

## Compounded medication for patients with rare diseases

Pharmaceutical compounding is the preparation of unlicensed medicines in order to meet specific patient needs that do not have a licensed (commercial) medicine available on the market. Especially for patients with rare diseases it is complementary when there is no interest from a sponsor in the marketing of an orphan drug. Today there are only 171 orphan drugs with a marketing authorization in the European Union for the prevention, diagnosis and treatment of several thousands of rare diseases. Furthermore, “one-size” does not fit all patients and the existing drugs may not always be adequate regarding the pharmaceutical dosage form and dosage strength, raw materials (hypersensitivity reactions and drug shortages) or the organoleptic characteristics.

The EU Pharmaceutical Legislation, Directive 2004/27/EC allows Member States to supply compounded medication in accordance with the legislation in force and to fulfil special needs.

Pharmacists already compounded medicinal products for their patients with rare diseases long before they became authorized orphan drugs in the EU (Cystadane, Cystadrops, Cystagon, Firdapse, Gliolan, Granupas, Kolbam, Orphacol, Pedeia, Peyona, Procysbi, Wilzin). Sterile and non-sterile compounding for these patients is routine pharmaceutical practice today such as infusion bags for total parenteral nutrition (neonates with inborn errors of metabolism) and (repurposed) medicaments awaiting orphan drug authorization or during drug shortages. The article lists many validated examples of standardized formulations (Standard Operating Procedures) to be used in the compounding for this group of patients. These procedures need to be followed to compound a medicinal product conform to all the quality standards of the European Pharmacopoeia. All ingredients used in the compounding must be accompanied by a certificate of analysis and full records of the pharmaceutical production need to be kept for full traceability and accountability.

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### Publication

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