Optimising resources

Focus group: Incentives for Academia Hospital and charities

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Fondazione Telethon

CAT Stakeholders workshop

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Agenda

Questions, examples and proposals

In the context of advanced therapies development:

- How can we facilitate the submission and the assessment of data submitted by hospital, charities and academia?
- How can we optimise resources from the charities point of view?

The ideal scenario

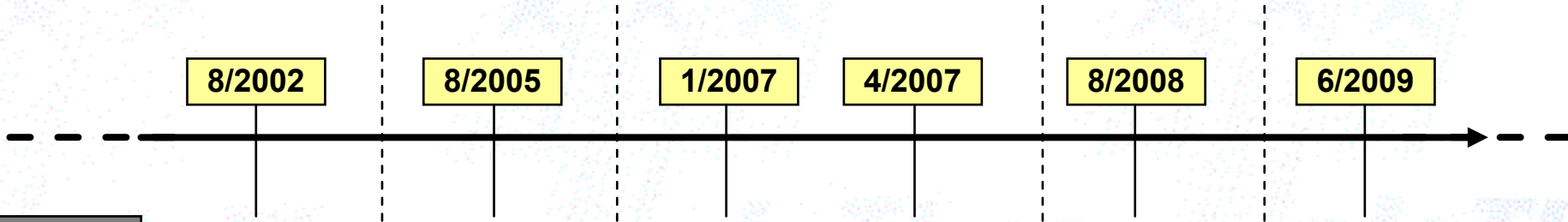
- Medicinal products are developed by pharmaceutical industries;
- Big Pharma or Small and Medium Enterprises have dedicated resources for the regulatory activities and the interaction with Regulatory bodies;
- Competent Regulatory Authorities have defined procedures designed for the interactions with pharmaceutical industries and SME

The real world

- New advanced therapies are very often based on data developed by academia, non profit organizations, or by institutions without a proper drug development programme
- Such institutions rarely have the regulatory expertise and competence necessary to support marketing Authorization applications of new advanced therapies
- The competent Regulatory Authorities do not have defined procedures designed for the interactions with charities and non-profit organizations in the context of a marketing authorization procedure

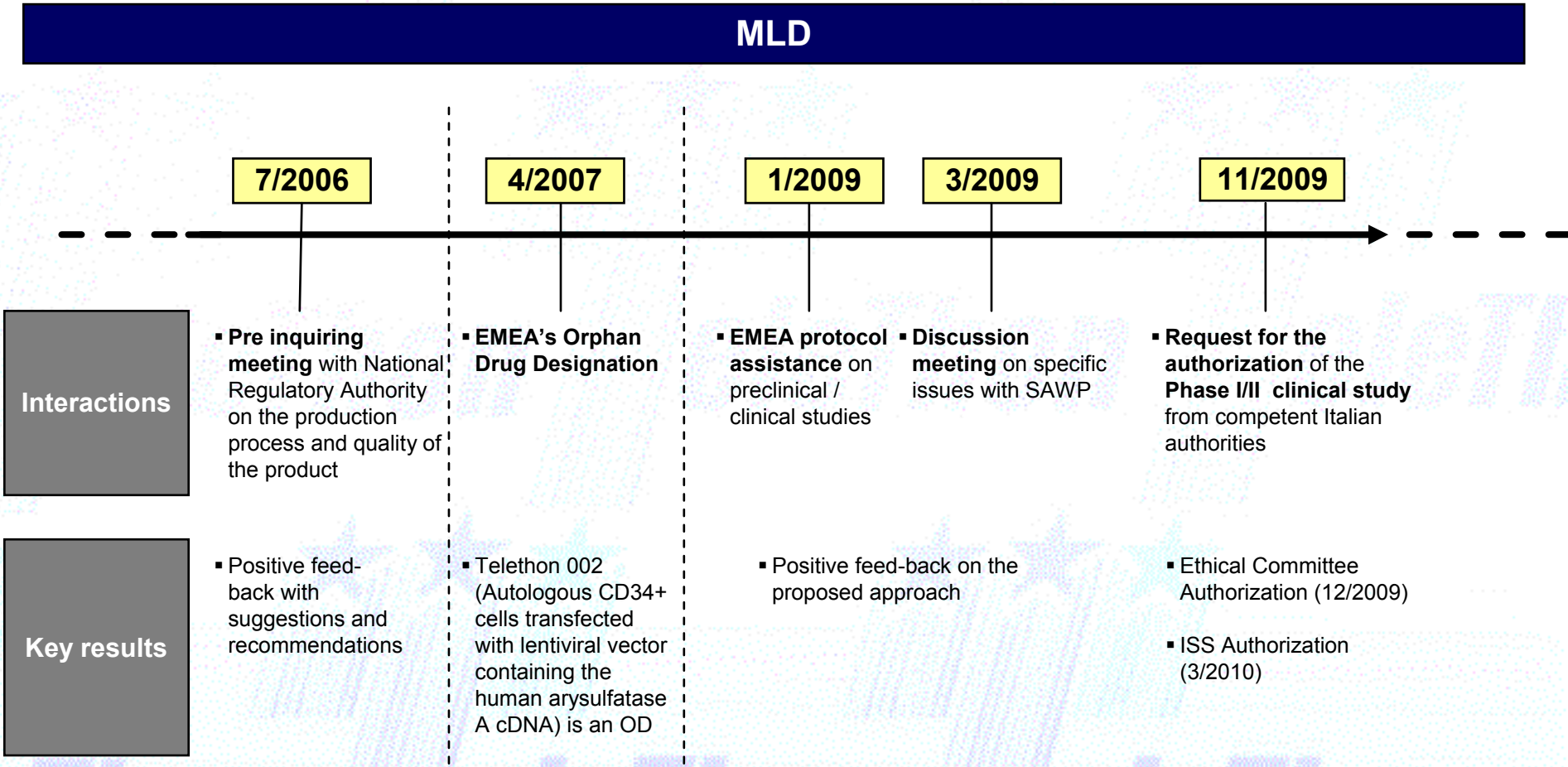
Ex vivo gene therapy for ADA-SCID: interactions endorsed and supported by regulatory authorities

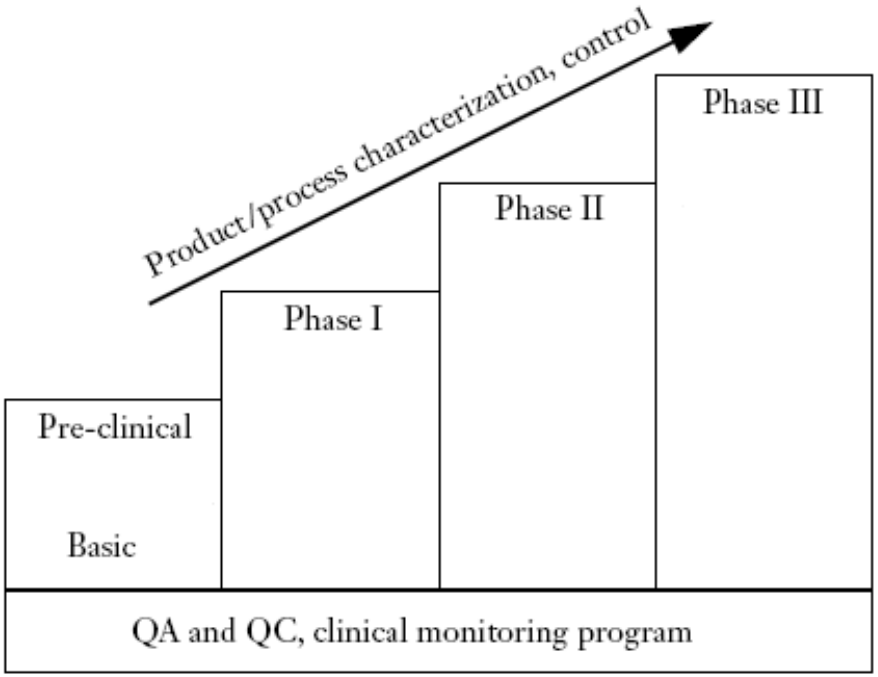
ADA-SCID



	8/2002	8/2005	1/2007	4/2007	8/2008	6/2009
Interactions	▪ **Pivotal Clinical trial** authorization	▪ **EMEA's Orphan Drug Designation**	▪ EMEA **pre-submission meeting**	▪ **EMEA protocol assistance** on preclinical and clinical studies	▪ **EMEA protocol assistance** on production process and quality	▪ **FDA's Orphan Drug Designation**
Results	▪ Beginning of the Clinical Trial	▪ ODD for Telethon 001 "Autologous CD34+ cells transfected with retroviral vector containing ADA gene"	▪ Positive feed-back and suggestions for revision	▪ Recommendations provided on the acceptability of a single clinical trial supporting MAA under exceptional circumstances	▪ Positive feed-back with suggestions and recommendations	▪ ODD for Telethon 001 "autologous CD34+ cells transfected with retroviral vector containing ADA gene"

Ex vivo gene therapy for Metachromatic leukodystrophy: interactions with regulatory authorities





- At Phase I a more basic level of production control and product characterization in keeping with the fundamental principles of GMPs should be expected

- More extensive process and analytical development and validation should take place over time in the next phases to result in full GMP compliance by the pivotal trial stage

Optimizing resources -1

- During the last decade several tools have been proposed for optimizing the drug approval process:
  - Disease Registries
  - Clinical Trial databases for regulatory authorities
  - Networks
  - …
- It is now time to verify whether and how such tools provided any real contribution to the drug developments in area like rare dieses, advanced therapies and unmet medical needs

- In this context a common effort involving all stakeholders and regulatory bodies will be important. In particular CAT, could coordinate and guide this activities providing transparency and supervision of the expertises and with the production of specific Guidance.

Optimizing resources -2

- At the European level it would be worth having some kind of initiative (e.g., workshop, training) supporting non-profit organizations in their regulatory efforts.

- Taking into account CAT suggestions and indications the FP7 research programme funding should support:

  - large scale production of GMP grade advanced therapies (e.g., nucleic acid, zinc finger nucleases)
  - CROs for animal toxicology testing
  - Phase I and Phase II clinical studies for Orphan drugs

Conclusion

In Tuscany, looking at the landscape sometimes you can clearly identify the line of the horizon, even if the road is not easy to find