News bulletin for small and medium-sized enterprises

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This news bulletin is published by the SME Office of the European Medicines Agency. It will be published three times a year.

The news bulletin aims to bring to the attention of SMEs, and their stakeholders, documents and activities related to the European regulatory environment.

Good manufacturing and good clinical practice (GXP)

Annex 2 of the GMP Guide was published in September 2007 (Link). It has been revised as a result the introduction of GMP for active substances used as starting materials, the increasing types of biological products and the new regulation on advanced therapies. It is released for public consultation with a deadline of March 2008. Feedback from organisations with experience in advanced therapies is particularly expected.

A draft reflection paper on advice to applicants, sponsors, contract research organisations (CROs) carrying out bioequivalence studies was released for consultation in October 2007 (EMEA/INS/GCP/468975/2007). Many bioequivalence studies are well conducted. However, in recent years, a number of cases have raised concerns amongst regulators which lead to the preparation of this paper. It underlines the responsibilities of the parties involved and reinforces the steps to be taken by the applicants, sponsors and CROs to ensure the quality of bioequivalence trials submitted in marketing authorisation dossiers. It is released for public consultation until 31 March 2008.

A draft reflection paper on expectations for electronic source documents used in clinical trials was released on 17 October 2007 for consultation (EMEA/505620/2007). Source data and documents form a cornerstone of the approach to record keeping in clinical trials conducted in accordance with Good Clinical Practice. With increasing use of information technology in pharmaceutical development there is a need to have clear guidance on the use of electronic source data and principles that should apply to them. It is released for public consultation until 30 April 2008.

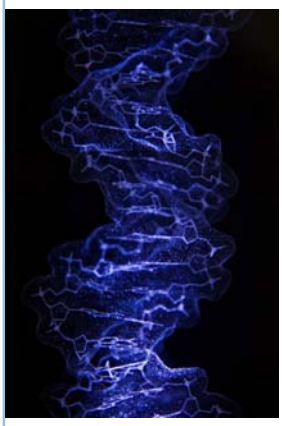
New scientific guidelines for human medicines

Genomics

An ICH note for guidance on definitions for genomic biomarkers, pharmacogenomics, pharmacogenetics, genomic data and sample coding categories was released in November 2007 (ICH Topic E15-EMEA/CHMP/ICH/437986/2006). It aims to harmonise terminology across the International Conference on Harmonisation (ICH) regions to facilitate pharmacogenomics and pharmacogenetics integration into global drug development and approval. It will come into effect in May 2008.

A reflection paper on pharmacogenomic samples, testing and data handling was released on 15 November 2007 (EMEA/CHMP/PGxWP/201914/2006). It highlights key principles to consider for PGx sampling in clinical trials intended for regulatory submissions.

A reflection paper on the use of genomics in cardiovascular clinical trials was released on 15 November 2007 (EMEA/CHMP/PGxWP/278789/2006). It reviews scientific matters concerning the use of genomic data in assessing therapeutic efficacy and tolerance of drugs in cardiovascular clinical intervention trials, focusing on genetic association with clinical endpoints.



Pharmaceutical development

A guideline on potency testing of cell based immunotherapy medicinal products for the treatment of cancer was released in October 2007 (EMEA/CHMP/BWP/271475/2006). Current guidance on cell therapy based medicinal products is available in the guideline for human cell based medicinal products (CHMP/410869/2006) which includes details on quality control and lot release testing of cell therapy products as well as to tests to evaluate the shelf-life. The newly released document intends to provide further guidance on specific requirements related to the development and validation of potency assays for cell based immunotherapy products. It will come into effect in May 2008.

Non-clinical safety

A guideline on strategies to identify and mitigate risks for first-in human clinical trials with investigational medicinal products came into effect on 1 September 2007 (EMEA/CHMP/SWP/28367/07). It is intended to assist sponsors in the transition from non-clinical to early clinical development. It also identifies factors influencing risk for new investigational medicinal products and considers quality aspects, non-clinical and clinical testing strategies and designs for first-in-human clinical trials. Strategies for mitigating and managing risk are given, including the calculation of the initial dose to be used in humans, the subsequent dose escalation, and the conduct of the clinical trial.

Clinical efficacy

A reflection paper on methodological issues in confirmatory clinical trials planned with an adaptive design was released in September 2007 (CHMP/EWP/2459/02). It includes considerations for adaptive design trials involving design modifications based on the results of an interim analysis.

New scientific guidelines for veterinary medicines

A guideline on "Stability testing of existing active substances and related finished products" (EMEA/CVMP/QWP/846/99-Rev.1) was released for consultation in October 2007. It was revised to be brought in line with the requirements of the note for guidance on stability testing of new veterinary drug substances and medicinal products (EMEA/CVMP/VICH/899/99-Rev.1). It is released for public consultation until 30 April 2008.

A note for guidance on "Declaration of storage conditions in the product information of pharmaceutical veterinary products, for active substances: annex to guideline on stability testing of new veterinary drug substances and medicinal products, annex to note for guidance on stability testing of existing active substances and finished related products" (EMEA/CVMP/422/99/Rev.3) was released in December 2007. The purpose of this guideline is to set out uniform statements on storage conditions for inclusion in the labelling of medicinal products and to define when they apply.

Regulatory guidance for human medicines

A guidance on "New therapeutic indication for a well established substance" was released in November 2007 (Link). It outlines the principles, procedure and information required to support an extended data exclusivity period based on significant pre-clinical or clinical studies carried out in relation to the new indication for a well established substance, as referred to in paragraph 5 of Article 10 of Directive 2001/83/EC, as amended by Directive 2004/27/EC.

A guidance on "Elements required to support the significant clinical benefit in comparison with existing therapies of a new therapeutic indication in order to benefit from an extended (11-year) marketing protection period" was released in November 2007 (Link). It outlines the principles, procedure and information required to support an extended marketing protection period based on a new therapeutic indication which claims to bring a significant clinical benefit in comparison with existing therapies, as referred to in Article 14(11) of Regulation (EC) No 726/2004 or Article 10(1) fourth subparagraph of Directive 2001/83/EC.

A revision of the European Commission guideline on Summary of Product Characteristics was released in December 2007 (EMEA/299527/2007). It aims to reflect new requirements in relation to the paediatric regulation and clarify guidance for other sections e.g. section 4.8 on undesirable effects. It is released for public consultation until 28 March 2008.

A document on "Scientific aspects and working definitions for the mandatory scope of the centralised procedure" was released in December 2007 (EMEA/CHMP/121944/2007). It includes general principles and scientific aspects on the definitions of the diseases appearing in the Annex to Regulation (EC) No 726/2004 (i.e. acquired immune deficiency syndrome, cancer, neurodegenerative disorders or diabetes, autoimmune diseases, immune dysfunctions and viral diseases). It will come into effect in May 2008.

Regulatory guidance for veterinary medicines

A draft guideline on the evaluation of the risk/benefit balance of veterinary medicinal products (EMEA/CVMP/248499/2007) was released in September 2007. It aims to improve the methodology for benefit-risk analyses to provide a more systematic approach to improve the consistency and transparency of decisions taken for veterinary medicines. It is released for public consultation until 31 March 2008.

News from European Institutions

The Regulation on advanced therapy medicinal products (Regulation (EC) No 1394/2007) was published in the Official Journal of the European Union in December 2007. Further information on implementation plan of the regulation is available on the European Commission website (Link).

The Regulation setting up the Joint Undertaking for the implementation of the Joint Technology Initiative on Innovative medicines (IMI) was adopted in December 2007. One of its objectives is to improve the efficiency of the drug development process with a view to bring more effective and safer innovative medicines to patients. This should be achieved through support of research activities by pooling resources from the public and private sectors. To this end, the IMI will be organising competitive calls for proposals for supporting the research activities. The involvement of small and medium-sized enterprises (SMEs) in its activities will be particularly promoted. Further information is available on the European Commission website (Link).

SME companies registered with the EMEA

246 companies currently have SME status assigned by the EMEA. The list of companies is published on the EMEA website at: http://www.emea.europa.eu/pdfs/SME/registeredcompanies.pdf

Contact the SME Office

The SME Office has been set up within the Agency to address the particular needs of smaller companies. The Office aims to facilitate communication with SMEs through dedicated personnel who will respond to practical or procedural enquiries, monitor applications, and organise workshops and training sessions for SMEs. Any comments on this news bulletin should also be forwarded to the SME Office:

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