ESIP Position on

Implementing the European Medical Device Regulation and a New Regulatory Framework for Orphan Medical Devices

European Social Insurance Platform (ESIP)

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About the European Social Insurance Platform (ESIP)

The European Social Insurance Platform (ESIP) represents 46 national statutory social insurance organisations in 18 EU Member States and Switzerland, active in the field of health insurance, pensions, occupational disease and accident insurance, disability and rehabilitation, family benefits and unemployment insurance. The aims of ESIP and its members are to preserve high profile social security for Europe, to reinforce solidarity-based social insurance systems and to maintain European social protection quality. ESIP builds strategic alliances for developing common positions to influence the European debate and is a consultation forum for the European institutions and other multinational bodies active in the field of social security.

ESIP members support this position insofar as the subject matter lies within their field of competence.

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Position Paper
Implementing the European Medical Device Regulation and a New Regulatory Framework for Orphan Medical Devices

In the context of the ongoing evaluation of the European Medical Device Regulation (Regulation (EU) 2017/745; MDR) and In Vitro Medical Device Regulation (Regulation (EU) 2017/746; IVDR), the European Social Insurance Platform (ESIP) proposes the following actions to continue safeguarding patient safety and the quality of treatment with medical devices:

- **Conduct a thorough formal evaluation of the MDR and IVDR** in all their legislative components, before considering further legislative changes. Information about potential market withdrawals should be incorporated as a central element of the planned evaluation.

- **Establish a dedicated legal framework for orphan medical devices**, based on the below recommendations:

    - **Introduce a definition of orphan medical devices**, based on the recommendations of the Medical Devices Coordination Group (MDCG) e.g. devices intended for the treatment of diseases or conditions that affect no more than 12,000 people per year in the EU;

    - **Establish a central authority responsible for orphan medical devices**, among others for granting and where necessary revoking the orphan status upon well-defined conditions, greenlighting the clinical evaluation of orphan devices led by Notified Bodies (NB), ensuring uniform and transparent authorisation fees with possible financial and technical support to small and medium-sized enterprises (SMEs);

    - **Define post-marketing plans for the collection of the missing clinical data**, as part of the approval conditions of an orphan medical device;

    - **Establish diseases-specific central registries for orphan medical devices**, under the responsibility of the central authority mentioned above.

State of Play of the Medical Devices Regulations

The European Medical Device Regulation (Regulation (EU) 2017/745; MDR) and In Vitro Medical Device Regulation (Regulation (EU) 2017/746; IVDR) entered into force in 2017 with the ambition to achieve the following objectives: to increase the safety of medical devices placed on the EU market, to raise the requirements for the clinical evaluation of high-risk medical devices, to create transparency by implementing a central, in parts publicly accessible database to collect information about medical devices and their manufacturers (EUDAMED), and to harmonise the quality of work of Notified Bodies throughout Europe. The implementation of the MDR was necessary to adapt the classification, clinical evaluation and marketing rules of the old Medical Device Directive of 1993 to today's technological state of the art.

However, the MDR and IVDR are currently subject to intense political discussion and increasing criticism. Several stakeholders, especially representatives of the medical technology industry, have raised concerns on the implementation of the legislative framework on medical devices, claiming that Europe would face serious challenges in patient care in terms of market withdrawals and consequent shortages. They argued that due to insufficient Notified Body capacity and sharply rising certification costs and requirements, several critically important devices are already being withdrawn from the market by their manufacturers. Furthermore, they contend that future innovations in medical devices are likely to occur outside of Europe.

The European Social Insurance Platform (ESIP) regards **the implementation of MDR and IVDR as a necessary and important step towards improving patient safety and the quality of treatment with medical devices.**

ESIP acknowledges that some manufacturers have experienced problems in the transition to the new legal framework, in terms of Notified Bodies' capacity and possibly legal uncertainties regarding the marketing of so-called legacy devices. However, appropriate measures have been taken to address these issues through targeted amendments to the MDR and IVDR – subsequently expanding the transition period – and numerous guidance documents have been published by the Medical Device Coordination Group (MDCG).

Despite the concerns mentioned above, robust information about medical device shortages caused by market withdrawals is still missing. Only by providing this information, a thorough evaluation can be conducted and further steps toward full MDR implementation be considered. ESIP therefore calls for **increased efforts to collect and verify information about potential market withdrawals and to incorporate it as a central element of the planned MDR evaluation.**

In light of the European Parliament Resolution on the urgent need to revise the Medical Devices Regulation of 23 October 2024,[1] **ESIP cautions against the reopening of both MDR and IVDR until the formal evaluation is completed and all legislative components, particularly the EUDAMED database, are fully implemented**.

In parallel, ESIP acknowledges the need to introduce adapted rules for medical devices for the treatment of rare diseases (so-called orphan medical devices).

Proposals for a new regulatory framework on orphan medical devices amending the MDR

In order to promote the development and marketing of orphan medical devices (ODs) without compromising the safety and quality standards of the Medical Device Regulations, ESIP calls for the following actions:

1. Agree on a clear definition of "orphan medical devices"

The MDCG published a guidance document on the clinical evaluation of orphan medical devices (MDCG 2024-10)[2]. ESIP welcomes and broadly supports this initiative. In particular, ESIP supports the proposed definition for ODs, aimed to restrict the scope to those devices intended for the treatment of diseases or conditions that affect no more than 12,000 people per year in the EU. In addition, an OD must offer an expected clinical benefit compared to existing treatment alternatives.

ESIP calls to integrate the definitions of ODs, orphan population and orphan sub-population recommended by the MDCG into the definitions of Article 2 of the MDR. This would be crucial to avoid, on the one hand, legal uncertainty, and on the other, the so-called 'orphanisation' of a disease: the increased targeting of specific subgroups of broader disease groups to the point that they become rare.

2. Establish a central authority for orphan devices

The MDCG guidance document 2024-10 outlines mechanisms for the justification of the OD status, for the clinical evaluation process and for ODs post-marketing surveillance. For this purpose, **ESIP considers it crucial to establish a central authority for ODs**.

In consultation with the European Medicines Agency (EMA) expert panels established under Articles 106 and 48(6) respectively of the Medical Device Regulation (EU) 2017/745 (MDR) and In Vitro medical Device Regulation (EU) 2017/746, the central authority should be entrusted with the following tasks:

[1] European Parliament, JOINT MOTION FOR A RESOLUTION on the urgent need to revise the Medical Devices Regulation (2024/2849(RSP)

[2] Medical Device Coordination Group (MDCG). Guidance on the Clinical evaluation of orphan medical devices (MDCG 2024-10)

- assess whether medical devices meet the established conditions to receive the OD status;
- assess and approve the clinical evaluation dossier of the OD led by Notified Bodies, with focus on the assessment of the abovementioned expected clinical benefit;
- requiring a post-marketing surveillance plan to follow up on the clinical requirements set as part of the approval conditions to address unresolved clinical evaluation issues (e.g. long-term effectiveness);
- Review and where necessary revoke the orphan status if the conditions defined in the post-marketing surveillance plan are not met, either at the time of CE certificate renewal or at any other point specified in the post-marketing surveillance plan.

Only a central authority, legally empowered within the EU medical device legislative framework, can ensure consistent and transparent procedures for granting OD status and market access for ODs. An agreement from this central authority should be mandatory for granting the CE marking for ODs, similar to the existing requirement for certain high-risk or implantable medical devices, where an opinion of the expert panel is needed before issuing a CE certificate.

Centralising and concentrating medical expertise will ensure that medical devices are evaluated in a reliable, transparent and consistent manner. This will streamline and simplify the tasks of the existing Notified Bodies that certify medical devices, relieving them from the necessity of maintaining expert personnel for the highly specialised and relatively rare clinical evaluation of orphan devices. Assessment of the technical documentation, however, should remain in the responsibility of a Notified Body, as they have highly qualified personnel for this task.

Furthermore, a central authority could ensure uniform and transparent authorisation fees, early advice and possible discounts for SMEs.

3. Clearly define criteria for the clinical evaluation and approval requirements for orphan devices

The MDCG guidance document 2024/10 includes criteria on the clinical evaluation and clinical investigation of ODs. **Annexes XIV and XV of the MDR should be amended accordingly.**

As clinical evidence for orphan devices is often limited at the time of marketing authorisation, binding requirements should be defined for the collection of the missing clinical data as part of the approval conditions. Post-Market Clinical Follow-Up (PMCF) plans should be specifically designed to address the missing clinical evidence. Post-marketing surveillance requirements should be defined by the central authority following consultations with manufacturers. The comparators ('standard treatment', if available) against which the orphan medical devices must be assessed should be clearly identified in the OD status decision.

Manufacturers should also be required to clearly define in advance specific quality assurance criteria for OD use (e.g. criteria for user/personnel training, structural criteria for facilities such as hospitals), to ensure safe use of ODs.

Furthermore, documenting the use of orphan devices through designated registries should become mandatory.

4. Ensure appropriate and timely collection of clinical data for orphan devices

ODs are used only in small patient populations. Accordingly, obtaining meaningful clinical data represents a major long-term challenge. To address this issue, **ESIP proposes entrusting the central authority with the establishment of EU-wide, disease-specific central OD registries.** The central authority should be made responsible to organise, host, maintain and regularly update such registries. Ideally, these registries should include data on all affected patients across the EU and should be made publicly available.

As more new ODs for specific orphan populations or sub-populations enter the market, they should be added to these registries, enabling direct comparisons of their clinical outcomes.

Where national registries exist, a pooling of the relevant data for an EU-wide evaluation should be considered.

ODs with limited clinical data at the time of their certification should only be legally used in the European market if their usage is reported to the above-mentioned central registries. This approach will ensure an appropriate and comprehensive data collection, and will subsequently support informed treatment decisions and provide predictability for medical device manufacturers regarding market access. Accordingly, reimbursement of ODs by the national social security systems should be contingent upon the submission of treatment data by the manufacturer as part of a post-marketing surveillance plan mandated by the central authority. Member States should be encouraged to lay down "comparative effectiveness research (CER)" or "coverage with evidence development (CED)" programmes to ensure and streamline the required device usage reports to central OD registries.

5. Targeted support for developers of orphan devices

Measures to support the development, clinical evaluation and marketing of ODs in the EU would be crucial to promote access to healthcare and better health outcomes, as well as market competitiveness.

ODs are often developed by highly specialised small and medium-sized enterprises (SMEs) which do not have the financial and administrative resources, nor the risk management capacity of larger manufacturers. To reduce the financial burden on SMEs and de-risk their investment, **special fees for marketing authorisation as well as specific consultation programmes by the central authority could be considered.**

In conclusion, regardless of potential legislative changes intended to enhance patient access to (orphan) medical devices, the primary focus should be on ensuring patient safety and facilitating access to evidence-based innovations within a secure environment. For this purpose, it is essential to ensure that as many ODs as possible undergo the necessary clinical evaluations, while also minimising their use outside of approved indications ("off-label-use").