



**HAL**  
open science

## Improving access to innovative medicines: proposals from national authorities to revise the European legislation

Aurélie Mahalatchimy, Florence Lanson, Blanka Bartos, Florence Taboulet

### ► To cite this version:

Aurélie Mahalatchimy, Florence Lanson, Blanka Bartos, Florence Taboulet. Improving access to innovative medicines: proposals from national authorities to revise the European legislation. European Society for Gene & Cell Therapy Congress 2022, Oct 2022, Edinburgh, United Kingdom. halshs-03901248

**HAL Id: halshs-03901248**

**<https://shs.hal.science/halshs-03901248>**

Submitted on 15 Dec 2022

**HAL** is a multi-disciplinary open access archive for the deposit and dissemination of scientific research documents, whether they are published or not. The documents may come from teaching and research institutions in France or abroad, or from public or private research centers.

L'archive ouverte pluridisciplinaire **HAL**, est destinée au dépôt et à la diffusion de documents scientifiques de niveau recherche, publiés ou non, émanant des établissements d'enseignement et de recherche français ou étrangers, des laboratoires publics ou privés.

# IMPROVING ACCESS TO INNOVATIVE MEDICINES: PROPOSALS FROM NATIONAL AUTHORITIES TO REVISE THE EUROPEAN LEGISLATION

Aurélie Mahalatchimy[1], Florence Lanson[2], Blanka Bartos[3], Florence Taboulet[4]

[1] Permanent Researcher in law at the French National Centre for Scientific Research (CNRS, research fellow), Law Faculty, UMR 7318 International, Comparative and European law (DICE) CERIC, CNRS, Aix Marseille University, Toulon University, Pau & Pays de l'Adour University, Aix-en-Provence, 13628, France. ; [2] CERPOP (Center for Epidemiology and Research in POPulation health), UMR 1295 Inserm, University of Paul Sabatier- Toulouse 3, Toulouse, 31062, France. ; [3] PhD Student in law, CREDIMI, University of Burgundy, Dijon, 27877, France. ; [4] Professor in Pharmaceutical Law and Health Economics, CERPOP, UMR 1295 Inserm, University of Paul Sabatier- Toulouse 3, Toulouse, 31062, France. **Acknowledgement: This work has been supported by ANR-funded I-BioLex project (ANR-20-CE26-0007-01, coord. A. Mahalatchimy).**

80% of rare diseases are of genetic origin in the EU  
= Many advanced therapies have obtained the orphan medicine status under  
**European regulation (EC) n° 141/2000 of 16 December 1999 on orphan medicinal products**

Provides orphan designation and incentives (market exclusivity for 10 years)

Needs to be revised. Why?

- Ensure the relevance of incentives granted to industry (to avoid abuses)
- Reward risk-taking and innovation more
- Improve equitable patient access in the EU

## Public consultations on revision of European legislation

Orphan medicines legislation	General pharmaceutical legislation
7 May - 30 July 2021	28 Sept. - 21 Dec. 2021
303 answers including 12 public authorities from 9 Member States*	478 answers including 31 public authorities from 15 Member States**

\*AT, DE, DK, ES, FI, FR, HU, NL, SE ; \*\*AT, BE, CZ, DE, DK, EE, ES, FI, FR, HU, IS, MT, NL, PT, SE

## MAIN PROPOSALS FROM NATIONAL AUTHORITIES

### On the criteria for orphan designation

**Prevalence** : < 5 in 10,000 persons in the EU

- To be adjusted to avoid "salami slicing" (segmenting therapeutic indications)
- To be taken into account with the severity of the disease (**level of unmet medical need - UMN**)

**Significant benefit** : no EU satisfactory method of diagnosis, prevention or treatment, or drug significant benefit

- To be clarified and **graduated**

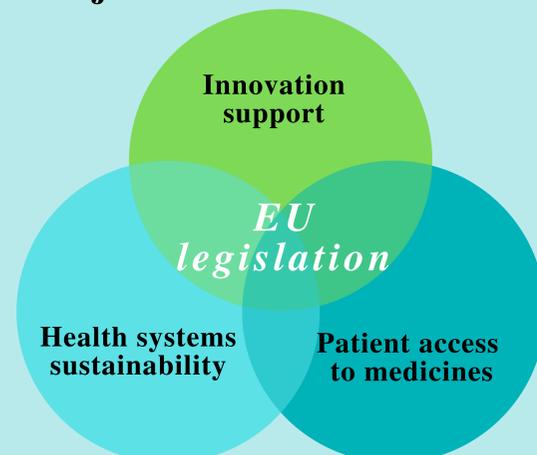
### On the incentives granted to sponsors

- **Proportionate to the level of UMN** (e.g. variable duration of market exclusivity)
- Focused on neglected therapeutic areas
- Conditional to certain obligations
  - Be transparent on R&D costs and actual prices
  - Launch in all or most Member States
  - UMN re-evaluated after commercialization
- Use experiences developed from covid-19 crisis
  - Joint procurement
  - Flexibility in clinical trials conduct

## DISCUSSION AND CONCLUSION

- Many isolated proposals : difficult to identify a consensus among the authorities
- Request of better cooperation between all agencies and more EMA support
- BUT emphasis on respecting the EU and Member States competences sharing

### Objectives to be balanced



> Orphan single status transformed into a mosaic of statuses  
↳ Complexities : how to quantify ? which indicators ?

> Future EU considerations of national authorities proposals and answers from other respondents ?