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

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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**

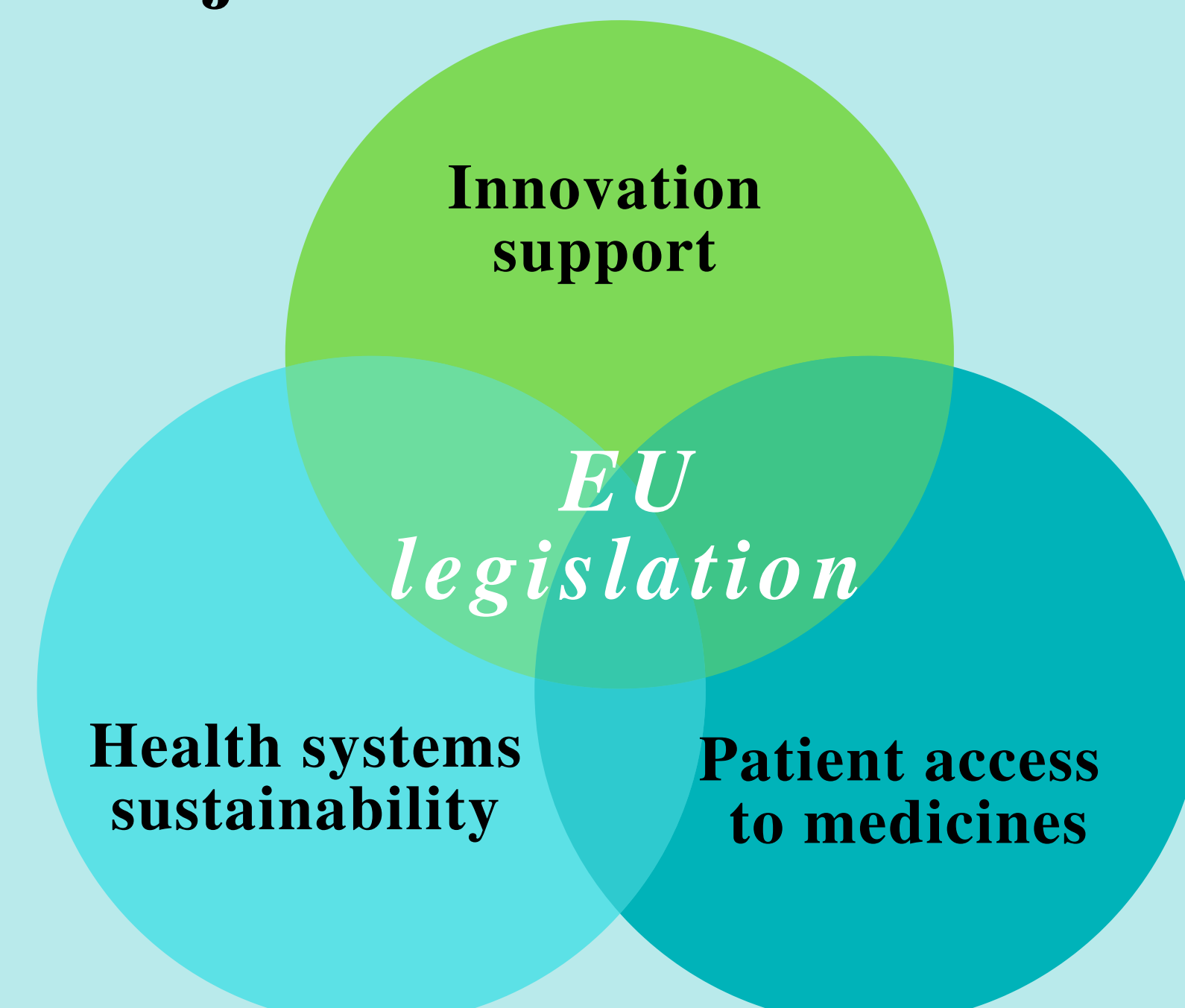
On the incentives granted to sponsors

- **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 ?