Involving Patients in Oncology Drug Development
Opening Remarks

Industry Perspective

Elevate multi-stakeholder involvement

Aim for Impact: Decisions, including on product information more adapted towards patients' needs while ensuring the patients are appropriately engaged during the process. Broader applicability of joint advice to further streamline the new medicines development process. Study design that serves multiple stakeholders moving away from sequential data generation.

Key metrics, EXAMPLE:
Evidence that patient's needs are in the center throughout the lifecycle of a medicine. Implement and optimise electronic product information (ePIL) project (infrastructure and legislative framework).

*Slide presented at EMA Regulatory Science to 2025 Multi Stakeholder Workshop 24 October 2018, Alan Morrison, EFPIA Regulatory Strategy Committee

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Industry is committed to bringing patients' perspective to drug development.

Pathways to do this include:

- Listening and understanding the patients' need throughout drug development (e.g., focus group, patient interview, ad board, literature review, Social media review)
- Including patient-relevant endpoints in clinical trials to collect standardized data on the burden of the disease and the impact of the treatment
- Generating patient preference data and evidence reflecting patients' treatment experience (tolerability, symptoms and functional impact, HRQoL, convenience) to complement traditional evidence (OS, PFS, Safety) for R:B evaluation
- Broadening of selection criteria and access in CT for more representative population (e.g., increased diversity)
- Increasing partnering with academic researchers & patient communities to inform protocol development

Industry's use of patient experience throughout the to drug' lifecycle.

Engagement Target and Population

Patient access

Clinical benefit

Preclinical | Phase 1 | Phase 2a/b | Registration | Post-approval

What is the treatment landscape and what are the medical needs?
Program viability

What are the disease manifestations? How do patients vary within a condition?
Target population

Which concepts are patient-relevant? What change constitute a meaningful outcomes?
Endpoints

What magnitude of effect is meaningful?
Sample size

What are the maximum acceptable risks? The minimum required benefit?
Defensible B:R

How does clinical trial experience apply to me?
Treatment decision, Adherence

What is an acceptable trial design?
Trial duration, visit schedule

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Opportunities:

- ☆ Seamless and more rapid drug development: drugs are reaching patents faster then before (PRIME, Accelerated approval, breakthrough designations)
- ☆ Better strategies to target unmet medical need (rare disease, personalized medicine)
- ☆ Ongoing discussions (Industry-Regulators-Patients) to define "patient-relevant evidence" including preference and how to provide this to patients for a better informed decision-making
- ☆ Increased partnership with patients group, academia, and decision-makers (regulators, payers).
- ☆ Standardization of collection, analyses and interpretation of PRO data to achieve more robust and credible evidence
- ☆ Use of digital health applications to increase trial access (e.g., decentralized trials), decrease completion burden (home completion) and generate additional evidence of treatment experience

Challenges

- ☆ International drug development and regional differences in clinical practices and patient's input (e.g., pain, opioid use)
- ☆ Regulatory, payers frameworks, and legislation differ
- ☆ Lack of harmonized standards to have quantitative data on relative weight of product attributes to enable patient focused decision making by all stakeholders involved.
- ☆ Lack of consensus regarding dissemination of clinical trial findings including patient-relevant information in a format that effectively inform patients

Aligning the stars

- Patients' experience definition
- Pathways to better engage with patients
- Opportunities to bring evidence of patients experience for decision-making