31st October 2016

Submission of comments on public consultation document 'Concept of 'similar medicinal product' in the context of the orphan legislation'

Comments from:

Name of organisation or individual
Cell and Gene Therapy Catapult (CGT) The Cell and Gene Therapy Catapult was established in 2012 as an independent centre of excellence to advance the growth of the UK cell and gene therapy industry, by bridging the gap between scientific research and full-scale commercialisation. With more than 100 employees focusing on cell and gene therapy technologies, we work with our partners in academia and industry to ensure these life-changing therapies can be developed for use in health services throughout the world. We offer leading-edge capability, technology and innovation to enable companies to take products into clinical trials and provide clinical, process development, manufacturing, regulatory, health economics and market access expertise. The Cell and Gene Therapy Catapult is a trading name of Cell Therapy Catapult Ltd and works with Innovate UK. For more information go to ct.catapult.org.uk or visit www.gov.uk/innovate-uk.

1. General comments

General comment (if any)
The Cell and Gene Therapy Catapult appreciates the opportunity to comment on the EC DG Health and Food Safety's recommendations for improving the implementation of the orphan medicinal products regulation through a targeted review of Commission Regulation (EC) No 847/2000 on the concept of 'similar active substance'. This revision, particularly as it relates to ATMPs is welcome given the increasing number of ATMPs in preclinical and clinical development. In light of the continuing progress in this field, CGT recognises the value to adopting a definition of similarity which is not overly prescriptive however this remains very broad for ATMPs, clarification and greater explanation would be appreciated. Guidance in the form of a Question & Answer document, including some practical examples would be helpful for developers of these products. General advice for Tissue Engineered Products and we would suggest differences between in vivo and ex vivo Gene Therapy Products should be considered more fully within Commission Regulation (EC) No 847/2000 would also be appreciated.

2. Specific comments on text

Line number(s) of the relevant text	Comment and rationale; proposed changes
Line 109-111	**Comment**: Developers will typically not have knowledge of "differences in the manufacturing technology" for competing products and would therefore have difficulty applying this criteria. Further clarification on the term 'manufacturing technology' in this context would be helpful. **Comment**: How would the impact of any differences on the biological characteristics be measured? E.g. will this be based on potency assay in vitro / in vivo animal model / clinical trial.
Line 116	**Comment**: How would changes aimed at improving the relative safety profile, which may also be long term, be measured?
Line 112-117	**Comment**: The evidence base upon which to compare two products may be very difficult to judge given that these products may come to market based on abbreviated packages