Building Real-World Evidence (RWE) to inform HTA/Payer decisions

Infarmed 30th Anniversary Conference: Use of Health Data
European Developments Panel - 25 September 2023

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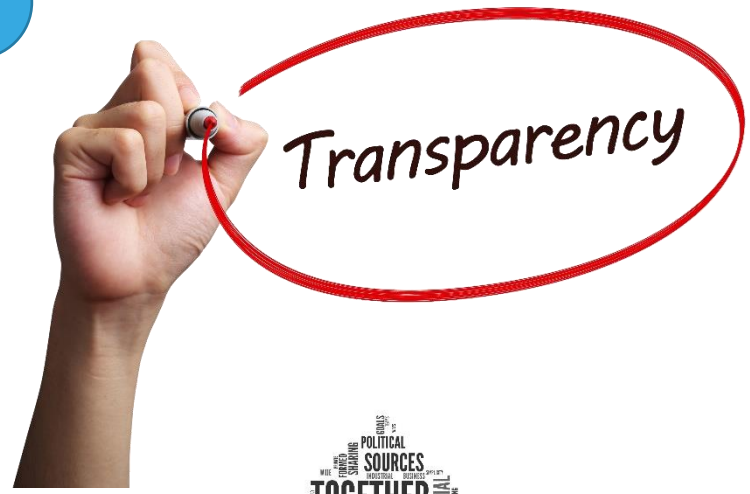
Principles

Payer-Led Multi-Stakeholder Learning Network

Highly innovative technologies often have immature clinical evidence (and high prices)

Potential for RWE
- to fill gaps in clinical development, and/or
- resolve uncertainties post-launch?

Can requirements be aligned across stakeholders and health jurisdictions/payers?



…working with the RWE4Decisions multi-stakeholder community

INAMI-RIZIV

Secretariat: FIPRA

MULTI-STAKEHOLDER COMMUNITY

Clinicians/Researchers/ Registry-holders

UZ Leuven, ECO, EORTC, EBMT, Canadian Registry

Patients/Foundations

EURORDIS, ECO, ECPC AfM-Téléthon, SMA UK

Regulators

European Medicines Agency (EMA)

HTA bodies, Payers and Health Ministries

AITHA, Austrian Social Insurance Association; INAMI-RIZIV, KCE; CADTH; Danish Medicines Council, Amgros; NICE; FIMEA; HAS; G-BA, AOK; NCPE

AIFA; ZIN; NoMA; AOMIT; INFARMED; Health Insurance Institute; Health Ministry; TLV

Analytics experts/Statisticians

Aetion, Flatiron, EFSPI

Academia

Università Cattolica del Sacro Cuore, University of Edinburgh, Mc Master University, University of Quebec, University Lyon, University of Helsinki

Industry

EUCOPE, AstraZeneca, Boehringer Ingelheim, Novartis, Pfizer, Roche, Takeda

"Learning by Doing"

RWE4Decisions REAL WORLD EVIDENCE 2023 STEERING GROUP

HTA/Payers

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President of Comm. of Drugs Reimbursement, **INAMI-RIZIV**

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Antonella Cardone

CEO, **Cancer Patients Europe**

Chris Sotirelis

Patient Advocate for Thalassemia

Insurer

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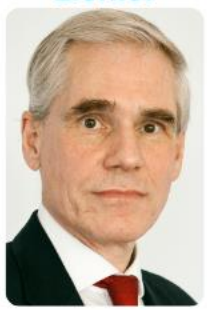

Consulting physician, **Austrian Social Insurance Institutions**

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Industry

EUCOPE

AstraZeneca

Boehringer Ingelheim

NOVARTIS

Roche

Pfizer

Takeda

Secretariat provided by FIPRA funded by EUCOPE and member companies

RWD for pricing and reimbursement decisions?

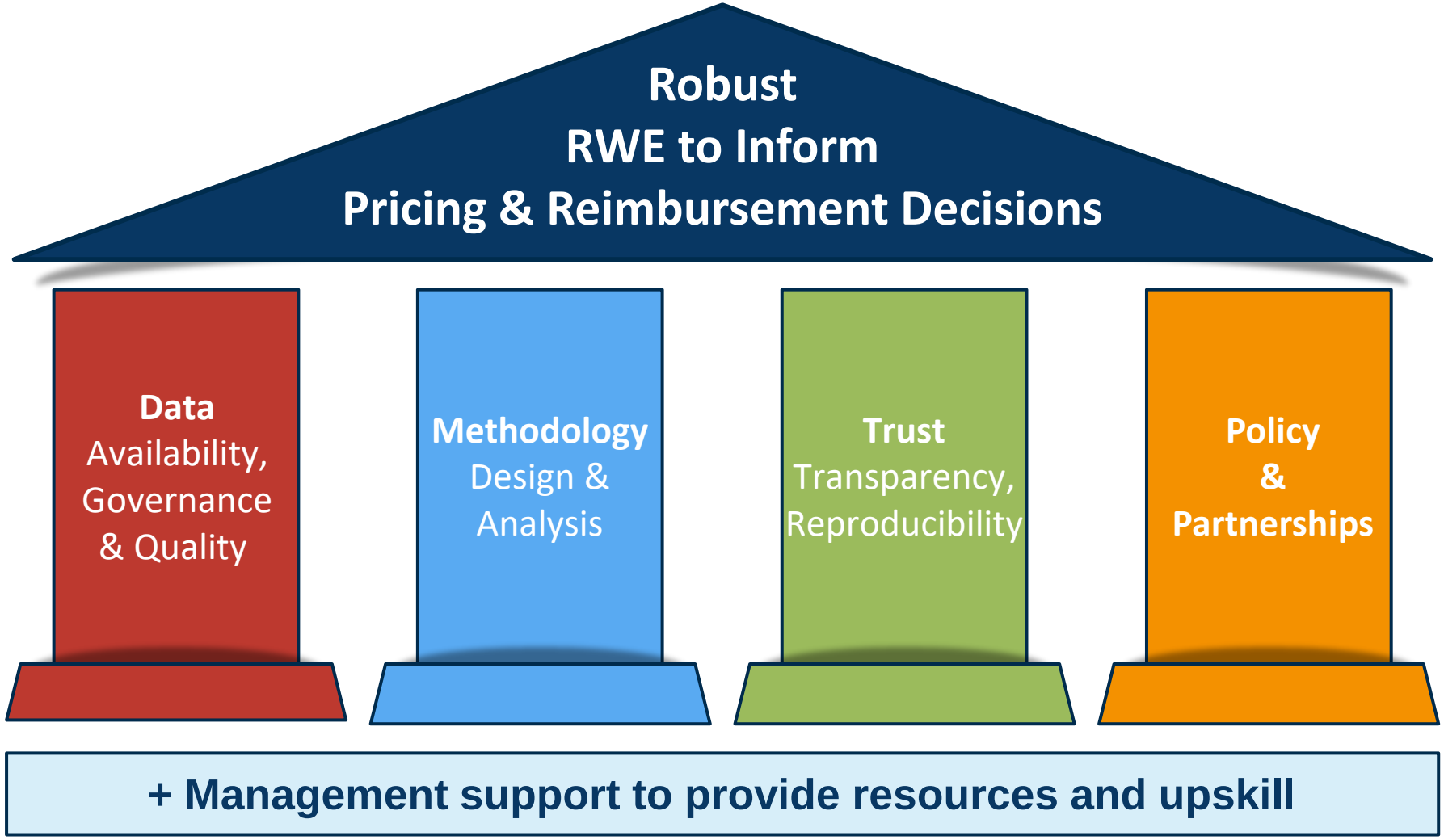
Economic modelling

- Characterizing population
- Understanding local care pathway
- Natural history
- Long-term effects
- ….

But what about as more pivotal evidence of clinical effectiveness?

- Extending population beyond that in clinical trial/initial reimbursed population (e.g. SMA)
- To demonstrate comparative effectiveness

Four pillars to support development of robust Real-World Evidence (RWE) for HTA/Payer decision-making



Capkun, G., Corry, S., Dowling, O., Asad Zadeh Vosta Kolaei, F., Takyar, S., Furtado, C., . . . Facey, K. (2022). Can we use existing guidance to support the development of robust real-world evidence for health technology assessment/payer decision-making? *IJTACC, 38*, E79. doi:10.1017/S0266462322000605

RWE4Decisions REAL WORLD EVIDENCE

Registries for Evaluating Patient Outcomes: A User's Guide

Fourth Edition

AHRQ Agency for Healthcare Research and Quality

HAS
HAUTE AUTORITÉ DE SANTÉ

ASSESS
HEALTH TECHNOLOGIES

METHODOLOGICAL GUIDE

Real-world studies for the assessment of medicinal products and medical devices

10 juin 2021

aihta
HTA Austria
Austrian Institute for Health Technology Assessment GmbH

(Good) practice organizational models using real-world evidence for public funding of high priced therapies

NICE National Institute for Health and Care Excellence

NICE real-world evidence framework

Corporate document
Published: 23 June 2022
www.nice.org.uk/corporate/ecd9

IQWiG

IQWiG Reports – Commission No. A19-43

Concepts for the generation of routine practice data and their analysis for the benefit assessment of drugs according to §35a Social Code Book V (SGB V)[1]

cadth Canada's Drug and Health Technology Agency

CADTH Methods and Guidelines

Guidance for Reporting Real-World Evidence

May 2023

Valtermed protocols and reports

https://www.sanidad.gob.es/en/profesionales/farmacia/valtermed/home.htm

GOBIERNO DE ESPAÑA MINISTERIO DE SANIDAD

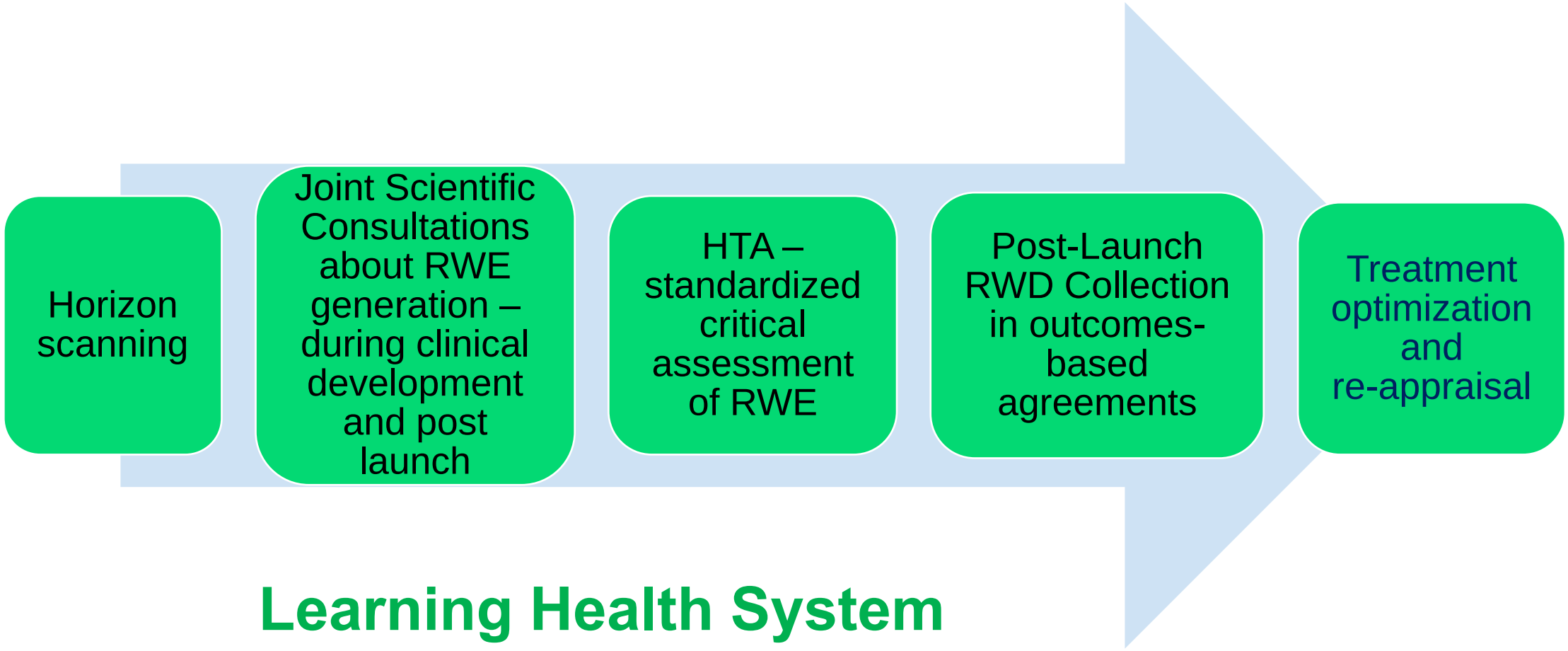
› Protocolos Farmacoclínicos:

- Tisagenlecleucel en leucemia linfoblástica aguda de células B **Escuchar** (versión en inglés **Escuchar**)
- Tisagenlecleucel y axicabtagén ciloleucel en linfoma B difuso de células grandes **Escuchar** (versión en inglés **Escuchar**)
- Inotuzumab ozogamicina en leucemia linfoblástica aguda **Escuchar** (versión en inglés **Escuchar**)
- Darvadstrocel en fístulas perianales complejas en enfermedad de Crohn **Escuchar** (versión en inglés **Escuchar**)
- Lumacaftor/ivacaftor y tezacaftor/ivacaftor en el tratamiento de la fibrosis quística **Escuchar** (versión en inglés **Escuchar**)
- Dupilumab en el tratamiento de la dermatitis atópica grave en pacientes adultos **Escuchar** (versión en inglés Will open in a new window to the page docs/20200131_Protocolo_dupilumab_dermatitis_atopica__grave_adultos.pdf
- Remdesivir en el tratamiento de la enfermedad por COVID-19 **Escuchar** (versión en inglés **Escuchar**)
- Burosumab en el tratamiento del raquitismo hipofosfatémico ligado al cromosoma X **Escuchar** (versión en inglés **Escuchar**)
- Voretigén neparvovec en el tratamiento de la distrofia retiniana asociada a la mutación *RPE65* bialélica **Escuchar** (versión en inglés **Escuchar**)

National Health Data Sources

https://rwe4decisions.com/documents/country-responses/

Life cycle of RWE generation



Learning Health System