Ethics and Equipoise in PBC Clinical Trials

Keith Berelowitz - Chair of Fulham Research Ethics Committee/Founder of pRxEngage

Disclaimer: "The views expressed in this presentation are my own and do not necessarily reflect the official position of The Fulham Research Ethics Committee, the Health Research Authority, or any other affiliated organization."

The Role of the Ethics Committee

- Independent review of research proposals.
- Ensuring adherence to ethical principles and regulations (HRA, MHRA).
- Focus on patient safety, rights, and well-being.
- Multidisciplinary expertise for robust evaluation.

Key PBC Consideration: Due to PBC's chronic nature, we must ensure long term safety is considered.

My Role as Ethics Committee Chair

- Guiding ethical deliberation and decision-making.
- Maintaining impartiality and transparency.
- Ensuring clear communication and understanding.
- Staying up-to-date with ethical guidelines.

Key PBC Consideration: Due to the rare nature of PBC, I must ensure that the committee understands the complexities of the disease.

Key Ethical Considerations: Equipoise & Scientific Rigour

- **Equipoise:** Genuine uncertainty about treatment benefits.
- Justification for control group (placebo/comparator).
- Robust study design and methodology.
- Clear research question and objectives.
- Justification of sample sizes.

Key PBC Consideration: Balancing the need for scientific rigor with the potential burden on PBC patients.

Patient-Centric Focus: PPI & Information

- Meaningful Patient and Public Involvement (PPI) throughout.
- Clear, accessible patient information sheets and consent forms.
- Addressing patient burden (visits, tests, quality of life).
- Ensuring that the patient information sheets are written in easy to understand language.

Key PBC Consideration: The impact of PBC on quality of life necessitates minimizing patient burden.

Diversity, Accessibility, and Informed Decisions

- Diversity, Equity, and Inclusion (DEI) in recruitment.
- Accessible research opportunities for all PBC patients.
- Ensuring the patient has the capacity to make the decision to join the trial.
- Providing resources and support for informed decision-making.
- Considering the specific needs of vulnerable populations.

Key PBC Consideration: Due to the potential disparities in access, we must ensure trials reach diverse populations.

Actionable Insight: We need to provide patients with accessible platforms (e.g., online registries, patient advocacy groups) to find relevant research opportunities.

Data Safety & Post-Trial Access

- Robust data protection and confidentiality.
- Clear safety monitoring and adverse event reporting.
- Plans for post-trial access to beneficial treatments.
- Plans for informing participants of the results of the trial.

Key PBC Consideration: The need for long-term follow-up in PBC trials.

Key Takeaways

Ethical research prioritizes patient well-being.

Equipoise and scientific rigor are essential.

Meaningful PPI and accessible information are crucial.

We must provide accessible ways for patients to find trials.

We must ensure informed consent.