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European Federation of Pharmaceutical
Industries and Associations



Patient experience data in decision making and future guidance

EMA Multi-stakeholder workshop

Patient experience data in medicines development and regulatory
decision-making

21/09 /2022

EMA – Industry interactions

Early advice on collecting patient experience data

Early EMA advice

Early interaction with EMA is important to discuss the proposed patient experience data, methods of collection and analysis and ensure these are fit-for-purpose to support regulatory decision-making

Agreement on timing

A joint agreement between applicant and EMA on optimal time and process to include patient(s) input would be helpful

Standard core measures

EMA is uniquely positioned to advance the development of standard core sets of measures for patients' disease and treatment burden at a disease or functional level. This would reduce the need to develop and review treatment-specific instruments.

Greater certainty

Multistakeholder collaboration is crucial to increase efficiency of gathering representative patient input on diseases, save patients' time and deliver greater certainty and fit for purpose tools

Patient experience data in decision making

- ▶ **Patient experience data (PED)** can provide **useful information to patients, prescribers, regulators** and other stakeholders and is important for HTA decisions on pricing & reimbursement
- ▶ Guidance is needed on how **meaningful PED** can be collected, analyzed and applied during drug development so that it is **fit-for-purpose for regulators and HTA bodies decision making**
 - ▶ Lack of clarity and guidance creates uncertainty that limits research into patient evidence
- ▶ Agencies provide **limited information on how PED has been used during the regulatory assessment and for decision-making** and what (if any) impact it had on the decision
 - ▶ This limits learning opportunities, and may lead to inefficiencies
- ▶ **Multi-stakeholder focus groups** could present an opportunity for sharing, discussing and documenting best practices



Transparency on use of patient experience data

- ▶ To encourage a more evidence driven, patient centric approach to drug development, **greater transparency on the use of patient experience data in regulatory decisions is needed**
- ▶ Propose that **transparency** on how patient experience data has been considered in the regulatory decision is an **explicit goal in the EMA plans for the development of patient centric drug development**
- ▶ Guidance should cover **what is needed to ensure that patient experience data is fit-for-purpose to be included in the regulatory decision-making process and documents** (e.g. EPAR) as well as, where appropriate, in the product information (SmPC)



What can be done to optimise the use of patient experience data in regulatory decision making?

Development of COAs and patient relevant endpoints for specific diseases and core COAs across indications



Use of IMI PREFER recommendations to develop robust and validated patient preference studies



Development of best practices for transparency in PED use in regulatory decision making



ICH guidance to ensure a global approach



PED and its impact on regulatory benefit-risk decision included in the EPAR and product information

Conclusion & next steps

- ▶ **Industry is strongly committed to patient focused drug development** and to substantiate the value proposition and increase the uptake of patient engagement
- ▶ EMA's engagement with patients is highly appreciated and an **ongoing multistakeholder dialogue, including sharing of case studies, would be beneficial** to learn from each other and develop global best practices under ICH
- ▶ **Alignment of PED requirements across regions would be beneficial**
- ▶ **Strengthening of EMA and EU medicines agencies network capacity** will be needed to facilitate the use and acceptance of patient evidence
- ▶ To maintain the momentum, **continuous dialogue and more in-depth discussion e.g. in multi-stakeholder EMA/HMA focus groups**, is needed