EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Non-clinical data for regulatory decision-making on the efficacy of medical countermeasures

24-25 Nov 2025
Hybrid

Background

The increase in global emergencies and the need to prepare for health threats—from emerging infectious diseases to bioterrorism, radiological, nuclear and chemical threats (CBRN)— advocates for efficient regulatory and scientific pathways for licensing medicinal products when human efficacy trials are not feasible because of absence of affected patients, or not ethical as humans cannot be challenged with threats for which there is no effective vaccine or treatment.

In these scenarios, regulators traditionally have relied on non-clinical data (usually animal models) as key demonstration of efficacy for decision-making in the intended indication(s).

In the two decades since the first medical countermeasures were approved based on non-clinical data, the scientific, regulatory, and societal contexts have evolved substantially, and there is now the possibility to discuss and critically review, based on concrete cases, the outcomes, translation, and methodology around non-clinical data as key evidence of efficacy for medical countermeasures.

Aims of the workshop

The workshop will bring together academics, regulators, developers and healthcare professionals to:

- Discuss the current regulatory frameworks for approval of medical countermeasures when no human efficacy studies can be conducted
- Building on examples from vaccines and therapeutics, review the translational outcomes of non-clinical data utilized in regulatory decisions as key evidence of efficacy
- Discuss how to: establish and choose non-clinical models that could reliably predict efficacy in humans; interpret and to bridge non-clinical results to expected clinical efficacy; identify success criteria for regulatory decision-making
- Review alternative approaches to the use of animal models and their potential for use in regulatory decision-making on medical countermeasures

Non-clinical data for regulatory decision-making on the efficacy of health threats medical countermeasures: current approaches and future directions

Chaired by Marco Cavaleri (EMA, Chair of Emergency Task Force - ETF) and TBD

Organising committee: Regine Lehnert (BfArM), Sun Yuansheng (PEI), Kaatie Smits (AFMPS), Edwige Haelterman (AEFMPS), Ecaterina Golea (EMA), Stephanie Buchholz (EMA), Laura Fregonese (EMA)

Day 1: 24 November 2025

09:00 Joining and technical checks (10 min)

09:10 Welcome and opening remarks (10 min)

09:10 **Welcome remarks, housekeeping and objectives** **10'**
Emer Cooke/ Steffen Thirstrup (EMA, Netherlands)

09:20 Session 1: Current regulatory frameworks and challenges on the use of animal models (85 min)

Chair: Bruno Sepodes (Infarmed, Portugal), TBC

09:20 **Challenges in developing medical countermeasures for public health threats** **10'**
Speaker: TBD

09:30 **FDA regulatory framework** **15'**
Speaker: TBD

09:45 **EMA regulatory framework** **15'**
Speaker: Stephanie Buchholz (EMA, The Netherlands)

10:00 **How to build the right animal model for efficacy demonstration?** **25'**
Speaker: TBD

10:25 **Animal models for emerging infectious diseases** **20'**

Speaker: TBD

10:45 Coffee break (30 min)

11:15 Session 3: Non-clinical in vivo data in the demonstration of efficacy of Vaccines (120 min)

Chair: TBD

11:15 **Animal models for Ebola vaccines** **20'**
Speaker: TBD

11:35 **Regulatory approval of Zabdeno/ Mvabea** **20'**
Speaker: TBD

11:55 **Chikungunya vaccines: the road to regulatory approval in the EU** **20'**
Speaker: TBD

12:15 **Integrating human immunogenicity and animal challenge Data: Imvanex as a case study** **20'**
Speaker: TBC, Industry

12:35 **Bridging non-clinical models to human vaccine efficacy** **20'**
Speaker: TBD

12:55- 14:00 Lunch Break

14:00 Panel discussion and Q&A (50 min)

Moderator: Marco Cavaleri

14:00 **Panel discussion** **30'**
Panelists: TBD/TBC

14:30 **Q&A with the Audience** **20'**

14:30 Session 4: Non-clinical in vivo data in the demonstration of efficacy of Therapeutics (80 min)

Chair TBD

14:30 **The regulatory approval of Tecovirimat for orthopoxviruses in the EU** **20'**
Speaker: TBC: Jane Crowe (HPRA, Ireland)

14:50 **Non-clinical models for orthopoxviruses (mpox)** **20'**
Speaker: TBC (Industry)

14:50 Coffee break (25 min)

15:15 **Which models for anthrax monoclonal antibodies?** **20'**
Speaker: TBC (Industry)

15:35 **Modelling and extrapolation to human case example Ebola and orthopoxviruses** **20'**

Speaker: TBD

15:55 Panel discussion (50 min)

Moderator: Filip Josephson (MPA, Sweden)

15:55 **Panel discussion** **30'**

Panelists: TBD/TBC

16:25 **Questions from the audience** **20'**

16:45 End of day 1

Day 2: 25 November 2025

09:00 Welcome and outline of the day (5 min)

Welcome remarks, housekeeping and objectives **5'**
(EMA, Netherlands)

09:05 Session 5: Non-clinical data for CBRN Medical Countermeasures (75 min)

Chair: TBD

09:05 **EU authorisation of Sagramostim for Acute Radiation Syndrome** **15'**
Speaker: Laura Fregonese (EMA, The Netherlands)

09:20 **Non-clinical development of countermeasures for radio-nuclear threats** **20'**
Speaker: Yannick Saintigny (CEA, France)

09:40 **Animal models for botulism anti-toxins** **20'**
Speaker: TBD

10:00 **Models in Chemical Threats** **20'**
Speaker: TBD

10:20 Discussion (40 min)

Moderator: TBD

10:20 **Panel discussion:** **20'**
Panelist: TBD/TBC

10:40 **Questions from the audience** **20'**

11:00 Coffee break (20 min)

11:20 Session 6: Alternative methods for animal models for the demonstration of efficacy (70 min)

Chair TBD

11:20 **EMA 3R regulatory framework** **10'**
Speaker: Sonja Beken (FAMHP, Belgium)

11:30 In vitro and ex-vivo methods

- ***Lungs- on a chip to test viral infections and medicines efficacy*** ***15′***
  Speaker: TBD

- ***Mini-brain organoids for biological and radiological countermeasures*** ***15′***
  Speaker: TBD

12:00 Computational methods for efficacy generation: how far are we? **15′**
Speaker: TBD

12:15 From innovation to regulation: Opportunities and limitations of on-animal evidence for efficacy **15′**
Speaker: TBD

12:30 Discussion (30 min)

Moderator: TBD

12:30 Discussion with the audience **30′**

13:00 Lunch break (60 min)

14:00 Session7: Post-authorisation efficacy data in humans (45 min)

Chair: TBD

14:00 Regulatory perspective **15′**
Speaker: TBD

14:15 Industry perspective **15′**
Speaker: TBD

14:30 Clinical perspective **15′**
Speaker: TBD

14:45 Discussion (40 min)

Moderator: Marco Cavaleri

14:45 Panel discussion: **20′**
Panelists: TBD/TBC

15:05 Questions from the audience **20′**

15:25 Coffee break (20 min)

15:45 Gaps and ways forward (40 min)

14:45 ***General guided discussion*** ***40'***

16:15 Closing remarks (10 min)

Wrap up **5'**

Marco Cavaleri (EMA, Netherlands)