

# Market access environment & policy engagement

## Context

- Many countries are looking to reform their pricing and reimbursement (P&R) systems for rare diseases and are soliciting input from manufacturers as part of this process

## The Challenge

- Lack of consistency in payer assessment systems for rare diseases across countries globally
- Client needed a coherent policy position on the most appropriate mechanisms and processes for value assessment and P&R decisions for orphan medicines

## Our Solution

- Reviewed existing value assessment and P&R systems for rare diseases across key markets globally
- Deconstructed different components of systems and assessed patient access
- Identified components of P&R processes that help facilitate patient access and those that act as barriers to successful outcomes
- Reviewed rare disease policy literature for recommendations on best practice for orphan drug value assessment
- Worked in collaboration with key client stakeholders to define a framework of key principles for value assessment and funding for orphan drugs
- Work was summarised and published in a peer-reviewed journal



 Gutierrez et al. Principles for consistent value assessment and sustainable funding of orphan drugs in Europe. Orphanet J Rare Dis. 2015;10:53

## Outcome

- Based on this work, our client engaged with a European-wide policy initiative to establish a common set of principles for assessment of rare disease treatments
- At a cross-industry meeting in 2016, Dolon assisted in facilitating workshops on the topic and assisted in driving European-wide consensus