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IPOPI's response to the European Commission's public consultation on the draft Guidelines on Good Clinical Practice for Advanced Therapy Medicinal Products

The International Patient Organisation for Primary Immunodeficiencies (IPOPI) welcomes the consultation launched by the European Commission on the draft Guidelines on Good Clinical Practice for Advanced Therapy Medicinal Products and would like to contribute to the Commission's reflection by providing the following ideas:

- Primary immunodeficiencies (PIDs) are a group of over 350 rare and chronic diseases caused when some components of the immune system (mainly cells and proteins) do not work properly. These deficiencies lead to increased susceptibility to a wide range of infections and means that infections can reoccur and leave the individual vulnerable to permanent organ damage, physical disability or even death. PIDs can be treated and, when the right diagnosis and treatment has been given, patients can live normal lives.
- Advanced Therapy Medicinal Products can be life-saving therapies for many patients with rare diseases. In the field of PIDs, some of the most severe types (such as Severe Combined Immunodeficiencies (SCID)) could be treated, even cured, by advanced therapy medicinal products, such as gene therapy or bone marrow transplantation. In fact, the European Commission authorised its first gene therapy in May 2016 for the treatment of adenosine deaminase deficiency (SCID-ADA).
- Patient participation in a clinical trial can be stressful for the patient and his/her family. Patients and their legally designated representatives do not have the medical knowledge of the medical experts and require that all informative materials that will be shared with them, should be written in a simple and lay-person manner. Information material and informed consent documents should be not only provided in writing, but also explained verbally (when possible) to the patient, so as to facilitate the understanding of the information provided. Additionally, a contact person from the investigator's team should be designated to reply to all the questions that patients or their legally designated representatives may have before, during or after the clinical trial takes place and also during the foreseen follow-up (when necessary).
- Long-term follow-up: IPOPI supports the idea of "*When clinical trial subjects should be followed after the investigational ATMP has been granted a marketing authorisation, it is recommended that the monitoring of the clinical trial subjects is integrated with the mechanisms foreseen in the marketing authorisation for the follow-up of subjects treated with the authorised product (e.g. registry, post-marketing studies)*".

  Data extracted from the follow-up of subjects treated with the authorised product after marketing authorisation has been granted, should ideally feed into the already existing patient registries or to a new one if none exists.

- Remote follow-up: detail arrangements for the remote conduct of follow-up activities should be discussed with the patient prior to the signature of the informed consent and also detailed in the informed consent and associated documents. The use of digital health application tools to optimise remote follow-up could also be encouraged.