# Paid early access in Europe – Is it needed, and what should it look like?

**ATTENDEES**

- 3x Dolon
- 6x Industry
- 2x Consultancy
- 1x Implementation services
- 1x Academia

## Necessity of paid early access

- Early access is a vital mechanism for expediting the availability of treatments in Europe
- Paid early access is important for rare diseases with small eligible patient populations, and advanced therapy medicinal products (ATMPs), where a single-administration results in a single point to realise commercial potential, as free-of-charge supply has a proportionally larger impact on return on investment
- Offering reimbursement ensures patients are able to receive faster access independent of whether a manufacturer is able to assume the financial burden associated with offering free-of-charge supply
- However, the establishment of paid early access in Europe could increase access disparity between higher income countries and those with more constrained healthcare budgets
- If Member States adopted pricing and reimbursement (P&R) processes that allowed immediate reimbursement following marketing authorisation (e.g., Germany), the need for paid early access would decrease, only being necessary to bridge the gap between pivotal trial results and regulatory approval

## Barriers to paid early access

- Fragmented decision-making
- Complexity/administrative burden of implementation
- Challenging pricing dynamics balancing affordability, evidential uncertainty and impact on formal P&R
- Perceived duplication of formal P&R processes

## Key questions and considerations

### 1 At what level should decision-making and funding take place?

National level decision-making and funding ensures country specific dynamics (e.g., willingness-to-pay) are accounted for, while reducing inequity or 'postcode healthcare' due to fragmented decision-making at a regional/local level

### 2 Should approval cover a single patient, or many?

Cohort programs may be more efficient and/or drive better equity in care than Named Patient approvals, however robust criteria are needed to ensure patients meet trial criteria and/or regulatory labels, providing confirmation of pivotal trial outcomes in the intended treatment population

### 3 What are the criteria that could be used to make approval decisions?

- High disease burden
- Significant unmet medical need
- Sufficient clinical evidence is available supporting therapeutic effect and safety
- Treatment offers a substantial improvement in disease management vs. standard of care
- Substantial impact on return on investment expected (e.g., orphan indications, ATMPs) if given free of charge

### 4 Is free pricing or pricing negotiations more appropriate?

- Price negotiations allow a dialogue between payer and manufacturer but may drive significant delays for patients, undermining the value of early access
- Free pricing offers a faster route to patient; compensatory mechanisms (such as the rebates seen with the French AAP) can be used to ensure prices acceptable for payers; however, they must be carefully calibrated to balance incentives/risk between manufacturers and payers to ensure the formal P&R process is completed

### 5 How should evidence collected through paid early access programs be leveraged, if at all?

- Data collected through paid early access programs can offer supplementary insights into the efficacy and safety of an innovation in clinical practice
- However, real world evidence may not be collected with sufficient patient numbers to provide a robust picture of therapeutic effect, or ultimately meet stringent payer evidence standards
- Robust protocols on how evidence is generated, and utilised or discounted in pricing and reimbursement decision-making, should be established through industry/payer dialogue