



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Workshop on endpoints for cystic fibrosis clinical trials

Call for expression of interest

27-28 September 2012
European Medicines Agency, London, United Kingdom



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Workshop on endpoints for cystic fibrosis clinical trials

Workshop objectives

This European Medicines Agency workshop will focus on outcome measures for cystic fibrosis (CF) lung disease and exocrine pancreatic insufficiency, with a view to informing regulatory decisions.

Therapeutic successes in CF have created challenges to the development of future CF therapies: statistically significant changes in pulmonary function and exacerbation rate become more difficult to demonstrate as baseline population values improve. A systematic approach to outcome measures development is needed to provide regulators with the tools necessary for evaluating new therapies.

Programme overview

Besides a panel discussion, it is planned to have break-out sessions on the following topics, to allow each to be discussed in greater depth:

- Pulmonary disease (clinical efficacy endpoints, biomarkers, surrogate endpoints, PRO).
- Bronchopulmonary infection (microbiological endpoints, PK/PD studies – particular focus on inhaled antibiotics, methodological challenges: inhaled antibiotics – on/off regime, alternating antibiotics).
- Exocrine pancreatic insufficiency (novel outcome measures).

A [draft workshop programme](#) is available on the Agency's website.

Participation

This workshop aims to bring experts in the field of CF together with regulators, industry and patients to discuss some of the challenges. The workshop is intended for participants directly involved in CF clinical trials or medicines development, and will not address regulatory affairs.

This is an invitation-only event, but the Agency will consider expressions of interest to participate from those who can actively contribute to the discussions. Please note that the number of places available at the workshop will be limited.

Expressions of interest should be sent by e-mail to: marketa.lisakova@ema.europa.eu

Deadline for receipt of requests to participate

31 July 2012.

Conference venue and secretariat

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