Abstract FIGON DMD Parallelsession Compassionate Use 09 October 2025

Background:

Access to medicines remains a critical issue in translational healthcare policy. This RSNN session, chaired by Marjon Pasmooij (head of the scientific department MEB, co-chair RSNN), focused on regulatory and practical aspects of early access to unauthorized medicines in the Netherlands and across Europe, with specific attention to **Compassionate Use Programs (CUPs)**

Regulatory framework (Presented by Cees Tuinenburg, MEB)

Cees Tuinenburg, Regulatory Project Leader, introduced the concept of CUPs and highlighted the differences to Named Patient Use (NPU). In the Netherlands, the Medicines Evaluation Board (MEB) is the competent authority for CUPs, while the Health and Youth Care Inspectorate (IGJ) oversees NPUs. CUPs are initiated exclusively by pharmaceutical companies for patient cohorts, whereas NPUs can be requested by physicians, pharmacists, wholesalers, or companies for individual patients. The number of requested CUPs has declined over the past decade, with approximately half of the applications approved. Regulatory uncertainty and operational complexity were identified as major barriers for pharmaceutical companies, prompting the MEB to publish new policy guidance to improve clarity. The key approval criterium remains the absence of satisfactory authorized treatment alternatives.

Research perspective (Presented by Colinda Post, PhD Student and medical oncologist at Amsterdam UMC)

The comparative analysis of Rosenberg, Post and colleagues revealed substantial variation among European countries in CUP and NPU policies especially regarding eligible applicants, reimbursement mechanisms, and duration.

These inequities could lead to patient self-funding and/or cross-border treatment, while lack of transparency may contribute to physician hesitation and compromised treatment options for patients with chronically debilitating and/or life-threatening diseases. Harmonization and regulatory clarity across Europe were recommended, and CUP routes were encouraged rather than NPU routes.

Industry perspective (Presented by Eugenie van Lieshout, Country Regulatory Affairs Lead Netherlands, Johnson & Johnson)

From the pharmaceutical industry perspective, CUPs are essential to ensure patient access during the period between EMA approval and national reimbursement—a gap often lasting two to three years in the Netherlands. CUPs also enable limited real-world data (RWD) collection, unlike NPU. However, differing national legislations and the absence of a legal framework post-EMA-approval complicate implementation. Industry advocates for extending CUPs until national reimbursement to maintain continuity of care and more clarity surrounding the current regulatory guidelines for CUPs.

Patient perspective (Presented by Cristina Guerrero Paez, Borstkankervereniging Nederland)

The patient perspective highlights that, after EMA approval, patients must have timely access to new treatments. Such access is essential to enable personalised treatment, where therapies are tailored to individual needs. It also requires creating the right conditions for shared decision-making, giving patients the opportunity to make informed choices together with their doctors. Timely access is furthermore crucial for generating real-world evidence—on effectiveness, side effects, and patient-reported outcomes. These insights are indispensable for understanding the true impact of new treatments and for improving care. Finally, access plays a key role in shaping the treatment landscape for those most in need, including patients with rare breast cancers and other subgroups who urgently require new options.

Patient organizations emphasize transparency, continuity, and involvement in early access decisions. Most patients are unaware of CUPs unless informed by clinicians. Access should be guided by value—balancing clinical benefits, side-effect burden, and affordability. CUPs and NPUs are viewed as important tools to bridge the gap between regulatory approval and clinical availability, while also generating RWD.

Discussion and outlook:

The panel highlighted unresolved legal ambiguities regarding the continuation of CUPs after marketing authorization, variations in European approval criteria, and the need for shared data collection frameworks as reflected in the 2023 EU Pharmaceutical Legislation revision. Stakeholders agreed on the importance of harmonization, transparency, and maintaining patient access during reimbursement negotiations.

Conclusion:

CUPs remain a vital mechanism for early patient access to innovative medicines but face declining use, regulatory inconsistency, and unequal access across Europe. Future efforts should prioritize regulatory harmonization, transparency, and continuity of treatment to ensure equitable and safe early access to therapies for patients with unmet medical needs.