

## EuropaBio key messages to 94th Pharmaceutical Committee - 28 May 2021

### Future Proofing

- 1) Introduce an exemption scheme by law for clinical trials with investigational ATMPs containing or consisting of GMOs from the currently applicable EU GMO legal framework to streamline and accelerate the assessment process of ATMPs, increase Europe's attractiveness for the conduct of clinical trials with novel biotechnology-derived cell and gene therapies, increase the number of innovative personalised orphan medicines, and foster patient access to transformative treatments for complex genetic and rare diseases.
- 2) Build on the COVID-19 regulatory experience to adopting innovative regulatory approaches, such as accelerated regulatory assessment based on the acceptance of staged submission of scientific evidence for new medicines and extension of indications, capacity to provide rapid scientific advice, including through a flexible PRIME scheme with earlier access and including new extension of indications, remote inspection alternatives, acceptance of decentralised and single-arm clinical trials offering a patient-centric approach and speedier access to novel technologies. Possibility to have applications for centralised marketing authorisations for new extension of indications in the context of full approval medicines. Flexibility in mixing orphan and non-orphan indications under the same marketing authorisation. CHMP/EMA commitment to maintain accelerated assessment during the evaluation of a medicinal product aligned with other regions. The proposal for an Emergency Use Authorisation, which is currently under discussion, should enable medicine developers, and not only the EMA and European Commission, to initiate an EUA pathway.
- 3) Accept, spell out the potential applications in guidance and encourage the use of fit-for-purpose RWE for regulatory benefit-risk assessment to drive clinical development and provide pivotal evidence of treatment benefits of new therapies, to complement possible evidence gaps. Tap into the potential of digital health tools and technologies to drive a more personalized medicine approach for the future.

### Incentives for innovation that reaches patients

- 1) The concept of "unmet medical need" already exists in the current legal EU framework in the context of the Conditional Marketing Authorisation, as defined by Article 4 (2) of EC Regulation 507/2006.  
The current EU legislative framework also includes the concept of "major therapeutic advantage" in a number of contexts:
  - o **Significant clinical benefit** – Art. 14(11)Reg 726/2004 and Art 10(1) Dir 2001/83/EC
  - o **Significant benefit** for orphan designation – Art 3 (1)(b)Reg 141/2000
  - o **Clinical superiority** for derogation from market exclusivity – Art 8(3)Reg 141/2000
  - o **(Lack of) significant therapeutic benefit** as grounds for a PIP waiver – Art 6(2) and 11.1 (c) Reg 1901/2006
  - o **Significant differences** in efficacy or safety for New Active Substances – Art 10 (2) Dir 2001/83/EC
  - o **Major public health interest** for accelerated assessment Art 14( 9) of Reg 726/2004



The General Court of the EU recently confirmed that unmet medical need can continue to exist despite multiple authorised treatments in respect of a condition or a disease. In Case T-11/18, the General Court noted the acknowledge that despite the fact 8 medicinal products had been authorised, schizophrenia patients continue to suffer unmet medical needs, in particular regarding the side effects associated with the authorised products.

Any criteria to identify “unmet medical need” should be broad and flexible enough to truly meet patients needs and foster therapeutic innovations that bring significant benefits. Moreover, the criteria should be agreed in a multi-stakeholder forum including patients, caregivers, regulators, HCPs, industry, HTA bodies, payers etc.

Any such discussion must consider a) adequacy of available treatments, disease severity, burden of disease/treatment, and b) population-based considerations (i.e. rare or paediatric).

- 2) Since drug development is a global undertaking – seeking convergence with other major international jurisdictions (e.g. US FDA) should also be taken into consideration, as it would facilitate and incentivise the global development of innovative therapies. From an EU viewpoint, there is a need for better alignment of processes and timelines between the various committees and working parties in order to ensure decisions on eligibility to various regulatory incentives and pathways are consistent.

Incentives may promote additional research and R&D investment, such as increased regulatory and/or scientific support during drug development (i.e. through PRIME). A future PRIME scheme could foster early interactions with HTA bodies (e.g. on orphan drugs). Furthermore, other options could be explored, such as ultra-accelerated review processes, and potential rolling reviews in order to accelerate approvals.

It should be recognised that there may still be remaining unmet need in disease areas which benefit from approved treatments. Hence there is a need to further incentivise all areas of unmet need by providing a predictable and favourable environment that supports innovation.

We stress that novel rewards should complement and not replace the existing incentives. Furthermore, any revision of the system of incentives requires an inclusive dialogue between the EC and industry.

- 3) The COVID-19 pandemic has shown how e.g. regulatory systems can be optimised to speed up access – learnings need to be taken from this.

Pathways for early dialogue with regulators and HTA bodies should be strengthened, which should also be introduced into appropriate study design. For example, in the field of rare diseases, the EMA's Committee on OMPs already assesses significant benefit of orphan medicinal products, it could be explored whether HTA bodies could be involved at an early stage in this process and their acceptance of significant benefit could be encouraged through more interactions between HTA/payers and the EMA. In general, increased early dialogue and involvement of HTA bodies/payers at an early stage of regulatory processes could be encouraged/explored in order to facilitate future acceptance of data and access for patients. RWE/RWD can complement the data generated from clinical trials.

