EMA roundtable with stakeholders
15 years supporting SMEs

Session 3

Stakeholder experience and feedback
Experience and feedback on EMA SME Initiative

- The EMA SME platform is a success story pushing innovation in the EU
- EMA addresses the unique needs of SMEs through specific incentives accessible through the SME status.
  - fee incentives
  - regulatory assistance
  - assistance with translation of the product information required for the granting of a MA
  - support through workshops and training sessions
- The PRIME scheme is viewed as highly valuable
  - It remains important to set up a regular discussion group on the PRIME to improve the scheme[1]
- All SMEs need easy access to appropriate expertise in crucial phases of medicines development
  - Rapid scientific advice
  - SMEs need to engage early in areas of innovation such as continuous manufacturing, genome editing, digital technology

1 – Only 35 SMEs out of 135 could benefit from PRIME – Why?

Stakeholder experience and feedback
SME definition

Table 1 Definition of SMEs

Enterprise Category	Employees	Turnover	Balance sheet total
Micro SME	0 to < 10	< €2 million	< €2 million
Small SME	10 to< 50	< €10 million	< €10 million
Medium-sized SME	50 to <250	< €50 million	< €43 million

Source: Commission Recommendation of 6 May 2003 concerning the definition of micro, small, and medium-sized enterprises (2003/361/EC), Official Journal of the European Union, L 124/36, 20 May 2003

A **large number** of **small European (bio)pharmaceutical companies does not comply** with the SME definition and therefore does not have access to any SME incentives, despite their need for support

Stakeholder experience and feedback
SME definition

EU SME definition can be difficult to comply with:

- The need for high capital investments often leads SMEs to “be linked” to other companies via venture capital companies, while maintaining operational independence
(risk investors acquire >50% shares, finances fluctuate highly over years)
- The need for high growth leads biopharmaceutical SMEs to increase their number of employees (> 250) even if their company is still in a high-risk unsustainable financial state (5 to 10 years from market)

The EC SME Strategy published on 10 March 2020 states that “complex ownership structures will be further assessed” in relation to the SME definition:

- Our different organizations representing SMEs stand ready to discuss further this opportunity with the European Commission and the EMA

Stakeholder experience and feedback
Competitiveness & viability aspects

SMEs congratulate EMA SME Office on the SME platform and newsletter and further supports closer interaction with stakeholder associations for streamlining communication

Regulatory harmonization, flexibility and simplification

- SMEs welcome and supports the EMA Q&A documents on regulatory flexibility measures introduced for the Covid-19 pandemic
- Consider to extend these measures to all medicines (not only essential medicines) and that some level of flexibility may continue in place even after COVID pandemic

Measures to incentivize R&D and incremental innovation could be strengthened:

- To simplify and facilitate access to EU funding programs
- To continue to support SMEs with easy access to early-stage scientific advice and administrative assistance

Measures to increase competitiveness of SMEs

- Measures to incentivize EU API sourcing for older mature products which are faced with continuous price erosions due to external reference pricing systems which in turn drive SMEs to source APIs from Asian countries, posing a higher risk of shortages in the EU market.
- Fee incentives for SMEs are indeed welcomed

Stakeholder experience and feedback

Extending the scope of SME office to academic and non-for-profit organisations

Alliance for Regenerative Medicine

Fee reductions

Access to regulatory advice impossible without fee reductions

Single point of contact

Increased awareness & education on regulatory requirements

Certification scheme

Increased quality of product development

Capital formation

Regulatory advice increases investors' confidence

Industrial partnership

Supports full product development and marketing authorisation

MEDTECH & PHARMA PLATFORM

Stakeholder experience and feedback
Combined products, devices and companion diagnostics

- EMA is responsible for human (veterinary) medicinal products
- Combined products are increasingly more being developed and marketed
- Majority of medical device/IVDs manufacturers are SMEs
- EMA will be involved in evaluation of integral drug/device combination products (Article 117 of MDR), companion diagnostics (IVDR) and in future also on not-integral combined products

"Combined products refer to combined use of a medicinal product, including biologics and advanced therapy medicinal products (ATMPs), with a device or diagnostic for medical purposes, without forming necessarily an integrated unit"

- **Points to consider**
  - **Ensuring sufficient and appropriate interactions between different stakeholders especially for not-integral combined products**
  - **Interplay between clinical trial regulation, and MDR and IVDR**
  - **Medical device/diagnostics companies to obtain SME status, benefits from SME incentives and access to interactions with EMA**

Stakeholder experience and feedback
Contract research organizations

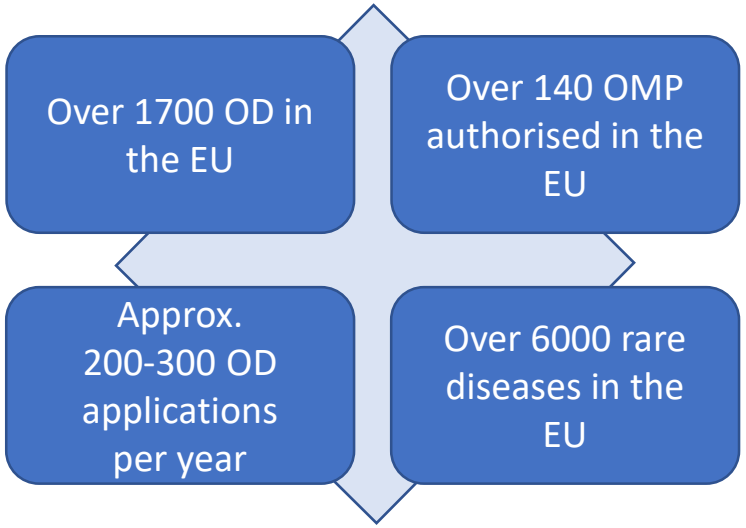
We sincerely appreciate the clarity and the openness of the EMA SME office to clarify all doubts and giving support to EUCROF SME members

Challenges exist for CROs:

- SME-EMA qualified CROs might be more and more involved in supporting Academia for new research initiatives, we believe this should be optimised
- The same would apply in case a CROs with SME status is part of a Consortium and would request EMA-SME office support

Stakeholder experience and feedback
Development of orphan medicines

- The Orphan Drug Designation (ODD) has a strong value because it is pre-condition for development of OMPs:
- To address unmet medical need
- For patients with debilitating or life-threatening diseases
- EU harmonised approach for orphan disease



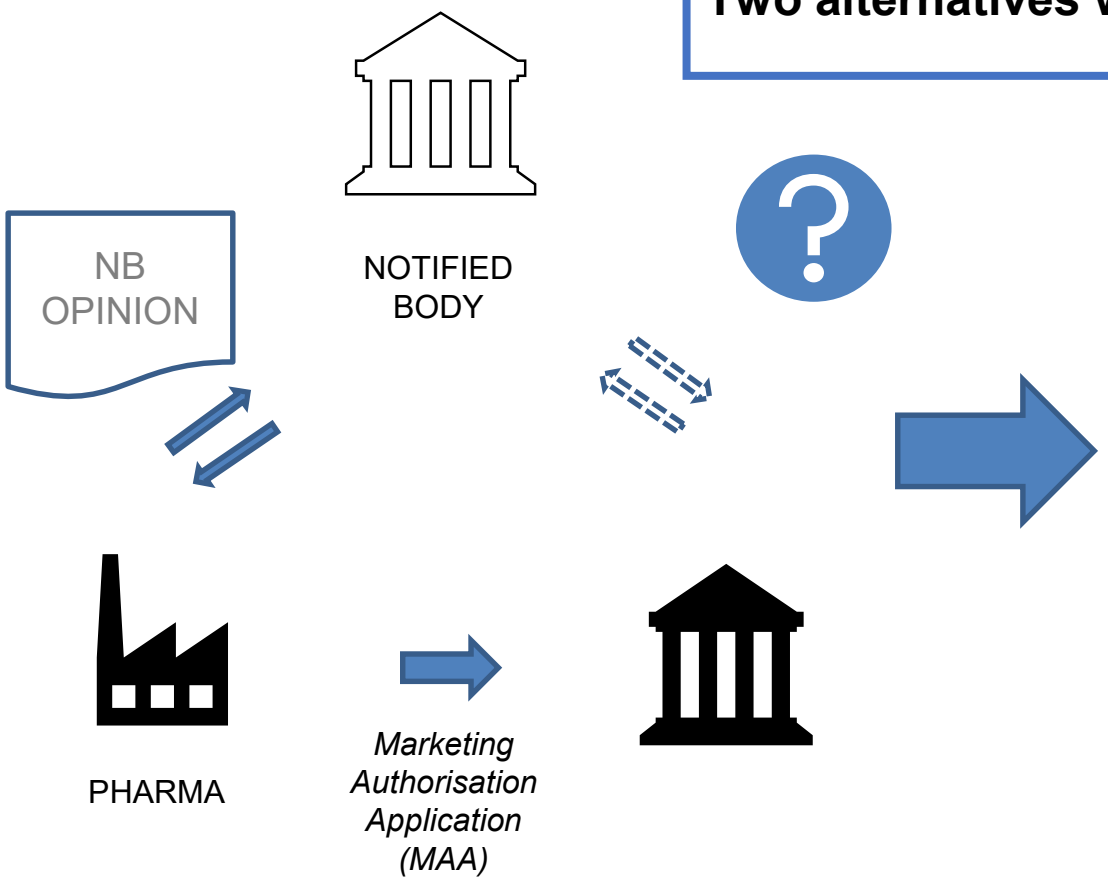
Challenges faced by SMEs

- Very challenging diseases (e.g. small patient populations to satisfy all stakeholders requirements)
- Recognition of rare disease through ODD
- Many single arm studies

Possible ways forward

- Provide a stable and positive environment for sustainability;
- Further incentivise research and development;
- Support HTA understanding of Orphan Medicinal Product specific requirements (patients and disease);
- Real-world-evidence (RWE)

An SME visionary view on an integrated pathway for Drug Device Combination Products



Two alternatives within the current legislative frameworks

Applicant option 1:

Liaise independently with the NB e.g. provide NB certificate as part of the MAA (as per today).

Applicant option 2:

One integrated pathway with EMA/NCA as the coordinator throughout the product's lifecycle e.g. the NB opinion is embedded in the MAA procedure.