ROLE OF REAL WORLD EVIDENCE TO FOSTER ACCESS TO INNOVATIVE DIABETES MEDICINE

Agathe Le Lay
Head of EU-HEOR
Novo Nordisk Region Europe, Copenhagen

EMA-EuropaBio Information Day, London, 22nd November 2016

novo nordisk®

PAYERS EXPERIENCING INCREASINGLY TIGHT BUDGETS ARE LOOKING FOR WAYS TO CURB COSTS

The Telegraph

Home Video News World Sport Finance Comment Culture Travel Life Women

Politics | Investigations | Obits | Education | Science | Earth | Weather | Health | Royal | Celeb

Thousands of cancer patients to be denied treatment

Common drugs for breast, bowel, prostate, pancreatic and blood cancer will no longer be funded by the NHS following sweeping cutbacks

The Economist Intelligence Unit

Country Industry Risk Data Special reports

Healthcare

Ageing populations and tight budgets

Over-65s now account for around 10% of the world's population. This demographic shift, combined with an economic slowdown, is threatening the stability of healthcare systems in many developed countries, and some developing ones.

INTERNATIONAL THE NEWS

Latest News | Today's Paper |

Pakistan | World | Sports | Business | Weird | Entertainment |

Budget for Health Insurance Scheme cut to Rs9 billion

Waseem Abbasi - Thursday, April 02, 2015 - From Print Edition

ISLAMABAD: While the PML-N government has managed to comple

FOR HEALTHCARE LEADERS HSJ

ALASTAIR MCLELLAN

One minister should join the hundreds of DH staff who will be made redundant

SECTORS TOPICS HSJ LOCAL COMMENT HSJ KNOWLEDGE MORE... JOBS SUBSCRIBE

HSJ Live 24.07.2015: 'Budget constraint' to blame for hepatitis C drug delays, says NHS England director

24 JULY, 2015

THE GLOBE AND MAIL

Enter a term, stock symbol or company name

BARRIE MCKENNA

Health care costs our single most pressing budget item

BARRIE MCKENNA

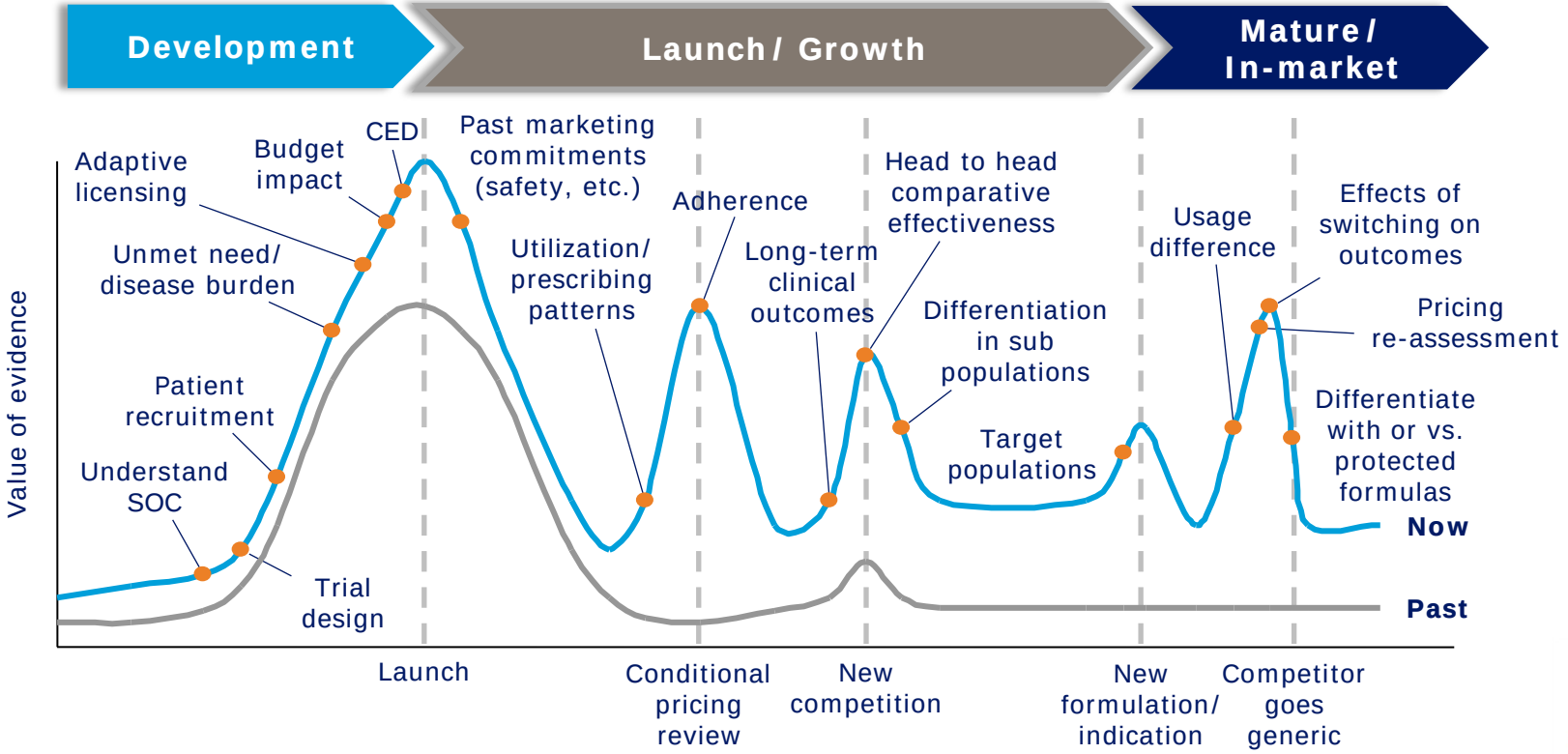
The Globe and Mail

Published Sunday, Feb. 27, 2011 5:38PM EST

Last updated Monday, Sep. 10, 2012 10:59AM EDT

novo nordisk®

EVIDENCE REQUIREMENTS TO OBTAIN AND MAINTAIN MARKET ACCESS ARE HENCE INCREASING



Source: QuintilesIMS

WHILE CLINICAL TRIALS ARE CONSIDERED "GOLD STANDARD", OUTCOMES MAY NOT REFLECT REAL-WORLD CONDITIONS

COMMON ISSUES

- **Too small** to detect drug adverse events
- May exclude **vulnerable groups** such as the elderly, children, pregnant women, chronic disease patients
- Often too **short** to assess long-term safety or effectiveness
- Certain trials may be **unethical**

novo nordisk®

EVIDENCE PREFERENCES AND REQUIREMENTS DIFFER BETWEEN LOCAL AND CENTRALISED DECISION-MAKERS

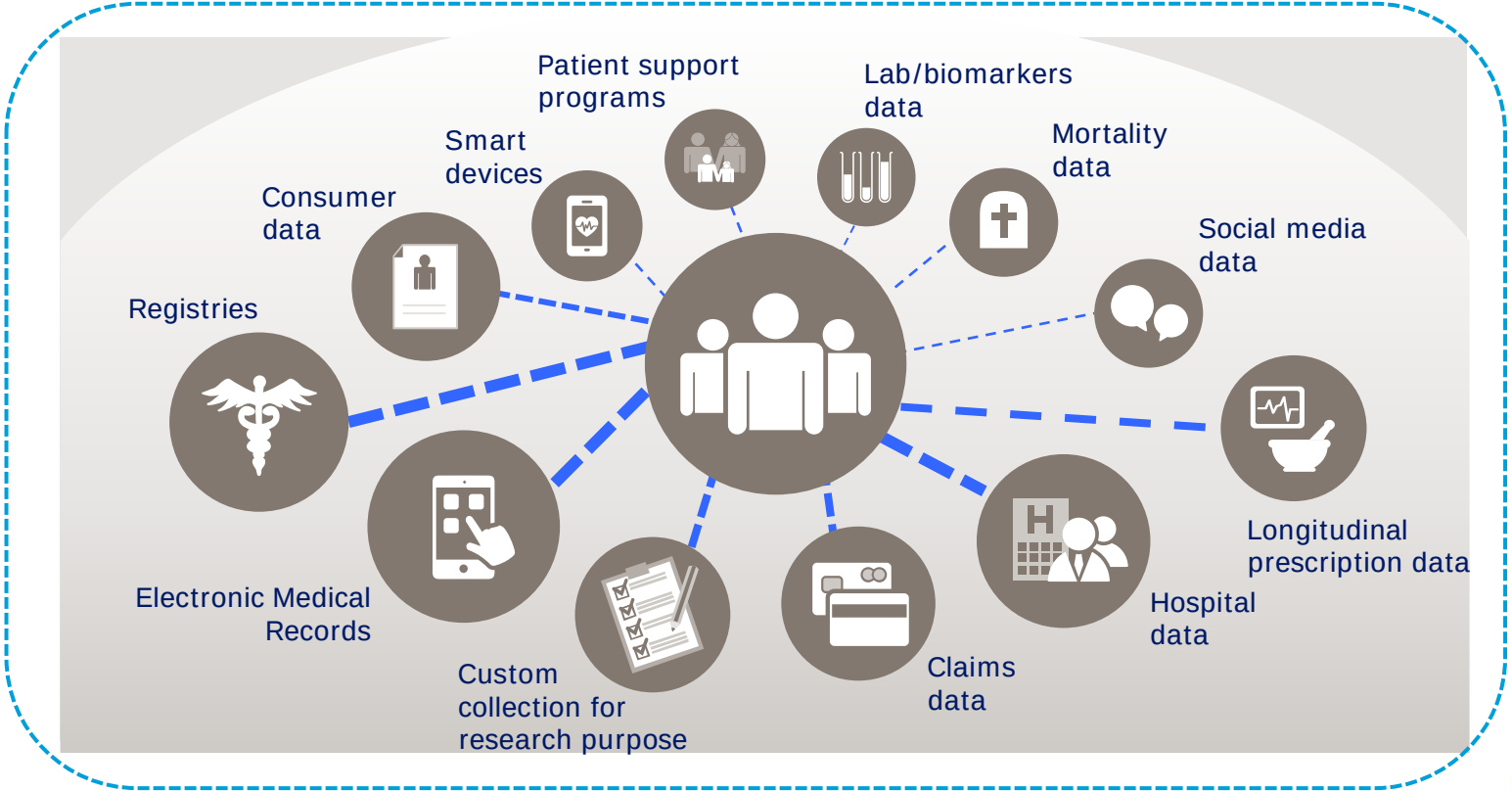
Clinical trial data is the core requirement for all agencies. Safety data, quality of life data and economic analyses are also commonly requested but not required by all agencies, with the latter being a requirement for national HTA agencies only

Agency accept/offer guidance on evidence type	EMA	EUnetHTA	HAS	G-BA	AIFA	ZINL	Spain	TLV	NICE	SMC
Trial data	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓
Indirect comparison	NS	✓	✓	✓	NS	NS	NS	✓	✓	✓
Systematic Reviews/ Meta-analysis	NS	✓	✓	✓	✓	NS	NS	✓	✓	✓
Real-world evidence	NS	NS	✓	NS	NS	NS	NS	NS	✓	NS
Safety data	✓	✓	✓	✓	✓	✓	✓	NS	✓	✓
QoL data	✓	✓	✓	✓	NS	✓	✓	NS	✓	✓
Economic evidence	NS	NS	✓	NS	✓	✓	✓	✓	✓	✓
Other sources (e.g. dose ranging studies)	✓	NS	✓	NS	✓	✓	✓	NS	✓	NS

Source: DecisionResources Group, 2016

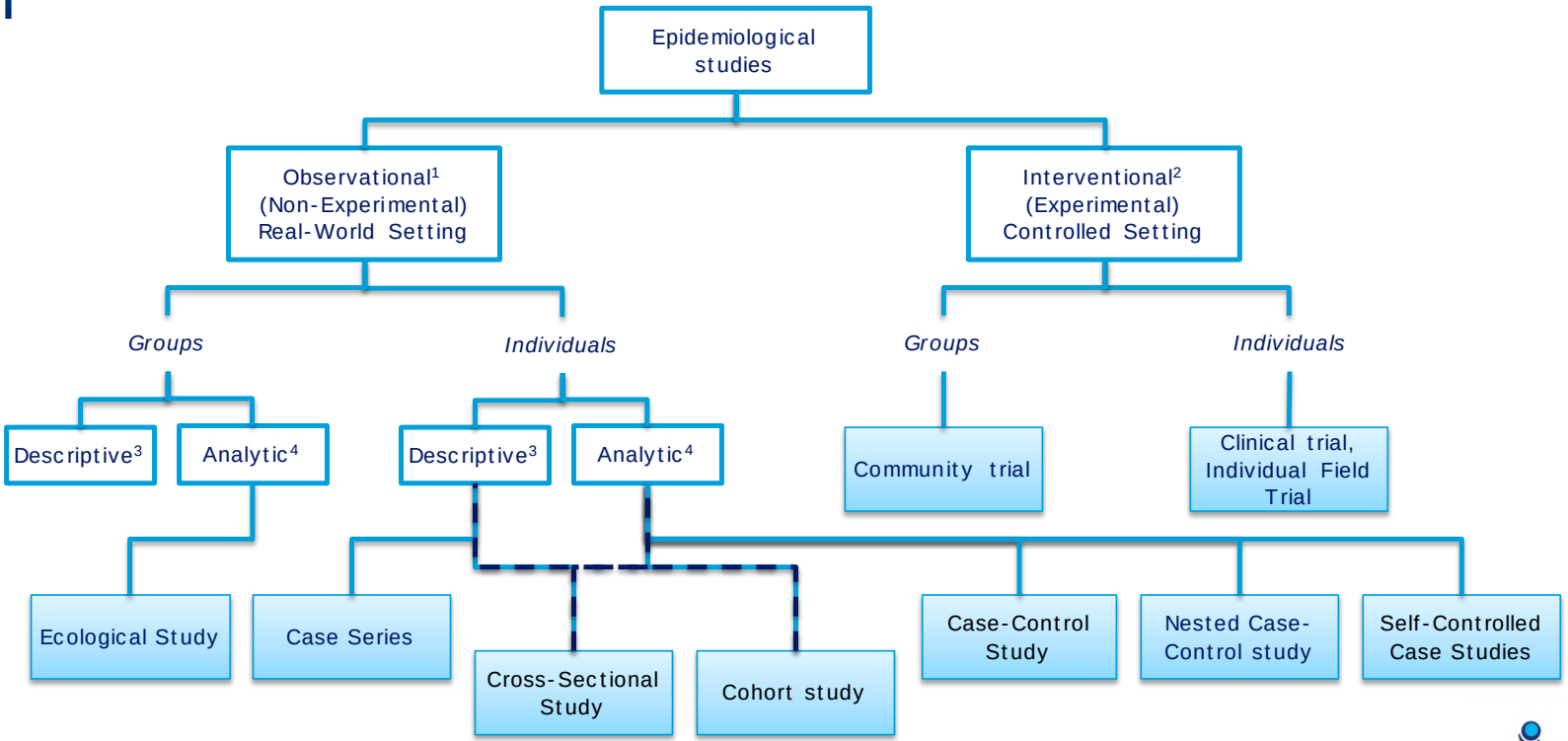
novo nordisk®

COMPLEXITY: REAL-WORLD DATA CAN COME FROM A VARIETY OF SOURCES



Source: QuintilesIMS

AND A WIDE RANGE OF EPIDEMIOLOGICAL STUDY DESIGNS EXIST



1. Observe only
2. Allocate exposure
3. Exposure or outcome
4. Exposure and outcome

novo nordisk®

ANTICIPATING FUTURE NEEDS EARLY MAY RESULT IN SYNERGIES AND POTENTIALLY STRONGER EVIDENCE

BEST PRACTICE

- **Anticipate** gaps to be filled with RWE 3-5 years ahead
- **Realise synergies** between products and studies
  - Data purchases, collaborations, alignment of protocols
- **Start investing early** in accessing and enhancing “grey” RWD if needed
- **Prioritize** based on budget and business need

BENEFITS

- **Better data** for retrospective studies through early planning
- Overall **cost savings**
- **Faster** turn-around times

novo nordisk®

CASE STUDY OF A SINGLE ARM OBSERVATIONAL STUDY TO SUPPORT MARKET ACCESS

- Insulin degludec was evaluated in a large **3a clinical trial programme** in adult patients with type 1 and type 2 diabetes (BEGIN®).

- Key benefits of this new therapeutic alternative: reduction of the risk of hypoglycaemia. However, **patients experiencing hypoglycaemia** who may benefit from the new treatment were, for regulatory reasons, **not included** in the initial clinical trial program.

- **Real-world evidence (RWE)** complemented the evidence base established by the ph3 program and **helped demonstrate the product value in a subgroup** of patients relevant to payers.

novo nordisk®

CONCLUSION

- This case study exemplifies how the use of RWE helped local payers (e.g. local formulary committees) in getting **additional insights into the clinical benefits** of a novel intervention in addition to evidence from the initial RCT program.

- Demonstrating **product value in real clinical practice** is increasingly important to achieve successful reimbursement, and central to evaluating the costs and benefits of health technologies competing for funding decisions.

- However, significant **challenges in the development and acceptance of RWE** remain to be addressed.

novo nordisk®

POINTS FOR CONSIDERATION

- Challenges with RWD: **fragmented** sources of data, **methodologies** & evidence standards
- **Acceptability/validity** of RWE: early engagement with payers & regulators e.g. payer partnerships
- Increasing schemes of **coverage with (RW or RCT-based) evidence development** to manage uncertainty and information asymmetry

novo nordisk®

THANK YOU
ANY QUESTIONS?

novo nordisk®

SHORT BIOGRAPHY

- Agathe Le Lay holds a PhD in Health Economics from the University Claude Bernard Lyon 1 and has more than 10 years of experience in developing and leading Health Economics & Market Access strategies related to both product development and life cycle management. Agathe is currently heading the European Health Economics & Outcomes Research team at Novo Nordisk, based in Copenhagen, Denmark.

novo nordisk®