EnprEMA CG Survey Results: Opportunities for European patients in global programmes

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Enpr-EMA Face to Face Meeting

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6 responses to survey: Opportunities for European Patients in Global programmes (Aug 2019)

- **HunPedNet (Hungarian NW):** EU and US patients have same opportunities, don't see this problem.
- **ITCC (oncology NW):** Early phase oncology trials start approx. at the same time in EU and US. EU patients have equal opportunities and often accrual is better in Europe. Sometimes an approved drug is not available/accessible in all EU countries. **Could Enpr-EMA do something to harmonise HTA assessment in different countries?**
- **RITIP (Spanish NW):** General impression is that recruitment starts faster in US and that more trials are available to patients in the US. Don't think that medicines of global trials are only authorised in US. HTA assessment and reimbursement is different per country which can be a problem for EC to address. Sometimes children do not have access to appropriate formulations which are authorised (e.g. fixed dose combinations (FDC) for TB).
- **BFM Study Group (leukaemia/lymphoma NW):** Yes, it's a problem. EU follows after US. There should be no reason to have two different marketing approvals (US and EU) and efforts should be shared between the two continents given that equal resources and opportunities are ensured. **Need for harmonised legislation across legislatures, more funds, more resources, prioritizing studies and avoiding duplications, unified procedures and ethical committees in the different Member States.**
- **MHRA experts (UK):** In terms of simultaneous participation of EU and US patients regulatory barriers do slow things down but are not insurmountable. It can swing both ways, some companies engage first and foremost with the FDA and others with the EMA/EU. Not aware of European patients participating in CTs that would only lead to FDA approval but sometimes FDA wider label via extrapolation (e.g. CF ivacaftor). However, sometimes product not authorised in all countries where patients are from (e.g. CF). (1) **Discussions between FDA & EMA important to align trial protocols.** (2) **Identify through a working group those products that are licensed under PIP or orphan disease categorization and where study sites were based (via clinicaltrials.gov) and what impact trial location had on EMA licensing and availability of therapeutics within member states. Request information from pharmaceutical companies limiting their sites to outside the EU as to the perceived barriers to working within the EU (cost regulatory etc).**
- **ECFS-CTN (Cystic Fibrosis):** Considerably better chances for patients in US of being able to enter a study than in Europe. Moreover, sites in EU open late which leads to a rushed and impractical experience for patients. FDA approval of ivacaftor (CF) much wider than in EU following global trials. Moreover, patients in some countries have no access to some licensed products due to them not being reimbursed by healthcare system (e.g. Orkambi and Symkevi in UK). **We would welcome more alignment between FDA and EMA, and other global regulators, on the approval label given to drugs, and the accepted study design that would lead to approval. Sponsors seem to find it slower to open studies in Europe than US, often due to the multiple ethical approvals needed across European countries and regions – could EnprEMA have a role in making this easier? Can we lobby for access to trials for people living in Europe to be more equitable?**

For discussion: Some aspects (e.g. multiple ethics approvals, lengthy CT authorisation process) will significantly improve when the CT Regulation comes into force. However, for other aspects, some highlighted by the respondents, Enpr-EMA might be able to make an impact, including with HTAs.