Industry Partnering Panel (IPP) closed breakfast meeting

07:00-08:30 Tuesday 21st January

Parkside Plaza London at EB2020

Summary

At the 2nd IPP in London during EB2020, industry participants recognised the necessity of creating appropriate collegial channels to share good clinical practice in EB research. Delegates widely appreciated the importance of working in a pre-competitive space, exchanging the experience amassed by single organisations to shorten the path to translation. Some of the generally problematic areas that would significantly gain from such a dialogue could include:

- The harmonisation of procedures for biopsy collection, wounds' documentation, and their follow up.
- Bridging apparent discrepancies between HTA agencies, regulators, and the industry, on what clinical endpoints constitute an efficacious readout of therapeutic effect.
- Acceptance of patient-based outcomes is variable among regulators.
- Clinical studies in the EB field can be potentially flawed due to the infrequency of data collecting. To counter insufficient data, some participants advocated the more widespread introduction of sparse data statistics in clinical studies to increase the robustness of the conclusions drawn from them.
- When too few participants can be recruited, a larger multi-centre trial might represent the only practical means of accruing sufficient subjects to satisfy the trial objective. However, this comes at a price: they are considerably more expensive and challenging to coordinate, and if a trial is to be meaningfully interpreted and extrapolated, then how the protocol is administered should be straightforward and similar at all centres.
- Industry delegates also underlined the necessity of educating the FDA and EMA to acknowledge the strength of valuable clinical evidence accumulated from piecemeal studies like case series and reports.
- Pain and itch in EB patients frequently lead to a dramatic reduction in their quality of life (QOL), an aspect of the disease that has not yet met with an entirely satisfactory therapeutic response.
- Lack of structure in QOL questionnaires and difficulties in harmonising the information obtained across different methodologies makes it difficult to establish clear correspondences between categories, rendering navigation of existing protocols problematic.
- Likewise, there are subsisting doubts as to whether QOL indicators are currently considered when calculating QALYs.
- Approved products may take up several years before they reach the market as they struggle to get reimbursed by agencies. From an industry perspective, this could be in part attributed to the lack of consensus on standard-of-care practices, bonafide QOL comparators and the absence of a unified clinician-reported registry for patient data.