Prospective planning of evidence generation for orphan medicinal products – opportunities for multi-stakeholder dialogue

The Payers' Perspective

EMA - Payer community meeting

Diemen, 18 June 2019

What's in it for Payers?

- 👍 "Heads-up" about new products which may – or may not – pose reimbursement challenges
- 👍 Opportunity to voice concerns:
  - Selection of population – will this be the population that most urgently needs treatment? If not, will the results be generalizable to this population?
  - Trial design – will this be acceptable?
  - Will the endpoints be relevant for decision-making?
  - More fundamentally, will the product offer a desirable treatment option?
- 👎 Fear of "committing" to a product if no objections are made during the discussions
- 👎 Potential conflicts of interest later on, if assessors/negotiators were involved in the discussions
- 👎 Scarcity of resources to participate in many projects
- ? Is input taken into consideration?

(Tafuri, G., Lucas, I., Estevao, S., Moseley, J., d'Andon, A., Bruehl, H., … Vamvakas, S. (2018). The impact of parallel regulatory-health technology assessment scientific advice on clinical development. Assessing the uptake of regulatory and health technology assessment recommendations. British Journal of Clinical Pharmacology, 84(5), 1013–1019. https://doi.org/10.1111/bcp.13524)

MoCA

- Multi-Stakeholder, so it's not only company, but patients, too
- "Safe harbor"
- Additional topics covered, eg access
- No additional travel (costs)

Status at "first contact"	Nr.	Current Status as of March 2019
Pre-Clinical Stage	3	1 progressed to clinical stage 1 discontinued 1 in development
Phase 1/2	3	2 Development ongoing 1 approved by EMA
Phase 2	3	1 approved by EMA 1 terminated 1 in development
Phase 3	5	1 approved by EMA 1 terminated 3 in development
MAA submitted	2	Both approved by EMA
Already authorised	3	1 additional indication in development

What *should* be in it for Payers?

- Dialogues should cover generating evidence pre-MA for all aspects of access – this can include economic aspects
- Dialogues on post MA evidence generation (setting, transparency on data.
- No redundancy with EMA (Prime, parallel scientific advice), EUnetHTA (Early Dialogues) or regional consortia /BeNeLuxIA), but…
- Coordination of payers' views – there should be a flow of information among the institutions (acknowledging the need for commercial confidentiality), with opportunities for cross-consultation
- Timing considerations – early enough to make a difference but not too early, otherwise resource constraints…
- Ideally, payer participation should lead to products that are easier to assess and pay for!