EMA
EUROPEAN MEDICINES AGENCY

Reframing the voluntary data submission to foster regulatory acceptance of NAMs

15th industry stakeholder platform on R&D support

4 December 2025

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Problem statement – VDS proposal

Current issues

- **No NAMs qualified yet**, and **no data seen in MAA dossiers** using the safe harbour approach
- *Sometimes industry may fear NAM data will be negatively considered in regulatory decisions* (EFPIA presentation at EPAA meeting)
- By the time of MAA some NAMs could be outdated – not latest advancements
- Regulators struggle developing regulatory acceptance criteria for a CoU without having access to the data

Reframing the VDS procedure

Independent procedure from MAA and product specific procedures

Specific call for data to address a particular context of use / regulatory applicability area

Safe space: confidential data only used for supporting regulatory acceptance of NAMs

Ad-hoc group of experts from the EU reg network and EMA staff will review the data

Benefits of the reframed VDS procedure

For regulators

- Gain confidence in and better understand NAM data
- Review data to help define and/or finetune context of use for a NAM
- Readiness and limitations for regulatory acceptance of a NAM within a specific context of use
- Support the drafting of context-of-use-based qualification criteria for NAMs

For method developers and applicants

- Confidential and independent procedure
- No interference with product specific assessment
- Additional support in reaching EMA qualification
- Appropriate/useful guidance with acceptance criteria supporting use of NAMs in CTA and MAA
- Harmonisation of regulatory requirements across the EU

Stakeholders - past activities

Initial engagement with companies and trade associations:

- Introduce the VDS concept
- Assess willingness to share NAM-related data via VDS
- Be informed on NAMs in development and intended Contexts of Use (CoUs)

Feedback:

- Overall positive response to the revisited VDS procedure
- Companies willing to provide more information on CoU and NAMs
- How will the data submission work?
- What level of data EMA want to receive?
- Which context of use?

Examples

NcWP stakeholders meeting
Regulatory applicability areas for VDS of NAMs

- *When a biologic drug class presents high target specificity such that there is no pharmacologically relevant species due to the lack of target expression or to the insufficient homology with the human target – NAMs can be used in a WoE approach to support initiation of FiH trials.”*
- *“When a biologic drug class elicits a response in NHPs that is (partially) representative of human biology and if there is extensive clinical experience with the modality, the mechanism of action (MoA) is well-understood, the target is extensively characterised, and safety liabilities are known or could be mitigated — NAMs can be used to address specific safety concerns for FiH dose selection in lieu of additional NHP studies.”*
- *“In line with ICH S5(R3), when a synthetic small molecule moiety in development demonstrates evidence of risk based on NAM and WoE data, this can be used to waive subsequent DART studies if adequate risk mitigation in clinical trials can be ensured.”*
- *Within the frame of safety pharmacology testing (CNS, CDV, RESP), NAMs can be used within WoE approaches for screening purposes or to provide specific information that can be used to address risk in regulatory decision making.*

Industry View
(Beilmann et al., 2025)

CTAs experience

NHPs RP

FDA roadmap

ICH S6

ICH S5

EMA

Ad-hoc meeting with Industry: Co-creating the pilot project with industry/CROs

Draft Agenda: voluntary data submission pilot project ad-hoc meeting with stakeholders

11 December 14:00-17:00 CET, Teams*

Agenda items	Speaker(s)/topic lead(s)	Time
Session 1 - VDS pilot project		
Setting the scene - EMA Industry view on VDS	EMA Industry	15′ 15′
Session 2 - Context of use/regulatory applicability areas		
• EMA examples • Industry perspective • Discussion	EMA Industry (nominated speakers)	5′ 25′ 60′
Session 3 – Data sharing		
• Submission at EMA/briefing document • Discussion	EMA	15′ 15′
Session 4 – Outcome of the pilot		25′
Conclusions	EMA	5′

VDS pilot project - Preliminary timeline

- Co-creation of the pilot - Kick off meeting with industry – **11 December 2025**
- Written exchange on key aspects of the pilot – **Q1 2026**
  - Refinement of applicability areas
  - Briefing document
- Follow-up meeting with industry (if needed) – **Q2 2026**
- Start of the VDS pilot – **Q2-Q3 2026**
- End of VDS pilot – **Q2-Q3 2027**

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Thank you

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