Expanded Access - Challenges

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Access Challenges for Companies

Demand for medicines has never been greater, timelines for approval uncertain

Requests often begin early when company lacks alignment, provision or budget

Dynamic legislation requires regulatory expertise & experienced interpretation

Managing unpredictable, complex requests requires significant resources

EAPs - Myths & Misconceptions

Cannot start an access program prior to having Ph III data	• Ability to launch is not tied to the phase of development. It is dependent on licensed status of drug and sponsor risk assessment
Unplanned safety events arising from EAP could derail approval	• HAs understand access programs are conducted in uncontrolled environments and focused on critically ill patients, no evidence of drug not being approved due to EAP safety event
There's no funding for global chargeable programs, so will not have any uptake	• There are many examples of successful ex-US chargeable programs in oncology, CNS, rare disease
Sponsor will lose control of drug	• Sponsor can have extremely tight control of who gains access and the specific conditions where they will permit access
Risky, could damage relationships with global regulators	• Majority of countries have stated legislation for access, HAs are open to finding a compliant path forward to meet patient needs
EAPs are cost prohibitive	• Companies can consider hybrid approach, keep program small initially, expand after first approval

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Key Commercial Challenges

- **EAP strategy - Alignment with commercial strategy?**
  - **Countries?**
  - **Physicians?**
  - **Patients?**
  - **Timing?**
- **Resource constraints / Lack of regulatory knowledge**
- **Drug supply limitations**
- **Physician demands for payment**
- **No budget to support program**
- **Charging limitations (US – only direct cost)**
- **Unsure of RWD feasibility**
- **Compliance concerns – cant be seen as 'seeding market'**

Thank you!

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