ARM's Statement on the EU's Health Technology Assessment Guideline

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Today's publication of the EU Health Technology Assessment (HTA) Coordination Group's 'Methodological Guideline for Quantitative Evidence Synthesis: Direct and Indirect Comparisons' is discouraging news for rare disease patients and the Advanced Therapy Medicinal Products (ATMP) sector.

The Joint Clinical Assessment (JCA) had the potential to accelerate patient access to ATMPs by centralizing the clinical assessment step in HTA and reducing duplication across EU Member States. However, with this guideline, the Coordination Group has ignored the HTA Regulation's call to adopt fit-for-purpose methodologies for ATMPs by insisting on randomised controlled trials (RCTs) even in cases where RCTs are not feasible.

The result is a final guideline that is very similar to the initial draft shared by EUnetHTA21 in May 2022. Indeed, according to the guideline, single-arm or non-randomised evidence 'may well be … insufficient for estimation of the relative treatment effectiveness in the context of JCA.' This appears to go against the specific mandate of the HTA Regulation, which leaves such a judgment to Member States, by limiting the JCA to a "description [not an evaluation]…of the degree of certainty of the relative effects."

The guideline ignores the compelling evidence presented by ARM, patient groups, and academic centres that single-arm trials to evaluate rare disease treatments are sometimes necessary for ethical, scientific, and practical reasons. Moreover, single-arm trials can appropriately demonstrate clinical benefit when informed by real-world data, such as from disease registries. The Coordination Group's approach will likely result in inconclusive JCA reports for many ATMPs, significantly delaying patient access and jeopardising the implementation of the EU HTA Regulation at the EU and national levels.

The guideline — together with the draft Implementing Act, which restricts developers' involvement and contains unrealistic data requirements and unworkable timelines — indicates that the JCA project is at real risk of becoming a failure. In turn, the EU's competitiveness as a region for ATMPs is in danger, especially for the smaller biotechnology companies that play a fundamental role in the sector's innovation globally. Ultimately, rare disease patients could see fewer transformative treatment options available in the EU.

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