
# Drug regulation in the 2020s
# Key roles to play by HCPs and Academia

# Collaboration with health professionals – the EMA journey

**1995**

EMA created

**1996**

HCPs as members of scientific committees

**2004**

Doctors' representative joins EMA Management Board

**2006**

Working group with HCP organisations created

**2011**

Framework of interaction with healthcare professionals and their organisations

**2013**

Healthcare professionals' Working Party (HCPWP) created

**2014**

Public Engagement Department

**2016**

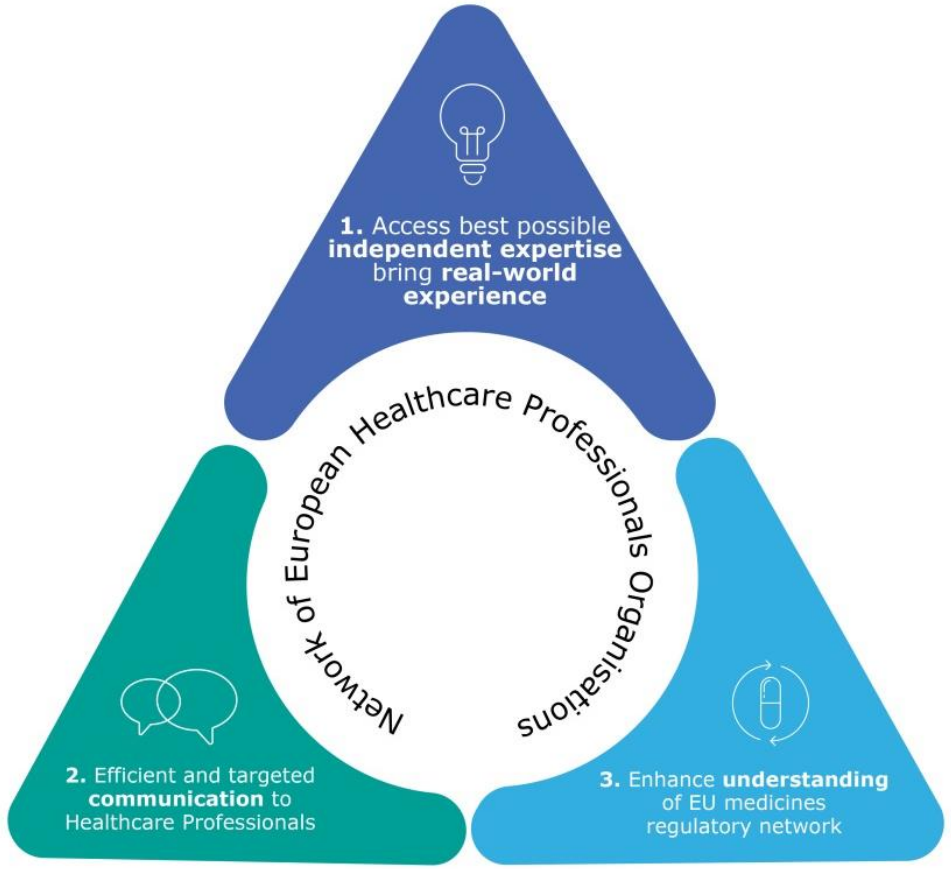
Framework of interaction updated

**Ongoing…**

Systematic inclusion of real world clinical experience in EMA regulatory output

Source: EMA

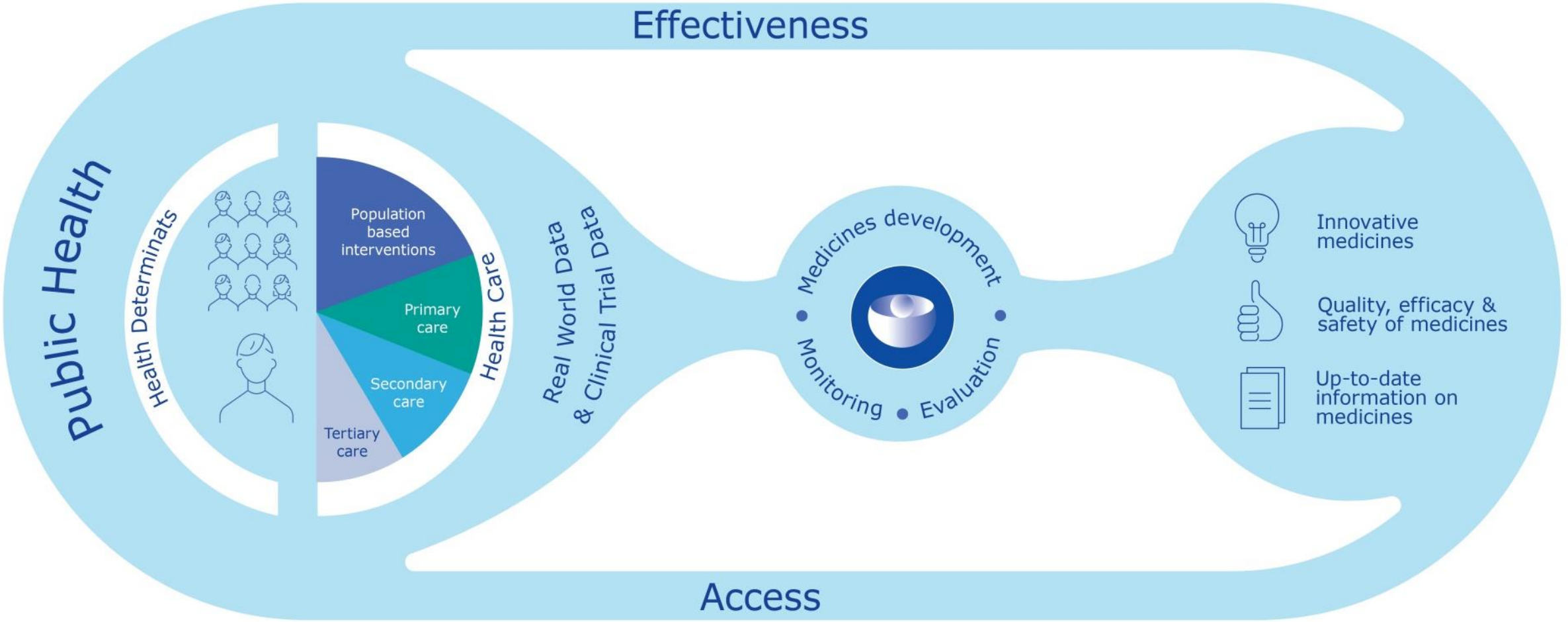
# Fostering EMA interaction with healthcare professionals




Source: EMA

- **Reinforce knowledge existing in the regulatory network** with additional valuable input from day-today clinical practice while **enhancing communication and outreach** to those impacted by EU decisions
- **Healthcare professional organisations**' including learned societies as key facilitators to channel targeted and meaningful messages to the wider community of healthcare professionals
- **Framework of interaction** adopted in 2011

# EMA in the Public Health context




Source: EMA

# STRENGTHS OF THE EU REGULATORY SYSTEM

Multicultural

Peer-reviewed

Reasonably foreseeable

Major achievement
of the EU

# Some fashion terms that you may be used (upset?) to hear …

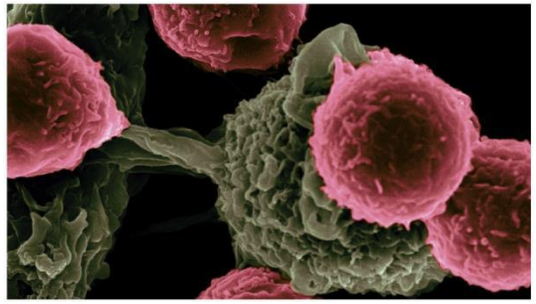
- Unmet medical need
- Innovation
- Timely access
- Targeted therapies
- Personalised medicines


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH
PRIME – PRIORITY MEDICINES

# CAR-T cell recommended for approval in the EU

KYMRIAH™ (tisagenlecleucel) Suspension for IV infusion

YESCARTA® (axicabtagene ciloleucel) Suspension for IV infusion



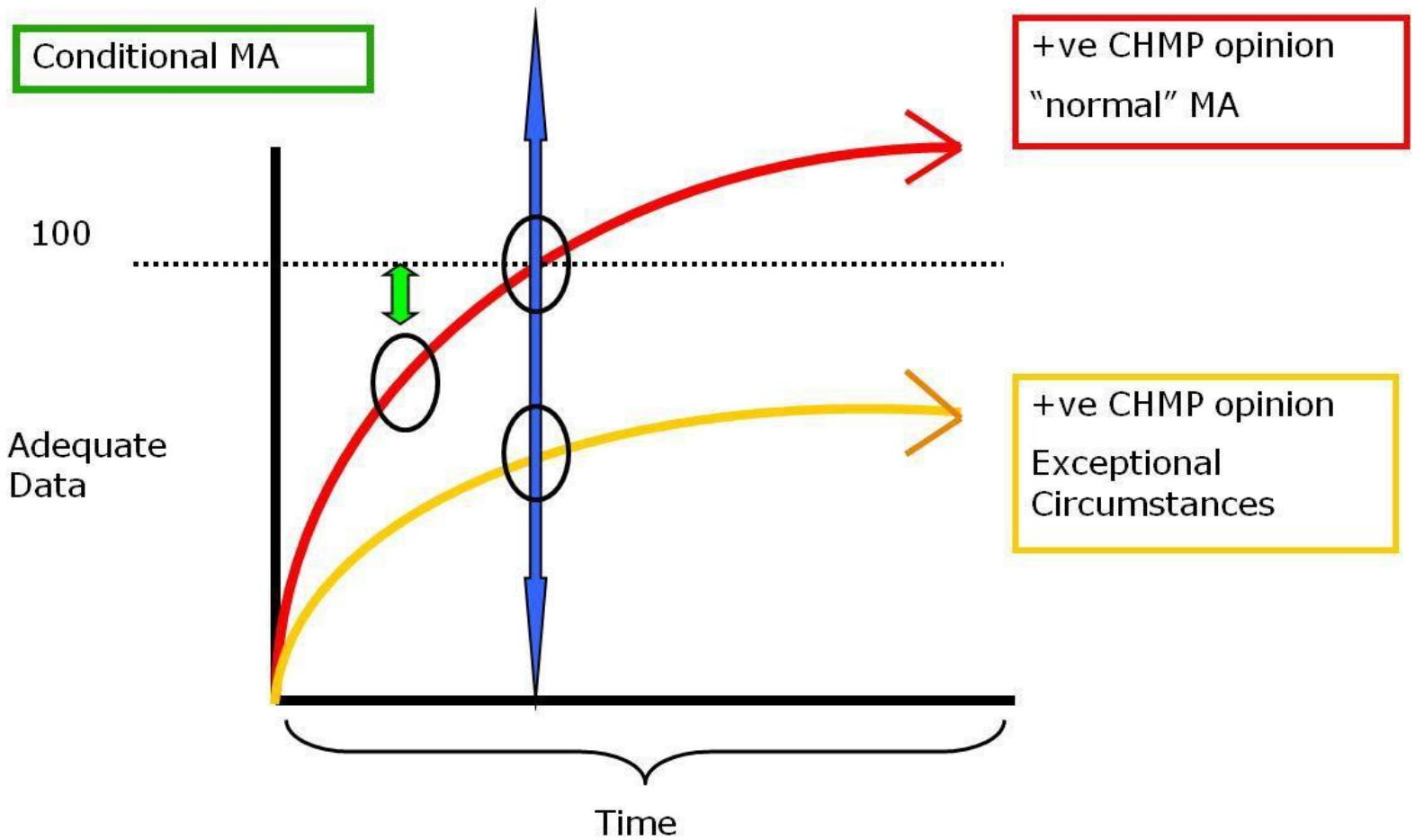
| Kymriah: regulatory tools and measures applied pre- and post-authorisation |
| --- |
| **Pre-authorisation** |
| PRIME designation - 23 June 2016 |
| Scientific Advice pertained to quality, non-clinical and clinical aspects – 2014, 2016 (twice) and 2017 (twice) |

[...]

| Yescarta: regulatory tools and measures applied pre- and post-authorisation |
| --- |
| **Pre-authorisation** |
| PRIME designation - 26 May 2016 |
| Scientific Advice pertained to quality, non-clinical and clinical aspects – 2015 (twice) and 2017 (twice) |

[...]

# Type of authorisation



# TIMELY ACCESS

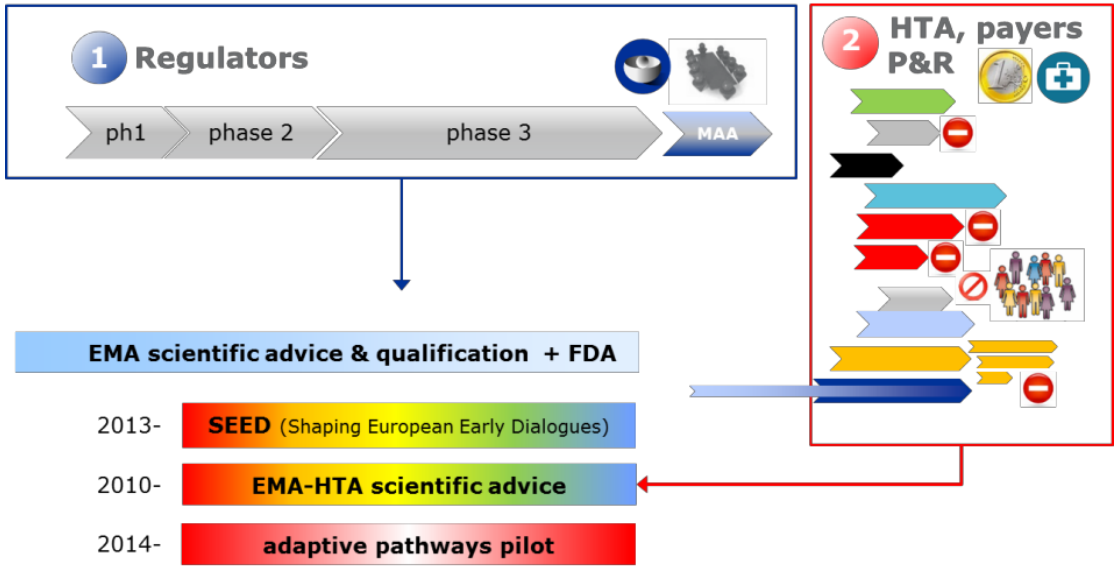
- PROS-
  - Earlier access for patients
  - Favourable for companies
  - Gradual entry into the market
- CONS-
  - Level of uncertainty
  - Over-interpretation of phase II data
  - Almost lack of safety information
  - Biomarkers rather than outcomes

# THE FUTURE

AREAS OF IMPROVEMENT

- HTAs (European HTA?)

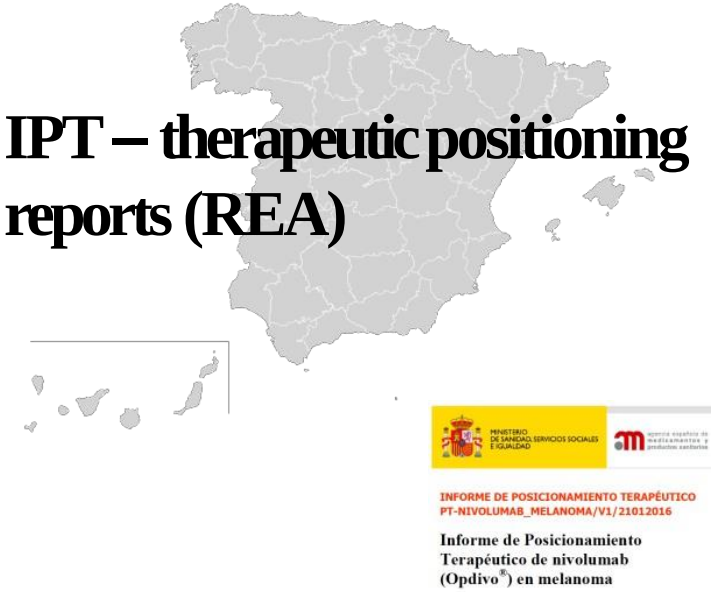
# health technology assessment (HTA)




eunethta
EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

## dialogue regulators &HTAs &payers


## IPT – therapeutic positioning reports (REA)




EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH


eunethta


Cantabria
Asturias
Euskadi
Galicia
Navarra
La Rioja
Cataluña
Castilla y León
Aragón
C. Madrid
C. Valenciana
Extremadura
Castilla la Mancha
Islas
Baleares
R. Murcia
Andalucía
Islas
Canarias
. Ceuta
. Melilla


SCOTLAND
NORTHERN IRELAND
ENGLAND
WALES

# ISSUES TO ADDRESS

- Harmonization: there is no scientific reason explaining differences in Access across the EU
- Inverse evaluation
- Pricing and reimbursement

# THE FUTURE

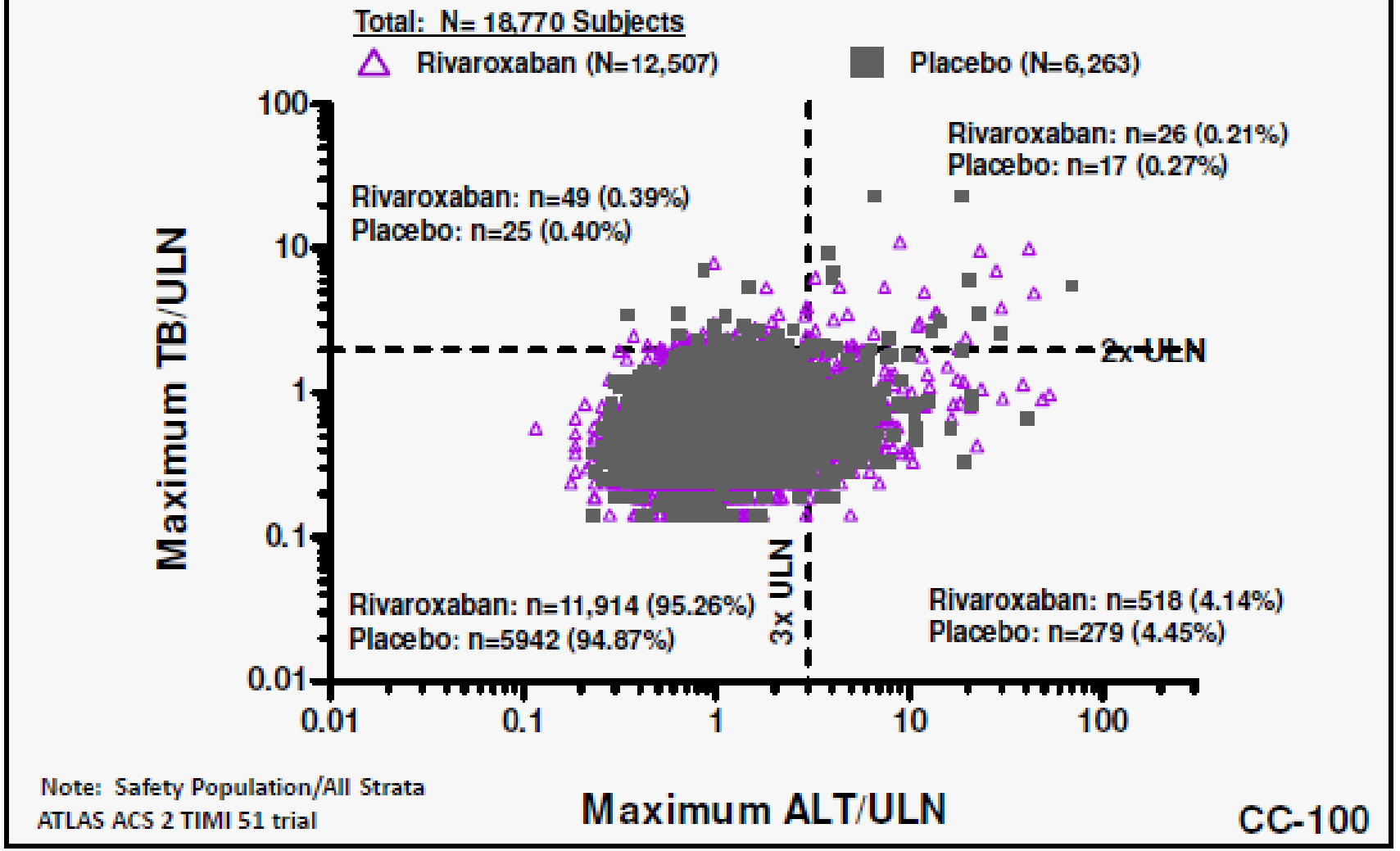
AREAS OF IMPROVEMENT

- HTAs (European HTA?)
- Efficiency of clinical research
    - Non-go decisions
    - Optimization of CTs
    - Relevant a easy-to measure endpoints

# CRF IN ACS STUDY

- 113 page CRF template
- Resulting in over 1000 pages for some subjects

# HEPATOTOXICITY




Note: Safety Population/All Strata
ATLAS ACS 2 TIMI 51 trial

CC-100

# PATIENT STATUS AT THE END OF THE STUDY

| | placebo | 2.5 | 5 |
|---|---|---|---|
| complete | 85% | 85% | 84% |
| death | 4% | 3% | 4% |
| good f/u | 89% | 88% | 87% |
| consent withdrawn | 8% | 9% | 9% |
| lost | 0.3% | 0.2% | 0.3% |
| other | 3% | 3% | 4% |
| bad f/u | 11% | 12% | 13% |

Source: Sponsor's TRLSTAT (Stfatus a En o Study) in ADSL.XPT

# GRAPHICALLY SPEAKING

## CTs population

## RW population

# THE FUTURE

AREAS OF IMPROVEMENT

- HTAs (European HTA?)
- Efficiency of clinical research
    - Non-go decisions
    - Optimization of CT
    - Relevant a easy-to measure endpoints
- Use of innovative tools
    - New CT design: framework, master, basket, adaptive … designs
    - RWE – Big Data

The NEW ENGLAND JOURNAL of MEDICINE

REVIEW ARTICLE

THE CHANGING FACE OF CLINICAL TRIALS

Jeffrey M. Drazen, M.D., David P. Harrington, Ph.D., John J.V. McMurray, M.D., James H. Ware, Ph.D., and Janet Woodcock, M.D., *Editors*

# Master Protocols to Study Multiple Therapies, Multiple Diseases, or Both

Janet Woodcock, M.D., and Lisa M. LaVange, Ph.D.

# A look over the fence - to other industries


E-controls, sensors → real-time analysis →

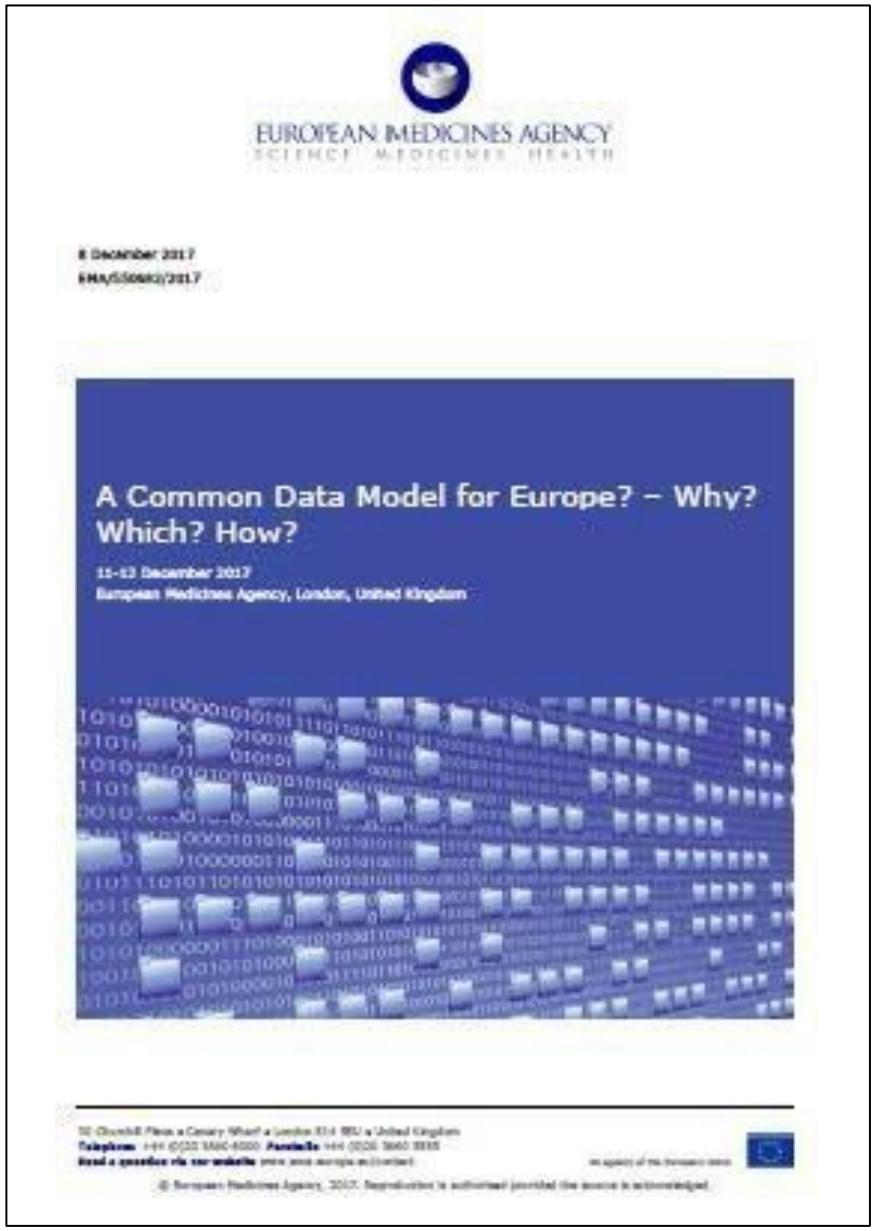
keep plane safe in the air **and** inform next-gen product design

Source: H-G Eichler

Sensors for real-time monitoring; geocoded maps; soil, weather conditions → raise agricultural productivity **and** inform next-gen product and services design

Source: H-G Eichler

# A Common Data Model for Europe?

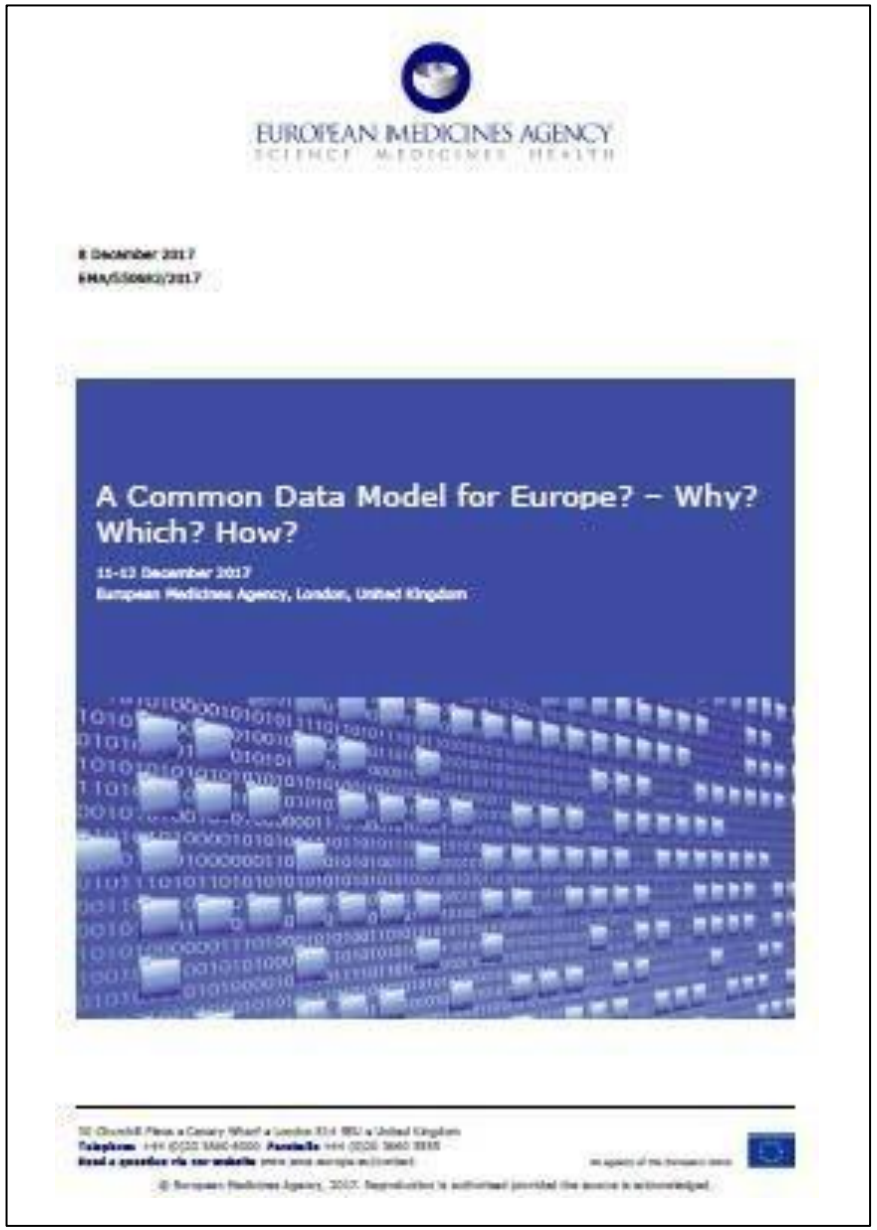


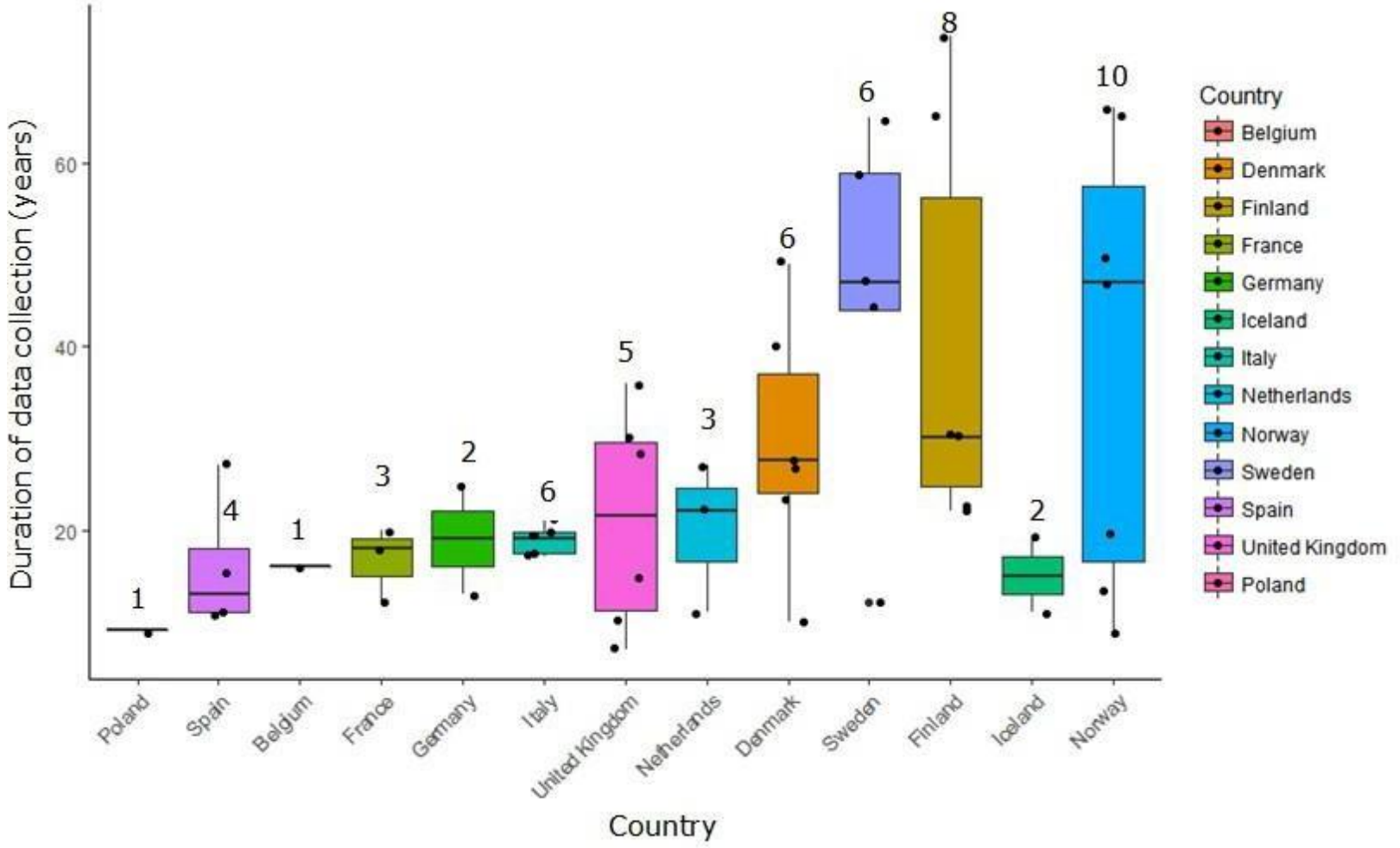

- Objectives:
  - To define the opportunities and challenges around implementation of a common data model in Europe to support regulatory decision making.
- Output:
  - To propose guiding principles for the development of Common Data model in Europe including key criteria for validation in the context of regulatory decision making.

# Enabling Sharing of Real World Data


**Three workshops on:**

- **Cystic Fibrosis: 14th June 2017**
- **Multiple-Sclerosis: 7th July 2017**
- **Car-T cells: 9th February 2018**
  **8th June 2018**






Following an analysis of electronic healthcare databases across Europe, only 34 databases across 13 member states relevant for regulatory decision making

# Healthcare Professionals Working Party (HCPWP)

**Platform for dialogue and exchange on relevant issues concerning medicines**

The HCPWP provides recommendations to the EMA and its Human Scientific Committees on all matters of direct or indirect interest to healthcare professionals