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by

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Specialized Regulatory Pathways
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White Paper

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SPECIALIZED REGULATORY PATHWAYS

This White Paper summarises discussions and observations arising from the International Medical Device Regulators Forum (IMDRF) 24th Session Joint IMDRF – DITTA and GMTA Workshop on 'Specialized Regulatory Pathways'. It was chaired by the European Commission, on behalf of the European Union under its role as IMDRF Chair.

The workshop kicked off with a reminder of how **IMDRF core documents** covering the medical device (MD) life cycle have evolved over time and have provided a valuable framework for the development and convergence of regulatory systems globally. IMDRF documents provide guidance on pre- and post-market topics that are suited to cover the wide array of products that fall under the definition of a MD. Technology-specific guidance has also been developed by the IMDRF for certain types of devices (e.g., personalised MDs), demonstrating the benefit of providing specialized pathways for certain device types.

Considerations for specific patient populations and devices with a potential to innovate how healthcare is delivered to patients, is a challenge faced in the rapidly evolving medical technology sector. Over the past decade, **specialized pathways** and other **innovative regulatory tools** emerging in different jurisdictions to facilitate and accelerate the access to market of MDs in these areas. Exchange of knowledge and experiences on a global level can help promote regulatory systems that are both robust and reliable, but also flexible enough to allow timely patient access to the best safe and effective technologies available for diagnosis and treatment.

Session 1 – Devices intended for specific patient populations

The session focused on approaches for devices intended for specific patient populations, with discussions on **orphan MDs and humanitarian-use MDs, paediatric MDs, and personalised and custom MDs**. In the case of orphan, humanitarian-use and paediatric MDs, an emphasis was put on the importance of establishing **clear criteria for the designation** of these devices. In addition, balancing careful assessment of the benefit-risk ratio in light of the available clinical evidence against the urgency to treat unmet needs for rare disease or conditions, was highlighted as key.

Various regulators and stakeholders shared perspectives on their desire to **incentivise activity** in these areas to ensure timely access for patients. A general support for the appropriate tailoring of clinical evidence requirements, whilst still ensuring patient safety, was observed. Accepting a wider **variety of evidence sources** including, for example, real world evidence (RWE) and information from patient registries was also discussed. Experts agreed that enhancing global perspectives and access can only be achieved through close cooperation among regulators, industry stakeholders and clinicians to overcome challenges around the generation of clinical evidence.

Important **trends on capabilities to tailor and personalise MDs** for individual patients were also presented. Whilst a welcome development to allow more effective patient care, challenges posed to standard regulatory frameworks were recognised. Regulators and stakeholders provided insights on questions pertaining to an increase in **Point of Care (PoC) manufacturing**. Innovative concepts such as **Medical Device Production System (MDPS)**, including the equipment and software used to produce patient-matched/personalised MDs are promising, but still in early stages. Participants noted the important contribution in this area of the IMDRF's recently published documents on Personalized Medical Devices.

Observations:

- **Regularly share international practice to stimulate the global ecosystem and promote agility in existing or emerging specialized pathways.**
- **For orphan, humanitarian use and paediatric devices, exchange experience regarding designation criteria and clinical evidence to enhance clarity and predictability.**
- **Consider reliance mechanisms between regulators with existing or emerging pathways.**
- **Encourage communication between industry and regulators to monitor the evolution of personalised MD, such as MDPS.**
- **Early communication is key in the regulatory approval process to ensure devices are developed in line with regulatory expectations.**

Session 2 – Innovative medical devices

This session discussed **specific pathways for innovative MDs**. Regulators outlined existing frameworks which facilitate access to innovative MDs for the diagnosis or treatment of life-threatening or irreversibly debilitating diseases and conditions and/or fulfil unmet clinical needs, where no appropriate alternatives exist. It was clarified that innovative MDs may consist **not only** of new **disruptive technologies**, but of significantly **improved existing technologies**, or where a **new intended purpose** is indicated.

Mechanisms such as interactions at early stages in the development of clinical strategies or pre-submission interactions were promoted. Furthermore, stakeholders called for interactive scientific discussion and timely communications throughout the regulatory process and increased incentives (e.g., grants for early clinical development). Participants also considered **opportunities for convergence and reliance** and their interrelation. Given the complexity of the sector, leveraging regulatory assessment knowledge from different jurisdictions can be a resourceful tool. The reinforcement of existing reliance models and/or expansion to additional lifecycle stages was also reflected upon.

Observations:

- **Exchange experience on the definition and assessments of innovative devices**
- **Continue to explore the value of pre-market interaction between regulators and stakeholders to support innovation.**
- **Promote continual data collection, including via post market clinical studies to monitor patient safety and device improvements.**
- **Consider global dialogue on data-driven development, clinical validation and the generation and use of real-world evidence.**

Session 3 – Regulatory toolboxes to foster innovation

The third session focussed on **regulatory tools** that can be used to foster innovation and facilitate access to emerging technologies. **Regulatory sandboxes** were explored as an interesting tool for the testing of design concepts and MDs in a controlled and collaborative environment, under a regulator's supervision. The importance of multi-stakeholder involvement (e.g., regulators, manufacturers, academics, healthcare professionals) in informing future policy was stressed.

It was discussed that the growing use of **digital medical devices**, including connected and AI-enabled MDs, can hold the potential to address some of the most critical challenges in healthcare, but may test existing regulatory landscapes. The use of **Predetermined Change Control Plans (PCCPs)** to help address challenges in the management of software as a MD (SaMD) was highlighted. The presentations on PCCPs explained that these plans would consist of pre-approved roadmaps for modifying MD software after it has undergone assessment by the relevant bodies. The potential benefits include further optimisation of technology without the need to conduct re-assessment while still ensuring safety and performance. It was recognised that, if used appropriately, the application of PCCPs could speed up access to new iterations of devices that are intended, by their design, to be modified and updated regularly.

Observations:

- **Consider the potential benefits of developing regulatory sandboxes for the testing of certain new technologies within a supported environment with regulatory oversight.**
- **Assess the implications and potential value of implementing PCCPs in SaMD and other technology areas.**
- **Seek greater convergence on the concepts of PCCPs including frameworks and terminology.**