Can PRIME attract innovation towards unmet needs / disruptive medicines?

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Pfizer

WORLDWIDE SAFETY & REGULATORY

Worldwide Research & Development

Application for PRIME (Guidance)

- Target medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation
  - Advanced therapies (gene therapies and cell therapies): 12 of 32 granted PRIME
  - Antibody therapies and combined antibody therapies: 8 of 32 granted PRIME
- Target conditions where there is an unmet medical need and demonstrate the potential to address the unmet medical need
- Support the claim that the product has the potential to bring a major therapeutic advantage to patients in a given indication, through a clinically meaningful improvement of efficacy
  - Apply based on the availability of preliminary clinical evidence in patients to demonstrate the promising activity of the medicinal product and its potential to address to a significant extent an unmet medical need (proof of concept)
  - In exceptional cases, (SME) may apply at an earlier stage of development based on
    - compelling nonclinical data
    - first in man studies

Application for PRIME

- Consider a PRIME application if:
  - Your product may treat an unmet medical need (even as a additional benefit to the main indication)
  - The product technology (or mechanism of action) is novel / transformative
  - You have Phase 1 and/or Phase 2a clinical data showing amazing efficacy or statistically very compelling efficacy
    - In very exceptional circumstances, consider applying with non-clinical and Phase 1 (healthy volunteer) data
  - Your competitors have PRIME status and are at roughly the same stage of development
- Simple application with quick turnaround of decision and option to re-apply
- EMA has indicated that for entry to the PRIME scheme consideration is given to the added benefit EMA can provide to the product development
  - Less likely if expertise and resources are available to the applicant (big Pharma)
  - Less likely at later stages of development (e.g., products already in Phase 3)

Why Apply for PRIME?

- PRIME is the equivalent of the US FDA's Breakthrough Therapy Designation and if you have one, apply for the other
  - This is NOT correct! The two systems are complementary , but breakthrough designation recognises 'priority' products, PRIME is intended to guide and support the development of priority products
  - But, basis for both applications should be consistent
- Publicity/recognition of EMA recognising priority medicine status
  - Recognition of EMA's recognition of the product when partnering or seeking additional investment for further development / clinical studies
  - e.g., Mereo BioPharma 16% share rise following PRIME designation for BPS-804 for the treatment of osteogenesis imperfecta

Benefits of PRIME

- Confirmation of entry to the centralised MAA review procedure
- Accelerated MAA assessment
- Identification of Rapporteur
  - Useful for seeking national advice/discussions with the Rapporteur regulatory agency
- Rapporteur involvement in EMA regulatory interactions (scientific advice, PIP, etc.)
- Recognition of potential to treat an unmet medical need
  - Conditional marketing authorisation
  - EUnetHTA consolidated advice in EMA-HTA parallel advice procedure (establishing product value and market access)
- PRIME kick-off meeting
  - Not another scientific advice meeting
  - Focus on areas for further discussion rather than trying to resolve issues
- Additional PRIME interactions
  - At key product development stages

Will PRIME benefit innovation?

- PRIME does not focus on very early, experimental innovative technologies or medicines
  - Clinical data or promise of clinical efficacy is needed
  - Role for the EMA's EU Innovation Network / Innovation Task Force (ITF)
- Recognition of highly promising transformative medicines focuses attention on these products and their developers, aiding investment and promoting their development
  - Particularly useful for SMEs
- Facilitate early MAA filing strategies (e.g., conditional approval) by greater understanding of product benefit-risk?
- Added benefit of EMA and Rapporteur expertise through PRIME and the PRIME meetings will facilitate the optimal development to reach the EU market
  - Parallel regulatory interaction to discuss strategy, identify gaps and ensure development is on-track