



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 October 2017
EMA/359806/2017
Human Medicines Research and Development Support

EMA expert meeting on genome editing technologies used in medicinal product development

18 October 2017
European Medicines Agency, London, United Kingdom

Meeting room 2F

30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom

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Send a question via our website www.ema.europa.eu/contact

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Objectives of the meeting

The European Medicines Agency (EMA) is convening an expert meeting (invitation only) on 18 October 2017 with the aim to discuss scientific and regulatory opportunities and challenges of medicinal product development using genome editing technologies.

Programme overview

Sessions

- Session 1:** Genome editing and technologies: state of the art and future outlook
- Session 2:** Application of genome editing by Academia
- Session 3:** Case studies using genome editing technologies: CRISPR-Cas9, TALENs and Zinc finger nucleases

Organising Committee

Committee for Advance Therapy (CAT)

Martina Schübler-Lenz	CAT Chair, Paul-Ehrlich-Institute (PEI), Germany
Bernd Gänsbacher	CAT member, Technical University Munich, Germany
Paolo Gasparini	CAT member, University of Trieste, Italy
Toivo Maimets	CAT member, University of Tartu, Estonia
Asterios Tsiftoglou	CAT member, Aristotle University of Thessaloniki, Greece
Maura O'Donovan	CAT member, Health Products Regulatory Authority (HPRA), Ireland
Dariusz Śladowski	CAT member, Medical University of Warsaw, Poland
Simona Badoi	CAT member, National Agency for Medicines and Medical Devices (ANM), Romania
Metoda Lipnik-Stangelj	CAT member, University of Ljubljana, Slovenia
Christiane Niederlaender	CAT member, Medicines and Healthcare Products Regulatory Agency (MHRA), United Kingdom
Belaïd Sekkali	CAT alternate, Federal Agency for Medicines and Health Products (AFMPS-FAGG), Belgium
Margarida Menezes-Ferreira	CAT member, National Authority of Medicines and Health Products (INFARMED), Portugal
Egbert Flory	CAT alternate, Paul-Ehrlich-Institute (PEI), Germany

Rune Kjeklen	CAT alternate, Norwegian Medicines Agency (NOMA), Norway
Marcos Timón	CAT alternate, Spanish Agency of Medicines and Medical Devices (AEMPS), Spain
Michele Lipucci	CAT alternate, European Organisation for Rare Diseases (Eurordis), Italy
Tiina Palomäki	Expert, Finnish Medicines Agency (FIMEA), Finland
Silke Schuele	Expert, Paul-Ehrlich-Institute (PEI), Germany

Pharmacogenomics Working Party (PGWP)

Krishna Prasad	PGWP Chair, Medicines and Healthcare Products Regulatory Agency (MHRA), United Kingdom
Magnus Ingelmann-Sundberg	PGWP Additional Expert, Karolinska Institute, Sweden

European Medicines Agency (EMA)

Falk Ehmann	Science and Innovation – Product Development Scientific Support
Patrick Celis	Scientific Committees Secretariat, CAT secretariat
Lucia D’Apote	SCiRS - Scientific Committee Regulatory Science Strategy
Lorenza Rattazzi	Scientific Committees Secretariat, CAT and COMP secretariat
Marie-Hélène Pinheiro	Liaison officer - Corporate Stakeholders

Programme

9:20–9:50 Registration

Registration and badge collection at the reception on the ground floor.

9:50–10:00 Welcome and opening

EMA: **Guido Rasi**, Executive Director

10:00-10:30 Session 1: Genome editing and technologies: state of the art and future outlook

Chair: **Krishna Prasad**, PGWP Chair

- **Genome editing - state of the art and future outlook**
Shengdar Tsai, *St Jude Children's Research Hospital – Memphis, US* (followed by Q&A)

Discussion on summary of comments

10:30-12:30 Session 2: Application of Genome editing by Academia

Chair: **Paolo Gasparini**, CAT member

- **CRISPR in drug discovery and development - achievements, opportunities and challenges**
Presented by Volker Lauschke, *Karolinska Institute, Stockholm, (SE)*
- **Specificity of genome editing tools - detection methods and ON/OFF target modifications**
Presented by Richard Gabriel, *National Center for Tumor Diseases (NCT), Heidelberg, (DE)*
- **Towards clinical translation of hematopoietic stem cell genome editing for the treatment of inherited diseases**
Presented by Pietro Genovese, *San Raffaele Hospital, Milan, (IT)*
- **Clinical trials using CRISPR-Cas**
Presented by Bernd Gänsbacher, *CAT member*

Questions and Answers session

12:30 Lunch break

13:30-16:00 **Session 3: Case studies using genome editing technologies: CRISPR-Cas9, TALENs and Zinc finger nucleases**

Chair: **Martina Schübler-Lenz**, CAT Chair

- **Case study 1 - A case study for developing a genomic medicine to treat an inherited retinal dystrophy**
Presented by Vic Myer, *Editas Medicines*
- **Case study 2 - Ex vivo genome editing with CRISPR-Cas9 to treat serious diseases**
Presented by Bill Lundberg, *CRISPR Therapeutics*
- **Case study 3 - Realizing the potential of CRISPR-Cas9 technology for therapeutic applications**
Presented by Sean Burns, *Intellia*
- **Case study 4 - Preclinical studies to support the clinical translation of ZFN-based Genome editing**
Presented by Michael Holms, *Sangamo Therapeutics*
- **Case study 5 – Development of TALEN-edited allogeneic CAR-T cell therapies**
Presented by Julianne Smith, *Collectis*
- **Case study 6 – Scientific and regulatory considerations for the development of megaTAL genome editing products for the treatment of cancer and severe genetic diseases**
Presented by Philip Gregory, *Bluebird Bio*

Questions and Answers session

16:00 **Coffee break**

16:15-17:00 **Roundtable discussion**

All speakers and sessions' chairs

17:00 **Closing remarks by the chairs**

Travel and Accommodation

Participants must possess valid travel documents and, where relevant, a visa for entry into the United Kingdom. Should you require an official letter of invitation, please contact PGWPSecretariat@ema.europa.eu.

Recording and Photography

The Agency records or broadcasts a number of its meetings, including some virtual meetings. This is part of the Agency's commitment to the principle of transparency as enshrined in the Treaty on the European Union. The conference will be recorded. By attending these events you consent to any photographing, recording, broadcast and publication of presentations on the EMA website.

WiFi access

WiFi is available throughout the EMA. Login details can be found on the back of your EMA access pass.

Getting to Canary Wharf

The EMA is located in Canary Wharf, a business district in the east of London.

Please find below the public transport options for travelling to Canary Wharf together with the approximate journey times and the map of the area.

Directions to European Medicines Agency and map of the area

HOW TO FIND US

- By London Underground** - take the Jubilee line to Canary Wharf underground station and follow signs to Churchill Place. Our office is just a few minutes' walk from the station.
- By Docklands Light Railway** - take the DLR from Bank, Tower Gateway, Stratford, Lewisham, City Airport or Beckton to Canary Wharf station and follow signs to Churchill Place.
- By Bus** - Canary Wharf is served by bus routes: 135, 277, D3, D7 & D8.
- By Air** - London City Airport is 10 minutes away. The DLR connects the airport to the Canary Wharf Estate.
- River Services** - Thames Clippers water-bus service runs between Waterloo, Embankment, London Bridge and Canary Wharf.

Inset Map: Shows the location of the European Medicines Agency (EMA) at 30 Churchill Place, Canary Wharf, London E14 5EU. It highlights the 'Step-free access route' and the 'Lift'.

Main Map: Shows the River Thames and surrounding areas including Westminster, Lambeth, Shoreditch, Bow, Stepney, Poplar, Isle of Dogs, Greenwich, and Deptford. The EMA location is marked with a blue arrow pointing to 30 Churchill Place.

European Medicines Agency
30 Churchill Place
Canary Wharf
London E14 5EU
Tel: +44 (0)20 3660 6000
Web: www.ema.europa.eu

By Docklands Light Railway (DLR)

Both venues are a short walk from Canary Wharf or Heron Quays station on the DLR. Services run from Bank, Tower Gateway, Lewisham, Stratford, King George V and Beckton.

By Underground

The nearest stop for both venues is Canary Wharf station on the Jubilee Line. From East exit (NB. This is the closest exit to 30 Churchill Place): exit the station and turn left into Upper Bank Street, turn right at Canada Square and continue straight into Churchill Place.

By Bus

Canary Wharf is serviced by local bus numbers D3, D7, D8, 135 and 277.

River services

River services run between Embankment, London Bridge and Canary Wharf throughout the day. Canary Wharf pier is roughly a 15-minute walk from the European Medicines Agency.

From London City Airport

Take DLR City Airport to Canary Wharf (journey time is around 20 minutes).

From Gatwick Airport

Take a mainline train to London Bridge, then the Jubilee Line to Canary Wharf (journey time around 50 minutes).

From Heathrow Airport

Take the London underground Piccadilly Line to Green Park, change to the Jubilee Line to Canary Wharf (journey time around 1hr 20 minutes).

Alternatively, take the Heathrow Express train to Paddington, then the Circle or Bakerloo line to Baker Street, then the Jubilee Line to Canary Wharf (journey time around 1hr 20 minutes).

Alternatively, you can take the Heathrow Express train to Paddington, then the District or Circle Line to Tower Hill then the Docklands Light Railway (DLR) to Canary Wharf (journey time around 1hr 30 minutes).

From Stansted Airport

Take the Stansted Express to London Liverpool Street then the Circle Line to Tower Hill and change onto the DLR to Canary Wharf (journey time around 70 minutes).

From Luton Airport

Take a first Capital Connect train to London Bridge then the Jubilee Line to Canary Wharf (journey time around 60 minutes).

From St Pancras International train station

Take the Northern Line to London Bridge then the Jubilee Line to Canary Wharf (journey time around 45 minutes).

Contact

Should you have any questions, please contact Monica Simeoni or Falk Ehmann via PGWPsecretariat@ema.europa.eu.

Useful links

<http://www.tfl.gov.uk/>

[National Rail](#)

[Gatwick Express](#)

[Heathrow Express](#)

[Stansted Express](#)

[Eurostar](#)

[Heathrow Airport](#)

[Gatwick airport](#)

[London City](#)

[London Stansted](#)