

Access to medicines for all patients with rare diseases

ECL & EFPN recommendations for revision of the EU legislation on medicines for rare diseases

1 Accelerate research & development of medicines in the areas of highest unmet medical needs

2 Improve availability and timely access to affordable medicines for patients with rare diseases



Foster academic pathway for medicines development



Modify designation criteria for orphan medicinal products



Significant benefit should reflect quality of life and survival



Identify criteria to define unmet medical needs



Base incentives on R&D cost transparency and ROI



Foster competition and guarantee market continuity