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Dilemmas in Early Access Programmes

IQPC Conference on Early and Managed Access Programmes, 22-24 October 2018, London/UK

Hartwig Gajek, M.D., Hesyra-Medical GmbH

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So many good reasons for Early Access

- Humanity
- Ethics
- Responsibility
- Perception of company
- ... potentially others

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... and a few aspects to be considered

- Patients
- Doctors/ Pharmacists
- Industry
- Regulators
- Payers/Healthcare providers

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Serving patients

- Driven by expectation for personal relief, rather than standards of ‚clinical equipoise'
- Hope overriding fear of potential harm, or sense of futility
- Terminally ill patients, and relatives, taking chance of ‚last resort'
- May opt to exit clinical trials, especially if allotted to control/ placebo arm, hoping to improve their odds

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Doctors and Pharmacists

- Typically, a key stakeholder in EAP
- The man in the middle – between pressure from patients, and needing support from industry
- Should receive adequate support in patient interaction
- Increased liabilty with prescription of unlicensed medicine, esp. if based on scarce data (e.g., ph1/2a only)
- Administrative burden (submissions, reporting, diversity of EAP, ...)

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Industry

- EA being rationed
  - Quantity of drug available
  - Competition with clnical trial recruitment
  - Accessible only where framework exists
- Internal rules for decison making to be set
- Struggle to match external expectations with reality
- Need to determine objectives, and adequacy of approach

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Industry

- Need to balance fostering awareness, or creating advocacy for drug, and potential frustration by patients/HCP's
- Mindful of accrual rates in development programmes, and EAP
- Consumption of internal resources
  - Review committee (internal/external)
  - Internal administration
  - Production logistics
  - RA/PV management
  - Unpredictable timelines
  - Diverse local requirements

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Regulatory Authorities

- Primary mission
  - Risk-Benefit Ratio
  - Public health issues
- RA usually relaxed about confounding of clinical trial data, but potential exists
- Requiring structured clinical evidence for efficacy and safety
- Visibility of EAP, in the absence of critical contribution of information for drug licensing

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Payers or Healthcare Providers

- Although reimbursement for EA not ‚inherent', remuneration of ‚reasonable' cost may be negotiable
  - Manufacturing, logistics
  - Administration/monitoring ?
  - Insurance ?
- Mechanism of charging unclear
  - Direct-to-patient?
  - Hospital/pharmacy?
  - Healthcare provider?
- Pricing models for EAP potentially impacting HTA – unclear whether positively or negatively

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Payers or Healthcare Providers

- Period of coverage beyond RA approval, until reimbursement
  - Regulatory paths designed as ‚adaptive licensing', but no corresponding mechanisms for reimbursement
  - Transition to regular reimbursement may be challenging
- Overall, payers reluctant to pay, struggling to cover licensed medicines
  - Issue of ‚equity of access'?
  - Bigger companies afford EA, while for smaller enterprises, the burden may besignificant

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Concluding remarks

- Check your objectives: being good Samaritan, or collect data
- Consider involving healthcare providers, being responsible for ‚equity in access'
- Regulatory Authorities have a degree of oversight, but EAP are not within their original remit
- RA usually helpful in designing practical data collections
- If you require payer-relevant data, you may be anyway too late with EAP