European Commission

HERA HEALTH EMERGENCY PREPAREDNESS AND RESPONSE AUTHORITY

#HealthUnion

HERA Industry days

Health Emergency Preparedness and Response Authority

2 & 3 June 2025, Brussels

European Commission

Side session

Sustainable regulatory pathways for MCM innovation, a focus on SMEs

European Commission

Sustainable regulatory pathways for MCM innovation, a focus on SMEs

Olivier GIRARD
HERA 03

Marco CAVALERI
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EMA
EUROPEAN MEDICINES AGENCY

EMA support

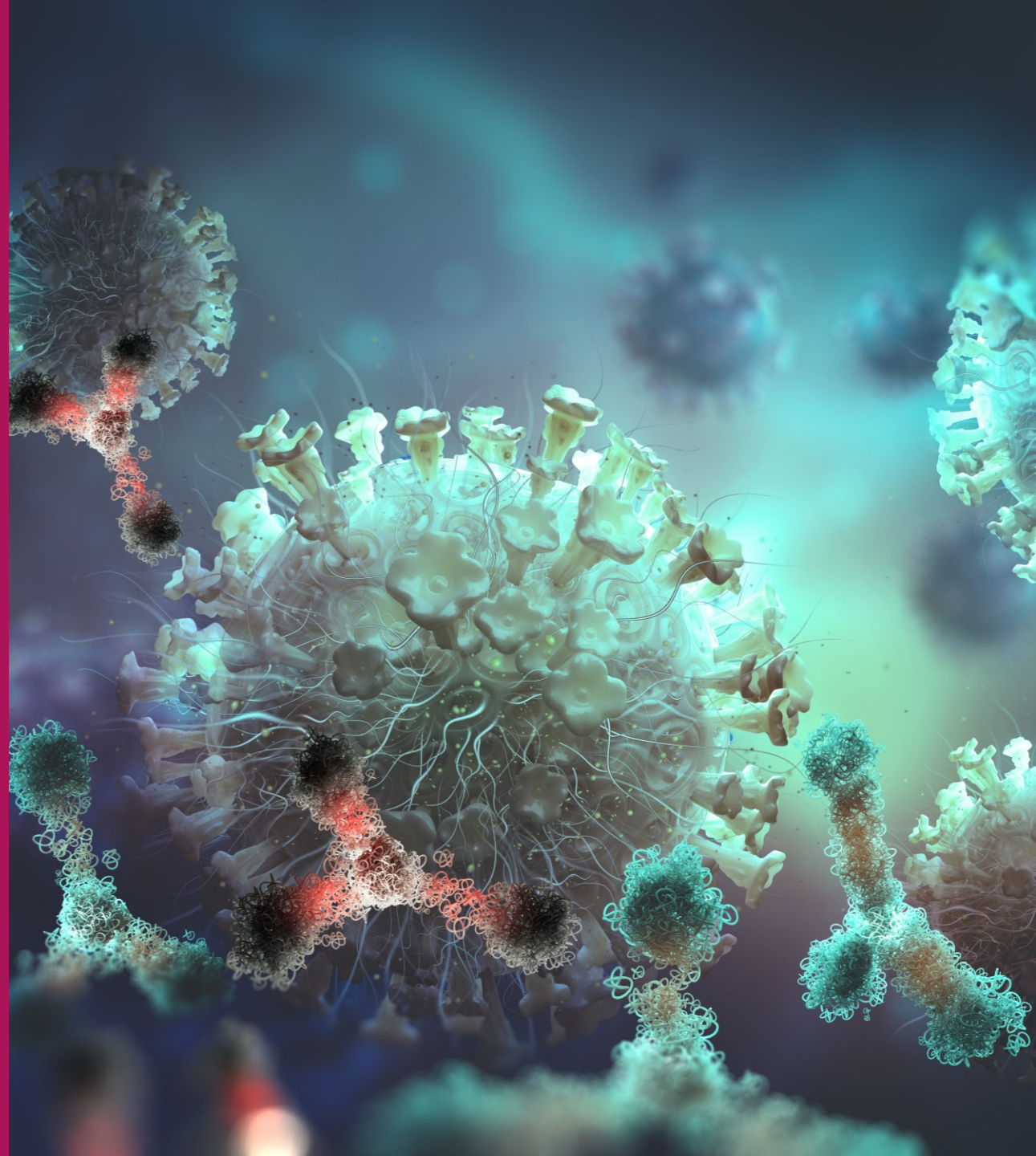
HERA

3 June 2025

Dr. Marco Cavaleri

Head **of** Public Health Threats Department

Chair of the Emergency Task Force, EMA



About the Emergency Task Force

- An advisory and support body on medicines for public health emergencies and preparedness, with composition is to be adapted for each emergency
- Strengthened ETF responsibilities, based on successful past experiences

Scientific advice and support to clinical trials

- assessed **directly** by ETF
- free of charge & fast-track for clinical trials and protocols
- **support study conduct**

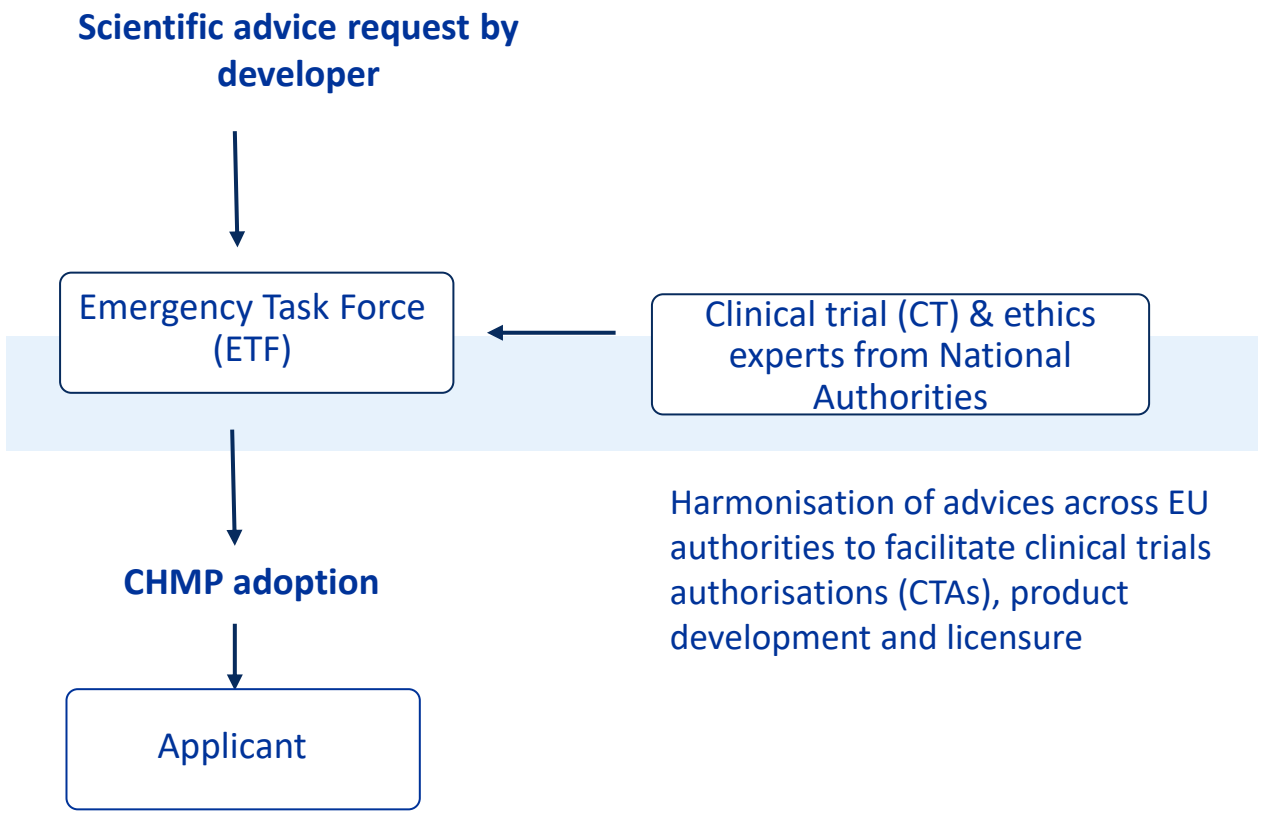
Scientific reviews

- **systematic** assessment of evidence on medicines

ETF recommendations

- **on medicines not yet authorised**
- on scientific or **public health matters**

ETF provides scientific advice to developers

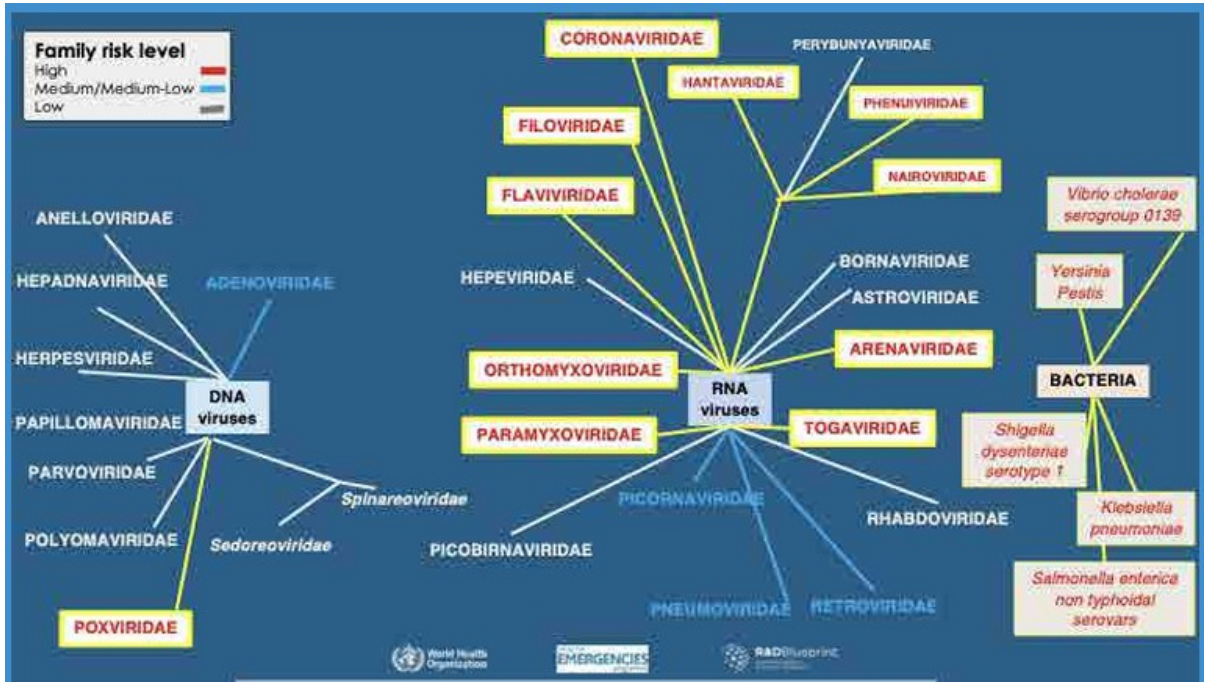


Harmonisation of advices across EU authorities to facilitate clinical trials authorisations (CTAs), product development and licensure

- CTAs under national remit; ethics committees differ by Member State
- Risk of discrepant views/advices across EU
- Bring together regulators and Clinical Trials assessors
- Involve ethics committees' experts

Regulation - 2022/123 - EN - EUR-Lex

ETF activities in preparedness and link to WHO priorities



https://cdn.who.int/media/docs/default-source/consultation-rdb/prioritization-pathogens-v6final.pdf?sfvrsn=c98effa7_7&download=true

EMA ETF focus :

- Airborne viruses with pandemic potential, Influenza & Coronaviruses
- Haemorrhagic fever viruses, e.g. Ebola/Marburg, Lassa
- Vector-borne diseases (Zika/WNV/TBE/Dengue; CHIKV; CCHF)
- Paramyxoviruses Nipah and Hendra
- Re-emerging viruses, e.g. polio and mpox
- AMR including TB
- Chemical, Biological, Radio-Nuclear (CBRN) threats including bioterorrism and biowarfare agents

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 Jul 2024
EMA/323691/2024 Corr.
Emergency Task Force (ETF)

EMA guidance document on the use of medicinal products for treatment and prophylaxis in case of exposure to biological agents used as weapons of terrorism, crime or warfare

Biological and chemical threats | European Medicines Agency (EMA) https://www.ema.europa.eu/en/human-regulatory-overview/public-health-threats/biological-chemical-threats

- Revising guideline on influenza vaccines and on COVID vaccines
- Drafting new guidelines on:
  - antivirals and monoclonals against COVID-19
  - vaccines against orthopoxviruses
  - use of animal models to demonstrate efficacy of medicinal products targeting health threats

Category	Biological agent
Category A	Anthrax (Bacillus anthracis)
	Plague (Yersinia pestis)
	Tularaemia (Francisella tularensis)
	Botulism *(Clostridium botulinum toxin)*
	Smallpox (Variola major)
	Viral haemorrhagic fever • Filoviruses (Zaire ebolavirus, Sudan ebolavirus and Marburg) • Arenaviurses (Lassa, Machupo)
Category B	Brucellosis (*Brucella species*)
	Q fever (*Coxiella burnetii*)
	Epsilon toxin of Clostridium perfringens
	Glanders (Burkholderia mallei)
	Melioidosis (Burkholderia pseudomallei)
	Epidemic Typhus fever (Rickettsia prowazekii)
	Food and water safety threats: • Salmonella species • Shigellosis • Escherichia coli 0157:H7 • Vibrio cholerae • Staphylococcal enterotoxin B • Cryptosporidium parvum
	Psittacosis (*Chlamydia psittaci*)
	Ricin toxin from *Ricinus communis* (castor beans)
	Viral encephalitis (alphaviruses) • Eastern equine encephalitis • Venezuelan equine encephalitis • Western equine encephalitis
Category C	Nipah virus
	Hantavirus

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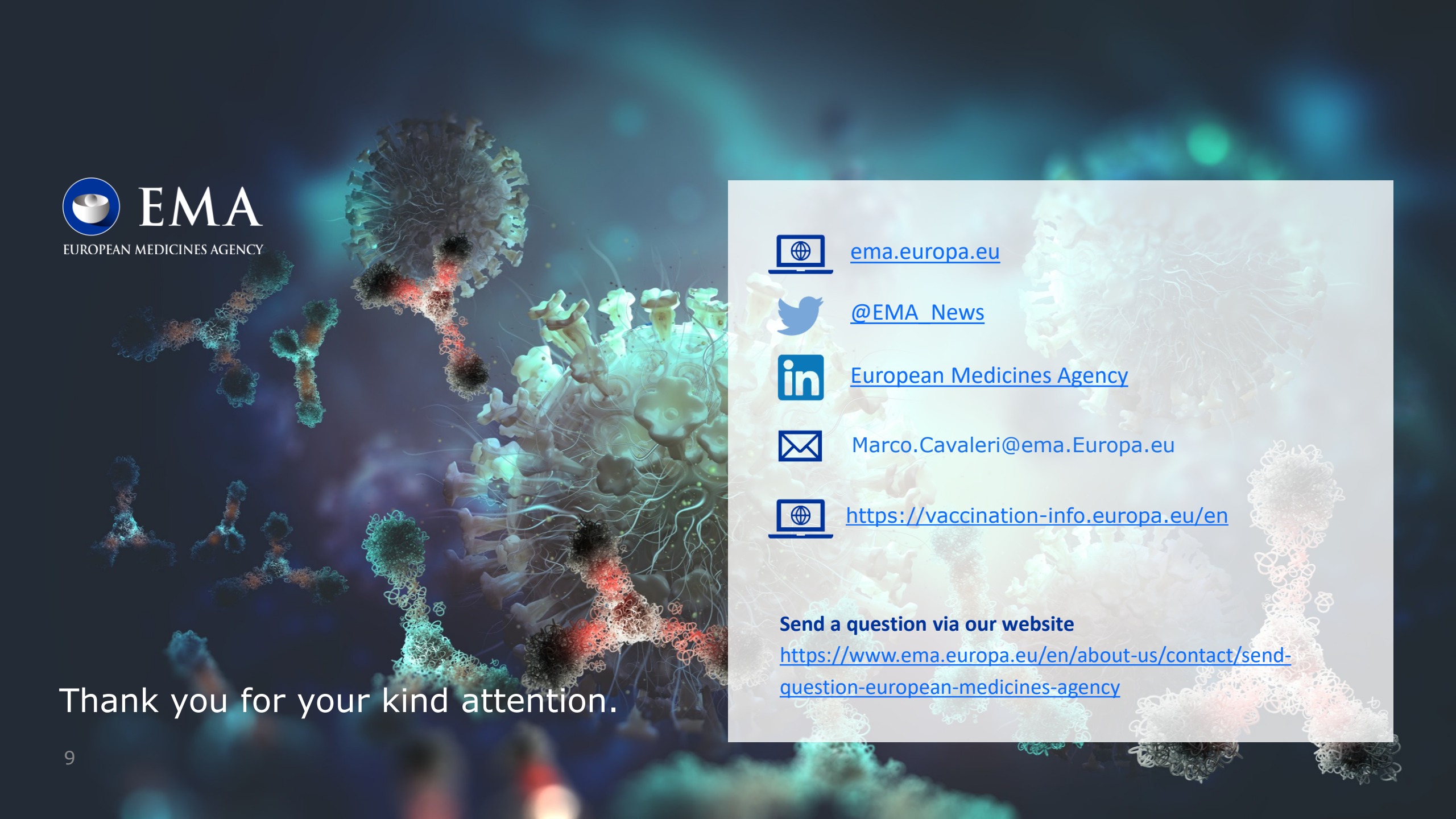

Thank you for your kind attention.

ema.europa.eu

@EMA_News

European Medicines Agency

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https://vaccination-info.europa.eu/en

Send a question via our website
https://www.ema.europa.eu/en/about-us/contact/send-question-european-medicines-agency

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Sustainable regulatory pathways for MCM innovation, a focus on SMEs

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experience from a Small and Medium-sized Enterprise (SME) – PHAXIAM Therapeutics

The operational reality for SMEs

Why They Matter?

- SMEs often lead breakthrough innovations in biotechnology
- They bring agility, creativity, and a fast response to health crises
- The vast majority are micro or small enterprises (e.g. 76% in France)

What Holds Them Back?

- Limited funding hinders long-term regulatory planning
- Insufficient regulatory resource and limited internal expertise
- A complex and fragmented regulatory landscape, hard to navigate

Result: Despite their key role in innovation,
SME struggle to optimize their regulatory development

Case Study: PHAXIAM Therapeutics

2024 | 2025 | 2026 | 2027 | 2028

Staphylococcus aureus

Prosthetic Joint Infections (PJI)

PhagoDAIR - Pilot Study — R — Follow-up

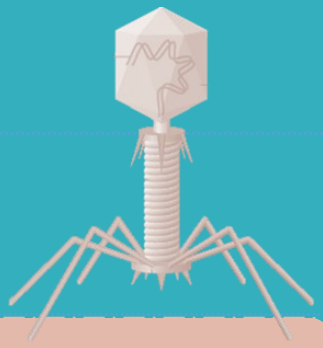
GLORIA - EU/US Phase 2 — R

Early Access / CMA

Scientific advice 2020: General development questions

Scientific advice 2024: Confirm study Design Pathway to registration

Positive opinion:



AAC: Compassionate Access

PHAXIAM

Accelerated Access: What Works and What Hinders SMEs

✅ What Helps

- Growing recognition of SME-specific regulatory challenges (e.g. EMA SME Office)
- Early and structured dialogue with agencies accelerates development
- Availability of adaptive regulatory pathways: PRIME, CMA, Early Access (EAP), compassionate use

🚫 What Hinders

- Regulatory frameworks often designed for big pharma
- Early access data often difficult to leverage for regulatory submissions
- Patient access pathways are complex and vary by country
- Free-of-charge models challenge SME sustainability

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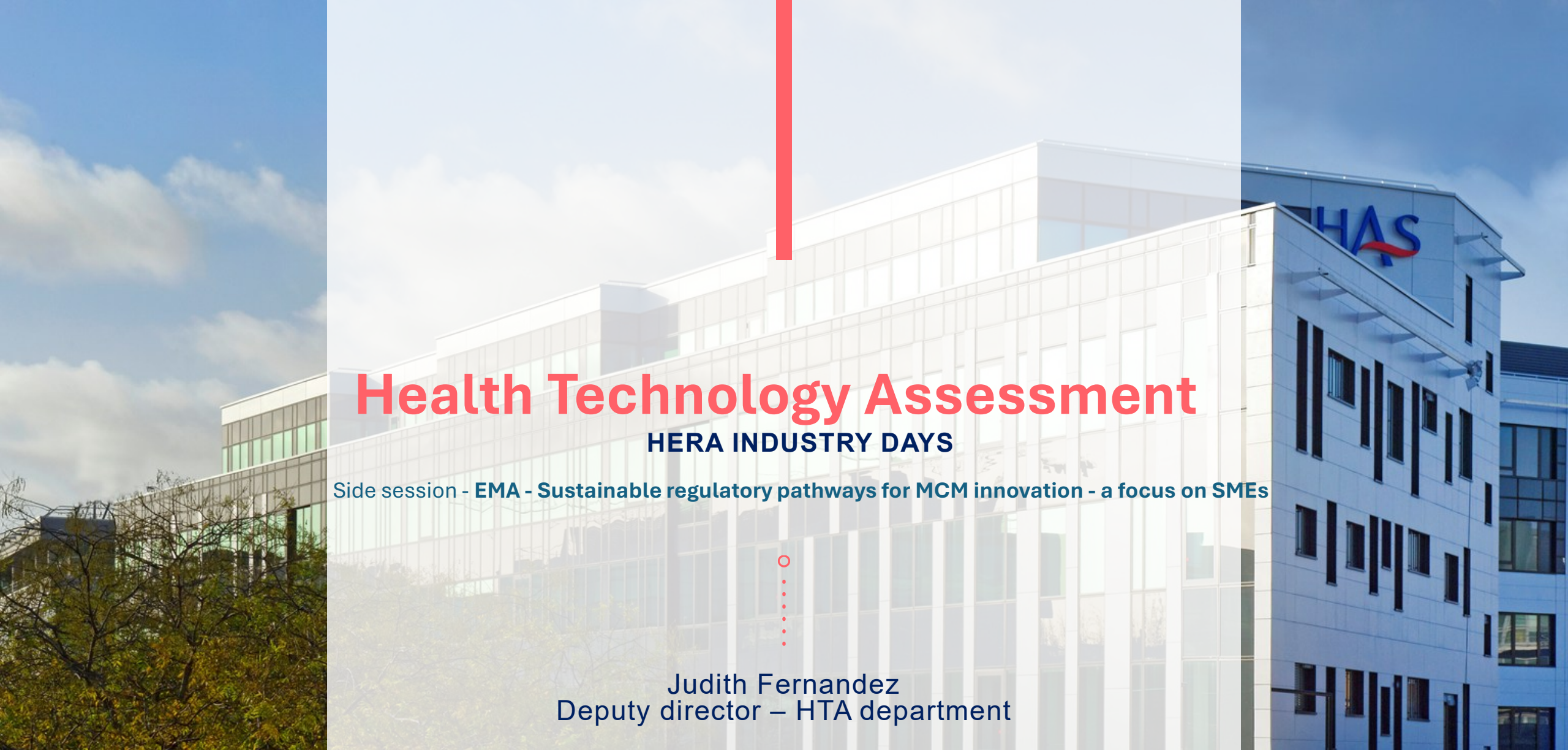


Health Technology Assessment

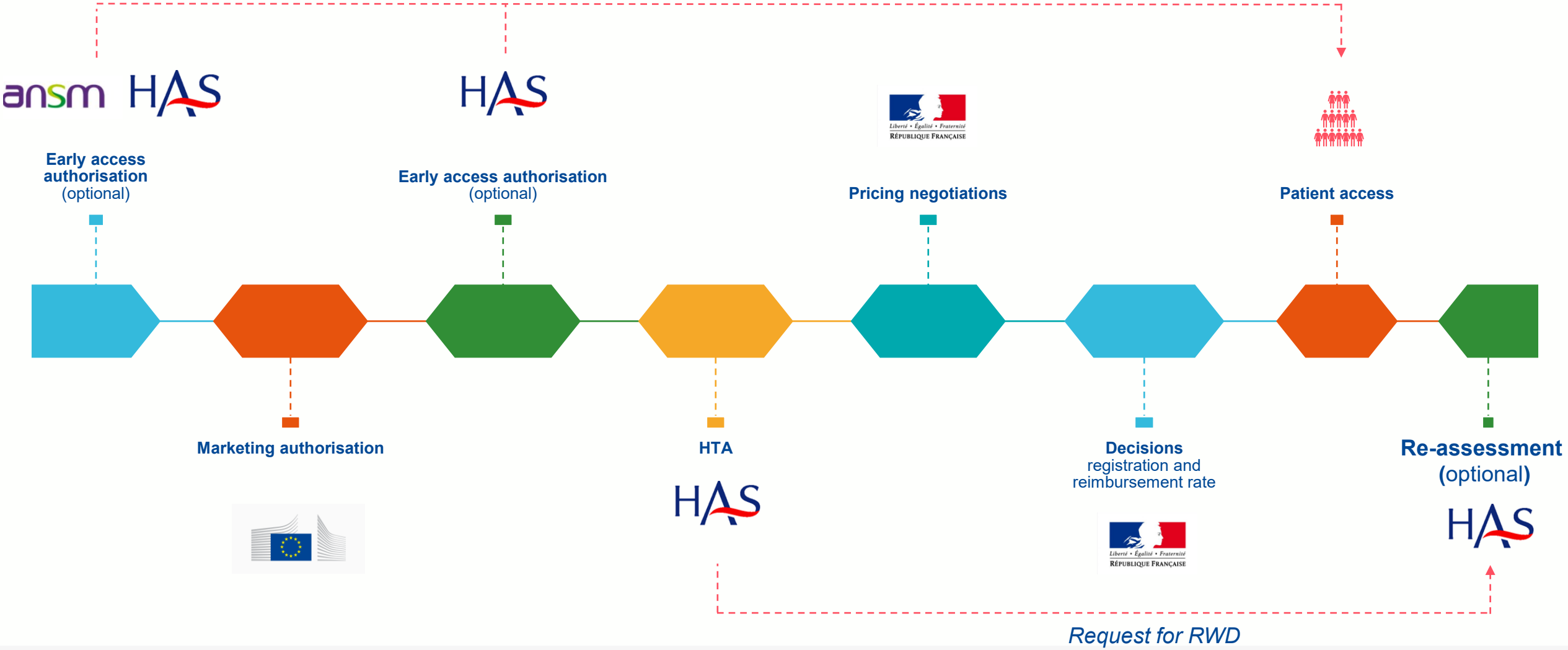
HERA INDUSTRY DAYS

Side session - **EMA - Sustainable regulatory pathways for MCM innovation - a focus on SMEs**

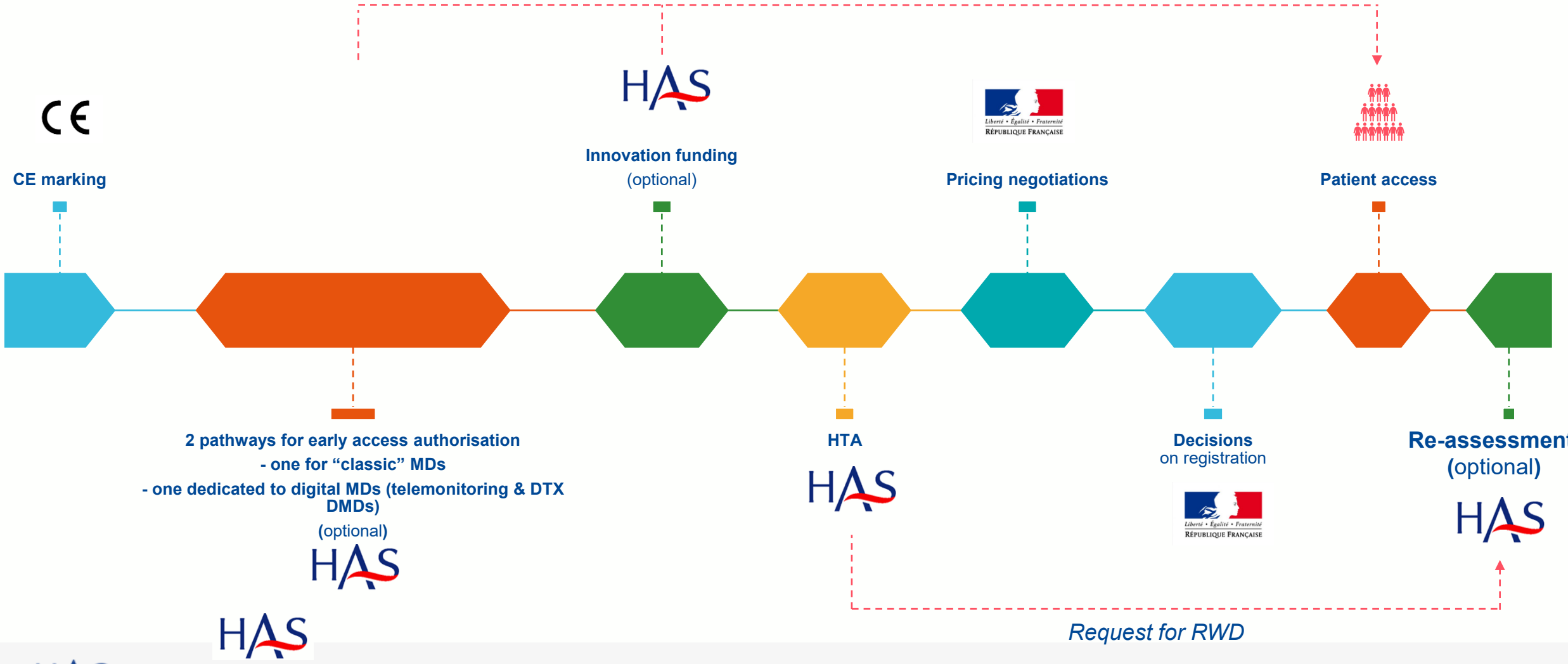
Judith Fernandez
Deputy director – HTA department

HAS
HAUTE AUTORITÉ DE SANTÉ

Market access pathway in France - *pharma*



Market access pathway in France - *MD*



Focus on early access - *pharma*

Allow access and coverage before final decision on registration

- Can be granted by HAS before MA (MA must be submitted within 2 years) or after MA
- Fast track process : 90 days maximum
- HAS = decision making body

5 eligibility criteria

- Acceptable preliminary benefit/risk ratio
- Serious, rare or debilitating disease
- Responding to unmet need (no treatment or only unsatisfactory treatment available)
- Access to treatment cannot be delayed
- Assumed innovative, notably compared to current standard of care

HAS

Mandatory real world data collection defined by HAS (+/- ANSM)

- Minimum data set: PROMs; Mortality; Primary endpoint used in pivotal trial

More information here and here

HTA regulation

Regulation (EU) 2021/2282 "HTAR"

- **Reduce the duplication of efforts by national HTA authorities** and industry by centralizing the submission of clinical data
- **Produce public, transparent, and high-quality scientific clinical reports**
- **Provide a sustainable framework for HTA cooperation in Europe**

The regulation provides for the production of four types of scientific outputs:

Joint Clinical Assessments – JCA

Compilation of comparative clinical evidence accompanied by an analysis of the certainty of the available data.

Joint Scientific Consultations- JSC

Exchange of information and development of recommendations for health technology developers regarding the clinical development plan to enable the production of robust evidence.

Identification of emerging health technologies (EHT)

Annual report identifying emerging health technologies that have a major impact on patients, public health, or healthcare systems.

Development of methodological and procedural guidance (MPG)

Methodological guidelines and detailed steps for joint scientific work.

Member States remain responsible for conclusions on their added value, access to reimbursement, and pricing.

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SUSTAINABLE REGULATORY PATHWAYS FOR MCM INNOVATION - A FOCUS ON SMES

Cornelius Schmaltz, Head of Unit, Intelligence Gathering, Analysis and Innovation Unit, DG HERA

HERA Industry Days

INTRODUCTION

HERA
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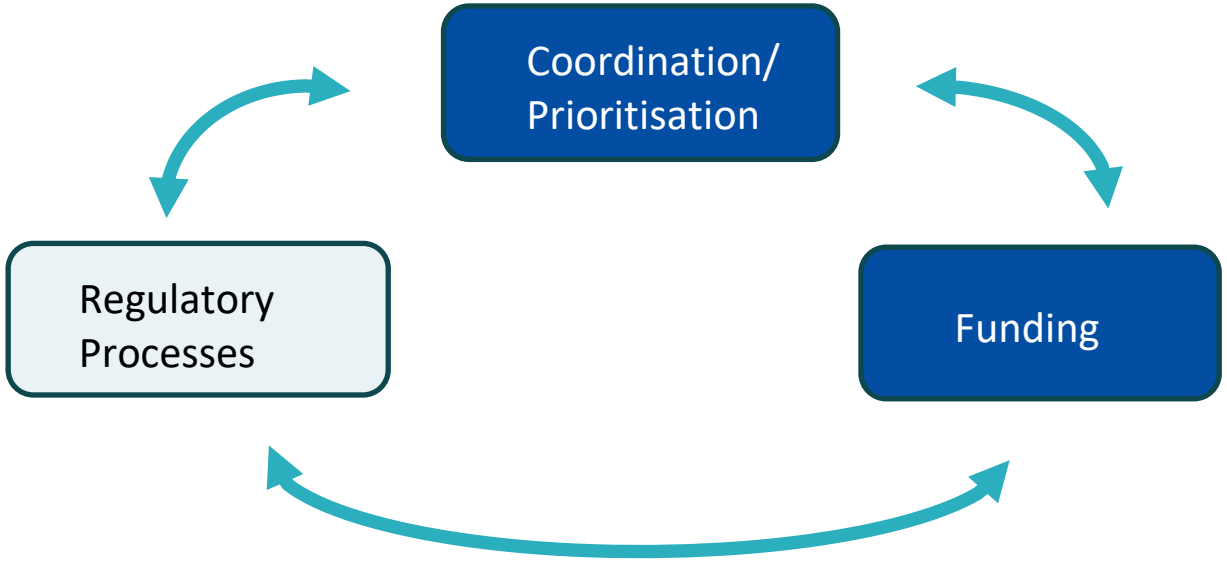
Core Mission: "***HERA shall be responsible for the following tasks promoting advanced research and development of medical countermeasures and related technologies;***" Decision of 16 September 2021 establishing HERA



To strengthen a competitive and resilient research and innovation ecosystem in the EU/EEA, regulatory processes, coordination, and funding for crisis-relevant medical countermeasures must be streamlined and closely aligned

EXAMPLES OF KEY INITIATIVES IN THE AREA OF COORDINATION, PRIORITISATION, AND FUNDING

- **Collaboration and coordination with Member States and industry**

  - **Clinical Trial Coordination Mechanism** - This mechanism brings together Member States to jointly identify and support the most promising clinical trials for public health threats.
  - **Joint Industrial Coorperation Forum and workshops with industry** to work towards novel funding tools that allow to compensate for funding gaps in the health sector.

- **Through EU4Health and Horizon Europe** - HERA supports the development and deployment of countermeasures such as influenza vaccines and diagnostics for antimicrobial resistance (AMR).

- **Setting up hubs of scientific excellence - European Vaccine Hub**

INTERPLAY – REGULATORY PROCESSES & NON-REGULATORY ACTIVITIES

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Strengthening alignment between regulatory processes and coordination/funding mechanisms:

- Foster close collaboration with regulatory authorities.
- Include requirements in tender specifications to pursue EU/EEA marketing authorisation.
- Provide funding to support regulatory processes, *e.g.* the development of pre-approved master protocols, activities that generate the necessary evidence (GLP, and GMP) for compliance with the regulations

Challenges faced with current regulatory framework

- Dealing with high-threat pathogens that have not yet caused widespread outbreaks:
  - No or limited number of cases required for approval of medical countermeasures.

- Innovation in diagnostics faces its own set of challenges, particularly with the introduction of the new In Vitro Diagnostic (IVD) Regulation:
  - Under the IVDR and MDR, manufacturers must provide robust clinical evidence to demonstrate the safety and performance of their devices.
  - This requirement can be challenging for devices intended to detect emerging or rare pathogens.

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Thank you