

Gene editing for advanced therapies

**governance,
policy,
and society**

OECD Expert Meeting

6-7 July 2017, Berlin, Germany

Scope

The 1.5-day expert meeting “Gene editing for advanced therapies: governance, policy, and society” will discuss governance mechanisms for the responsible use of gene editing in somatic cells for the purpose of promoting human health. The expert meeting is organised under the auspices of the OECD Working Party on Biotechnology, Nanotechnology and Converging Technologies (BNCT). It will be hosted by the Federal Ministry of Education and Research (BMBF) in Berlin, Germany.

Gene editing using CRISPR/Cas9 and comparable methods offers great promise for better understanding, diagnosing and treating diseases and conditions. A number of promising applications are already entering research and therapy. At the same time, the trajectory of these disruptive tools and their uptake in the clinic remain unclear due to uncertainties in the scientific, regulatory, and economic landscapes. Policy makers, researchers and the public are confronted with new regulatory, ethical and social questions around the development and implementation of gene editing technologies and applications. Implications of gene editing in the human germline are discussed in other fora and will not be in the scope of this meeting.

The purpose of the expert meeting is to explore the core scientific, legal, regulatory and societal challenges facing the responsible development and use of gene editing in somatic cells for advanced therapies, such as regenerative medicine, cell therapy, and precision medicine. International stakeholders will aim to identify where new forms of collaboration across science and society may help to promote a reasonable balance of risk and benefit in personalised health and well-being.

The desired outcome of the expert meeting is to increase clarity around current and future innovation trajectories, promoting better policy, understanding, collaboration and alignment amongst stakeholders and countries.

Objectives

1. Pool ideas and approaches from countries for the responsible development of gene editing technologies in advanced and personalised therapies, especially in relation to research policy, ethical, legal and social aspects, regulation and governance, and innovation policy.
2. Examine options available to social actors in, and around, public engagement approaches designed to inform governance and regulation.
3. Draw more general policy lessons for the responsible development of emerging technologies.



Day One; 6 July 2017

9:00-9:15

Welcome messages

Dr. Stephan Roesler (Deputy Director, Ethics and Law in the Life Sciences, BMBF, Germany)

Prof. Dr. Francesc Gòdia (Professor of Chemical Engineering, Autonomous University of Barcelona, Barcelona, Spain)

Dr. David Winickoff (Senior Policy Analyst, Secretary of Working Party on Bio-, Nano- and Converging Technologies (BNCT), OECD, Paris, France)

Moderation: **Dr. Mark Bale** (Deputy Director, Science Research and Evidence Directorate, Department of Health, UK)

9:15-9:40

Opening talk: Scenarios of gene editing in research and medicine

Prof. Dr. Jin-Soo Kim (Director and Professor, Center for Genome Engineering, Institute for Basic Science and Department of Chemistry, Seoul National University, Seoul, South Korea)

The opening talk will present a scientific overview of the scientific, regulatory and social trajectories of gene editing in research and clinical use. Opportunities, possible applications and challenges of CRISPR/Cas9 and targeted gene editing technologies in medicine. Future scenarios for how these new techniques will engage the field of advanced and personalised therapies.

9:40-10:05

Keynote: Responsible governance of gene editing for health innovation

Prof. Dr. Peter Dabrock (Chair, Department of Theology, Friedrich-Alexander-Universität Erlangen-Nürnberg, Germany)

This keynote will highlight societal trajectories of gene editing for health innovation. Ethical, legal and social tensions that policymakers and regulators need to balance. Potential pathways for realising the greatest social value.

10:05-13:00

Session 1

Governance approaches in an international context

Session moderator: **Dr. Lyric Jorgenson** (National Institutes of Health (NIH), Deputy Director, Office of the Director, Office of Science Policy, Bethesda, USA)

Gene editing techniques raise challenges for how current systems of governance – regulation, rules and soft law – ought to shape this emerging field. This session attempts to map recent national and international developments in the governance of gene editing for advanced therapies, e.g. regenerative medicine, cell therapy, precision medicine. Experts from different national contexts will discuss existing and future approaches to law, regulation, innovation policy and stakeholder engagement; what are the mechanisms for meaningful public input into regulatory and policy-making processes?

Key policy questions to be discussed:

1. What are the primary governance challenges in the arena of gene editing and advanced therapies across different regulatory systems? For example, assessing risk and benefit; distinguishing therapy and enhancement; research versus clinical use.
2. What kinds of government decision-making practices garner public trust and foster the responsible development and use of gene editing technologies for therapeutic applications?
3. Where might international cooperation on these challenges help?

Comments:

- **Prof. Paolo Gasparini** (Direttore, Genetica Medica, Direttore del Dipartimento, Dipartimento dei Servizi e di Diagnostica avanzata, IRCCS, Trieste, Italy)
- **Dr. Denise Gavin** (Chief, Gene Therapy Branch, Division of Cellular and Gene Therapy, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research, FDA, HHS, USA)
- **Dr. Srinivasan Kellathur** (Head, Advanced Therapy Products, Premarketing, Health Science Authority, Health Products Regulation Group, Singapore)
- **Dr. Debra Mathews** (Assistant Director for Science Programs, Johns Hopkins Berman Institute of Bioethics; Associate Professor, Department of Pediatrics, Johns Hopkins University School of Medicine, Baltimore, MD, USA)
- **Dr. Ritu Nalubola** (Senior Policy Advisor, Office of Policy, Office of the Commissioner, FDA, USA)
- **Dr. Martina Schüßler-Lenz** (Chair, Committee for Advanced Therapies CAT, European Medicines Agency; Deputy Head, Section Advanced Therapy Medicinal Products, Paul-Ehrlich Institute (PEI), Langen, Germany)

Group work and panel discussion:

- **Mr. Simon Burall** (Programme Director, Sciencewise, UK)
- **Dr. Heidi Howard** (Senior Researcher, Centre for Research Ethics & Bioethics (CRB), Uppsala University, Sweden)
- **Prof. Dr. Christof von Kalle** (Managing Director, NCT Heidelberg, Professor and Chair Translational Oncology, National Center for Tumor Diseases Heidelberg (NCT), Germany; Head Translational Oncology, German Cancer Research Center Heidelberg (DKFZ), Germany)
- **Professor Glyn Stacey** (UK Stem Cell Bank Director, National Institute for Biological Standards and Control (NIBSC), UK)
- **Prof. Dr. Andrew Webster** (Professor, Director SATSU, Department of Sociology, University of York Heslington, York, UK)
- **Prof. Dr. Christiane Woopen** (ceres - Cologne Center for Ethics, Rights, Economics, and Social Sciences of Health; University of Cologne, Germany)

13:00-14:00	Lunch break
14:00-15:00	Session 1 (cont.) Governance approaches in an international context
15:00-15:30	Coffee break
15:30-17:30	Session 2 Advancing regulatory science

Session moderator: **Dr. Ubaka Ogbogu** (Assistant Professor, Faculty of Law and Faculty of Pharmacy and Pharmaceutical Sciences, University of Alberta, Edmonton, Canada)

This session addresses issues of regulatory science in the field of gene editing for advanced therapies. It does so against the backdrop of growing challenges to regulatory agencies in the face of a rapid evolution of complex research tools and therapies. Participants will discuss how different regulatory systems are coping with fast-moving technological change in gene editing and advanced therapies. There is uncertainty among the public, policy makers, and the regulatory agencies about the actions required.

Key policy questions to be discussed:

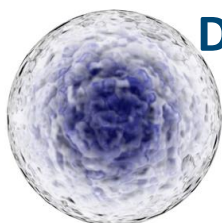
1. What are the main challenges for regulatory science in the field of advanced therapies, especially for those using gene editing?
2. How to stimulate the development, standardisation, and validation of gene editing tools in advanced therapies to assess safety and effectiveness?
3. How might collaborative, international efforts be helpful in developing robust approaches to regulatory science?
4. What are the potential benefits and pitfalls of using emerging forms of Artificial Intelligence (AI) systems in (pre-)clinical testing (e.g. to better understand off target effects)? What could be the process of integration and who develops the standards? Are there unique liability issues?

Comments & panel discussion:

- **Prof. Dr. Toni Cathomen** (Professor of Cell and Gene Therapy Center for Chronic Immunodeficiency at Center for Translational Cell Research, Director Institute for Cell and Gene Therapy, Medical Center, University of Freiburg, Germany)
- **Dr. Hervé Chneiweiss** (Directeur, Neuroscience Paris Seine, CNRS, Inserm/ Université Pierre et Marie Curie, Paris, France)
- **Prof. Dr. Maria Cristina Galli** (Department of Cell Biology and Neurosciences, Istituto Superiore di Sanità Roma, Italy)
- **Dr. Sol Ruiz** (Head of Division, Biologics and Biotechnology, Spanish Agency of Medicines and Medical Devices, Madrid, Spain)
- **Prof. Dr. Kenneth S. Taymor** (Deputy Director, Forum for Collaborative Research, UC Berkeley School of Public Health, USA)

17:30-18:30 Reception

Day Two; 7 July 2017



Moderation: **Dr. David Winickoff** (Senior Policy Analyst, Secretary of Working Party on Bio-, Nano- and Converging Technologies (BNCT), OECD, Paris, France)

9:00-9:25 Keynote

Open and responsible innovation – collaborative forms of research and product development

Prof. Dr. Tania Bubela (Professor, School of Public Health, University of Alberta, Canada)

This keynote will assess the current innovation landscape for the development of novel therapies using gene editing techniques, and help locate collective norms or approaches that may be useful from the perspective of public health goals.

9:25-12:45 Session 3

Between investment, access to innovation and public health

Session moderator: **Prof. Dr. Charis Thompson** (Chancellor's Professor, Center for Science, Technology, and Medicine in Society, UC Berkeley, Berkeley, USA; Professor, Department of Sociology, London School of Economics and Political Science, London, UK)

This session explores options to balance robust innovation, access, and health system sustainability in the context of gene editing for advanced health therapies. Within science and technology policy there is need for a deeper understanding of the investment mechanisms, collaboration, data and intellectual property (IP) policies that can support both innovation and equitable access to technology. In recent years, new models of “open science” and “open innovation” organised around hubs and centres of excellence have been one way to address high upfront costs, investment risks, fragmented policy, and lack of standards. The session will draw on real-world case studies in which policy actors engage the innovation process upstream through incentives and IP structures to minimize trade-offs between the rate of innovation and cost.

Key policy questions to be discussed:

1. What are the biggest barriers for the uptake of gene editing technologies and advanced therapies?
2. How is public benefit assessed and what will ensure the just distribution of benefits?
3. What are options and mechanisms to share and reduce upfront R&D costs (e.g. licensing rules, open science) across sectors?

Comments:

- **Dr. Lars Klüver** (Managing Director, Danish Board of Technology Foundation, Copenhagen, Denmark)
- **Prof. Dr. Bartha Maria Knoppers** (Full Professor and Director of the Centre of Genomics and Policy, Faculty of Medicine, Human Genetics, McGill University, Montreal, Canada)
- **Dr. Roli Mathur** (National Centre for Diseases Informatics & Research (NCDIR), Bengaluru, India)
- **Dr. Gunnar Sandberg** (Vinnova – Swedish Governmental Agency for Innovation Systems, Sweden)

Group work and panel discussion:

- **Dr. Richard Johnson** (BIAC, CEO, Global Helix LLC and member, National Academy of Sciences Board on Life Sciences, USA)
- **Prof. Dr. Won Bok Lee** (Professor, Ewha Womans University Law School, Seoul, Korea)
- **Ms. Katherine Littler** (Senior Policy Advisor, Wellcome Trust, UK)
- **Prof. Dr. Luigi Naldini** (Professor, Cell and Tissue Biology and Professor of Gene and Cell Therapy, “Vita Salute San Raffaele” University School of Medicine, Milan, Italy)
- **Dr. Françoise Roure** (Présidente de la section Sécurité et Risques, Ministère de l’Économie et des Finances, Paris, France)
- **Assistant Professor Dr. Krishanu Saha** (Assistant Professor, Biomedical Engineering, BIONATES, Madison, USA)

12:45-13:00 **Summary & conclusions**

13:00 **End of meeting**



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