



The MSP Advisory Group feedback

The European Hematology Association (EHA)

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Hematology – rare diseases, innovative treatments

Hematology is a rare disease specialty

- Relatively low patient numbers → **multinational collaboration**
- Limited economic interest in some diseases → reliance on **academic studies**
- Evidence generation → **innovative study designs**, real-world data
- Multi-agent therapies/post-exclusivity → **dose/combination-optimizations**

Hematology often “test bed” for innovation

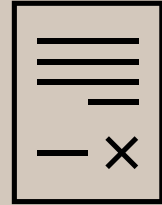
- ATMPs are already widely used in clinical practice, including hospital production
- Regulatory advice on academic ATMPs → product quality/evidence generation

Challenges often faced early by hematology, before wider populations

Multinational collaboration is essential

Problems raised

- Assessment under CTR, navigating legal agreements



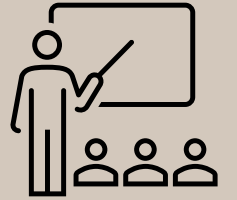
EHA supports ACT EU initiatives to:

- Streamline assessment process
 - Limit/align request for information during assessments
 - Empower reporting member state, including ethics
 - Flexibility in assessment timelines, will benefit assessors as well
- Standard EU templates for clinical research
 - Data sharing/collaboration agreements/IP rights
 - Only ad hoc contract in exceptional cases

Regulatory advice & educational activities for academia

Needed:

- Scientific/regulatory advice for academic sponsors
 - ATMP production, dose-optimizations/drug repurposing in academia
- Educational activities -> typically few trials in a career
 - Use the opportunities in CTR to minimize administrative burden



EHA supports ACT EU initiatives to develop:

- Framework for fast/fit-for-purpose advice to academic sponsors
 - Fees need to reflect academic needs/budgets
- EMA led educational activities/curriculum for sponsors/investigators
- Overview of support structures already in place