EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Expert panels orphan device pilot

Annual meeting of EMA eligible patient and healthcare professional organisations

Presented by Michael Vogl on 20 November 2024
Expert Panels and Groups Office (H-QA-EPG)

An agency of the European Union

Current challenges

- For developers
  - Transition period from the Medical Device Directive to the Medical Device Regulation brought new challenges to the development of devices
  - Notified Body (NB) have no possibility to advice on the development
  - Orphan devices present a unique challenge to the medical device industry
- For clinicians
  - It needs to be ensured that devices that are intended for a small (sub)-population stay on the market
  - Innovation in the area of orphan devices needs to be promoted in the EU to ensure it reaches European patients as soon as possible

Orphan device criteria (proposed additional slide)

- MDCG 2024-10 Clinical evaluation of orphan medical devices
- A medical device or an accessory for a medical device should be regarded as 'orphan device' if it meets the following criteria:
  - the device is specifically intended to benefit patients in the treatment, diagnosis, or prevention of a disease or condition that presents in **not more than 12,000 individuals in the EU per year**, and at least one of the following criteria are met:
  - there is **insufficiency of available alternative options** for the treatment, diagnosis, or prevention of this disease/condition, or
  - the device will offer an option that will provide an **expected clinical benefit** compared to available alternatives or state of the art

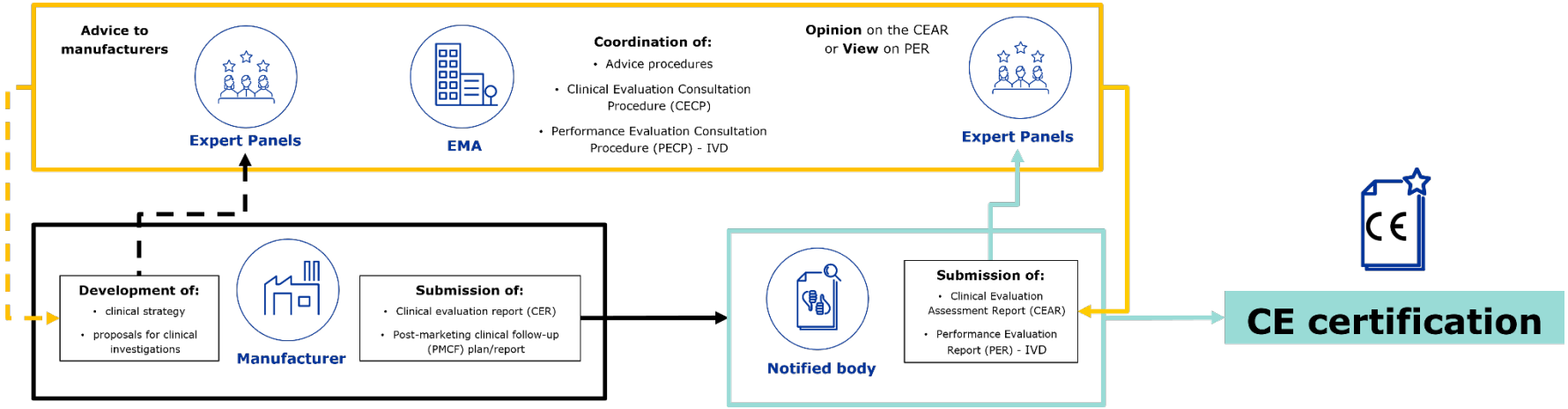
Expert Panels involvement

- Early-stage advice
  - Advice on the orphan status of the medical device
  - Advice on the manufacturer's clinical development strategy and proposed clinical investigations
- Late-stage advice
  - Advice on the orphan status of the medical device
  - Advice to Notified Bodies and manufacturers possible

Early-stage advice

- Devices for the pilot: class III and class IIb intended to administer and/or remove a medicinal product
- Applicants: manufacturers
- Timing: prior to clinical evaluation and/or investigation
- Content: clinical development strategy and proposals for clinical investigation; always preceded by orphan status designation
- Impact: due consideration given by the manufacturer and documented in the clinical evaluation report (CER)
- Advice optional/voluntary and independent of the clinical evaluation consultation procedure (CECP)

Pilot on advice to NBs on orphan devices – early-stage



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Late-stage advice (requested by a manufacturer)

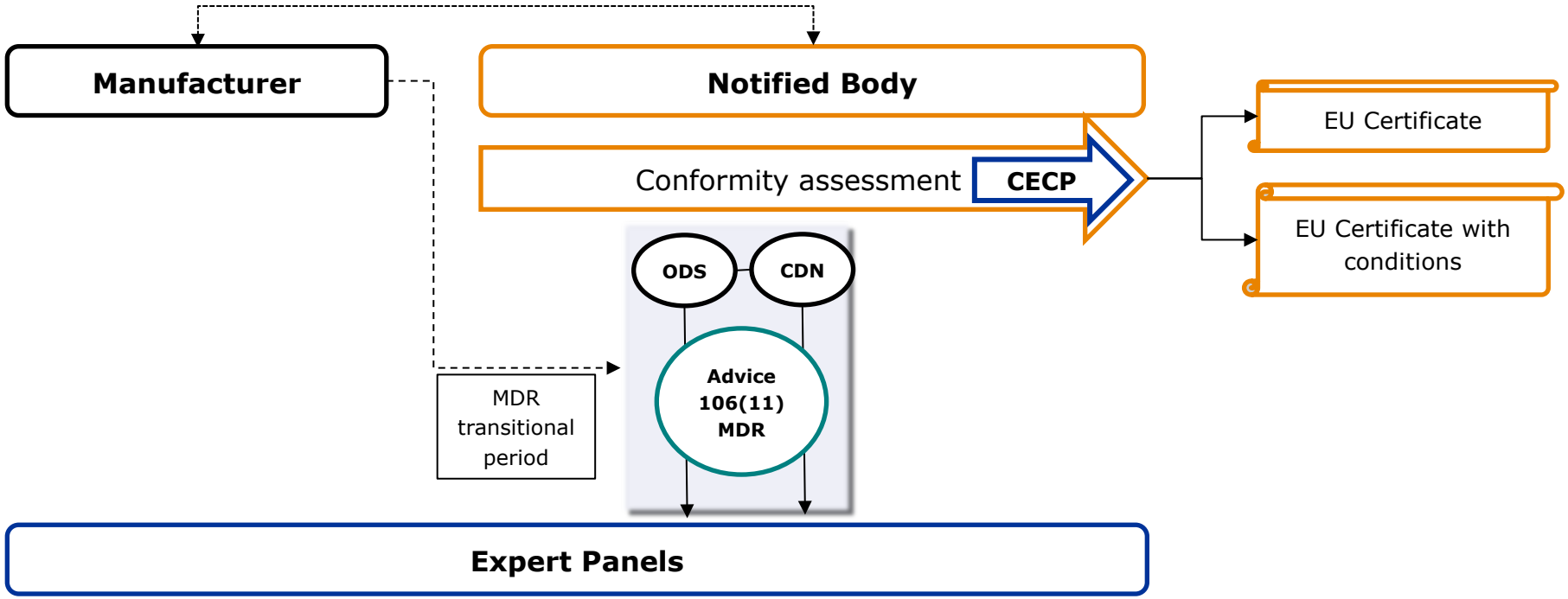
- Early scientific advice (under MDR 61(2)) might not possible:
  - clinical development is finalised
  - device already undergoing conformity assessment (in agreement with NB)
- In accordance with MDR Article 106(11) and as extraordinary measure during the MDR transitional period* (if it does not interfere with the assessment by the NB)
- Manufacturer needs to be able to update its clinical evaluation report (CER) taking into consideration the expert panel's views
- Manufacturer makes the advice available to the NB (e.g. as an annex to the CER).

*until 31.12 of 2027/2028, depending on the risk class of the device

Late-stage advice (requested by a notified body)

- In accordance with MDR Article 106(11)
- NB should consult the manufacturer before (for information and/or request further input)
- Advice requested in the form of specific questions from the preliminary analysis of the clinical evaluation
- Particularly useful if the NB does not agree or is uncertain with the manufacturer's orphan device claims
- If the NB has divergent views from the expert panels those reasons should be registered in its clinical evaluation assessment report (CEAR)

Pilot on advice to NBs on orphan devices – late-stage



ODS – Orphan device status
CDN - Clinical data needed

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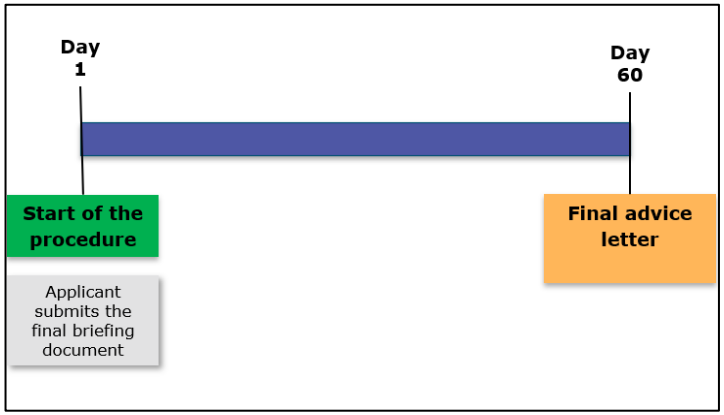
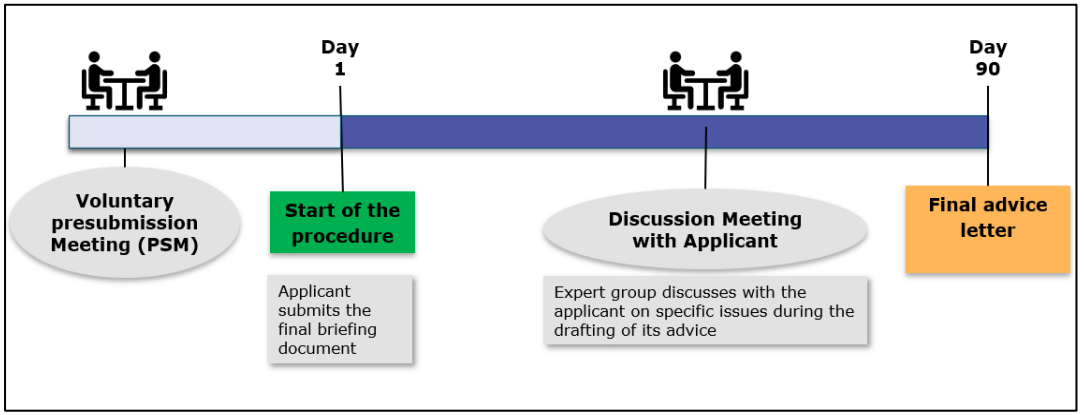
Procedural timeline in the pilot (tentative)*

OD status



OD status + clinical development



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Any questions?

Further information

[Insert relevant information sources or contact details as applicable.]

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