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European Confederation of Pharmaceutical Entrepreneurs AISBL

Platform approaches in practice: experience from small and mid-sized innovative developers

EMA Webinar, 2 March 2026

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Platform approaches – industry realities

Strategic platform use

Platform development employs a **well-characterised and validated technology**, manufacturing process and/or data package across multiple therapeutic products for **efficiency and predictability**

Benefits for smaller companies

Small and mid-sized biotech companies, especially those more active in the rare disease space, can meaningfully reduce non-clinical and early clinical uncertainty by reusing prior safety, mechanistic and regulatory experience, thereby **lowering technical risk**, **shortening development timelines**, **reducing costs**, and **reinforcing their capacity to sustain innovation**

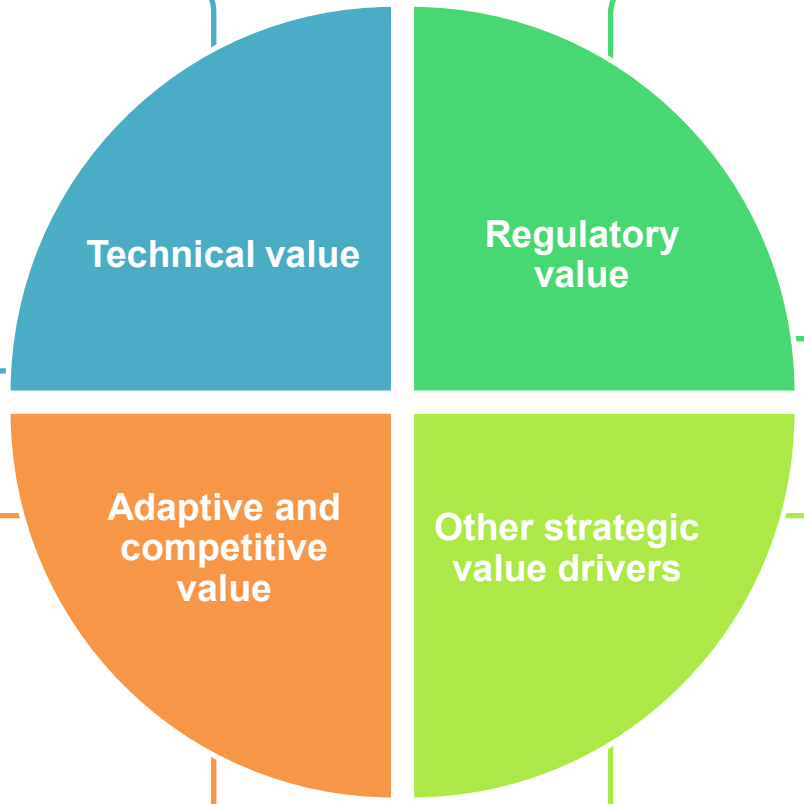
Personalised and rare therapies

Platform approaches are especially relevant and valuable in individualised treatments for **ultra-rare diseases** by enabling **focused clinical programs** and reducing duplication where data is most scarce

Regulatory innovation

Evolving EU regulations signal a **positive shift towards greater support** for platform marketing authorisations, facilitating adaptable and efficient drug approval processes

Value delivered through accumulated knowledge



- Accelerate early-stage development
- Reduce technical uncertainty
- Optimise dose & endpoints
- Speed up patient recruitment

- Enable data reusability
- Shorten review timelines
- Establish predictable regulatory outcomes
- Maintain safety rigour

- Enhance portfolio flexibility
- Support go/no-go decisions
- Enable efficient resource allocation
- Strenghten scientific credibility

- Demonstrated efficiency gains beyond clinical dimensions
- Improve access metrics
- Support strategic biotech objectives

EU landscape for platform technologies

What are the opportunities?

- Reduce repeat and duplicative filings via a platform marketing authorisation
- Reduce assessment burden and speed up product iterations via platform technology master files
- Accelerate learning, reduce ambiguity and foster future-proof regulations via well-implemented sandboxes
- Leverage validated platform knowledge across multiple clinical trial applications within simplified and core (investigational product) dossiers
- Reduce animal testing by leveraging prior non-clinical data
- Align regulatory and HTA evidence expectations via unified, early scientific consultations

What are the open points?

- Unclear criteria as to what counts as a “platform” in early-stage science
- Uncertain regulatory trajectory for platform technology master files
- Limited alignment on what constitutes comparable data across products
- Challenges in demonstrating that platform modifications don’t change safety/efficacy
- Difficulty demonstrating platform investments’ value for resilience, innovation and long-term affordability
- Need for alignment on payment / reimbursement frameworks for platform-enabled therapies

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Platform sciences in the context of individualised medicines

How does it work in practice?

Developer is working on a customised antisense oligonucleotides (AOS) targeting unique genetic mutations in rare CNS disorders in children

Although each ASO sequence is unique to a child's specific mutation, 90%+ of the product is identical: same backbone, same chemistry, same manufacturing controls, same PK behaviour, and highly consistent class-specific safety expectations

Variable components introduce mutation-specific sequences to customise therapy within predefined scientific and technical limits

Instead of running a mini-trial for each child, the Master Protocol unites individualised ASO treatments under a single harmonised clinical design with standard assessments

Dose-escalation follows predefined criteria with independent oversight to ensure patient safety and methodological rigor

Insights from EUCOPE Members

Platform sciences in the context of individualised medicines

What does this unlock for patients?

Faster access for children with rapidly progressing, ultra-rare disorders

Less unnecessary duplication while preserving scientific rigour and public trust

A model that could define how n=1 medicines move from “exceptional” to “systematically and safely delivered”

Insights from EUCOPE Members

Platform sciences in the context of individualised medicines

What system-level benefits emerge?

Combining fixed and variable components enables scalable, reproducible development of high-precision personalised medicines

The platform supports EU regulatory frameworks with data reuse, reducing redundancy and enabling predictable therapy development

This structured platform supports expansion to new mutation targets with a predictable and scalable therapeutic framework

What needs to be defined?

How much variation is acceptable in the sequences before the product falls outside the platform?

When is prior platform safety/PK data enough to justify reduced new data?

Can oversight focus increasingly on the platform, rather than re-reviewing each variant in full?

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Thank you!

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