2ND MULTI-STAKEHOLDER

Symposium ON IMPROVING PATIENT ACCESS TO RARE DISEASE THERAPIES

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HOTEL LE PLAZA
BRUSSELS

20 years
EURORDIS
RARE DISEASES EUROPE

EMA, PRIME, Adaptive pathways

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#RareEU2017

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA's efforts to enable timely access: PRIME, Adaptive Pathways

Eurordis, Brussels, February 2017

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EMA's commitment to promote timely access

- **Front end**: PRIME; pilot programs to elicit patient preferences; early dialogues with HTA bodies (ample experience with early parallel scientific advice)
- **Back end**: data infrastructure/analysis development (registries project), 'late' interaction with HTA bodies (starting)
- **Product lifespan**: Adaptive Pathways pilots, IMI ADAPT SMART project

Requirement for multi-stakeholder engagement is high in all initiatives (no 'regulator-only' initiatives)

Focus on PRIME and Adaptive Pathways

Two complementary strands of activity:

- **PRIME:** focus on earlier, efficient interactions; resource prioritisation to address unmet medical need; fast-moving procedures

- **Adaptive Pathways:** focus on lifespan approach to drug development, authorisation, reimbursement and use of product

Added value? PRIME

- Reinforce scientific and regulatory advice → increase likelihood of robust and useful data generation
- Enable accelerated assessment → accelerated availability
- Dedicating extra resources to unmet medical need (→ steer R&D towards unmet need?)
- Provide platform for patient and HTA input
- Enhance transparency (active substances, broad characteristics,…)

Added value? Adaptive Pathways

- Address the ‘access versus evidence’ conundrum
- Reduce (unavoidable) uncertainties fast
- Enable development of non-conventional (‘difficult’) products
- Help enlarge the tool box for evidence generation
- Help address sustainability of access (?)
- Leverage multi-stakeholder collaboration

Thank you for your attention

Further information

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