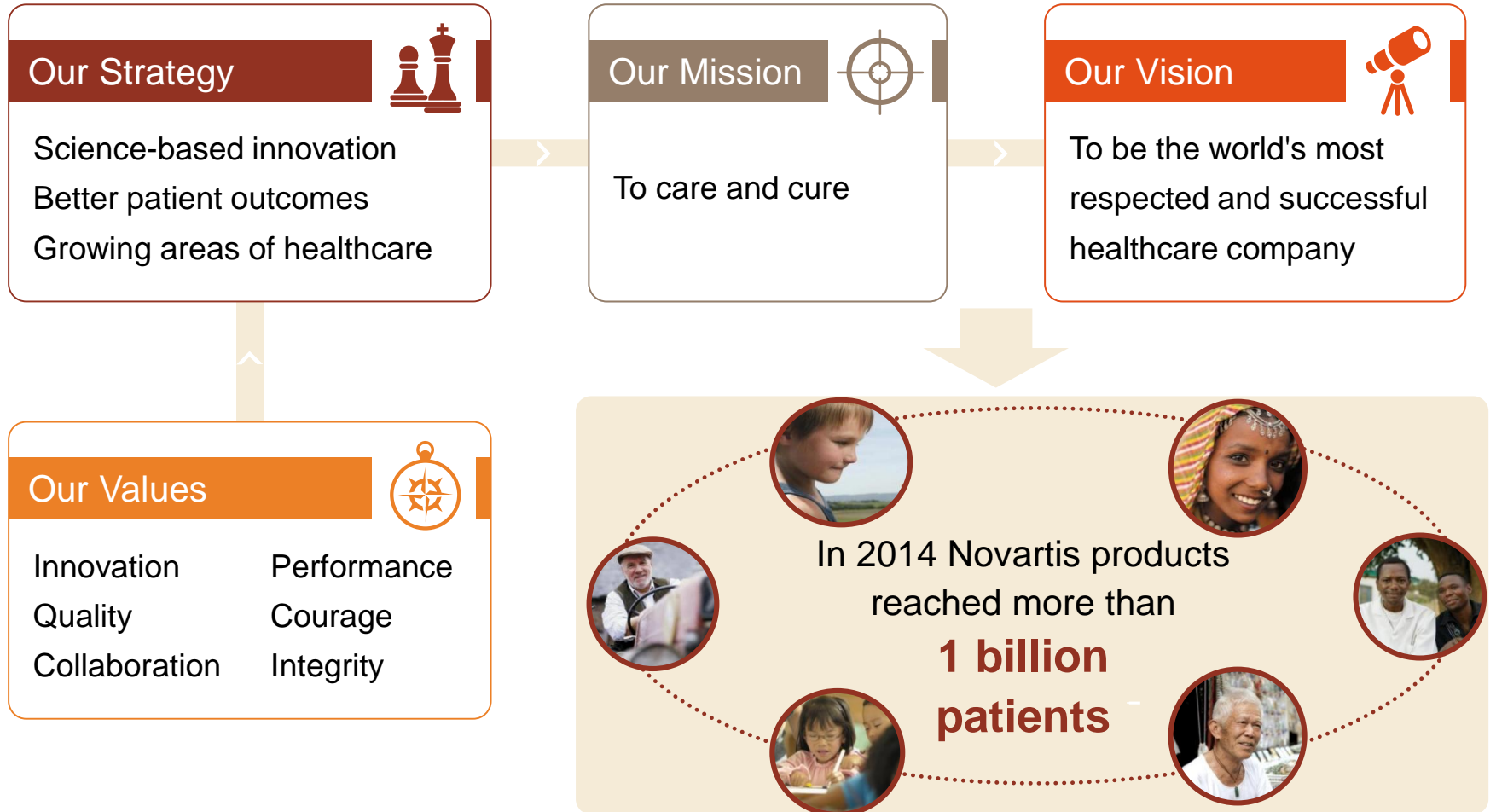
Key figures

2014	USD billion
Net sales:	58.0
Net income:	10.3
R&D investment:	9.9

Sales by region 2014



Always with a focus on patients



We have an industry-leading pipeline

FDA breakthrough therapy designation for two drug candidates in 2014, making **5** in total

Studying **25** biological pathways associated with cancer progression

20 compounds in development for a range of disorders in dermatology and rheumatology

6 Sandoz biosimilars in Phase III trials or undergoing registration

13 major approvals in US, EU, Japan in 2014



Innovation overview 2014

9.9 USD billion invested in research and development of new drugs and medical devices

More than **200** R&D projects underway, 135 of them in the Pharmaceuticals Division

The Novartis Institutes for BioMedical Research (NIBR) is the research engine of Novartis, with more than **6,000** scientists

Focus on **molecular pathways** shared by several diseases

Research-to-Development transition determined by **fast and rigorous** “proof-of-concept” trials

Strategic alliances with academia and other companies **strengthen** our preclinical pipeline

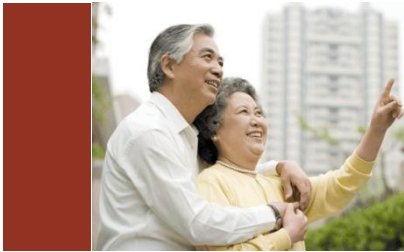


Cambridge global NIBR headquarters



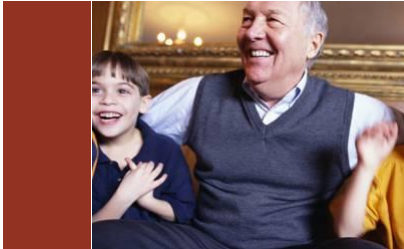
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Facing huge changes in Healthcare



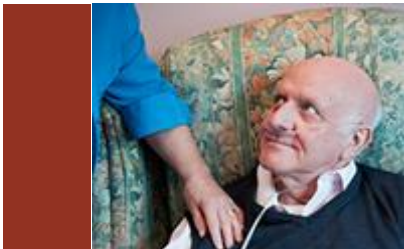
Growing populations

Almost 1 billion more people are expected to inhabit the planet by 2025, driving up demand for healthcare worldwide



Aging

By 2025 there will be 500 million more people aged over 50+, posing a challenge for governments and health insurers as they try to keep spending in check



Rise of chronic diseases

Chronic illnesses such as cancer and heart disease are on the rise. By 2025 they will account for 70% of all illnesses as the population ages and standards of living improve



Increasing demand for healthcare

These factors and the accelerating pace of innovation will contribute to increasing demand for healthcare. By 2025 global healthcare spending is expected to more than double to over USD 15 trillion

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Impact of Innovative Medicines

CONTRIBUTION OF INNOVATIVE MEDICINES TO INCREASE IN LIFE EXPECTANCY (2000-2009)

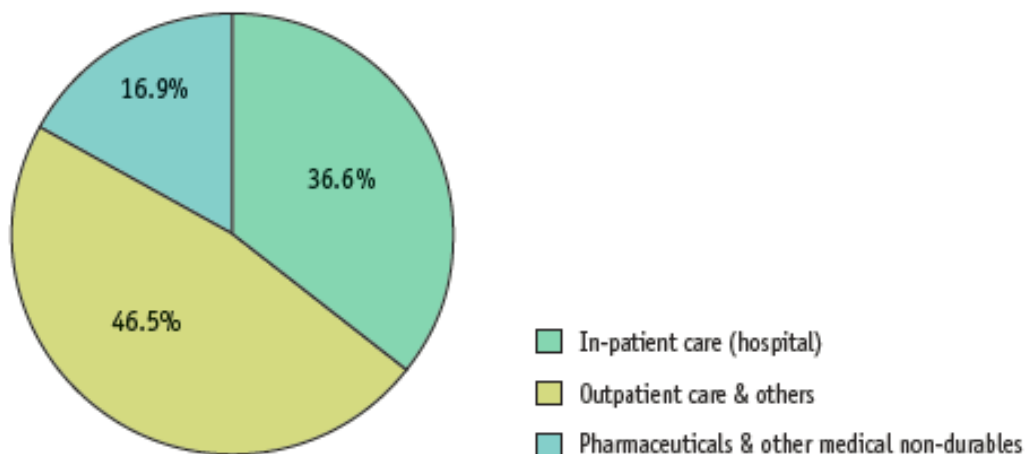


Source: Lichtenberg, F: Pharmaceutical innovation and longevity growth in 30 developing OECD and high-income countries, 2000-2009 (2012)

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Medicine Costs

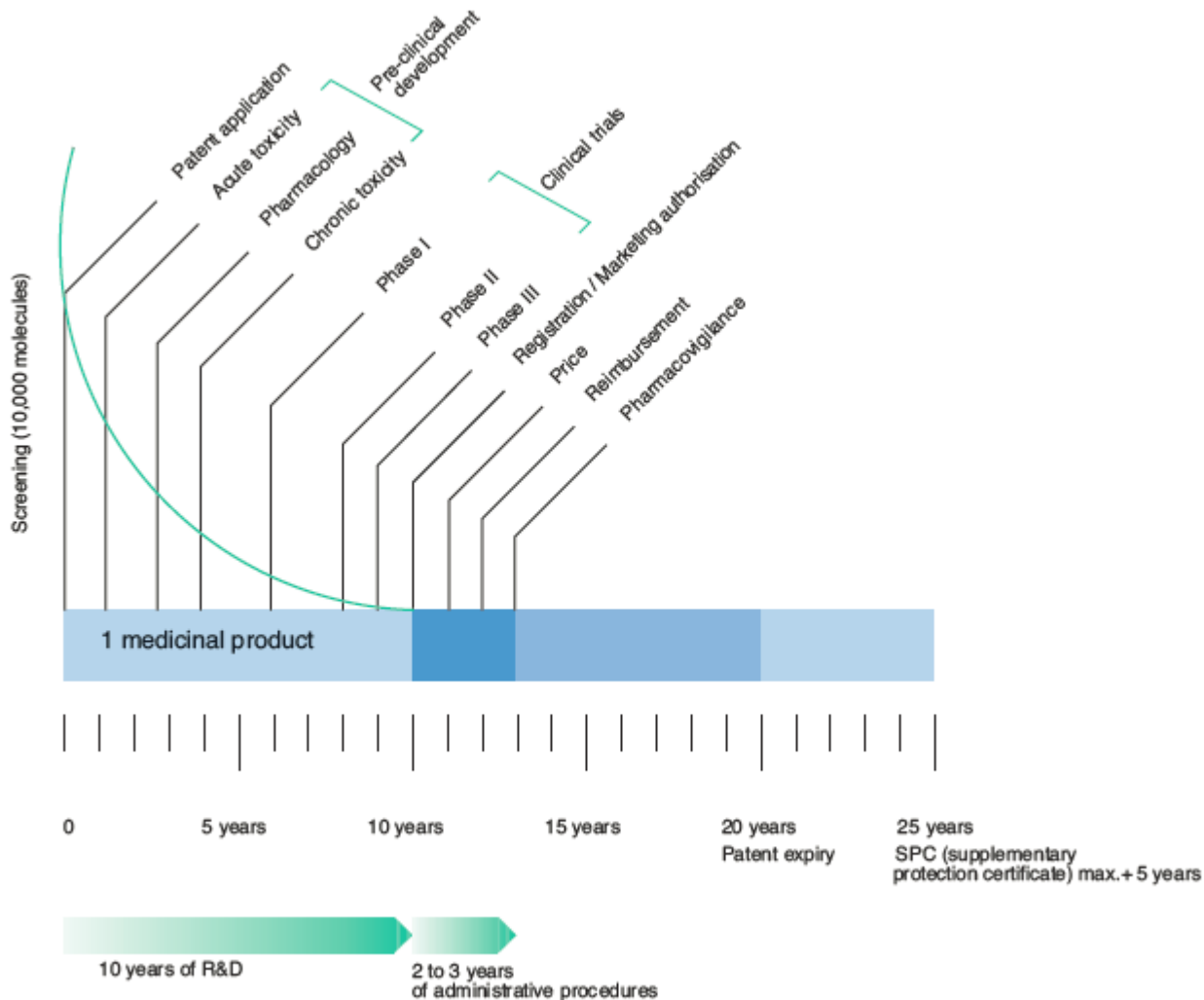
BREAKDOWN OF TOTAL HEALTH EXPENDITURE IN EUROPE – 2012



Source: OECD Health Data 2014, November 2014 – EFPIA calculations (non-weighted average for 21 EU & EFTA countries)

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Phases of the research & development process



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Need for Additional Information/Data

THE PAST

RCT

Controlled trials,
manufacturer led

Few

Few evaluators at launch, mostly
regulators and large payers



Efficacy and Safety

Initial view of
benefit-risk

THE PRESENT

RCT and RWE

Shift to secondary patient-level
data across sources

Many

Many groups over time
including clinical and
small payers



Almost everything

Insights on environment,
outcomes, costs,
comparative effectiveness

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What is real-world evidence/data?

- RWE is based on data collected in clinical practice and is defined as follows

Data used for clinical, coverage and payment decision-making that are not collected in conventional RCTs

understanding healthcare data collected under real-life



European Commission
Working Group on
Relative Effectiveness



ISPOR Task Force

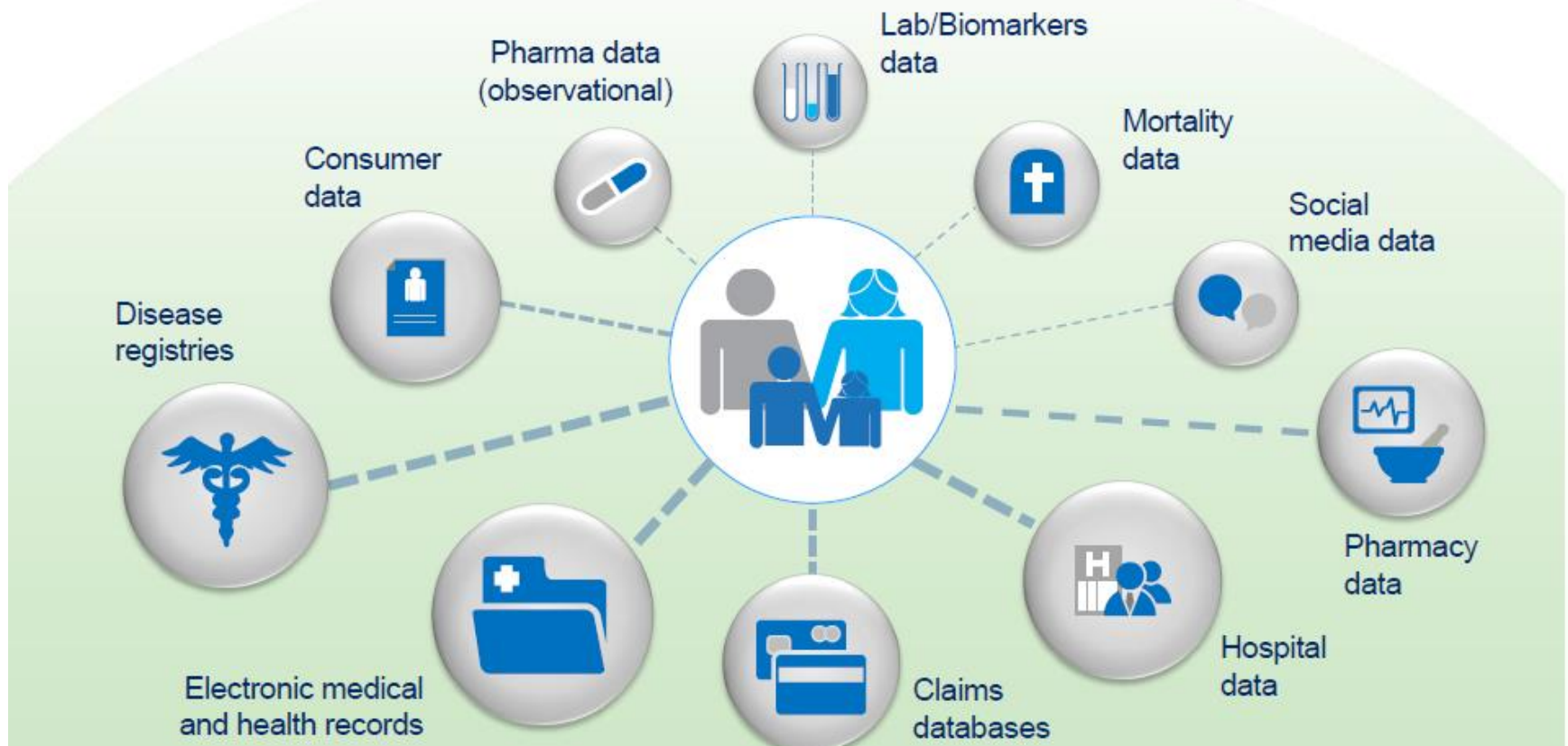
Data that are collected outside the controlled constraints of conventional RCTs that describe what is really happening in everyday normal clinical practice

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Source for RWE/RWD

“90% of the world's data has been produced in just the last two years”
- U.S. Chamber of Commerce Foundation

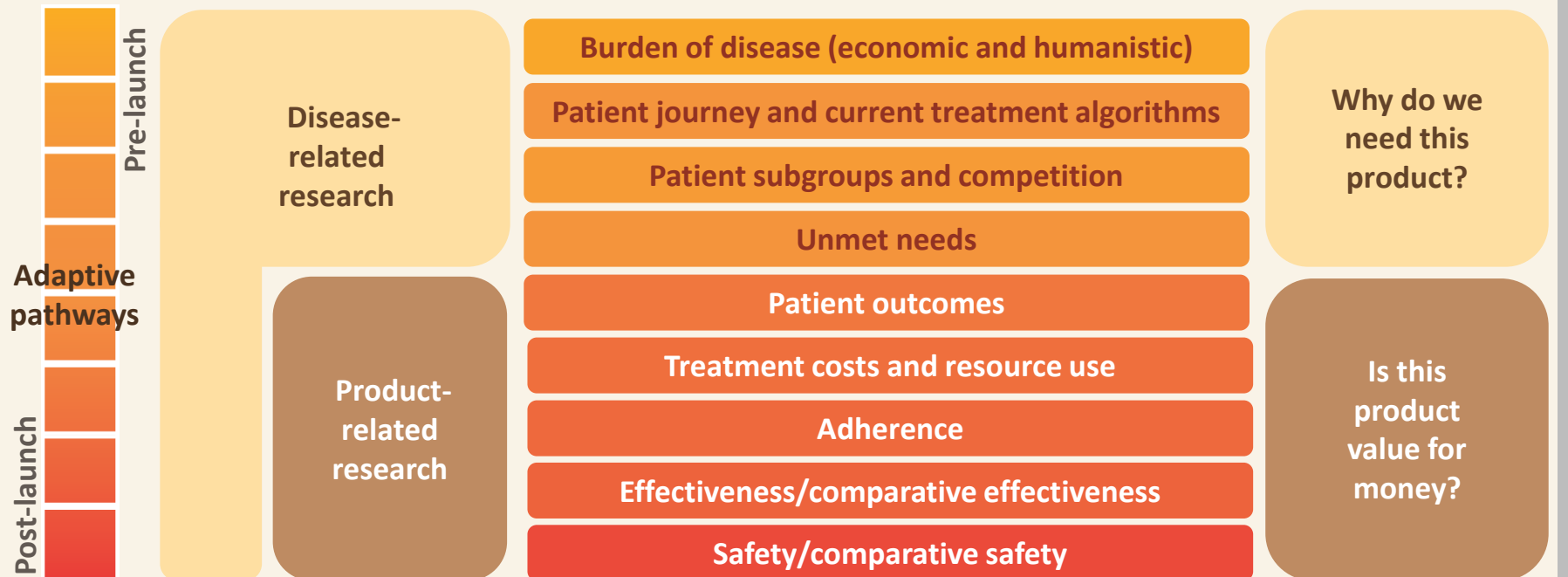
RWD is PATIENT data = BIG DATA



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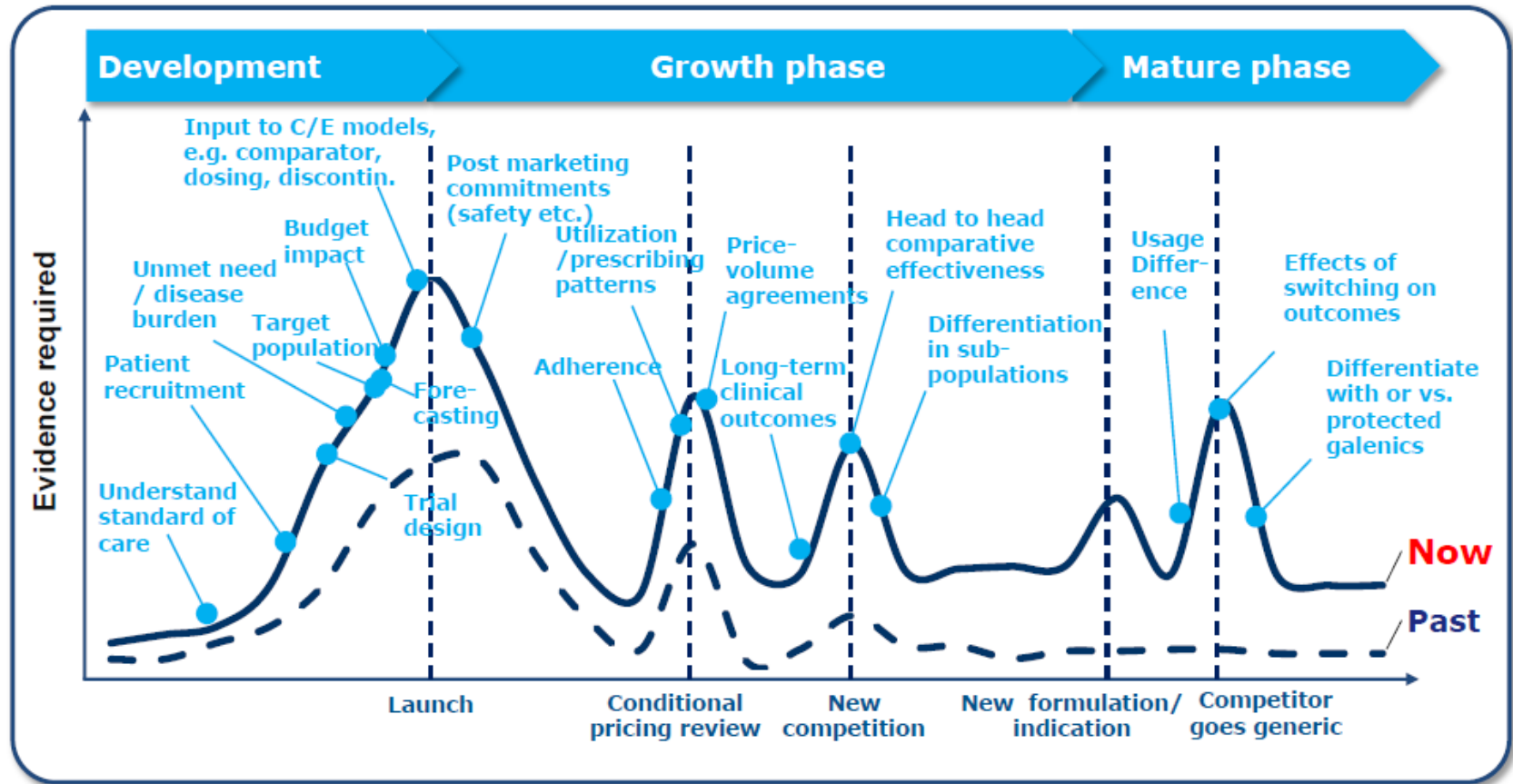
Issues that RWE can address

- RWE generation from a range of real-world data sources during the life cycle of a product can address a wider range of outcomes and research questions than RCTs alone



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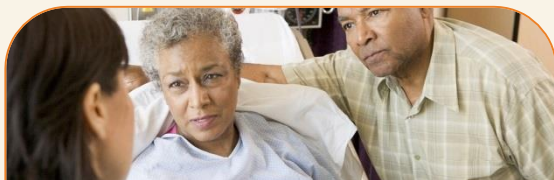
Timing for RWE/RWD collection



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RWE can benefit all stakeholders

Patients



- Facilitating early access to medicines, e.g. through adaptive pathway designs
- Improving understanding of effectiveness in patient populations that are often excluded from clinical trials
- Building knowledge of rare diseases

Payers



- Enabling comparisons of effectiveness of different interventions and assessments of value
- Supporting risk-sharing models based on outcomes in a real-world setting

Regulators



- Informing decisions on conditional approval or expansion of an adaptive licence to a broader population
- Providing opportunities for trade-offs, such as earlier access before making a full assessment of the real-world benefit

Healthcare systems



- Optimizing identification and delivery of effective and affordable treatments
- Improving healthcare delivery and disease outcomes
- Improving understanding of outcomes and costs/savings in the local setting

Manufacturers



- Informing planning and budgeting for development and commercialization
- Allowing confirmation of medical and economic value in the real world

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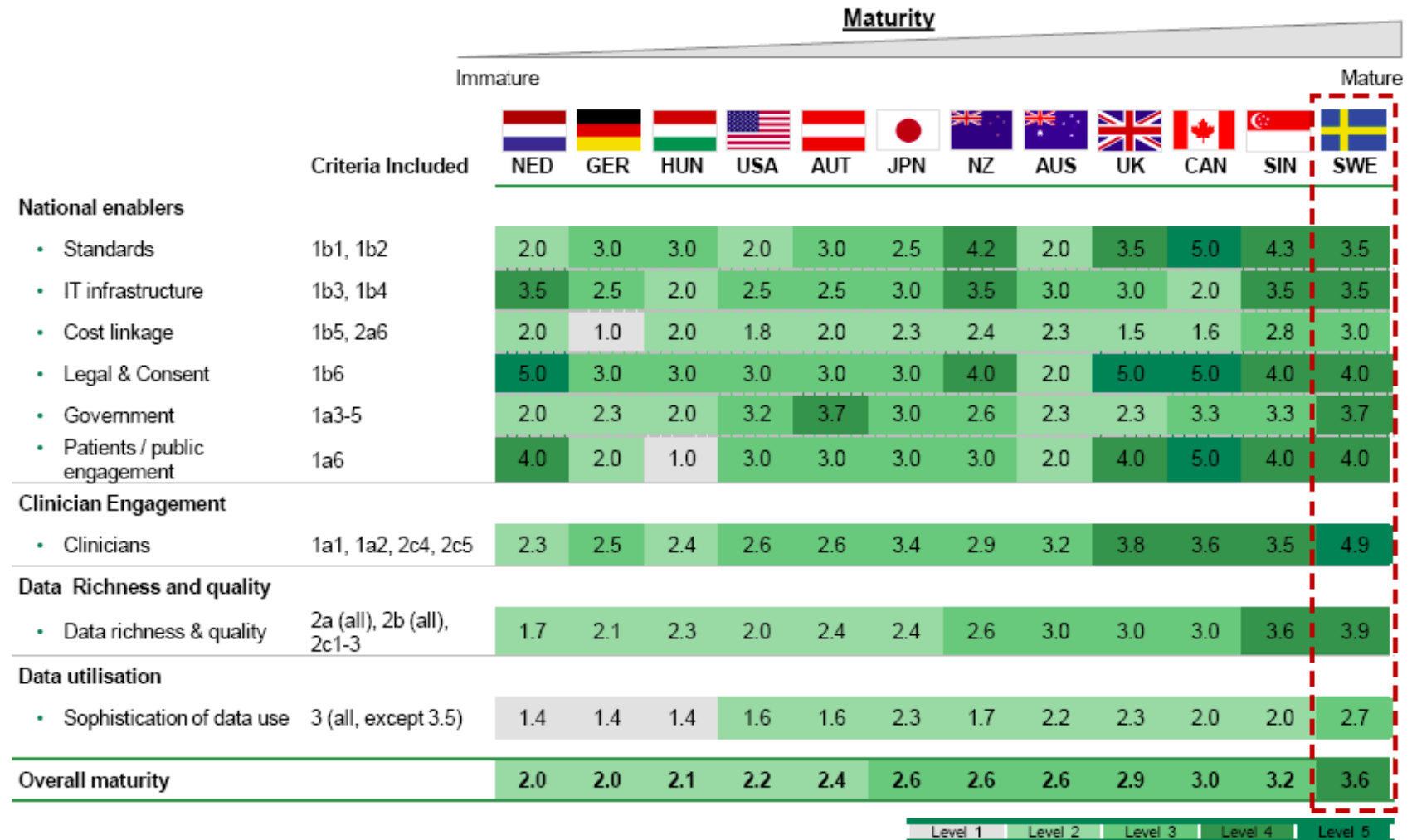
Nordic Opportunities for RWE/RWD



- Well structured public **health care system**
- Universal implementation of **EMRs** since 1990's
- National and population-based health **registers** in the region since 1950's
- **Common** set-up of health care, health information and classification systems
- Strong **academic** hubs to collaborate with in the region

Maturity for RWE and Value Based Health Care

Source: BCG report; Progress toward value-based health care, 2012

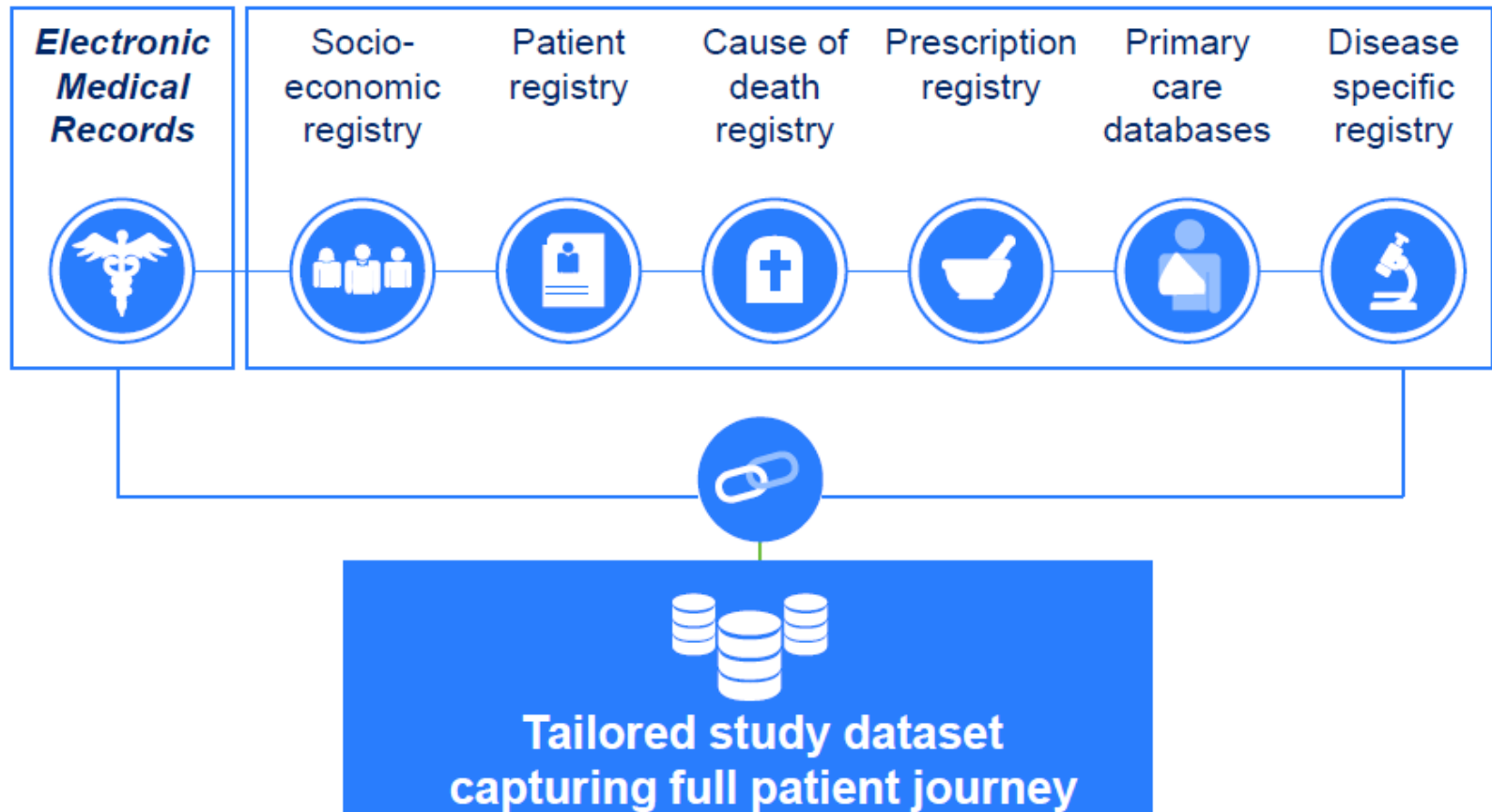


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RWD from Nordic Registries

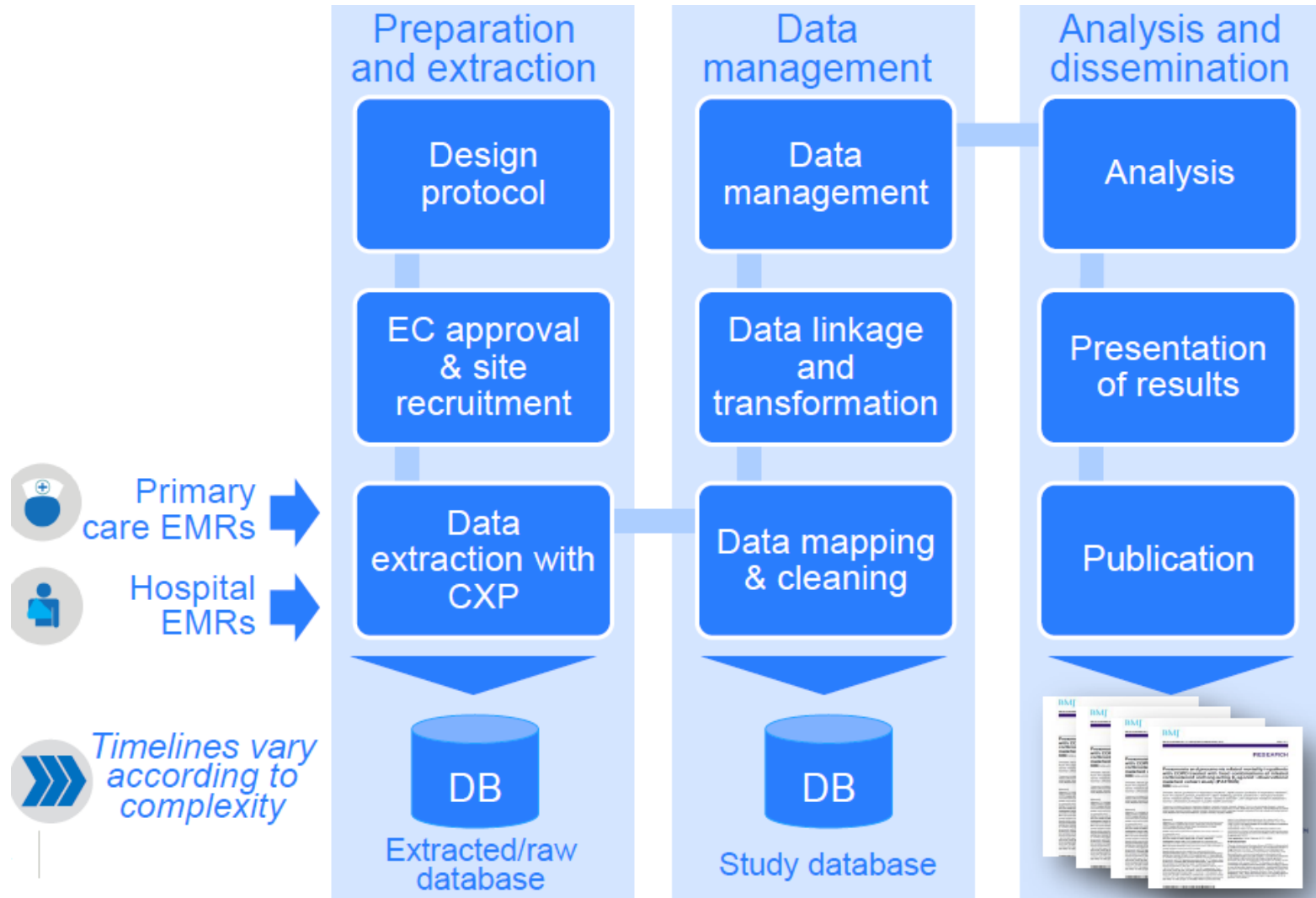


Via CXP



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Collection of RWE/RWD



Example: The PATHOS study (Sweden)

Overall rationale



To better **understand the epidemiology** of COPD including treatment practice, co-morbidity and mortality during the last decade

Study Objectives



Describe the long-term **epidemiology** of COPD and the health care **structure** in primary care



Assess the **comparative effectiveness** between fixed ICS/LABA combination treatment in COPD



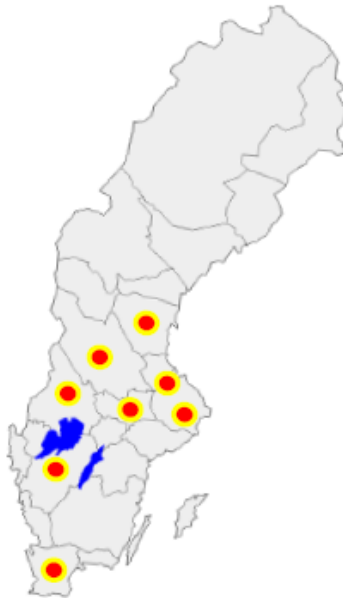
To investigate occurrence of **pneumonia** in a COPD population treated with fixed combination



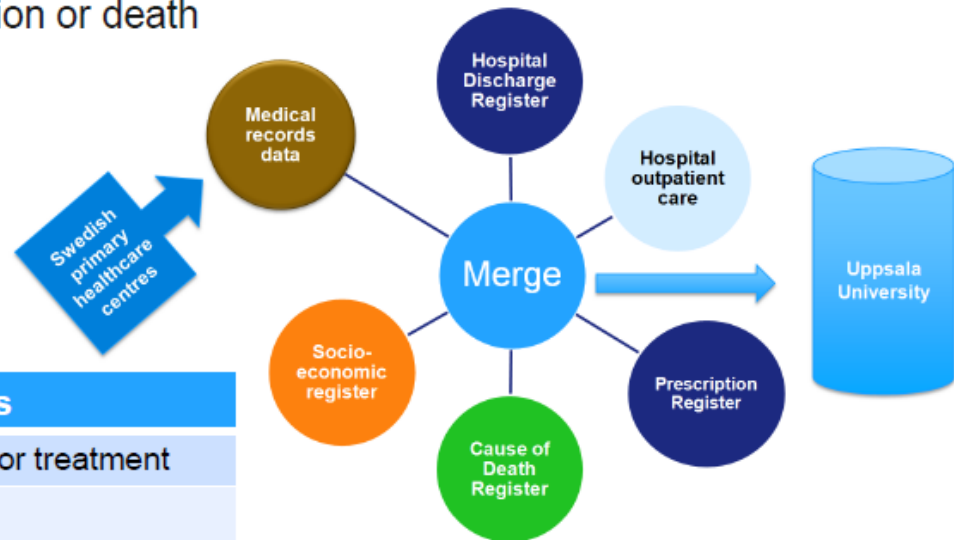
Estimate the **cost-effectiveness** amongst fixed combination treatments in COPD

The PATHOS study design

Retrospective, observational, population-based, propensity score matched study



- EMR data from 76 primary health care centres linked with data from Swedish registries (8% of the Swedish pop)
- Patients followed from January 1999 to December 2009, or end of treatment with fixed ICS/LABA combination therapy, emigration or death



The PATHOS study in numbers

27 934	Patients with COPD or treatment
21 361	COPD patients
190 000	Visits at hospital
3 500 000	Contacts with primary care
1 300 000	Prescriptions

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The PATHOS study

COPD Case study

The PATHOS study



Background

- COPD associated with considerable morbidity, mortality; pneumonia is a frequent complication
- To prevent exacerbations, patients are frequently prescribed fixed-dose ICS/LABA combinations
- Pharma client wanted to develop credible insights about the best treatment course for COPD patients to work with stakeholders to improve guidelines, COPD management

The Study

Retrospective, observational, population-based study of 21,361 COPD patients in Sweden over 11 years to focus on

- Prevalence and incidence
- Co-morbidities
- Disease management
- Complete clinical data on each patient
- Mortality and life expectancy

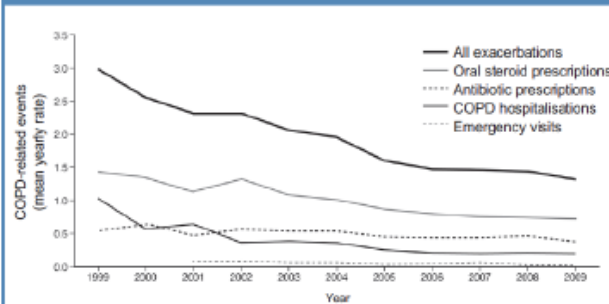
The results

- Improved outcomes
 - Earlier diagnosis
 - Primary care focus
 - Changed treatment options
 - Decreased exacerbations
- TA-specific impact on exacerbations, pneumonia-related mortality
- 4 publications in BMJ and J Int Medicine, Prim Care Resp Journal
- 5 scientific abstracts presented

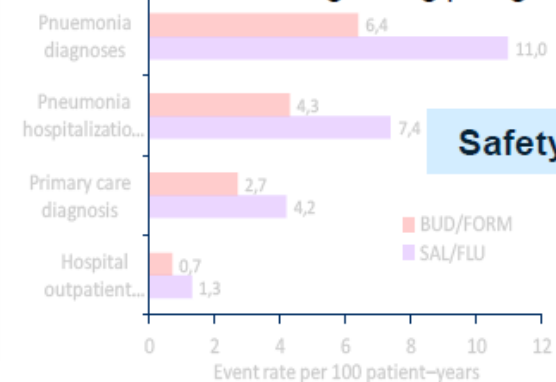
The PATHOS study results showed better disease management, safety, and comparative effectiveness

Disease management evolution over 11 years showed dramatic decrease in number of exacerbations

Figure 3. Chronic obstructive pulmonary disease (COPD)-related events (mean yearly rate) during the 11-year study period (1999–2009)



Pneumonia and related mortality in COPD treated with fixed combinations of inhaled corticosteroid and long acting β 2 agonist

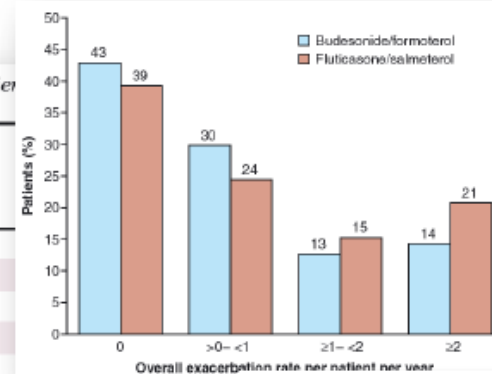


Safety

Comparative effectiveness Combination of budesonide/formoterol more effective than fluticasone/salmeterol in preventing exacerbations in COPD

Table 2 Yearly occurrence of events among pairwise (1 : 1) propensity score-matched populations of COPD patients with budesonide/formoterol versus fluticasone/salmeterol

Variable	Fluticasone/salmeterol (n = 2734)	Budesonide/formoterol (n = 2734)	Treatment contrast ^a
Events, per patient-year	Mean (95% CI)	Mean (95% CI)	Rate ratio (95% CI)
All exacerbations	1.09 (1.05–1.14)	0.80 (0.77–0.84)	0.74 (0.69–0.79)
COPD hospitalizations	0.21 (0.20–0.23)	0.15 (0.142–0.163)	0.71 (0.65–0.78)
COPD-related hospital stay, days	0.95 (0.88–1.02)	0.63 (0.58–0.67)	0.66 (0.62–0.71)
Emergency visits	0.034 (0.031–0.037)	0.027 (0.025–0.030)	0.79 (0.71–0.89)
Oral steroid use	0.85 (0.81–0.90)	0.63 (0.60–0.67)	0.74 (0.68–0.81)
Antibiotic use	0.54 (0.52–0.57)	0.38 (0.37–0.40)	0.70 (0.66–0.75)



<0.0001

Jansson et al. *BMJ* 2013
Larsson et al *JIM* 2013
Ställberg et al. 2014

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Questions/Projects (1)

1) How to capture patient journal data from Primary Care & Hospitals to Registries? How many patients can that cover?

- Diabetes
- Hypertension
- Heart failure

Possible information sources:

- COPD, IMS Health, Pygargus CPX 3.0?
- Osteoarthritis?
- Nodes, Gothia Forum?

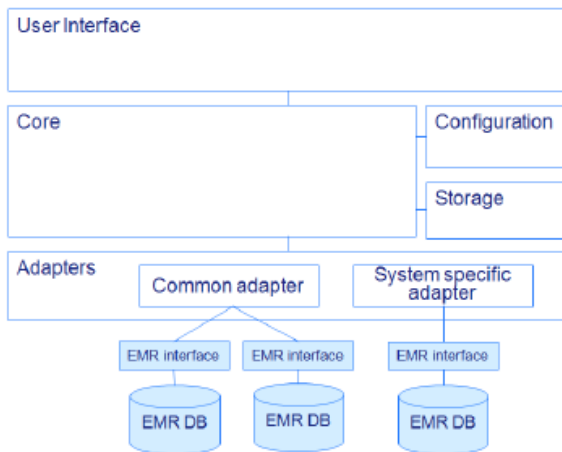
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Additional Information

Pygargus CXP 3.0

- ✓ Application for extracting relevant data from EMRs
- ✓ Modern extraction tool that delivers anonymized patient data for clinical studies
- ✓ Supports extraction from different health care (EMR) systems at hospitals, GPs, psychiatric care, geriatrics, spirometry, E-archive systems, etc
- ✓ Makes the complex task of collecting data from different sources into a single, standardized and quick task
- ✓ International development focus

CXP 3.0 – Architecture



CXP 3.0 – Supported information objects

- Demographics
- Diagnoses
- Encounter Diagnoses
- Measurements
- Case Notes
- Medications
- Prescriptions
- Vaccinations
- Laboratory results
- Radiology results
- Pathology results
- Encounters
- Procedures
- Consultation referrals
- Sickleaves
- Chemotherapy

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Questions/Projects (2)

2) How to use Registries to screen patients for participation into clinical trials?

Possible information sources:

- Cancer registry Norway