

Regulatory experience on the use of prior knowledge and platform approaches

Webinar on the use of platform approaches in the non-clinical and clinical domains

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PART 1: Use of prior knowledge in quality CMC for **human medicines** – Ragini Shivji



PART 2: Use of platform technologies for **veterinary vaccines** – Javier Pozo

PART 1: Use of prior knowledge in quality CMC for human medicines

- What is prior knowledge?
- Evolution of EU CMC regulatory position
- Why use prior knowledge?
- How to justify and use prior knowledge

What is prior knowledge?



- Prior knowledge includes company knowledge from **development** and **manufacturing experience** (e.g. experience based on similar compounds, products and processes) as well as reference to **scientific and technical publications** or application of established scientific principles e.g. within chemistry
- References to prior knowledge and/or platform approaches appear throughout ICH guidelines Q8-Q14 and in various EMA guidelines.

Evolution of EU Regulatory position on prior knowledge for quality



22 March 2018
EMA/CHMP/BWP/187162/2018
Human Medicines Research and Development Support Division

Meeting Report:
Joint BWP/QWP workshop with stakeholders in relation to
prior knowledge and its use in regulatory applications
23 November 2017, European Medicines Agency, London

Introduction

Prior knowledge has always been an important tool in designing both manufacturing processes and control strategies for medicinal products. In recent years, it has gained more focus in EU guidelines (e.g. process validation for biotech drug substances¹; process validation for finished products²), and has been a regular topic of conversation at various conferences, symposia and meetings.

Prior knowledge
definition

Using prior
knowledge

Justifying use

Presenting prior
knowledge in the
dossier



Meeting Report: Workshop with
stakeholders on support to quality
development in early access approaches
(i.e. PRIME, Breakthrough Therapies)



Toolbox guidance on
scientific elements and
regulatory tools to
support quality data
packages for PRIME and
certain marketing
authorisation applications

EMA-FDA joint Q&As on
Quality and GMP aspects
of PRIME/Breakthrough
therapy applications

- **Development & approval timelines compressed**
- **Innovation & complexity**
- **Global development**
- **Same legal requirements** for CMC as other EU medicines



How to justify & use prior knowledge

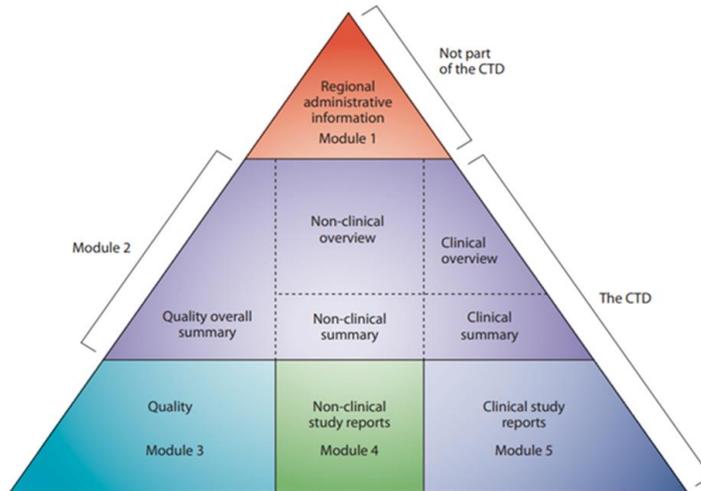
- **Context and relevance**
Show applicability of prior knowledge to new product

- **Explain & justify**
Clearly describe extent of prior knowledge

- Use [Scientific advice and protocol assistance](#)

Note:

- *Appropriate dossier location*
- *Level of granularity*
- *Summary discussion useful*
- *Differentiate prior knowledge & new product data*





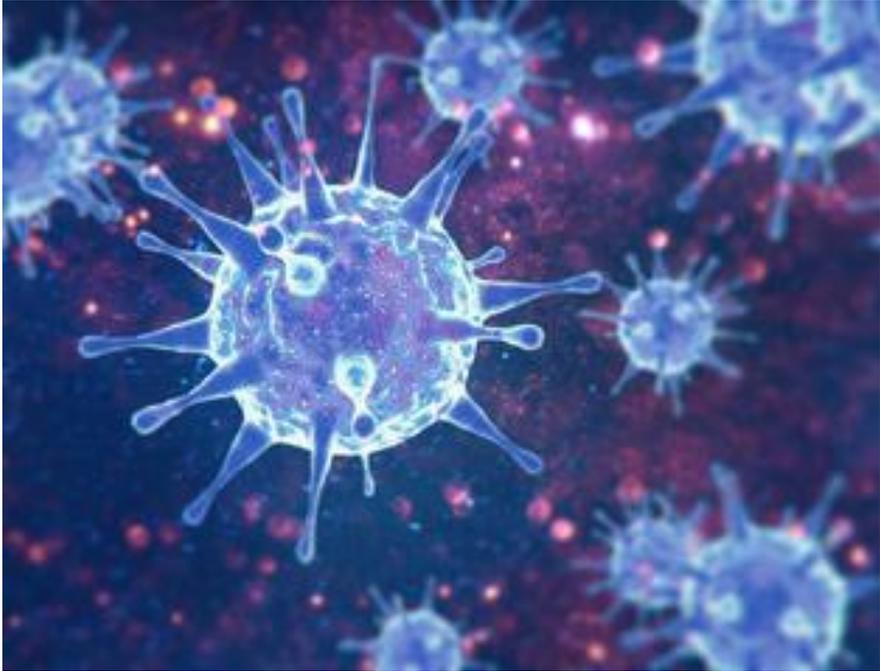
Examples of use of prior knowledge

- **Seasonal influenza vaccines** - relies on relatedness/ prior knowledge of authorised strains, minimises data required for annual updates
- **COVID Vaccine MA** - prior knowledge of viral vectored vaccine used in a COVID-19 vaccine MA
- **COVID vaccine updates** - several variant updates approved; prior knowledge reduced submission requirements.

PART 2: Use of platforms for veterinary vaccines

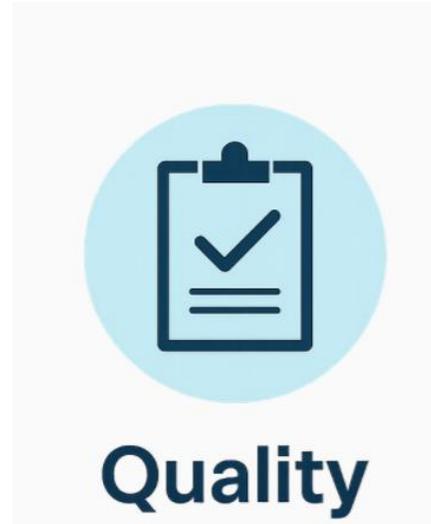
- Concept of platform technology and master file
- Content of vPTMFs
- Certification and benefits of using vPTMF
- Experience to date

Platform technology master files - concept



- For veterinary vaccines, **platform technology** is a collection of technologies that use a common 'backbone' (vector or carrier) which can be adapted by inserting different antigens or genes to produce new vaccines.
- A **platform technology master file** is a stand-alone, platform-specific dossier containing **all scientific data expected to remain unchanged** regardless of the antigen or gene inserted into the platform.
- Regulation (EU) 2019/6 – Veterinary Medicines

vPTMF Content



- **Mainly quality data**, focus on the **backbone vector or expression system**, production process, validated control strategies

Depending on the platform, may also include:

- **Safety data:** platform-specific biological properties (e.g. spread, dissemination, user safety)
- **Efficacy data:** intrinsic characteristics of the platform (e.g. immunogenic mechanism, delivery efficiency)

Certification process



- In **parallel to a new marketing authorisation application** or as a **separate procedure** (for platforms used in authorised vaccines)
- Assessment of vPTMF application results in a **vPTMF certificate**—valid throughout the European Union than can be referenced

Benefits of vPTMF approach



- **Faster development and authorisation** of new vaccines by reusing already-certified platform data
- **Rapid adaptation** of platform vaccines to emerging diseases
- **Reduced regulatory and administrative burden**, avoidance of repeated data submissions
- A well-defined platform offers a consistent basis for Q, S and E, enabling developers to focus on evidence generation for insert-specific data

Experience to date

From January 2022:

- **4 vaccine platform technology master files certified** (viral vectored vaccines)
- vPTMF certificates used in new MA applications, including emergency vaccines
- Positive feedback from regulators and vaccine companies: streamlined assessment
- EMA procedural and technical guidance published

Future:

- vPTMF expected to extend to other types vaccine platforms
- Gain experience from the lifecycle management of vPTMFs
- Advancing global alignment to vPTMF approach (veterinary)

Acknowledgements

**Veronika
Jekerle**

**Brian
Dooley**



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Thank you

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