Initiatives on ATMPs

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Health and Food Safety

1. ATMPs

- Investments on ATMPs in the EU are growing at significant pace and amount of clinical trials on ATMPs hosted in the EU are indicative of a promising pipe-line of new products.
- The development of ATMPs poses unique challenges linked to the complexity of these products.
- The Commission is committed to ensure that the regulatory framework is well-adapted.
- We need to work together: MS are responsible for the assessment of the clinical trials, for the application of the GMO legislation, for the hospital exemption, *etc.*

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2. Initiatives on ATMPs

- ***Development of specific GMP for ATMPs***.
  - Application of GMP to ATMPs has consistently been signalled as a problem for developers of ATMPs.
  - Draft Guidelines adapt GMP requirements to specific characteristics of ATMPs, address novel questions, bring consistency with ATMP scientific Guidelines, scientific advices, and with approaches taken by assessors of clinical trials.
  - Two consultations have confirmed wide support from developers.

2. Initiatives on ATMPs (cont.)

- ***Development of pragmatic approach to GLP:***
  - Preclinical studies for ATMPs may not always be GLP-compliant.
  - Adaptation of GLP to ATMPs developed by CAT.
  - COM discussed issue with national authorities responsible for clinical trials.
  - Pragmatic approach developed by CAT was endorsed by national competent authorities.

2. Initiatives on ATMPs (cont.)

- ***Facilitating the understanding of regulatory requirements:***
  - Guideline on investigational ATMPs under development by CAT in collaboration with CTFG.
  - Q&A on application of risk-based approach to non-substantially manipulated products.
  - Scientific guidelines to clarify regulatory expectations in a rapidly-evolving field: *e,g.* Guideline on gene therapy medicinal products, Guideline on genetically modified cells, Guideline on comparability.

2. Initiatives on ATMPs (cont.)

- ***Adaptation of procedures:***
  - Streamlining marketing authorisation procedures (improve interface between various committees involved in the assessment of ATMPs).
  - Adapted scientific advice procedures for ATMPs with increased involvement of CAT.

2. Initiatives on ATMPs (cont.)

- ***Hospital exemption:***
  - Common approaches to address possible public health risks, sharing of information on safety/efficacy, increased transparency, sharing of best practices, etc.
  - Nominations of experts are welcome, preferably **by April, 18.**

- **Interface with GMO legislation**:
  - Dialogue between competent authorities responsible for medicinal products and GMO to address the shortcomings identified by developers of gene therapy, within current legal framework.

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3. Conclusion

- Exciting times for ATMPs.
- We need to work together to foster the development of these promising type of products.
- Close monitoring and sharing of information is key: products have great potential but also entail risks.