From: Gavin McGowan <gavin.mcgowan@ncrc.ie>
Sent: mercredi 31 octobre 2018 19:30
To: SANTE PHARMACEUTICALS B5
Subject: TSC 01/2018 on GCP for ATMPs

Dear Sir/ Madam,

Please find comments from the National Children's Research Centre, Ireland, on the Targeted Consultation on the draft guideline on Good Clinical Practice for Advanced Therapy Medicinal Products (ATMPs) outlined below.

I can confirm that I am providing these comments as a representative of the National Children's Research Centre (NCRC). The category of the NCRC is 'other'. The NCRC is registered in Ireland as a Charity.

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2. Clinical Trial Design
Given the nature of the products/therapies involved, a statistical analysis plan to be presented/published at the outset for a given study design as part of the regulatory requirements.

3.2 Specific considerations regarding the Investigator's Brochure (IB)
(iii) *Reconstitution:* Line 248-249, typographical error (e.g. handing instructions). Should read as (e.g. handling instructions)
(iv) *Administration procedure:* Line 257, typographical error (e.g. handing instructions). Should read as (e.g. handling instructions)

5. Administration procedures
Line 305-306, "If the presence of the administration is envisaged…", amend to 'If the presence of the Sponsor during administration is envisaged…'

8.2 Long-term follow-up
More explicit longer term follow up requirements for trial sponsors of certain paediatric studies may be more appropriate, especially for those that may have malignant potential.

8.2.2. Remote follow-up
Line 378, typographical error 'Detail arrangements' should read as 'Detailed arrangements'
Line 379-381, suggest change to text from "In accordance with applicable requirements, the sponsor should ensure that approval of a clinical trial protocol is obtained in the country where the long term follow-up takes place" to 'In accordance with applicable requirements, the sponsor should ensure that the long term follow-up takes place in the countries where approval of a clinical trial protocol was obtained.'

8.3. Administration of out of specification products

Suggest to include obligation to inform trial participant in cases where the product is out of specification to enable their informed consent

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The National Children's Research Centre, located on the grounds of Our Lady's Children's Hospital, Crumlin, in Dublin is the largest children's research centre in Ireland. It has been at the heart of paediatric research in Ireland for over 50 years. Supported by donations to the Children's Medical and Research Foundation (CMRF), the NCRC is a key enabler of paediatric research in Ireland. Through our competitive research grants, state of the art laboratory, training and education, Discovery Banks and Children's Clinical Research Unit, we provide all of the infrastructure needed by clinicians and scientists to conduct high quality research into childhood disease. It is our mission to support internationally competitive, high quality research that has a real and lasting impact on child health.

Kind regards,

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NATIONAL CHILDREN'S RESEARCH CENTRE