Lessons learned from academic development of ATMPs

What worked!

- Start the project "with the end in mind", early interaction with regulators to design trials and development path
- Importance of infrastructure, organization, and regulatory support
- Collection of all relevant pivotal data and good quality of data during early phase clinical trial for registration and access to market
- Hybrid model (academia/not for profit) and industry (late stage &commercialization + CDMO) leading to MA
- Approval (EMA) and P&R (AIFA) based on the totality of evidence from early to pivotal (single arm!)
- Access from other EU-MS is possible for an ultra-rare disease with strong support for pts

Hurdles and bottlenecks

- Disinvestment of industry for rare/ultra rare diseases with limited/no commercial interest
- Need to repeat scale up, validation, animal experiments → duplication and difficult reuse of data
- Difficulties to introduce innovation and optimization during development
- Lack of funds for GMP manufacturing, late stage clinical trials, commercialization and long-term FU
- Challenges in conducting randomised trials for rare diseases with ATMPs
- Fragmented P&R in EU
- Difficulties in S2 cross-border scheme for patient mobility from some MS

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