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SCIENCE MEDICINES HEALTH

EMA support to innovation

Translating innovation into access for ATMPs:
3rd EU-Innovation network multi-stakeholder meeting

15 November 2024 – Rome, Italy

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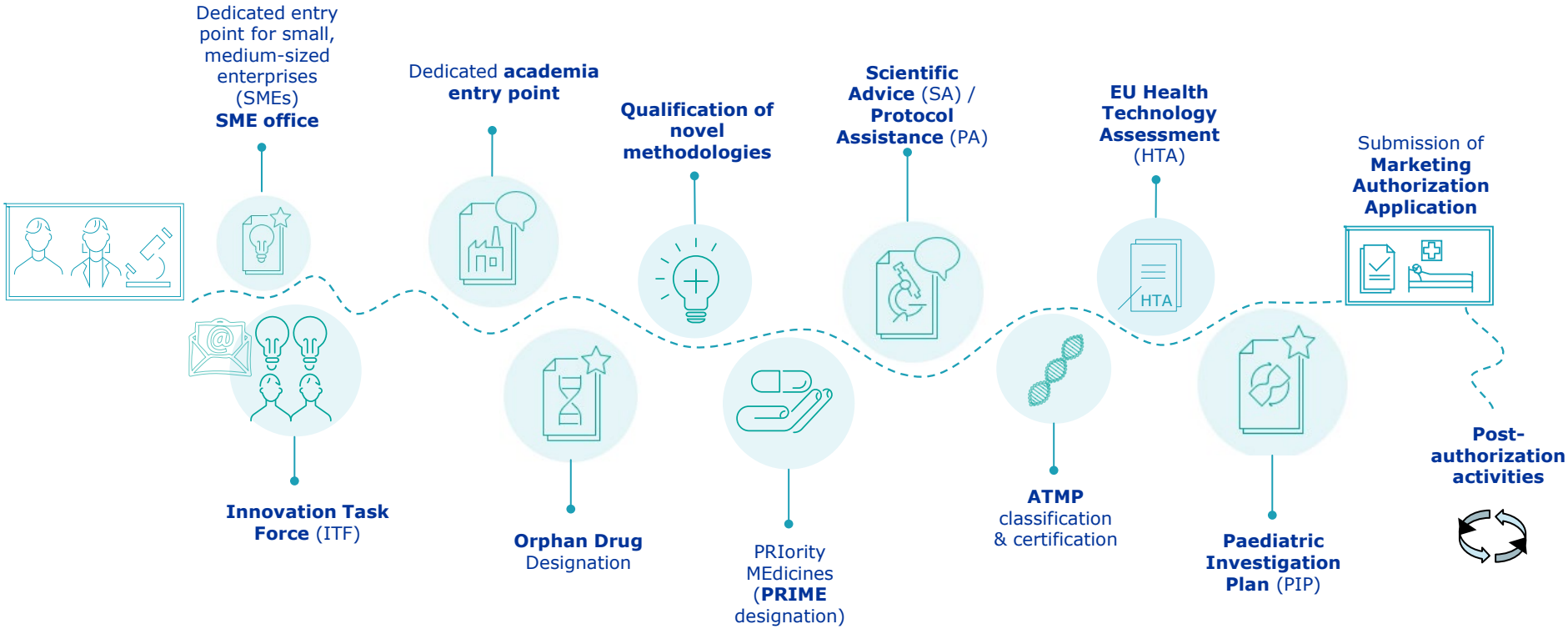
- The presenter does not have any conflict of interest.

EMA interactions across the medicine life cycle



ITF briefing meetings

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Multidisciplinary platform

for preparatory dialogue and orientation on

innovative methods, technologies and medicines

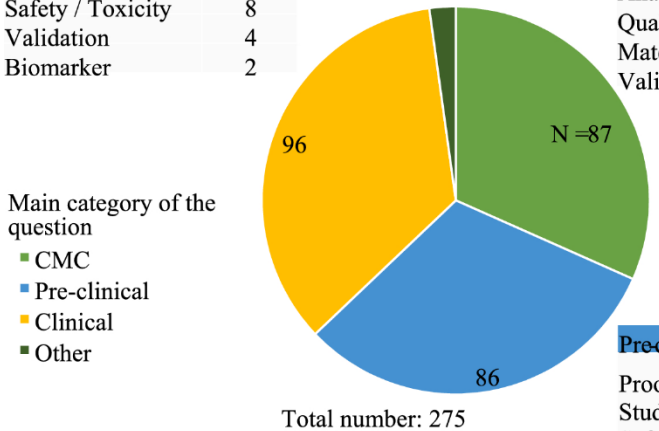
- Support **innovative** drug development
- **Early informal** dialogue with opinion leaders
- 1,5-hour discussion – *Free of charge*
- Brainstorming "style" on innovation in areas without existing guidance
- First step to engage is submit completed 3-page template

Which type of questions can be asked? Which type of feedback will I receive during an ITF meeting?

Classification of questions asked during ITF meetings (2021-2022)

Clinical	Top 5 (%)
Study design	46
Endpoints	15
Safety / Toxicity	8
Validation	4
Biomarker	2

CMC	Top 5 (%)
Manufacturing technology	21
Analytical technology	18
Quality	18
Materials	14
Validation	9



Total number: 275

Pre-clinical	Top 5 (%)
Proof of Concept	22
Study design	21
Safety / Toxicity	20
Biomarker	9
Mechanism of Action	8

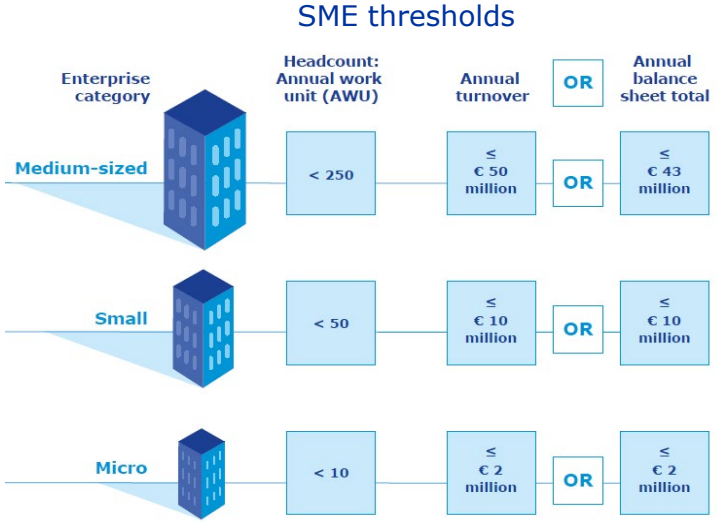
In 22% of the feedbacks provided, experts recommended to continue their engagement with the regulators through either another procedure at EMA, such as scientific advice or qualification of novel methodologies, or through national competent authorities' regulatory advice. […] **Actionable initial guidance was provided in 85% of the cases**. Moreover, for 16% of the questions, a clarification in writing was requested by the applicant through the ITF briefing meeting report which was followed by a written post-meeting explanation.

Uster, Cordo' et al., Insights into Early Interactions on Innovative Developments with European Regulators, TIRS 2024

Dedicated support to micro, small and medium-sized enterprises (SME)

SME Office

- Assignment and renewal of SME status
- SME briefing meetings: early dialogue with regulators to discuss regulatory strategy and navigate the range of procedures and incentives available
- Regulatory support (e.g., eligibility for EMA procedures)
- Fee incentives
- Translation assistance for product information
- Training and awareness via info days & SME newsletters



EMA has a **framework for collaboration with academia** since 2017

- Promote and further develop regulatory support **to translate academic research into novel methodologies and medicines**
- Ensure that the **best scientific expertise** and academic research is available to inform regulatory decision-making
- **Collaborate on areas of research on regulatory science** (novel approaches, endpoints, methodologies)

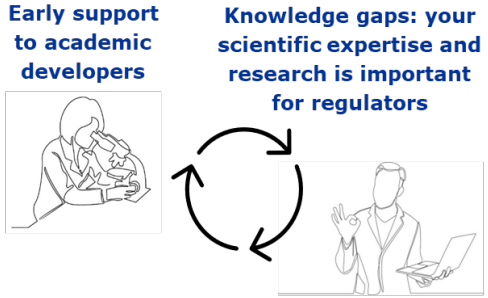
Incentives for Academia:
Scientific advice/protocol assistance **free-of-charge** to academic developers of orphan medicines

Dedicated entry point for Academia:
Academia@ema.europa.eu

EMA interactions with academia

Academia briefing meetings

- EMA listens to researchers' challenges
- Bidirectional exchange
- Opportunity to discuss regulatory aspects
- Support on how to advance R&D
- No fee, no briefing book
- Possibility of iteration and follow-up



European Platform for Regulatory Science Research – launch in 2025

Virtual event

Advancing Regulatory Science Research

18 November 13:30-17:30 CET

Registration via EMA website

Supporting the development of advanced therapies

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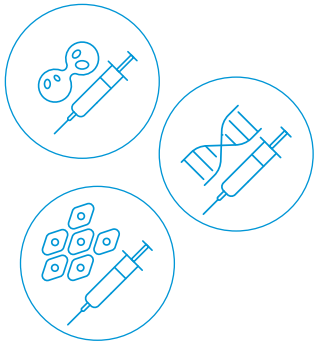
ATMP specific tools



- **Classification:** developers can consult the EMA Committee for Advanced Therapies (CAT) to confirm that their product is classified as an ATMP
- **Certification** of ATMP quality and non-clinical data from SMEs by the EMA CAT

Identification of any **potential issue** to be addressed **at early stage**, before the marketing authorization application

Scientific Advice

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Advising developers on product-specific questions during development of medicines *to meet regulatory and scientific requirements:*

1	how to manufacture them
2	how to test them in non-clinical studies
3	how to test them in humans in clinical trials
4	how to study them in specific populations (e.g., rare diseases and children)
5	prospective in nature, it is NOT a pre-assessment

Provided by the Committee for Medicinal Products for Human Use (CHMP) on the recommendation of the Scientific Advice Working Party (SAWP)

Qualification of novel methodologies

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Procedure to guide the development of novel, more efficient ways to develop medicines (development of new endpoints for clinical trials, new imaging method, new biomarker…)

Who can apply?

- Consortia / networks
- Public / Private partnerships
- Academic developers
- SMEs
- Pharmaceutical industry

Qualification advice

- On future protocols and methods for further development towards qualification
- Evaluation of scientific rationale and preliminary data
- Confidential

Qualification opinion

- On the acceptability of a specific use of the proposed method in an R&D context
- Assessment of submitted data
- Publicly available

Examples: *methods to predict toxicity, strategies to enrich a patient population for a clinical trial, surrogate clinical endpoints, patient/caregiver reported outcomes, patient registries…*

PRiority of MEdicine (PRIME) scheme

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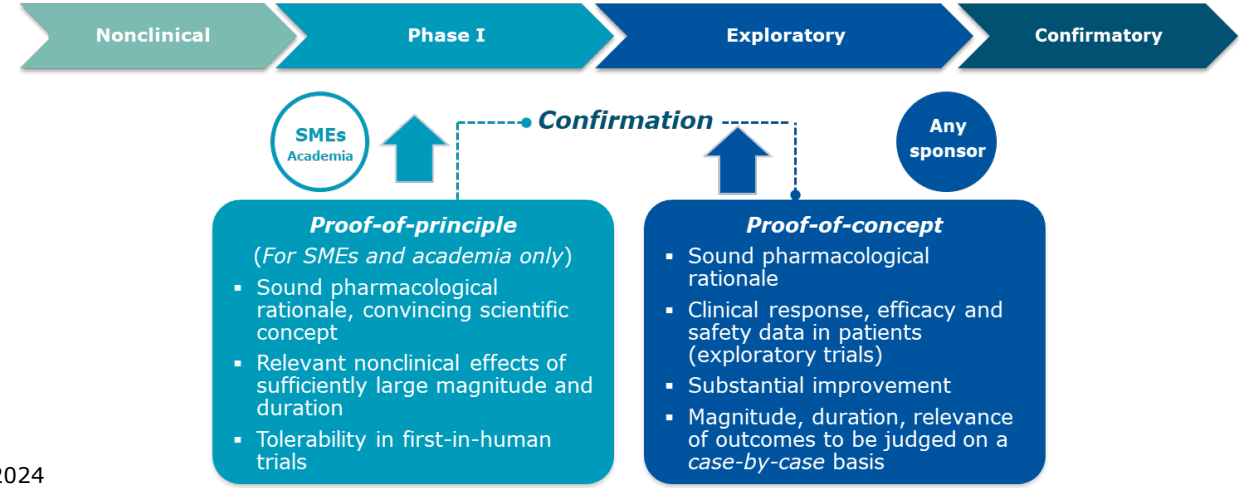
Foster the development of medicines of major public health interest, with a focus on *therapeutic innovation*. Such medicines have the potential to address an *unmet medical need.*

Reinforce scientific and regulatory advice

Optimise development for robust data generation

Enable accelerated assessment



Orphan designation

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A medicinal product can obtain an *orphan designation* if:

- it is intended for the treatment, prevention or diagnosis of a **disease that is life-threatening or chronically debilitating**;
- the **prevalence** of the condition in the EU is **less than 5 in 10,000** or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment for its development;
- **no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised**, or, if such a method exists, the medicine must be of significant **benefit** to those affected by the condition.

- Sponsors who obtain orphan designation benefit from **protocol assistance**, a type of dedicated scientific advice from CHMP-SAWP
- **Academic applicants receive free protocol assistance** for developing orphan medicines

Conclusions

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Looking into the future:

- Promotion of research and innovation by **supporting developers** throughout the medicine lifecycle
- Facilitate engagement and **dialogue with SMEs, academia** and their stakeholders
- Active contribution into relevant **EU initiatives enabling innovative medicines development and access to patients**

Thank you for your attention!

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