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Summary

Requirements for clinical trials and for the authorisation of new medicines in Europe

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Mechanisms & Personalised Medicine

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The presentation reviewed the European Medicines Agency's regulatory framework, approval pathways including conditional authorization, and guideline development processes for gastroenterological therapies.

KEY POINTS

- EMA licenses granted through the centralised procedure are automatically valid across all of Europe, with the CHMP committee providing recommendations directly to the European Commission.
- Conditional approval is restricted to life-threatening diseases with unmet medical needs, requires a positive benefit-risk ratio, and imposes obligations to complete data post-approval.
- The speaker stated that Ocaliva, licensed in 2016 for PBC based on surrogate markers, had its license revoked after confirmatory studies showed no real effect on hard outcomes and raised safety concerns.
- Two new PBC products were licensed by end of 2024, both with conditional approval based on 12-month trials using surrogate biomarkers, and both face obligations to conduct confirmatory long-term outcome studies.
- EMA and ICH regulatory guidelines are recommendations, not legal obligations; methodological approaches like hierarchical testing can address differences between FDA and EMA endpoint requirements.
- EMA welcomes scientific advice requests from academic consortia and start-ups, offering fee reductions, and develops disease-specific guidelines reactively based on industry pipelines and unmet regulatory needs.

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