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Why EU Patients Need Regulation That Aids Access To Orphan Devices

by Eliza Slawther

Children and patients with rare diseases across the world need better access to medical devices, but new EU regulation fails to support manufacturers of such products. A recent journal article highlights why orphan devices are vital and the need for improved regulation.

The *Working Group on MedTech For Rare Diseases* was established last year by international experts to research and improve the regulatory landscape for medical devices and technologies used to diagnose, treat or support individuals with rare conditions. The group has now published its first article, entitled "*Orphan medical devices have come a long way*" which argues that global regulators, including in Europe, should do more to support the development of and access to these products. It provides a succinct but impactful overview of the history of orphan devices.

The piece forms part of a thematic collection focused on tackling specific challenges faced by manufacturers of orphan devices in the Orphanet Journal of Rare Diseases.

While 92 countries in the world have introduced regulatory provisions for orphan drugs, just two – the US and Japan – have orphan device regulation in place, author Marc Dooms notes in the piece.

In the US, a program known as the Humanitarian Use Device designation offers an alternative regulatory pathway for the compassionate use of orphan devices. Meanwhile, Japan's Pharmaceutical and Medical Device Act

The Working Group on Medtech For Rare Diseases was established by the International Rare Diseases Research Consortium (IRDiRC) and the Netherlands' University of Twente in early 2022. Its members represent countries from across the globe, with more than half of these being European countries.

contains provisions for drugs and medical devices to be designated as orphan drugs or medical devices.

"The recent Medical Device Regulation (MDR) in Europe does not even mention this unmet need," says Dooms, adding that the MDR has made it "even more difficult to place custom made devices on the market, including 3-D printed devices" (more below).

In a previous interview with the organization, *Medtech Insight* detailed the group's call for experts to author articles as part of its thematic journal collection. (Also see "*International Initiative Tackles Paucity Of Rare Disease Devices*" - Medtech Insight, 17 Jan, 2023.).

Dooms, who is a senior orphan drug pharmacist at the University Hospitals Leuven, Belgium and expert in the rare disease field, claims that both medical specialists and patients have strongly indicated the need for more orphan medical devices.

Improvements Needed

Most orphan devices, Dooms explains, are initially developed in either academic, collaborative settings or from existing devices for other indications when used off-label. Surgeons, in particular, have used off-label or self-assembled medical devices for rare disorders "for many years," he says, yet more support is needed to improve the safety of and access to orphan devices.

Children tend to be impacted even more than adults, given that their body parts are smaller and some diseases even more rare. Additionally, children may need devices that grow with them or that can be replaced as they develop.

Examples of such orphan devices are:

- Prosthetics used after rare cancer surgery in children that grow with the child;
- Exoskeletons used for patients with rare diseases causing muscle weakness;
- Implantable pacemakers for children

Defining Rare Diseases

The European Medicines Agency (EMA) defines orphan as conditions that affect no more than five in every 10,000 people, while the US Food and Drug Administration (FDA) considers an orphan device to be one that is designed to treat or diagnose a disease or condition that affects or is manifested in not more than 8,000 individuals in the US per year.

As these products are used in small numbers of patients, they offer little profit for

with rare heart conditions;

- Smartphone apps that help support parents with their child's nutrition and drug regimen.

companies. This means that too few new orphan devices are developed, while existing products are the most vulnerable to market withdrawals due to cost pressures.

Experts have called for regulatory provisions to be made, particularly in the EU, to prevent existing orphan devices disappearing from the market. (Also see "*Pediatric Cardiologists Told To Prepare For Critical Devices Disappearing Due To MDR*" - Medtech Insight, 25 Oct, 2022.).

"Similar to orphan drugs, there is much research and development needed in this area, [as well as] incentives to stimulate authorization and reimbursement, tools for patient involvement and materiovigilance," Dooms says.

Incentives for companies such as those already used in the medicinal sector could also be introduced, he notes, while supportive frameworks should be put in place to avoid withdrawal for financial reasons, to regulate off-label use of products and manage medical device vigilance.

Such measures are particularly important given that many orphan device manufacturers are small and medium sized enterprises, focused on single products.

Off-Label Use

In the article, Dooms points out that the off-label use of drugs for rare disorders has, in several instances, led to marketing authorizations being granted for these conditions.

He questions whether medical devices used off-label could be granted indication extensions in the same way, noting that there are caveats such as the need for medical device hardware and software to be refined and developed continuously.

The off-label use of medical devices is widely accepted in instances where medical professionals have no other viable treatment options and are acting in the best interest of their patient. However, under the MDR, manufacturers are required to identify whether systemic off-label use of a product is occurring in their post-market phase and take steps to reduce this or where appropriate seek regulatory approval for such indications (Also see "*TEAM-NB Position Paper Helps Manufacturers Manage And Benefit From Off-Label Use*" - Medtech Insight, 26 Oct, 2022.).

Custom-Made Devices

Dooms notes in his article that the EU MDR has made it more difficult for manufacturers of custom-made devices to place their product on the EU market. Custom-made devices are products made for specific patients at the request of an authorized person under a prescription

(e.g. a specialist doctor.)

Manufacturers of these products must meet nearly all requirements set out in the MDR, although they are exempt from some obligations, as detailed in a previous *Medtech Insight* article. (Also see "*Regulating Custom-Made Devices Under EU MDR – Clear Distinctions To Replace Overlap Confusion*" - Medtech Insight, 16 Mar, 2021.).

Editor's note: This article has been amended to correct details about the US HUD program.