Criteria for orphan device status

Current proposals and definitions

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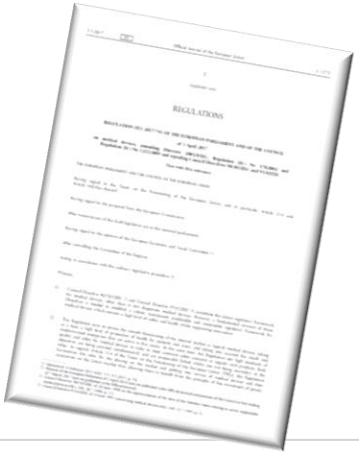
HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

ESC
European Society of Cardiology

Orphan Medical Devices– the challenge

- **Challenging to define**
  - Medical device intended to benefit patients in the treatment or diagnosis of a rare disease or condition
- **Challenging to develop and regulate**
  - Clinical Evidence
    - How to generate & demonstrate
    - How to evaluate pragmatically
- **Many OD in CVD therapeutic area**
- **Significant public health concern**



Unique challenges for Orphan Devices

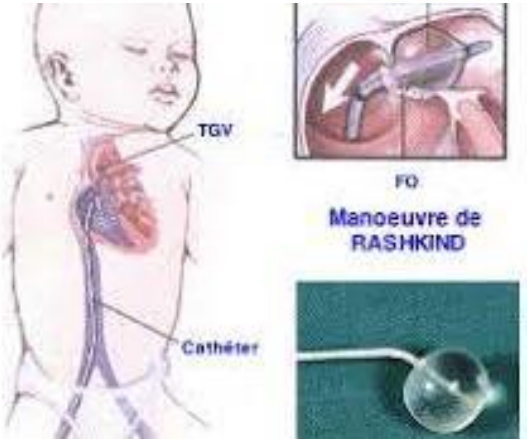
Development and Assessment challenges

Clinical Evidence

- What's required for safety
- What's required for performance*

Multifactorial barriers

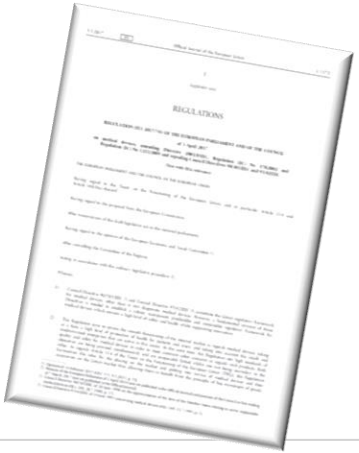
- Regulatory
- Economic



IMDRF Berlin 2023

EU Regulators & Priority for Orphan Devices

- *The MDCG acknowledges the specific situation of 'orphan devices' and will pursue work with a view to providing a definition for 'orphan devices' and suggesting specific guidance or other means of assistance for those products to be able to meet the legal requirements. Sustainable solutions are also needed in the mid- and long-term for orphan devices MDCG 2022-14*



MDCG TF on Orphan Devices

- co-chair: DE/COM, IE
- extension of membership 2023
- NCAs of 11 MS: BE, CZ, DE, ES, FI, FR, HR, IE, IT, NL, PT
- Team-NB, NBCG-Med
- ***ESC***, BioMed Alliance, CMPE, EAP
- MTE, COCIR, EAAR, EUROM, Euromcontact
- observers: EMA, IRDiRC, Connect4Children
- ‘open’ meetings with stakeholders & ‘closed’ meetings only with NCAs
- **Specific WP: Criteria and specific guidance for clinical requirements for certification of OD under MDR**

Criteria for OD status –principles for a definition

- Epidemiology
  - Orphan populations
  - Sub-populations
  - Conditions and indications
- Additional criteria
  - Unmet need or insufficiency of alternative treatment/diagnostics
  - Potential safety/benefit

CORE–MD
Coordinating Research and Evidence for Medical Devices

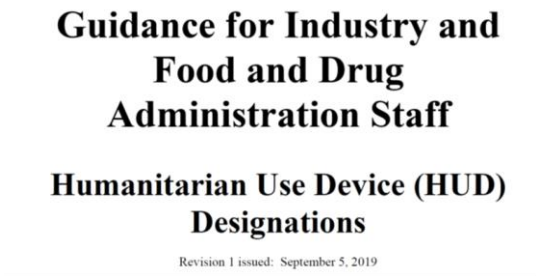
OD status as a tool for approval & access

- EMA : Orphan Medicines
  - it must be 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 must not be more than 5 in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed 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.
- **Orphan Designation**
  - Defined incentives
  - Regulatory assessment similar

OD status as a tool for approval & access

- US FDA : HUD/HDE
  - medical devices intended to benefit patients in the treatment or diagnosis of diseases or conditions that affect or are manifested in not more than 8,000 individuals in the United States per year

- Designation step
- Recognition of orphan subsets
- Probably benefit concept
- Some market restrictions



Guidance for Industry and Food and Drug Administration Staff

Humanitarian Use Device (HUD) Designations

Revision 1 issued: September 5, 2019

OD status as a tool for approval & access

- Japan PMDA: Orphan Device Designation

- **Small number of patients**
  - < 50,000 in Japan (Prevalence Rate < 3.9 in 10,000 people)
  - Or designated intractable disease
- **High medical needs**
  - Unmet needs - No alternative medical intervention is available
  - Significant benefit - Significantly improved efficacy and/or safety expected compared to existing products
- **High probability of successful development**
  - Strong rationale to use the product, and an appropriate development plan
- Incentives for R&D and priority review

OD status in EU under MDR –current proposals & definition considerations

- Epidemiology
  - Orphan Populations
  - Sub-populations / Orphan subsets
    - Patient factors and medically plausible
  - Conditions and indications
- Additional criteria
  - Unmet need or insufficiency of alternative treatment/diagnostics
  - Potential safety/benefit
    - Description of alternatives, patient device characteristics
- Option to use Expert Panels

Conclusion –OD definition and criteria

- Selective & objective
- Feasible to demonstrate and assess
- Capture authentic use cases
- Possible to evaluate and revise
- Transparent
- Justify principle of certification with more limited clinical evidence
- Balancing protecting patients with allowing access

Thank you

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