Qualification of disease registries & PRAC
Some considerations and reflections

Sabine Straus,
MEB Netherlands

Stating the obvious

- All medicines have **benefits and risks**
- **At the time of approval:** evidence comes mainly from controlled, randomised clinical trials
- **After approval:** medicines will be used in real life conditions by a larger, heterogeneous, less healthy population under less ideal conditions
- Post-marketing **safety monitoring** is important **to further characterize**

> Therefore, the post-marketing assessment of medicines plays a key role for better defining drugs' safety profile in real-world setting and filling the evidence gap of pre-marketing studies.

- Data can come from multiple sources including registries

What data are relevant for PRAC?

- Post marketing safety data
- Real world use
- Data on drug utilisation patterns including off label, discontinuation, switching, dosing
- Data to assess the effectiveness of risk minimisation measurements
- Data on patient experience
- Background incidence of adverse events
- Data disease epidemiology/natural history
- Post marketing efficacy/effectiveness data
- Data to assess the feasibility of planned studies

Regulator perspective characteristics of registry data that can provide 'useable' evidence for regulatory decision-making

- Accurate — Precise, reliable
- Adequate — Adequate range of characteristics of population covered & duration of follow-up
- Consistent — Across countries / data sources - or differences can be explained
- Complete
- Quality
- Timely — Lag time for data availability
- Valid — Internal and external validity
- Access
- Governance
- Interoperability /linkage

Strengths and usefulness of patient registries in post-authorisation setting

- Rare disease, certain populations, natural course
- Facilitate background data, reduce bias
- Actual use (duration, dose, holiday, switch)
- Decrease uncertainty of estimates (exposure data)
- Allow comparisons (treated/untreated) safety
- Long term data under real life conditions
- Ideally all approved drugs, by indication and by patient characteristics, (including untreated)
- Publicly available?

Can qualification help us understand if a registry can deliver what we need ?

Early days

So-far no results from qualified registries

First registry qualified in 2018 – ECFS patient registry on cystic fibrosis

Qualification status is not considered during decision making at PRAC currently

Too little experience on the impact of qualification to prove relevance for PASS over a non-qualified registry

Registries used eg

- **PedNet, EUHASS** –*Data collection* *(thromboembolic events, thrombotic microangiopathy, and anaphylaxis)*

/Long term safety haemophilia patients, **Hemlibra**

- **Disease registry in patients vision loss due to inherited retinal dystrophy** caused by confirmed biallelic RPE65 mutations *long-term safety*, **Luxturna**
- **IPIG registry** International Paroxysmal Nocturnal Haemoglobinuria Interest Group *long term safety* in a real-world setting/risk of adverse *pregnancy outcomes*, **Aspaveli**
- **EPP registry** erythropoietic protoporphyria *long term safety data /Treated Untreated /evaluate compliance to RMM*, **Scenesse**

Some take home considerations/thoughts

Registries have an aim/objective/mission

Is current qualification process shaped in an optimal way

(More involvement of PRAC during the qualification procedure?)

What does qualification add for all involved

Need for further analyses to understand better the added value