A INOVAÇÃO NO SETOR DOS DISPOSITIVOS MÉDICOS

CAMINHOS REGULAMENTARES ESPECÍFICOS PARA OS DISPOSITIVOS ÓRFÃOS E INOVADORES

MARIANA MADUREIRA

Webinário 2, INFARMED, 26/11/2025

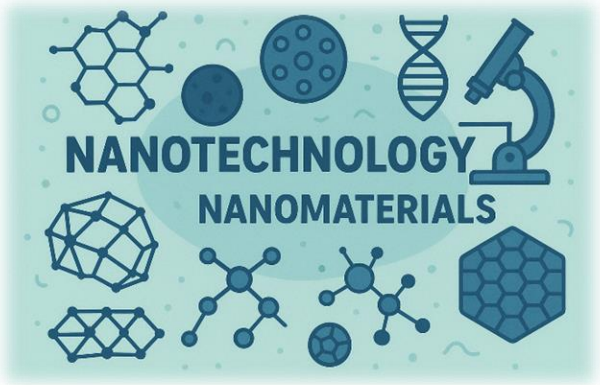
REPÚBLICA PORTUGUESA
SAÚDE

Infarmed
Autoridade Nacional do Medicamento e Produtos de Saúde, I.P.

AGENDA

DISPOSITIVOS MÉDICOS

INOVAÇÃO NO SETOR

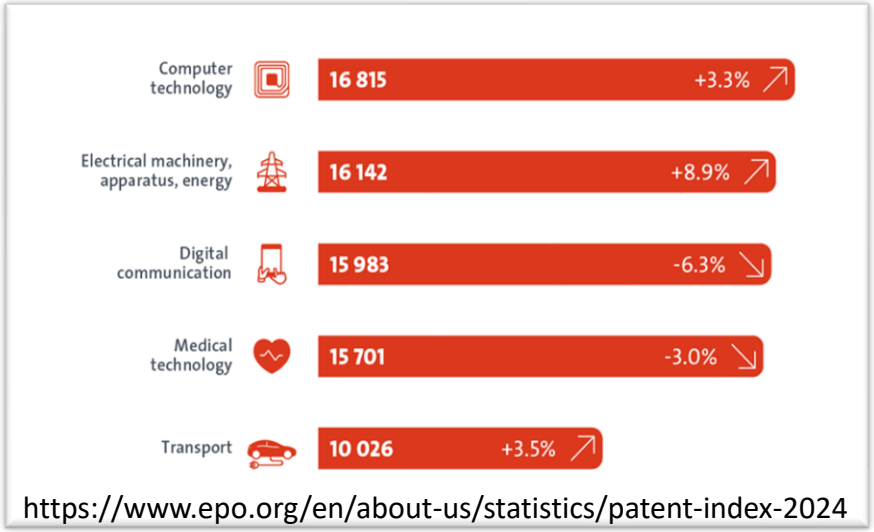


Wearable technologies

3D Printing

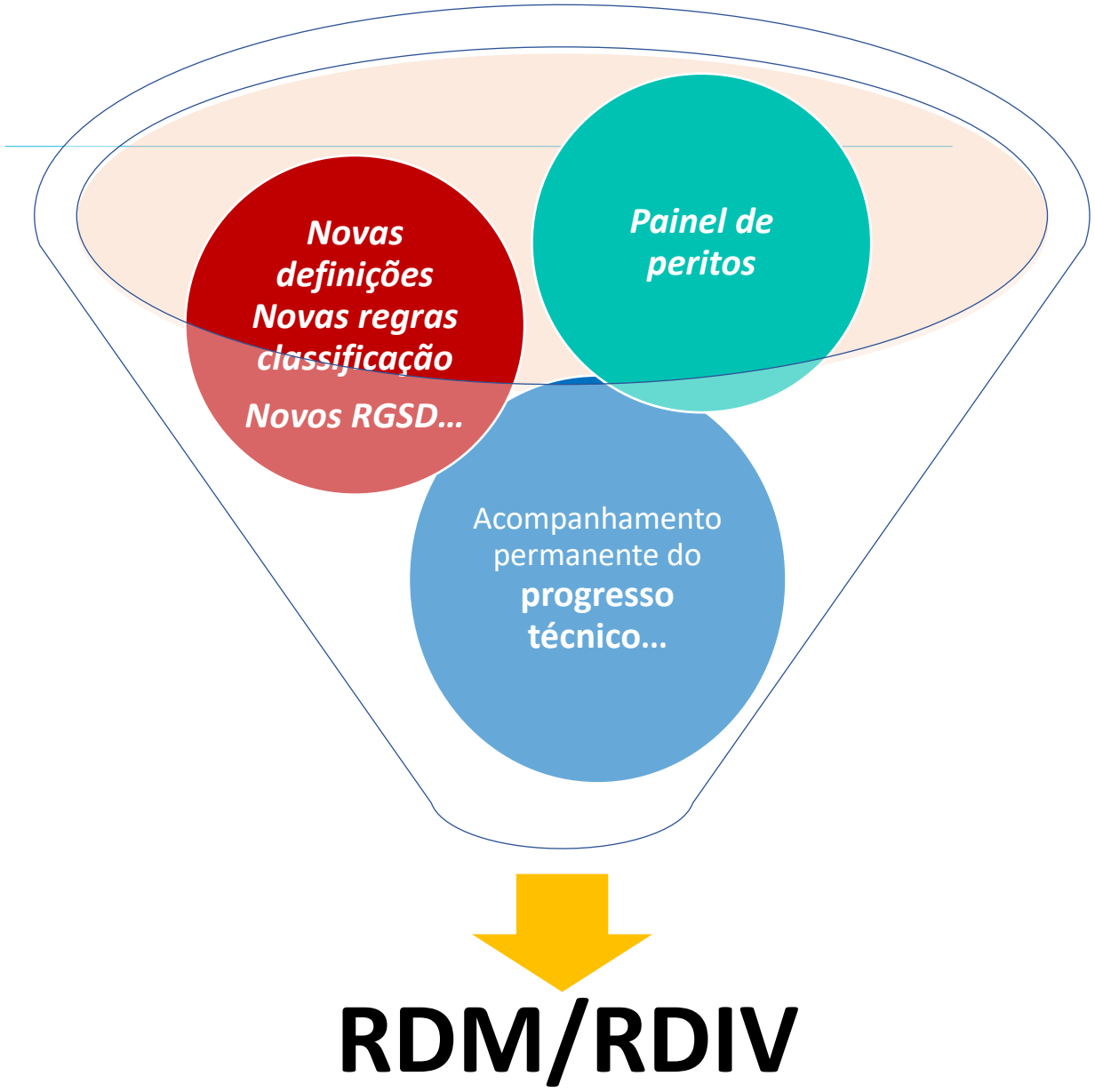
Robotics

...



https://www.epo.org/en/about-us/statistics/patent-index-2024

DISPOSITVOS MÉDICOS – ELEMENTOS DE INOVAÇÃO



RDM/RDIV

European Commission

EUROPEAN COMMISSION EXPERT PANELS
ON MEDICAL DEVICES AND IN VITRO DIAGNOSTIC DEVICES

- **Avaliação da conformidade de certos dispositivos inovadores de classes de risco elevadas**
- **Aconselhamento Científico** - apoio ao desenvolvimento de dispositivos médicos

7. New technologies – Terms of reference

Advises on issues related to application of new and emerging technologies to medical devices, including software, apps and cybersecurity.

+ Horizon Scanning…

MDCG NT WG Chair & Co-chairs: COM, PT/INFARMED & DE/MoH

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CAMINHOS REGULAMENTARES

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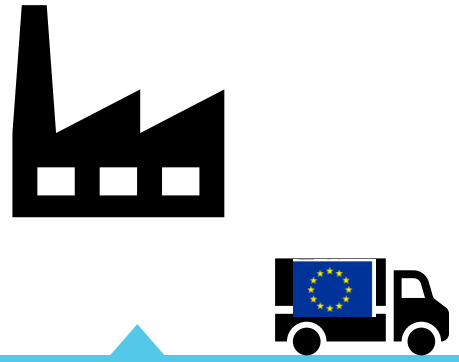
DISPOSIVOS MÉDICOS

CAMINHOS REGULAMENTARES PARA A MARCAÇÃO CE – DISPOSITIVOS PARA FINS ESPECÍFICOS



Dispositivos médicos

Dispositivos médicos feitos por medida

Dispositivos “In house”

Os dispositivos só podem ser **colocados no mercado** ou **entrar em serviço** se cumprirem o RDM

Decreto-Lei 29/2024, 04 de abril

Colocação no mercado

Entrar em serviço

DISPOSITIVOS MÉDICOS

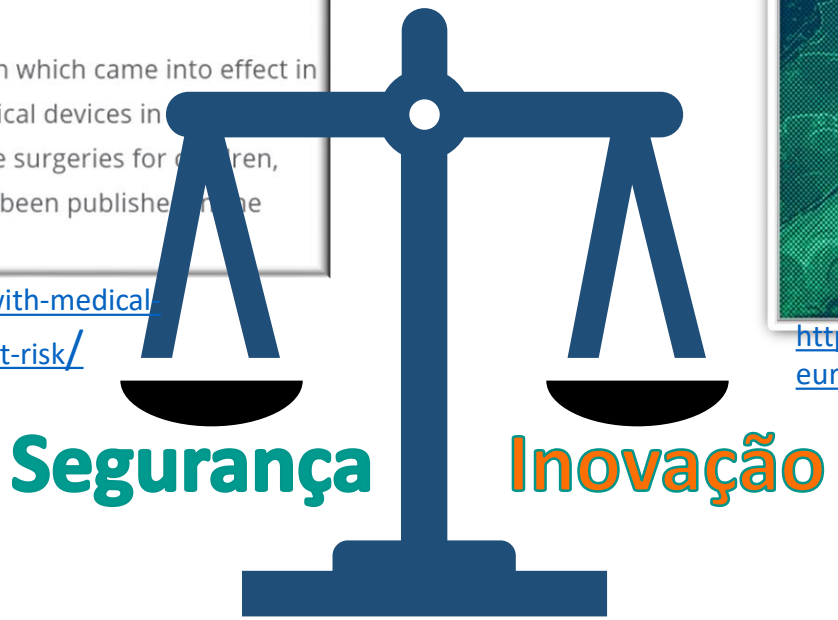
CAMINHOS REGULAMENTARES

Medical device regulation challenges put children's surgeries at risk

Posted on: 20 October 2022

Research led by Trinity College has found that a regulation which came into effect in May 2021 with the aim of improving the oversight of medical devices in leading to unintended consequences which may put some surgeries for children, and the treatment of rare diseases, at risk. The study has been published in the journal Pediatric Cardiology.

https://www.tcd.ie/news_events/articles/challenges-with-medical-device-regulation-put-necessary-paediatric-surgeries-at-risk/



https://www.euronews.com/next/2024/03/13/innovation-in-europe-is-falling-behind-what-can-we-do-to-catch-up

Short-term actions – Legislative

1 Implementing regulation for e-IFUs for medical devices

- Public feedback closed
- Planned adoption date: **Q2 2025**

2 Establishment of an Expert Panel on orphan and paediatric devices

- Planned adoption date: **Q2 2025**

3 Reclassification of well-established technologies (WET)

- Request for evidence: processing input
- Planned adoption date: **Q4 2025**

4 Expansion of the list of well-established technologies (WET)

- Request for evidence closed and analysed
- Consultation with MDCG ongoing
- Planned adoption date: **Q4 2025**

5 Implementing rules regarding requirements to be met by Notified Bodies

Implementing act according to Article 36 (3) MDR / Article 32 (3) IVDR will include;

- Timelines for conformity assessment, including clock-stops;
- Requirements for a reliable quotation;
- Monitoring of timelines and costs (KPI);
- Recertification

- Planned adoption date: **Q4 2025 /Q1 2026**

Medidas com impacto na inovação

Short-term actions – Non-legislative

1. Guidance on **breakthrough technologies (BtX)**
2. Guidance on **orphan IVDs**
3. Guidance on **sampling of technical documentation**
4. Guidance on **certificates under conditions**
5. **IMDRF Guidance of high priority:** Pre-Determined Change Control Plans, Good Machine Learning Practices, Quality Management Systems, IVD Clinical Evidence and the Reliance Playbook
6. **MDSAP mapping activities** (NBCG-Med and MDCG)
7. **Support to other activities :** e.g. Horizon scanning, orphan devices, JAMS 2.0

Summit Presentations - https://nobocap.eu/nobocap-community-summit-2025/

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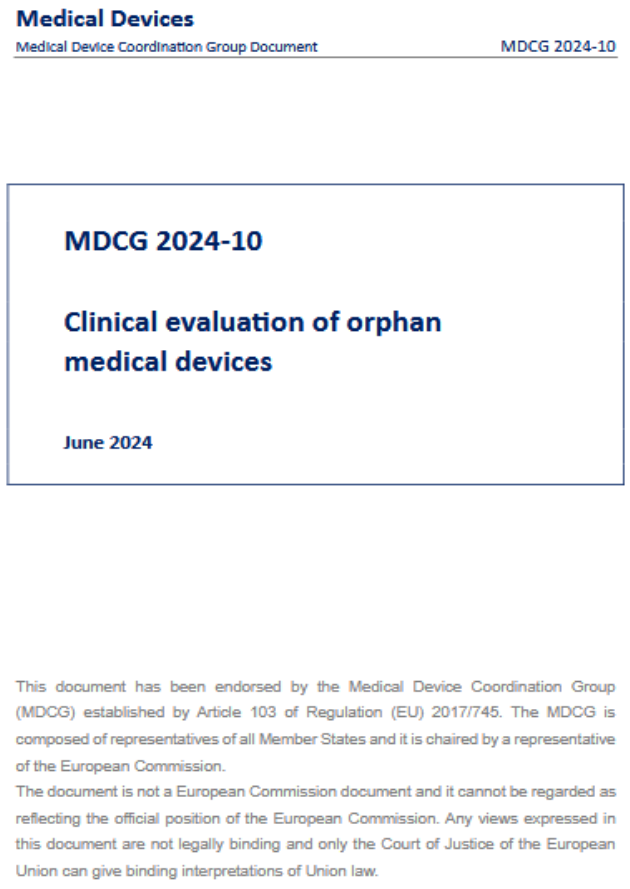
DISPOSITIVOS ÓRFÃOS

DISPOSITIVOS ORFÃOS

MDCG 2024-10



Medical Devices

Medical Device Coordination Group Document MDCG 2024-10

MDCG 2024-10

Clinical evaluation of orphan medical devices

June 2024

This document has been endorsed by the Medical Device Coordination Group (MDCG) established by Article 103 of Regulation (EU) 2017/745. The MDCG is composed of representatives of all Member States and it is chaired by a representative of the European Commission.

The document is not a European Commission document and it cannot be regarded as reflecting the official position of the European Commission. Any views expressed in this document are not legally binding and only the Court of Justice of the European Union can give binding interpretations of Union law.

Dispositivos órfãos são dispositivos destinados ao tratamento de doenças ou condições que **afetam apenas um pequeno número de pessoas a cada ano**. Frequentemente, são utilizados para **tratar doenças ou condições médicas raras** para as quais existem poucas opções de diagnóstico ou tratamento. Dispositivos órfãos podem ser **cruciais para suprir uma necessidade médica** que, de outra forma, não seria atendida.

Em muitos casos, os dispositivos órfãos **destinam-se ao uso exclusivo ou predominante em menores e populações pediátricas**, ... Gerar proactivamente **dados clínicos dentro de um prazo adequado em pequenas populações de doentes** é **particularmente desafiador**, como é o caso de populações vulneráveis, tendo em vista os requisitos éticos e regulamentares para proteger adequadamente essas populações…

https://health.ec.europa.eu/document/download/daa1fc59-9d2c-4e82-878e-d6fdf12ecd1a_en?filename=mdcg_2024-10_en.pdf

https://ec.europa.eu/newsroom/sante/newsletter-archives/54175

DISPOSITIVOS ORFÃOS

MDCG 2024-10

Critérios para determinar quando um DM ou um acessório para um DM deve ser considerado um "**dispositivo órfão (DO)"**

Medical Devices

Medical Device Coordination Group Document MDCG 2024-10

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4. Orphan device status and orphan indication

4.1. Orphan device criteria

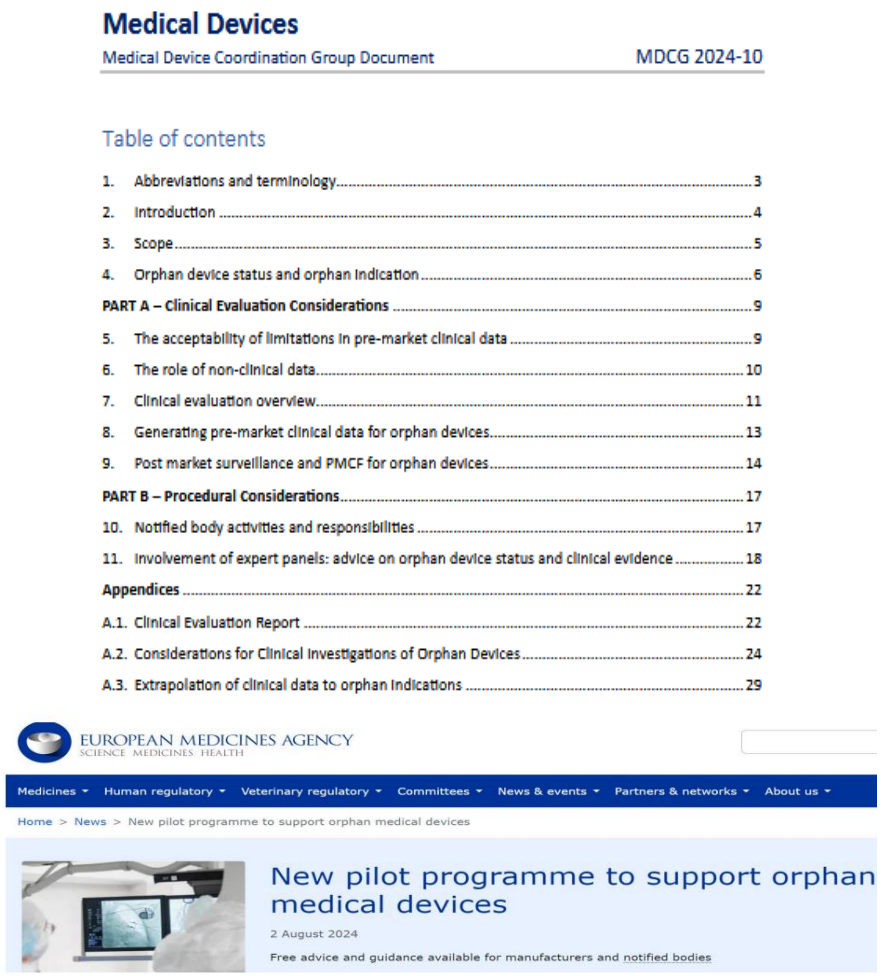
For the purpose of this guidance, a medical device or an accessory for a medical device should be regarded as 'orphan device' (hereafter also referred to as 'OD'), if it meets the following criteria:

- 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 European Union per year[5]; 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 for the treatment, diagnosis, or prevention of this disease/condition, taking into account both device and patient population-specific factors.

[5] Extrapolated from the population estimate criteria for Humanitarian Use Device (HUD) designation established by the U.S. Food and Drug Administration (FDA) and calculated on the basis of an EU population of **447** million, see www.fda.gov/regulatory-information/search-fda-guidance-documents/humanitarian-use-device-hud-designations

DISPOSITIVOS ORFÃOS

MDCG 2024-10



Medical Devices

Medical Device Coordination Group Document MDCG 2024-10

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Medicines · Human regulatory · Veterinary regulatory · Committees · News & events · Partners & networks · About us

Home > News > New pilot programme to support orphan medical devices

New pilot programme to support orphan medical devices

2 August 2024

Free advice and guidance available for manufacturers and notified bodies

Critérios para determinar quando um DM ou um acessório para um DM deve ser considerado um "**dispositivo órfão (DO)"**

PARTE A – Considerações sobre a avaliação clínica

- A **aceitabilidade das limitações nos dados clínicos pré-comercialização** para dispositivos órfãos,
- Principais considerações sobre **a avaliação clínica de dispositivos órfãos novos e *legacy***,
- Geração de **dados clínicos pós-comercialização** para dispositivos órfãos, incluindo PMS e PMCF.

PARTE B – Considerações procedimentais

- Orientações para **organismos notificados (ON)** sobre a avaliação de dispositivos órfãos - Atividades e responsabilidades:
  - ✓ Dialogo estruturado em fase prévia –ex: verificação do estatuto DO o mais cedo possível
  - ✓ Certificados com condições
- O papel dos **painéis de peritos** (EMA):
  - ✓ Designação de “dispositivo órfão”
  - ✓ Aconselhamento cientifico a fabricantes e ONs

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DISPOSITIVOS INOVADORES

DISPOSITIVOS INOVADORES

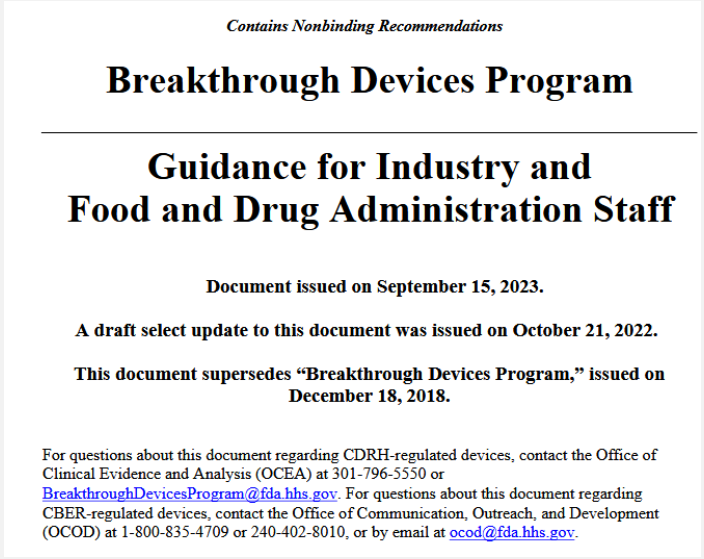
PANORÂMICA REGULAMENTAR (NÃO EU)

- Australia
- Canada
- China

United States

FDA - Breakthrough Devices Program

Este programa tem como foco proporcionar acesso atempado a dispositivos que ofereçam tratamentos ou diagnósticos mais eficazes para **doenças potencialmente fatais** ou **irreversivelmente debilitantes**.



Contains Nonbinding Recommendations

Breakthrough Devices Program

Guidance for Industry and Food and Drug Administration Staff

Document issued on September 15, 2023.

A draft select update to this document was issued on October 21, 2022.

This document supersedes "Breakthrough Devices Program," issued on December 18, 2018.

For questions about this document regarding CDRH-regulated devices, contact the Office of Clinical Evidence and Analysis (OCEA) at 301-796-5550 or BreakthroughDevicesProgram@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010, or by email at ocod@fda.hhs.gov.

Japan

PMDA – Sakigake Designation

A designação *Sakigake* promove dispositivos médicos inovadores por meio de processos de revisão acelerados e consultas prioritárias..

Criteria and Advantage of SAKIGAKE Designation

- **Criteria**
  - innovativeness
  - severity of disease
  - prominent effectiveness or/and safety
  - willingness and framework to first development in Japan
- **Advantage**
  - Prioritized Consultation: waiting time; 2 months →1 month
  - Pre-application Consultation: de facto review before application
  - Prioritized review: targeting total review time; 12 months → 6 months
  - Review Partner: assignment of PMDA manager as concierge

Priority review	Any product categories	Designated as: 1. Orphan Medical Devices 2. Apparent improvement of medical care for severe diseases
SAKIGAKE **(Forerunner designation)**		• Innovative medical products • For serious diseases • Development & NDA in Japan: The NDA submission being the world's first c simultaneous with other countries • Prominent effectiveness expected based on non-clinical and early phase clinical study data

Fonte IMDRF: https://www.imdrf.org/sites/default/files/2023-10/Innovative%20medical%20devices.pdf ; https://www.imdrf.org/sites/default/files/2024-03/Japan.pdf

DISPOSITIVOS INOVADORES
PROGRAMA EUROPEU - MDCG BTX TF

MDCG BTx TF was established Jan 2025

MDCG BTx task force

- Aims
  - Establish criteria for breakthrough devices (MD and IVD)
  - Guidance supporting accelerated conformity assessment
  - Clinical/Performance evaluation guidance
  - Procedural guidance
- Projected guidance publication - Q4 2025

Co-Chairs:

Member state members/respondents to consultations:
AT,ES, DE, DK, FI, FR, NL, PL, PT, SE.
Notified bodies: NBCG-Med / Team NB.
Industry: MedTech Europe, COCIR, MPP, EFPIA.
Clinical: ESC, Biomedical Alliance
Other: ESIP, EU4HS.

Contributions

21 May 2025 Workshop (Brussels)

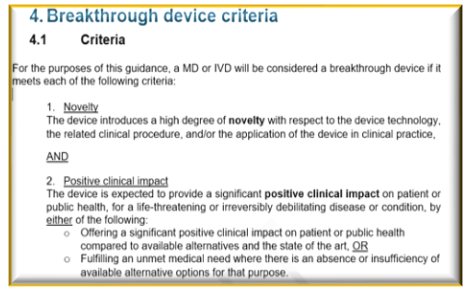
Extended consultation of all stakeholders, CIEPS, IVD, NET and NBO WGs

Respondents: >400 comments received from Member states (10), Notified bodies (NBCG-Med / Team NB), Industry (MedTech Europe, COCIR, MPP, EFPIA), Clinical (ESC, Biomedical Alliance), and ESIP, EU4HS.

Stakeholders workshop III, MDCG, CIE, IVD, NBO

MDCG for endorsement -> 1st December

DISPOSITIVOS INOVADORES
CRITÉRIOS E CONSIDERAÇÕES


4. Breakthrough device criteria

4.1 Criteria

For the purposes of this guidance, a MD or IVD will be considered a breakthrough device if it meets each of the following criteria:

1. Novelty
The device introduces a high degree of **novelty** with respect to the device technology, the related clinical procedure, and/or the application of the device in clinical practice,

AND

2. Positive clinical impact
The device is expected to provide a significant **positive clinical impact** on patient or public health, for a life-threatening or irreversibly debilitating disease or condition, by either of the following:
- Offering a significant positive clinical impact on patient or public health compared to available alternatives and the state of the art; OR
- Fulfilling an unmet medical need where there is an absence or insufficiency of available alternative options for that purpose.

draft

Dispositivo inovador (DM ou DIV) se cumpridos os seguintes critérios:

1. **Inovação** (tecnologia, procedimento clínico relacionado, e/ou aplicação na prática clínica

E

2. **Impacto clínico positivo** (na saúde do doente ou na saúde pública, para uma doença ou condição com risco de vida ou irreversivelmente debilitante, por meio de qualquer um dos seguintes:
   - Comparado com alternativas ou para satisfazer uma necessidade não atendida.

Inovação na **tecnologia** do dispositivo:
- Materiais, incluindo sua composição, …duração do contato dos materiais com tecidos humanos ou fluidos corporais…;
- *Design*, incluindo especificações e propriedades novas ou modificadas;
- Processo de fabrico;
- Mecanismo de ação,…

Inovação no **procedimento clínico** relacionado ou **aplicação** do dispositivo na **prática clínica:**
- finalidade ou indicação pretendida
- ou aplicação de tecnologias existentes num contexto novo…

Ex: Para tratar ou prevenir um efeito colateral grave associado ao tratamento

Ex: para tratar condição que apresenta um risco significativamente menor de efeitos adversos graves

- Natureza da doença ou condiçã
- Alternativas e estado da arte

DISPOSITIVOS INOVADORES
CRITÉRIOS E CONSIDERAÇÕES

draft

Considerations on meeting the criteria – Degree of Novelty

Additional considerations

Where a device's novelty constitutes a e.g. first-in-class device, the first certification of that type of device[4], or is introducing an innovation that is expected to result in a paradigm shift, that device is more likely to represent sufficient novelty with respect to qualifying as breakthrough.

Unless the device offers a substantial or clinically meaningful deviation from the relevant state of the art, it may be challenging for the device to qualify as breakthrough if the novelty is limited to incremental, sustaining improvement or iterative changes.

1115 **Appendix A.1 – Table on BtX determination**

Table 1: Illustration of Breakthrough criteria

Novelty (see 4.2.2) \ Positive Clinical impact (see 4.2.3)	Non-significant positive clinical impact *Does not contribute to clinically meaningful improvements in health outcomes compared with alternatives / SOTA*	Significant positive clinical impact on patient health* *Contributes to clinically meaningful improvements in health outcomes on an individual level*	Significant positive clinical impact on public health* *Contributes to clinically meaningful improvements in health outcomes on a population level*
Incremental / Sustaining Innovation *Low degree of novelty - Minor or iterative changes from alternative(s) / SOTA*			
Disruptive innovation *High degree of novelty - significantly differs from alternatives/ SOTA*		**Potential Breakthrough device****	**Potential Breakthrough device****
Paradigm shift *High degree of novelty - Transformative innovation representing a fundamental change in a health area*		**Potential breakthrough device****	**Potential Breakthrough device****

** For a life-threatening or irreversibly debilitating disease or condition, see. 4.2.4.*
*** Subject to Breakthrough Designation procedure, see Section 11.*

As novelty increases, uncertainty may also increase with respect to the expected safety or performance of the device, as less relevant supporting information from similar devices is available to help inform the risk evaluation for the novel device. Where a high degree of novelty is associated with increased uncertainty, the device may only be considered BtX if there is adequate justification for how the device is expected to provide a significant positive clinical impact, see 4.2.3. Three qualitative levels (low, medium, and high) of uncertainty are described below.

Low level of uncertainty: Expected safety and performance well understood. Unlikely to have unidentified risks

Medium level of uncertainty: Possible that there are existing unidentified new/emerging risks

High level of uncertainty: Likely to have existing unidentified new/emerging risks

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DISPOSITIVOS INOVADORES

AVALIAÇÃO PRÉ-CLINICA, CLÍNICA E DE DESEMPENHO CONSIDERAÇÕES

Considerações gerais:

- *Balance of pre-market and post-market clinical evidence*
- *Acceptability of limitations*
- *IVD and other considerations*

Evidência não-clinica:

- *Challenges due to novelty*
- *Biological safety*
- *In silico*

DISPOSITIVOS INOVADORES
AVALIAÇÃO PRÉ-CLINICA, CLÍNICA E DE DESEMPENHO - CONSIDERAÇÕES

draft

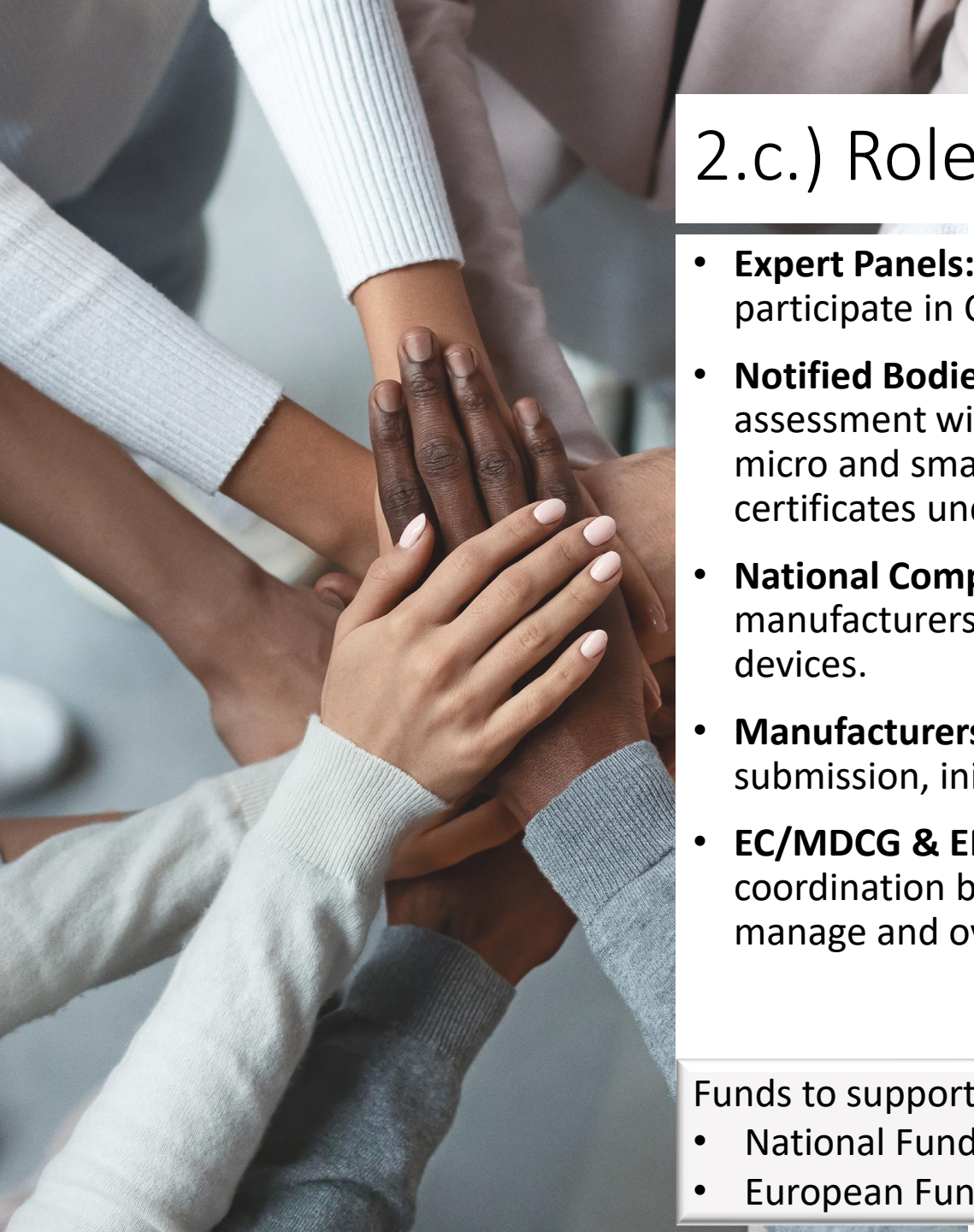
Considerations on pre and post market clinical evidence

- *Clinical Investigations / Performance Studies*
    - *Focus on safety and short/medium term performance*
    - *Appropriate study design and comparator*
- *PMCF/PMPF*
- *Certificates with conditions*

DISPOSITIVOS INOVADORES
OS PAPEIS DOS DIFERENTES ATORES

draft

Pre-clinical testing	Clinical evaluation (including CI/PS)		Regulatory submission & Certification			
Painel de peritos *provide opinion on designation status…*	**ACs** *NCAs provide dedicated support to confirmed BtX designtations*	**Painel de peritos** *Provide advice at earlier stages on clinical strategy and clinical development plan*	**ACs** *NCAs prioritise clinical investigations and clinical performance studies*	**ONs** *NBs provide discussion opportunity at pre-submission/structured dialogue prioritisation of files, cost appropriateness to SMEs.*	**Painel de peritos/ON** *sends CECP if it applies to a specific device according to Article 54(3) of the MDR*	**Painel de peritos/ON** Support to NBs in the clinical assessment of BtMDs



2.c.) Roles of key actors in the BtX pathway

- **Expert Panels:** provide BtX status opinions, provide early scientific advice and participate in CECP for high-risk devices.
- **Notified Bodies (NBs):** Provide structured dialogue, conduct conformity assessment with priority given to BtX files, take into consideration the role of micro and small companies, collaborate with expert panels* and issue certificates under specific conditions (if needed).
- **National Competent Authorities (NCAs):** Oversee notified bodies, support manufacturers (esp. SMEs), enable priority assessments of CI/CPS for BtX devices.
- **Manufacturers:** Innovate! & Ensure proper self-assessment prior to submission, initiate BtX designation.
- **EC/MDCG & EMA:** oversee and manage the expert panels, contribute to coordination between different actors, build knowledge in the expert panels, manage and oversee the transparency dashboard.

Pilot on scientific advice BTx

Funds to support innovation
- National Funding Mechanisms
- European Funding mechanisms

5

INOVAÇÃO
OUTRAS INICIATIVAS

NATIONAL COMPETENT AUTHORITY
IMPROVING COMPETENCIES/CAPACITIES

Ongoing projects

European and international interactions – participation in MDCG working groups, and IMDRF working groups

To start

HS-g-25-25 Direct grant to Member States to provide regulatory or scientific advice to small and micro-enterprises to support the development and carrying out of the conformity assessment of devices, particularly innovative devices, and to facilitate the Union level coordination on medical device safety issues

- Budget: EUR 4 000 000
- Expected project duration: 36 months
- Targeted applicants: EU Member States' authorities (+ NO + IS)

INICIATIVAS DE HORIZON SCANNING

Horizon Scanning System - MDs & IVDs

📑 **HADEA/2024/OP/0024**

🎯 **Objectives**

Put in place a **horizon scanning system** 🔭 in the area of **MDs and IVDs** 🩺 🧪 in order to maintain an up-to-date overview of **new & emerging technologies** 🚀.

Aim:

🔎 **Screen available sources** for novel & emerging technologies
⚙️ **Assess features** that may impact medical devices & IVDs
📈 **Identify opportunities, risks & trends** related to these technologies

This horizon scanning was identified as a need in the context of implementing **MDR/IVDR legislation** to support **competitiveness & innovation** in the EU market, while ensuring a **high level of protection of health** for patients and users. 💗

💶 **Contract value:** €896 400
🏢 **Contractor:** TECHNOPOLIS FRANCE
📅 **Period:** 30 months (contract signed on 06/06/2025)
⚡ **Status:** Ongoing

📎 👉 Read the contract award notice for more details.

NET WG Horizon scanning System

OBRIGADA
THANK YOU

Infarmed
Autoridade Nacional do Medicamento
e Produtos de Saúde, I.P.

REPÚBLICA PORTUGUESA
SAÚDE

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Autoridade Nacional do Medicamento
e Produtos de Saúde, I.P.