EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 May 2010
EMA/274082/2010
Press Office

Press release

European and international experts discuss the way forward in stem-cell research and development

European Medicines Agency hosts first workshop on stem cell-based therapies

The European Medicines Agency (EMA) assembled for the first time European and international experts from academia, regulatory authorities (from Europe, Japan and the United States) and pharmaceutical industry, in a workshop on 10 May 2010, to review opportunities and difficulties in developing stem cell–based therapies and to discuss regulatory challenges.

The workshop was part of the public consultation process on the first dedicated regulatory guidance document on stem-cell research and development. The draft reflection paper builds upon the experience gained so far through extensive dialogue with European experts and pharmaceutical industry and was developed by the Agency's Committee for Advanced Therapies (CAT), together with the Cell-based Products Working Party and Biologics Working Party.

"Today's discussion will pave the way for the first European marketing authorisation application for a stem cell-based product" said Thomas Lönngren, Executive Director of the EMA.

Research into stem cell-based therapies has increased dramatically in the past few years with ongoing clinical studies in adult stem cells and exploration of embryonic stem cells and induced pluri-potent stem cells (artificially reprogrammed adult cells) for possible future clinical applications. "Stem cells hold the promise of an unlimited source of cells for therapeutic applications to treat patients who have no or only unsatisfactory treatment options. However, these therapies bear certain risks, such as tumourigenicity and immunorejection, and hence need to be carefully regulated with the input from multi-disciplinary expertise", said Christian Schneider, chair of the CAT.

Within the European Union, some 40 clinical trials are currently exploring the use of stem cells in regeneration of lost or damaged tissue (heart, skin, bone, spinal cord, liver, pancreas and cornea) and in haematological or solid-organ malignancies. The majority of these trials are using mesenchymal cells derived from adipose tissue, bone marrow, stromal cells and connective tissue. A small proportion of the trials are using haematopoietic stem cells. The Agency has been informed about the intent of a

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8427 **Facsimile** +44 (0)20 7418 8416
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

European manufacturer to submit the first application for marketing authorisation for a stem cell-based product.

The Agency's committees have been advising pharmaceutical companies on stem-cell research at different stages of development for several years. The CAT has confirmed classification as 'advanced therapy medicinal products' (ATMPs) of three different stem-cell therapies. The Scientific Advice Working Party, in conjunction with the CAT, has given scientific advice on the quality, preclinical and clinical development of seven stem-cell products. The CAT is currently evaluating, within certification procedures, quality and non-clinical data for stem-cell ATMPs under development by European small and medium-sized enterprises.

The attendees of the workshop welcomed this opportunity to discuss the draft reflection paper on stem cell-based medicinal products. The conclusions reached on the quality, non-clinical and clinical sections will be considered when finalising the paper. Any further comments are welcome until 30 June 2010. The reflection paper is expected to be finalised by the end of 2010 and will be published on the Agency's website.

The Agency will continue its public dialogue with academia, regulators and pharmaceutical industry on stem cell-based therapies.

Notes

1. The draft reflection paper on stem cell-based medicinal products was released on 16 March 2010 for consultation until 30 June 2010 and can be found here: http://www.ema.europa.eu/pdfs/human/cat/57113409en.pdf
2. Presentations of the keynote speakers at this workshop can be found here: http://www.ema.europa.eu/meetings/conference.htm
3. Podcasts of the keynote speakers at this workshop, together with a summary report, will be published on the Agency's website shortly.
4. This press release, together with other information on the work of the European Medicines Agency, can be found on the Agency's website: www.ema.europa.eu

Contact our press officers

Martin Harvey Allchurch or Monika Benstetter

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu