Medical Technologies in rare diseases and specialised domain – the status quo

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MedTech Europe

from diagnosis to cure

Medical Technologies in rare diseases

- Medical Technologies are of crucial and often vital importance for patient living with rare diseases.
  - **In Vitro Diagnostics** like next-generation sequencing, whole-exome analyses, are helping clinicians diagnose and treat patients who previously had no therapeutic options.
  - **Medical Devices** provide major contribution to life expectancy and quality of life both for children and patients with rare diseases

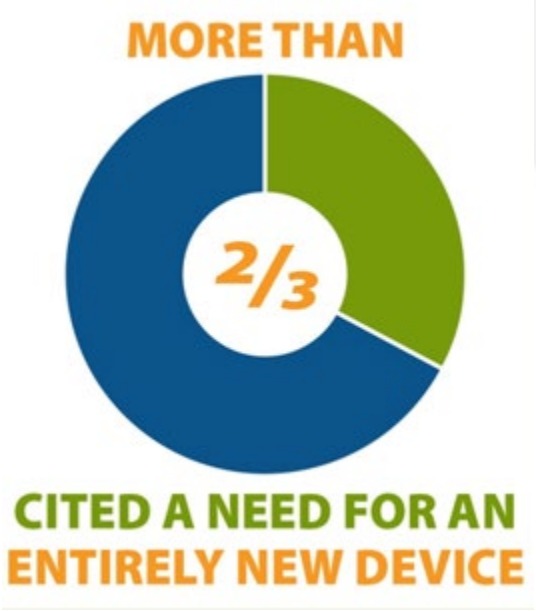
The challenge of device development for rare diseases

- Pharma Industry and MedTech Industry face the same challenges:
  - Small number of population affected
  - Lack of knowledge about the disease natural history
  - Lack of well-defined study endpoints

- In rare diseases efforts to accelerate research and product development clearly mainly focus on drugs and biological products through regulatory and economic frameworks.

R&D of Medical Devices

- Assessment of the needs and unmet needs for MD in rare diseases



- Innovation and development based on engineering process.

- How to Incentive R&D for Medical Devices?

Regulatory frameworks: from Directive to Regulation

- Until 2020 (IVD) and 2022 (MD) are regulated through 3 Directives
  - IVD Directive
  - MD Directive
  - Implantable MD Directive
- From 2020 (IVD) and 2022 (MD) will be regulated through 2 Regulations
  - IVD Regulation
  - MD Regulation

- Home-brew tests
- Custom made devices
- Off label devices
- EUDAMED
- Post marketing follow-up
- Cooperation between Member States

Clinical performance

Clinical evaluation

Classification

Companion Diagnostics

HTA, Procurement and reimbursement

- HTA Regulation proposal
  - MD value assessment and HTA are different for pharmaceuticals and in particular for rare disease.
- Procurement
  - 70% of MD purchasing are made through public procurement
- Reimbursement
  - More than 30 different healthcare systems in Europe, with there own reimbursement processes.

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