BBS EFSPI
Registry Studies and Health Technology Assessment (HTA)

Case studies using Registry Data for HTA in Scandinavia
Presentation by Quantify Research
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Disclosures

I have the following relevant financial relationships to disclose:

- CEO and board member of Quantify Research AB and affiliates
- Own stock and stock options in Quantify Research AB

Agenda

1. Nordic RWD assets – the Nordic goldmine
2. How is RWD used in Nordic HTA?

Nordic RWD assets

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Nordic data – societal context for the "goldmine"

The Nordic data landscape offers unique and global un-paralleled opportunities for RWE studies

Personal identification numbers and high trust in public institutions

- All residents have personal identification number, which is pervasive across society
- Linkage of patient-level data across datasets
- Data managed governments and authorities

Tradition of information archiving and transparency

- Decades of follow-up data
- In principle, all data is publicly available (required: ethics, approvals, experience, etc.)

Mandatory registration without opt-out and extensive welfare system

- Secondary data is administrative
- Complete coverage of populations across many dimensions of health and society
- Loss to follow-up only due to death or migration

Nordic data – what's in the goldmine?

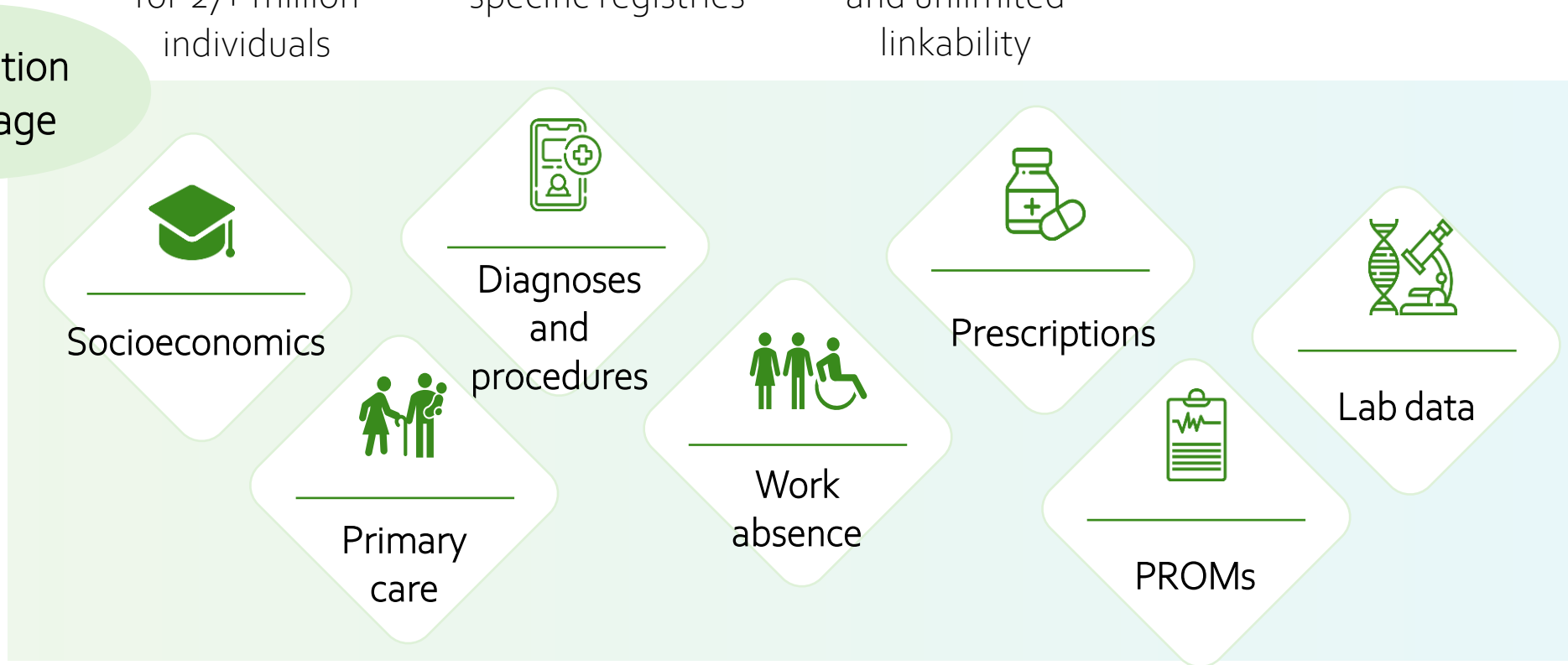
Patient level data for 27+ million individuals

100+ disease-specific registries

Unique coverage and unlimited linkability

Low extraction cost

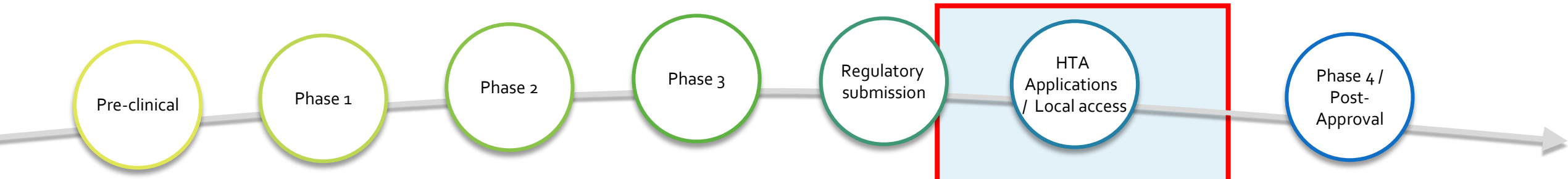
Population coverage



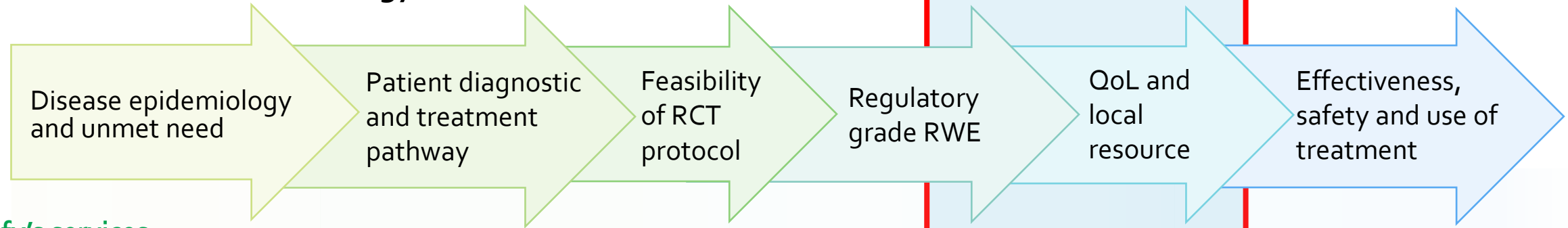
How is RWD used in Nordic HTA?

Today and tomorrow

RWE can be used throughout a Product's Life Cycle



Common RWE Evidence & Strategy needs



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RWE Strategy to address (local) regulatory/payer needs

Lead, design and execute RWE studies on
- epidemiology,
- unmet needs and future trends,
- DDI and treatment patterns

Feasibility of trial protocols
- Expected sample size
- Site identification
- SOC

Regulatory grade RWE
- Synthetic control arms
- rRCT

RWE studies for local HTA support
- Resource use
- Costs
- QoL
- Patient pathways
- Establish SoC
- Size of indicated patient group

PASS

Treatment persistence

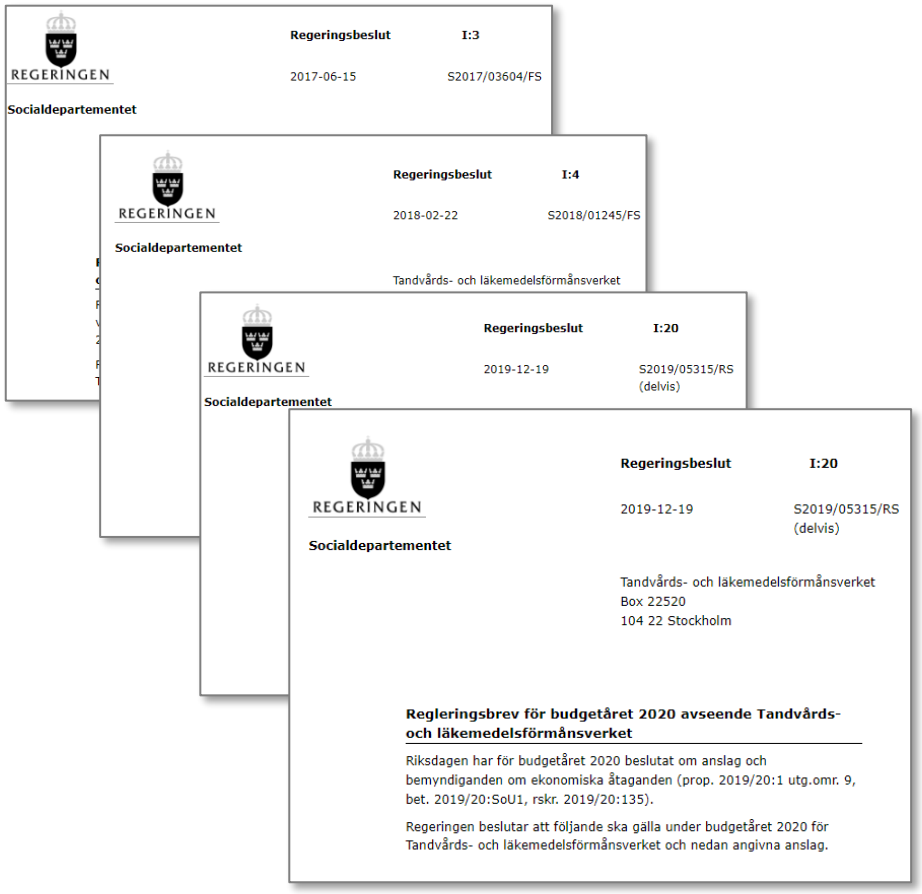
Comparator effectiveness with new comparators

Support for extended indications

Off-label use

RWE is high priority in the Nordics

Government Offices of Sweden
Ministry of Health and Social Affairs



Assignments from Swedish Ministry of Health and Social Affairs to the Swedish HTA authority TLV involving RWE

- Treatment effect in clinlical practice (2017)
- Follow-up oncology pharmaceuticals (2017)
- Follow-up oncology- and other pharmaceuticals through alternative data sources (2019)
- Develop health economic evaluations for precision medicine and examine possible payment models for ATMP (2020)
- Evaluate and develop the monitioring possibilities through the national IT-platform for health (2020)
- Evaluation of pharmaceuticals and their treatment effects in clinical practice using alternative data sources with focus on oncology (2021)

Source: https://www.esv.se/statsliggaren/regleringsbrev/?RBID=20589

Typical uses of RWE in Nordic market access

The need of RWE in Market Access

Informing submission documentation

- Patient pathways
- HCRU, PROs (e.g. QoL)
- Comparator assessment and selection
- Patient characteristics and sick leave

Indirect treatment comparison using RWD

- Assessment of RW patients vs trial population
- Synthetic control arms

Follow-up requirements from HTA agencies

- Conditional approval
- Possible off-label use
- Performance-based managed entry agreements

Options for RWE studies in the Nordics

1. Full scale RWE studies in all the Nordics with detailed patient level data (timeline ca. 1 year)
2. Aggregated statistics with reduced detail but no need for ethics approval (timeline ca. 3 months)

Real-world example of RWE to support reimbursement

The following evidence was missing for dossier and model:

- ✓Incidence and prevalence of disease
  - ➢ Budget impact
- ✓Frequency of certain comedication use
  - ➢ HCRU
- ✓Number of hospitalization days in RW
  - ✓ HCRU
- ✓Sequence and distribution of treatment
  - ➢ Model design to reflect patient pathways
- ✓Patient age at diagnosis and treatment
  - ✓ Model design to reflect timing of events and duration of model

Aggregated statistics was requested from all Nordic countries

Pop: Patients with indication diagnosis

+

Data collected:

Procedure code +
Patient characteristics +
Hospitalization days per treatment

SE: National Board of Health and Welfare
DK: Statistics Denmark
FI: Finnish institute of Health and Welfare
NO: Norweigan Directorate of Health, Helsedata

Importance:

- ➢ Model validity
- ➢ Decreased uncertainty
- ➢ Increased HTA confidence in submission

RWD used to create comparator arm to support HTA

Context: Relevant comparator for local HTA submission not available in RCTs

Solution: Create comparator arm from Nordic RWD and compare using statistical matching

- Quality, depth and breadth of Nordic data provides compelling evidence, especially locally

Other examples of RWE used in Swedish HTA (TLV)

Setting	Type of RWE	Comment
Hemophilia product review	• The **dosing and use** of factor VIII concentrate • Estimate **quality of life**.	• RWE evidence both submitted by manufacturer and requested by TLV
Hemlibra CE model in hemophilia	• Actual **drug usage** of comparator treatment to estimate actual **costs** (instead of indicated usage)	• For use in cost modelling
Xolair reimbursement follow-up	• **Treatment effect** after 16 weeks • Treatment effect if treated > 6 months • **AEs** if treated > 6 months • Treatment **discontinuation** due to no effect	• Requirement to submit within 4 years • Purpose: retain reimbursement status • Typically up to manufacturer to decide how to collect data
Eliquis reimbursemet follow-up	• Treatment **adherence**	• Later showed better adherence than comparator

2 common RWE scenarios in HTA:

1. **Offered by the manufacturer**
2. **Requested by the HTA agency**

Future use of RWE in Nordic HTA

- **Guidance from Nordic payers on RWD & RWE**
  - NICE has released guidance for the industry on the use of RWE – Hopefully the Nordic payers will follow suit
- **Pay-for-performance / Managed entry agreements**
  - Previously tried unsuccessfully - Too complicated, targets unclear, contracts too uncertain
- **rRCTs, target trial emulations to support decision making**
  - Alignment with payer question (effectiveness) vs regulatory (efficacy)
  - Already possible and in use
  - Low relative cost, long follow-up
- **Use of RWE to demonstrate value beyond the QALY**
  - Including patient-created RWE

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