Medical Devices

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Medical devices can be orphans, too

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Definitions

Whereas the definition of orphan drugs is well established in current EU legislations, orphan devices are relatively unknown. Not defined in the EU MDR 2017/745, the MDCG 2024-10 finally provided last year the first EU-based definition of an orphan medical device – as one "*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 European Union per year…*".[1]

In addition to rare indications, the paediatric population is also underserved in the field of medical devices as devices are, by default, designed for the adult population.[2] For paediatric purposes, instruments and implants may need to be customised or used off-label.[3] Industry experts therefore collectively call these products orphan and paediatric devices or OPDs.[3]

Below are some examples of OPDs

- *"Therapeutic devices such as microvascular plugs, which can be used for closure of patent ductus arteriosus in premature babies.*
- *Monitoring devices such as electroencephalogram… devices combined with artificial intelligence algorithms to detect seizure activity in neonates.*
- *Supportive devices such as exoskeletons used to assist mobilisation in patients with conditions such as spinal muscular atrophy, or Duchenne muscular dystrophy.*
- *Diagnostic devices such as genetic tests used for the diagnosis of many rare diseases."*[3]

Expert panels

In 2024, the EMA initiated a new pilot programme for expert panels to support manufacturers and notified bodies to address challenges faced by orphan medical devices (mainly high risk [Class IIb and Class III]), especially with respect to generating clinical evidence for these devices in the premarket phase. An information session was organised in September 2024,[4] followed by the release of a Q&A document.[5]

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The role of medical writers in orphan devices

To apply for expert panel consultation, an application form and a briefing document will be submitted. One of the key sections is the justification of the orphan status of the device based on the state-of-the-art (SoTA), e.g., the epidemiology of the disease or condition and insufficiency of current treatment options.

For legacy devices and devices in advance stages of development, the clinical evaluation report will need to include the rationale for the orphan designation that should be consistent with briefing document SoTA. Another component is the considerations for limited premarket clinical evidence, off-label use data, and extrapolation of these information to the orphan intended use.

For drug-device combination products, the link between the orphan medicinal product and the orphan device component has to be very clear.

In the nascent field of orphan devices, regulatory medical writers play a vital role in putting all these essential components together to support underserved "orphan" populations.

References

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Raquel Billiones, who has a PhD in Biology and is *Medical Writing*'s editor-in-chief, has been a regulatory writer for almost 20 years, covering both pharmaceuticals and medical devices. Her core competencies include clinical trials and marketing authorisation submission documents, data disclosure and protection, and project and people management. She is currently employed at Alexion (AstraZeneca Rare Disease).