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PATIENT REPORTED OUTCOMES (PROs) IN EARLY ACCESS

For some industry players, the idea of collecting and using real world PRO data as part of Early Access Programs may be a new concept. Clinigen presents insight sourced through separate panel and workshop discussions with patient group and HCP stakeholder groups respectively.

Focused within rare cancers in Early Access, it brings together both unique perspectives. The research highlights the willingness of patients to provide data, and the opportunities for best practice PRO collection and use. It also raises important questions about what level of assurance can really be given to patients that their data will be handled and used in the ways they would like it to be. This is an area where industry can take the lead on being ambitious whilst also managing expectations.

" TO GET PATIENTS TO PARTICIPATE, INDUSTRY HAVE TO STICK THEIR HEAD ABOVE THE PARAPET AND SAY THIS IS WHY I THINK IT'S IMPORTANT. "

Patient Representative

KEY FINDINGS

01 PRO CO-DESIGN:

- Complete consensus that patients should be involved in co-designing PROs, and that this could in turn encourage use of the eventual PRO that is developed, increasing data capture and quality, and potential effectiveness of the PRO in informing clinical practice.
- Patient input should be provided by a small number of "patient experts" who have in turn sourced the feedback of those they represent.
- Patient groups are open to receiving training and improving health literacy regarding PRO design, validation and use.
- Patient representatives felt that patient input on the duration of data collection should also be sought, as data is often not collected for long enough, for example into survivorship.

02 PRO CAPTURE & STORAGE:

- The onus is on industry to ensure that such initiatives are communicated – as a cancer sufferer, it's not a priority to seek out opportunities to provide data, but there is nonetheless a willingness to provide PRO data.
- Patient representatives indicated that patients:
  - Would like a variety of formats to input, away from the oversight of their HCP, and HCPs agreed with this concept.
  - Want to be able to see their own data and know who else is seeing it.
  - Want to have 100% confidence in data security, a particular concern for cancer patients.
  - Do not want to be remunerated; HCPs were surprised at the consensus on this.
  - Feel more comfortable when a non-profit is involved.

03 PRO USE & FEEDBACK:

- Patients would like increased transparency in advance, on how the data is intended to be used, and what it might realistically achieve. HCPs felt this would assist them in explaining the project to potential patients.
- Any role of PROs in informing pricing and reimbursement discussions would be welcomed by patients but was viewed with more scepticism by HCPs who caution about managing expectations.
- Patients want the feedback loop to be closed regarding how the data was eventually used, however HCPs pointed to the difficulty in delivering on this promise in every case.

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NEXT STEPS

- ✔ "Patient expert" involvement in PRO co-design should be considered early on in development, and proactively communicated to patient groups.
- ✔ In advance, as much clarity as possible should be provided on what PRO data collected during Early Access will be used for, which will help HCPs to explain projects, and patients to get involved.
- ✔ Any patients involved in the co-design process or collection could go on to become "patient mentors" to help and support those who will provide PRO data in the future. There is an opportunity for this to help combat the lack of awareness within Early Access and also create models which can be replicated more widely.
- ✔ Industry, ideally working with non-profits, should consider using systems that can foster patients' confidence in data security, allowing access to their own data and timely knowledge on who is using it.
- ✔ It is important to listen to patients to understand the best format, timing and length of questionnaires that are developed, as well as how long to collect data for.
- ✔ Wherever possible, efforts should be made to close the feedback loop regarding what the data showed and what use it came to.
- ✔ Industry could maximise on the strong appetite amongst patient representatives to see PROs from Early Access impacting pricing and reimbursement discussions. Work could be done with HCPs to overcome cynicism.

FIND OUT MORE

See our related content on Clinigen Insights:
www.clinigengroup.com/insights

Request the full proceedings of the patient group and HCP sessions:
patientadvocacy@clinigengroup.com

Make contact with our team to discuss real-world data collection and use within your Early Access Program:
pharmaenquiries@clinigengroup.com

WE WOULD LIKE TO THANK ALL THE PATIENT GROUP REPRESENTATIVES AND HEALTH CARE PROFESSIONALS WHO GAVE THEIR TIME TO BE PART OF THE WORKSHOPS AND PROVIDE THEIR INSIGHTS ON THIS TOPIC.

ABOUT CLINIGEN

Clinigen Group plc is a global pharmaceutical and services company with a unique combination of businesses focused on providing ethical access to medicines. Its mission is to deliver the right medicine to the right patient at the right time through three areas of global medicine supply: clinical trial, unlicensed and licensed medicines.

The Group has sites in North America, Europe, Africa and Asia Pacific. Through its unlicensed medicines division, Clinigen designs and implements Early Access Programs, many of which include real-world data and PRO measures.