Innovative Medicinal Products and Early Access Provision: challenges and opportunities to improve access to care for oncology patients

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Introduction & Background

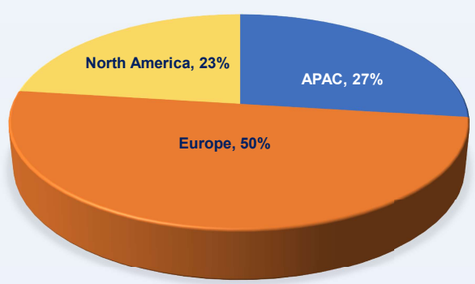
- An unmet medical need exists for many oncology patients who cannot be treated satisfactorily by available therapeutic options.
- Early access provision (EAP) is endorsed by competent authorities to improve patient access to innovative medicinal products (InMPs).
- Despite efforts made by competent authorities to expedite development, review and approval of InMPs, there may still be lengthy delays before oncology patients can gain access to InMPs.
- Recently reported data from the US Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) revealed that applications for expanded access pathways dealing with patient cohorts remain low, despite high approval rates.
- Challenges practicing oncologists face when dealing with EAP, together with limited education and support, may influence access of oncology patients to care.
- This research determined awareness and understanding among practicing physicians of EAP, along with specific educational needs that could improve oncology patients' access to care via implementation of EAP prior to InMP's marketing authorization.

Methods

- To explore the understanding, knowledge gaps and acceptance of EAP pathways, a fully anonymous on-line survey was conducted between November 2020 and January 2021.
- A total of 3,258 physicians (including practicing oncologists) were questioned about their knowledge and understanding of EAP, their level of knowledge and experience with the process, understanding of their own responsibilities, and their interest in further educational activities and opportunities to improve the process.
- Respondents were contacted and invited to participate using a mainstream survey platform: guidance and emails were sent explaining the purpose of the survey, the authors conducting the survey, and a link to the actual survey platform.
- Attributes were rated on a 5-point scale from 1 not important/strongly disagree/very low, to 5 very important/strongly agree/very high.
- Only fully completed responses were included in the analysis presented below.
- Special note: due to stringent regulations related to data privacy protection in the European Union, no personally identifiable data were requested or captured in the survey, and there was no tracking of individual participant completion.
- In addition a search of the ClinicalTrials.gov and EU Clinical Trials registers was performed for the period 1 Jan 2015 to December 2020 to determine how many cancer products had associated patient group applications for EAP prior to marketing authorization.

Results

- The majority of respondents were located in Europe and North America (50% and 23%, respectively), with additional representations from South-East Asia, China, Japan, and Australia (27%) ***Figure 1***.
- Most of the responders were oncologists (75%), but other specialties were also represented including pulmonologists, hematologists, and cardiologists.



*APAC: Asia –Pacific Region

Figure 1 Regional breakdown of on-line survey participants. Responders were located in Europe (50%), North America/the USA and Canada (23%); Asia-Pacific (27%)

- Majority of responders (56%) had limited experience with EAP in their daily practice (-<2 patients/year); 19% handled requests for > 5 EAP patients annually, and 25% for 2-5 EAP patients a year ***Figure 2***.
- Two-thirds of physicians indicated an average or lower level of understanding about the application process and regulatory requirements (65.2% and 66.0%, respectively).
- For data collection and serious adverse event reporting under EAP, 57.8% and 50.5% of respondents, respectively, had an average or lower level of understanding.

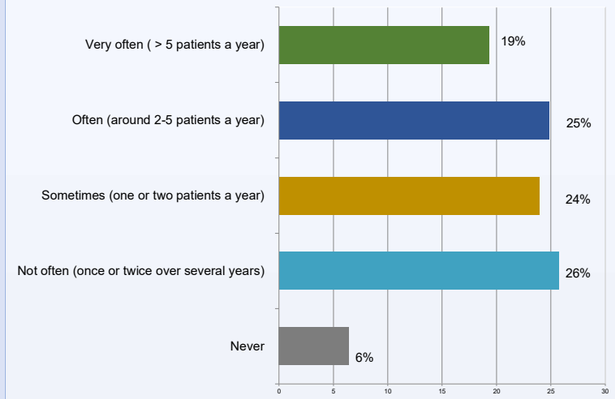


Figure 2 Responders experience with EAP in daily practice: never (6%); not often (26%); sometimes (24%); often (25%) and very often (19%)

- A high proportion of respondents reported that they would consider the availability of clinical efficacy and safety data from comparative phase III randomized controlled trials as of high and very high importance to support their decision to apply for EAP (93.4% and 86.8%, respectively). Other evidence and study types were rated substantially lower (e.g. pharmacokinetic, pharmacodynamic, mode of action, etc.) ***Figure 3***.
- In addition, search of the ClinicalTrials.gov and EU Clinical Trials registers for the period 1 Jan 2015 to December 2020 identified:
  - 38,407 cancer trials with a US location, of which only 149 (0.4%) offered EAP protocol.
  - 8,981 cancer trials listed with a EudraCT, only 21 (0.23%) included EAP protocol.

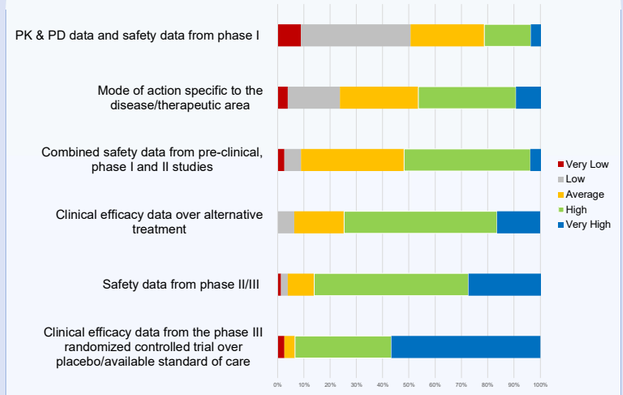


Figure 3 Evidence important to support practicing physician decision to request innovative medicinal product under early access provision

Conclusion

Challenges posed by the EAP process for InMP, together with a lack of education on this topic, might contribute to its under-utilization and influence early access of oncology patients to care.

Continuous educational efforts from different stakeholders, specifically during InMP clinical development stage, are required to better inform and support practicing oncologists dealing with EAP.

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Conflict of Interest

Beyond employment cited above, authors have neither conflict of interest nor financial support for this work to disclose. Mrs Yianick Green-Morrison was an employee of System Analytic at the time of poster development.